



**CANCER PREVENTION AND RESEARCH  
INSTITUTE OF TEXAS**

# **Oversight Committee Meeting**

**February 19, 2014**



## **CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**

### **Summary Overview of the February 19, 2014, Oversight Committee Meeting**

Please find enclosed the meeting packet for the next meeting of the CPRIT Oversight Committee to be held on Wednesday, February 19, 2014, at 10:00 AM. This summary overview of major agenda items provides background on key issues for Committee consideration.

#### **CEO Report**

Wayne Roberts will present the CEO's report and address issues assigned by the Oversight Committee at the January 24<sup>th</sup> meeting including reconstituting the University Advisory Committee (UAC) and proposed dashboard metrics for the agency.

#### **Chief Scientific Officer Program Portfolio Presentation and Grant Award Recommendations**

Dr. Margaret Kripke will present the Program Integration Committee's recommendations for scientific research awards. The research continuation grant recommendations are the first grant applications to be considered under the "new" review process set out by SB 149. SB 149 changed the way that grant recommendations are formally approved. A vote by two-thirds of the Oversight Committee that are present and voting (*i.e.* not recused because of a conflict of interest) is required to approve each funding recommendation. If two-thirds of the Oversight Committee does not vote to approve an award recommendation, then a statement explaining the reason for not following the PIC's recommendation must be included in the meeting minutes.

#### **Product Development Officer Program Portfolio Presentation and Grant Award Recommendations**

Kristen Doyle, acting Product Development Officer, and Dr. Jack Geltosky, CPRIT's Product Development Review Council Chair, will discuss CPRIT's product development portfolio and present the Chief Executive Officer's recommendations for product development grant awards. The applications recommended for product development grant awards were submitted to CPRIT prior to the passage of SB 149. The Oversight Committee's consideration of these awards is governed by the review process in place at the time the applications were submitted. The Oversight Committee will not vote to approve each application recommended by the Chief Executive Officer but may reject a slate of proposed grant awards by a two-thirds vote of the Committee. Nothing limits the Oversight Committee from discussing one or more recommendations on the slates individually. Following the Committee's ratification of the grant awards, the Committee will consider delegating authority to negotiate and execute grant contracts to the CEO and General Counsel.

*Because information related to the scientific research and product development grant applications recommended for funding is not publicly disclosed until the Oversight Committee meeting, the information has been made available to board members through a secure electronic portal.*

### **Strategic Communications Contract**

An effective, coordinated, strategic communications program is needed to inform the public, legislature, media, health professionals, and partner organizations about CPRIT's activities. CPRIT has completed the procurement process for the strategic communications program contract pursuant to the rules for procurement of services set forth by the Texas State Comptroller of Public Accounts. A recommendation will be presented to Oversight Committee by the Communications Officer.

### **Scientific Research and Prevention Programs Committee Appointments**

The Chief Executive Officer has appointed 27 new members to the CPRIT's Scientific Research and Prevention Programs Committee. CPRIT's statute requires the appointments to be approved by the Oversight Committee. The appointments will be discussed by the Nominations Subcommittee at its February 17 meeting. A biographical sketch for each appointee is included in the board packet.

### **Presentation by SRA, International Inc.**

SRA is CPRIT's third party grant administrator. Dr. Rajan Munshi, Project Director, Peer Review and Science Management at SRA International, Inc. will present a summary of the professional services provided to support CPRIT's peer review and grants management activities. The presentation will include an outline of the stages of the grant award life cycle, key aspects of these stages, and the processes that have been implemented to ensure a rigorous, robust, and transparent process for grant awards.

### **Program Priorities Project**

The Oversight Committee chair will report on efforts undertaken by the chair, CEO Roberts, and Dr. Becky Garcia (staff project lead) since the January 24 meeting to frame a process for the Oversight Committee to set annual priorities for CPRIT grant programs.

### **Executive Staff Reports**

Summary reports of important program, operational, and fiscal activities will be provided by the Chief Executive Officer, the Chief Prevention and Communications Officer, the Chief Operating Officer Report, and the Chief Compliance Officer. Memos from the appropriate subcommittees recommending action have been provided for items that the Oversight Committee is expected to act upon.



**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**

**Oversight Committee Meeting**

**Texas State Capitol Extension  
1400 N. Congress Avenue, Austin, Texas 78701  
Room: E1.012**

**February 19, 2014  
10:00 a.m.**

The Oversight Committee may discuss or take action regarding any item on this agenda, and as authorized by the Texas Open Meetings Act, Texas Government Code Section 551.001 et seq., may meet in closed session concerning any and all purposes permitted by the Act.

1. Call to Order
2. Roll Call/Excused Absences
3. Adoption of Minutes from January 24, 2014 meeting **TAB 1**
4. Chief Executive Officer Report **TAB 2**
5. Honoraria Policy **TAB 3**
6. Chief Scientific Officer Report and Grant Award Recommendations **TAB 4**
7. Chief Product Development Officer Report and Grant Award Recommendations **TAB 5**
8. Chief Prevention and Communications Officer Report **TAB 6**
9. Strategic Communications Contract **TAB 6**
10. Scientific Research and Prevention Program Committee Appointments **TAB 7**
11. Proposed Amendment to 25 T.A.C.703.13 and Authorization to Post in the *Texas Register*
12. Presentation by SRA, International Inc. **TAB 8**
13. Program Priorities Project **TAB 9**
14. Subcommittee Business
15. Chief Operating Officer Report **TAB 10**
16. Compliance Officer Report **TAB 11**
17. Consultation with General Counsel
18. Future Meeting Dates and Agenda Items
19. Public Comment

*Anyone wishing to make public comments is required to notify the Chief Executive Officer in writing prior to the start of the meeting. The Committee may limit the time a member of the public may speak.*
20. Adjourn





**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**

**January 24, 2014  
Minutes**

**1. Meeting Called to Order**

The meeting was called to order by Chair William Rice, Friday, January 24, 2014 at 11:07 a.m., at the Capitol Extension Building, Committee Room E1.012 in Austin Texas.

**2. Roll Call /Excused Absences**

**Board Members Present:**

Chair William Rice  
Angelos Angelou  
Gerry Geistweidt  
Pete Geren  
Ned Holmes  
Cynthia Mulrow  
Craig Rosenfeld  
Amy Mitchell

Chair Rice asked for a motion to grant an excused absence to Will Montgomery, whose flight was cancelled due to inclement weather.

A motion was made by Mr. Geren and seconded by Mr. Holmes to grant an excused absence to Will Montgomery whose flight was cancelled due to inclement weather.

**MOTION CARRIED UNANIMOUSLY**

A quorum being present, the meeting was called to order by Chair Rice.

**3. Adoption of Minutes from November 22, 2013 meeting**

Chair Rice entertained a motion to approve the Minutes as circulated.

Motion made by Ms. Mitchell and seconded by Dr. Rosenfeld to approve the Minutes for the November 22, 2013 meeting as circulated.

**MOTION CARRIED UNANIMOUSLY**

Chair Rice informed the members that the committee would not take up the Product Development award slates scheduled to be discussed under agenda item #6 due to the absence of Dr. Jack Geltosky, Chair of the Product Development Review Council. Dr. Geltosky's flight was cancelled due to inclement weather. The award slate has been rescheduled for the February 19<sup>th</sup> meeting.

**4. Chief Executive Officer Report**

CEO Wayne Roberts reported on the following issues presented by the memorandum included in the Board Meeting Packet:

***Status and summary of CPRIT staff vacancy postings***

- Chief Product Development Officer posting closes January 24, 2014.
- Internal Auditor posting extended until January 24, 2014.
- Attorney posting closed January 10, 2014.
- Procurement Specialist posting will close February 28, 2014.
- Grant Specialist postings are being refined and will be posted prior to February 19th Oversight Committee meeting.
- Chief Compliance Officer was filled December 16, 2013.

***Implementation of State Auditor's Recommendations***

CPRIT has fully implemented or is in the process of fully implementing all 51 recommendations from the SAO's January 2013 management audit.

***Annual Report***

The report will be submitted by January 31, 2014 and will, for the first time, meet all statutory requirements and contain no disclaimers.

***Electronic Bulletin Board***

CEO Roberts reported that changes made by the 83rd Legislature permit members of a governing body to communicate via an electronic bulletin board that is accessible through the agency's website and viewable in real-time by the public. Mr. Geren recommended that this item be referred to the Board Governance Subcommittee for consideration.

A motion to refer the electronic bulletin board to the Board Governance Subcommittee for consideration and a recommendation was made by Mr. Geren and seconded by Ms. Mulrow.

**MOTION CARRIED UNANIMOUSLY**

***Look ahead to the February 19 Oversight Committee Meeting***

- The OC will consider research grants under the new legislation using the Program Integration Committee (PIC). The product development applications that were originally supposed to be presented today will be presented at the next meeting;
- Presentation by SRA International, Inc, CPRIT's third party application and review contractor concerning its role and processes;
- Overview of grant award contract and monitoring processes;
- Finalization of the Strategic Communications Contract; and
- Program Priority Discussion.

## **5. Chief Scientific Officer Report and Grant Award Recommendations**

### ***Chief Scientific Officer Report***

Chief Scientific Officer Margaret Kripke provided an overview of the scientific research program. Detailed information is also provided in Dr. Kripke's report included in the Board Meeting Packet. She reiterated the original goals of the scientific research program which were to support the best science possible related to cancer research and also to recruit the best researchers who are interested in cancer research to the state of Texas. She then discussed the need for a more strategic approach for deployment of CPRIT funds and suggested that program priorities focus on prevention and early detection, rare cancers, and funding of projects that moved products and devices toward commercialization. Mr. Geren asked how these suggested priorities fit with the Oversight Committee's plan to establish program priorities over the next several months. Dr. Kripke explained that she would like to release new RFAs targeted toward some of these priorities in March; this would allow the program to move forward, but in no way precluded modification of these priorities by the Oversight Committee at a later date.

Dr. Rosenfeld inquired about the multi-investigator awards. He stated that he knew the primary investigator had to be Texas residents to receive CPRIT funds. He asked if that also applied to sub investigators on the award. Dr. Kripke explained that they do not have to be in Texas; however they cannot receive funding if they are not.

Dr. Mulrow asked if there were any special provisions for minority awards. Dr. Kripke stated that there are special provisions under the training awards for underrepresented minority applicants. Dr. Kripke stated that CPRIT will investigate ways to increase the pool of underrepresented minorities coming up through the ranks.

Dr. Kripke informed members that in consultation with our Interim Product Development Officer and the Product Development Review Council, she decided to transfer the Early Translation Research Awards to the Product Development arena. She stated that this decision was based on the fact that the Product Development program has

the appropriate expertise to review these applications and that this would eliminate the need to duplicate this expertise in a research review panel. Mr. Roberts commented that this is an example of an issue that could be addressed through the prioritization process in the future.

***CEO Recommendations for Scientific Research Grant Awards***

Dr. Kripke introduced the Chief Executive Officer's Grant Award recommendations for scientific research grant awards. Dr. Kripke reported that the three recruitment awards total \$6,000,000 and are under the First-time Tenure Track Faculty Members mechanism.

David Reisman, CPRIT's Chief Compliance Officer, provided the compliance certification for the award slates. Mr. Reisman certified that the application review process that resulted in the three research awards being presented today under the First-time Tenure Track Faculty Members mechanism have followed applicable laws and agency administrative rules.

Chair Rice noted for the record that Ms. Mitchell has reported a conflict of interest with application ID #s R13132, R13011, and R12949. In accordance with CPRIT's rules, Ms. Mitchell was recused from the discussion or action on these applications.

Chair Rice explained that this award slate recommendation is subject to the law in effect at the time that the applications were submitted meaning that the process to finalize the Chief Executive Officer's recommendation follows the procedure in effect prior to the enactment of SB149. The Chief Executive Officer's funding recommendations are final unless two-thirds of the Oversight Committee members vote to disregard the recommendation.

Chair Rice called for a motion to disregard the Chief Executive Officer's recommendation for the First-Time, Tenure-Track Faculty Members Award Slate.

Hearing no motion to disregard the slate, the chair called for a motion to delegate contract negotiation authority to the Chief Executive Officer and the General Counsel and to authorize the Chief Executive Officer to sign the contracts on behalf of the Institute.

Motion made by Mr. Angelou and seconded by Dr. Mulrow.

**MOTION CARRIED UNANIMOUSLY**

**6. Product Development Officer Report and Grant Award Recommendations**

As previously explained by Chair Rice, the Product Development report was deferred to the next Oversight Committee meeting on February 19<sup>th</sup>.

Chair Rice dismissed the Committee at 12:47 p.m. for a break.

The board reconvened at 12:57 p.m. with Chair Rice calling the Committee back to order.

**7. Chief Prevention and Communications Officer Report**

Dr. Becky Garcia provided the Chief Prevention and Communications Officer report.

***Communications Report***

Chair Rice recognized Board Governance Chair Amy Mitchell to give the Board Governance subcommittee report. Ms. Mitchell reported that the subcommittee met January 7, 2014 and discussed issues related to a 2014 CPRIT conference with staff. After reviewing the staff resources required to hold a conference in 2014, it was agreed that it would not be in the best interest of the agency to convene a conference this year. The subcommittee recommended instructing CPRIT staff to solicit proposals for venues in several major Texas cities to hold a CPRIT conference in 2015 and/or 2016 so that CPRIT can assess venue interest.

Dr. Rosenberg questioned why the proposed date is late 2015 or early 2016, to which Dr. Garcia responded that it may serve the agency better to wait until after the conclusion of the 2015 legislative session.

Chair Rice asked how the agency would pay for the conference costs. Dr. Garcia advised that conference costs would be covered by registration fees. A discussion ensued regarding sponsorship, with the general consensus being that CPRIT would not pursue sponsorships at this time.

Dr. Rosenberg inquired if there was a scientific element to the conference. Dr. Garcia explained that the conference mirrors the three program tracks and includes multiple scientific presentations from experts and grantees. She commented that grantees look forward to the networking opportunities at the conference.

The Chair called for a motion to direct CPRIT staff to release a Request for Proposal to solicit venues in major Texas cities to hold a CPRIT conference in 2015 and/or 2016.

Motion made by Dr. Rosenfeld and seconded by Dr. Mulrow.

**MOTION CARRIED UNANIMOUSLY**

***Prevention Program Report***

Dr. Garcia gave a brief overview of the Prevention program. The overview is included in the Board Meeting Packet.

**8. Final Order Adopting Proposed Changes to CPRIT's Administrative Rules**

Chair Rice recognized Kristen Doyle, CPRIT's General Counsel, to address the final orders adopting changes to CPRIT's administrative rules. Ms. Doyle recommended that the Oversight Committee approve the final orders adopting new administrative rules and rule changes and approve an implementation plan.

The Chair recognized Ms. Mitchell for the Board Governance Subcommittee recommendation. Ms. Mitchell related that the Board Governance Subcommittee met on January 7, 2014 to discuss the new rules and rule changes and recommends that the OC vote to approve such.

As there was no discussion, Chair Rice called for a motion to approve the final orders adopting CPRIT's new administrative rules and rule changes and to direct staff to file the orders with the Secretary of State.

Motion made by Dr. Rosenfeld and seconded by Mr. Holmes.

**MOTION CARRIED UNANIMOUSLY**

The Chair called for a motion to approve the plan as proposed by CPRIT's General Counsel to implement CPRIT's new administrative rules and rule changes.

Motion made by Dr. Rosenfeld and seconded by Mr. Angelou.

**MOTION CARRIED UNANIMOUSLY**

**9. Appointments to Scientific Research and Prevention Programs Committees**

The Chair recognized Mr. Holmes, Chair of the Nominations Subcommittee, to discuss the Chief Executive Officer's new appointments to the Scientific Research and Prevention Programs Committees. He stated that the Nominations Subcommittee recommended approval of these appointments as presented by the CEO.

Chair Rice called for a motion to approve the Chief Executive Officer's appointments to the Scientific Research and Prevention Programs Committee.

Motion made by Dr. Rosenfeld and seconded by Ms. Mitchell.

**MOTION CARRIED UNANIMOUSLY**

**10. Subcommittee Business**

***Subcommittee Chairs***

Chair Rice made a recommendation to remove the “interim” status from the subcommittee positions for all subcommittees except for the Diversity subcommittee, which will be addressed separately.

A motion was made by Mr. Geren and seconded by Mr. Holmes to approve removing the “interim” status from the subcommittee chair positions for all subcommittees except for the Diversity subcommittee, which will be addressed separately.

**MOTION CARRIED UNANIMOUSLY**

***Approval of Diversity Subcommittee Interim Chair***

Chair Rice reported that the Diversity Subcommittee has nominated Dr. Mulrow as interim chair. He called for a motion to approve.

Motion made by Mr. Geistweidt and seconded by Mr. Geren to approve Dr. Mulrow as the interim chair of the Diversity Subcommittee.

**MOTION CARRIED UNANIMOUSLY**

There was discussion among the Oversight Committee members regarding the vacant third position on the Diversity Subcommittee. Chair Rice announced that he will be the third member of the Diversity Subcommittee.

***Diversity Subcommittee Report***

Dr. Mulrow provided the Diversity Subcommittee report which included the following recommendations.

1. Request CPRIT staff provide aggregated information on the Historically Underutilized Business (HUB) data from CPRIT grantees and a copy of the HUB form for review. Staff also requested to incorporate the importance of the HUB procurement program in compliance training that is to be provided to grantees.
2. Task the University Advisory Committee with providing the subcommittee and the Oversight Committee information about their institutional HUB programs including purchasing statistics and efforts to increase HUB purchasing by April 18<sup>th</sup>.

3. Task the University Advisory Committee with providing demographic information about the general population of their faculty, medical students, and post-doctoral students, including information about those who are or may be focused on oncology-related prevention or treatment areas.
4. Requested the Oversight Committee include diversity issues in its priority setting process.

Mr. Geistweidt asked if it was a statutory requirement to purchase from HUB vendors. General Counsel Doyle responded that the statute requires the Oversight Committee to establish standards that ensure grant recipients purchase goods and services from HUBs. New administrative rule § 701.23 addresses this requirement.

Chair Rice called for a motion to direct the University Advisory Committee to provide the subcommittee information by April 18<sup>th</sup> regarding their institutional HUB programs, including purchasing statistics and efforts to increase HUB purchasing.

Motion made by Mr. Geistweidt and seconded by Dr. Rosenfeld.

**MOTION CARRIED UNANIMOUSLY**

Chair Rice called for a motion to direct the University Advisory Committee to provide the subcommittee information by April 18<sup>th</sup> about the demographics of the general population of their faculty, medical students, and post-doctoral students, including information about those who are or may be focused on oncology-related prevention or treatment areas.

Motion made by Mr. Angelou and seconded by Dr. Mulrow.

**MOTION CARRIED UNANIMOUSLY**

**11. Chief Operating Officer Report**

The Chair recognized Heidi McConnell to present the Chief Operating Officer's Report.

Ms. McConnell reported on the:

- Fiscal Year 2014 first quarter expenditures,
- Fiscal year 2014 first quarter performance measures,
- Issuance of \$55.2 million in commercial paper notes in November 2013,
- Fiscal year 2013 financial audit conducted by McConnell & Jones LLP,
- Fiscal Year 2014 Internal Audit Plan, and



- Contract with Grant Thornton LLP for fiscal year 2014 internal audit services.

Chair Rice recognized Mr. Angelou, Chair of the Audit Subcommittee to provide the report. Mr. Angelou reported that the Audit Subcommittee met on January 23<sup>rd</sup> to discuss the FY 2013 Internal Audit Annual Report and the FY 2014 Internal Audit Plan, as well as the contract with Grant Thornton for internal audit services. The Audit subcommittee recommended approval of the Internal Audit Annual Report and the FY 2014 internal audit plan together with a contract to Grant Thornton to provide internal audit services to implement the plan.

The Chair called for a motion to approve the FY2014 Internal Audit Plan. Motion made by Dr. Rosenfeld and seconded by Dr. Mulrow.

**MOTION CARRIED UNANIMOUSLY**

Chair Rice called for a motion to approve the FY2013 Internal Audit Annual Report. Motion made by Dr. Mulrow and seconded by Mr. Holmes.

**MOTION CARRIED UNANIMOUSLY**

Chair Rice called for a motion to approve the contract for internal audit services with Grant Thornton for \$200,000. Motion made by Dr. Rosenfeld and seconded by Dr. Mulrow.

**MOTION CARRIED UNANIMOUSLY**

Ms. McConnell advised that the agency would forward the approved contract to the State Auditor's Office for audit delegation authorization and would also seek approval of the contract from the Legislative Budget Board because it exceeds \$100,000. .

## **12. Compliance Officer Report**

The Chair recognized David Reisman to present the Chief Compliance Officer's Report.

David Reisman reported on the status of required grant recipient reports. He stated that as of January 16, there are 54 delinquencies with regard to reports not submitted to CPRIT. He stated that CPRIT staff would follow up with the grant projects and may cease reimbursement or advancement of grant proceeds if required reports are not on file for the grant project.

As there were no questions or comments, the Compliance report was accepted as presented.

### **13. Agency Planning and Operations**

Chair Rice informed members that because he would lay out the next two agenda items for Oversight Committee consideration, he would turn the gavel over to Mr. Geren as Vice Chair.

Dr. Rice presented the following items for discussion.

#### **A. CPRIT Strategic Planning**

Dr. Rice discussed the Oversight Committee's role in agency strategic planning, and referenced programs like the Baldrige Program that help organizations improve their performance. Mr. Geren asked if the Baldrige Program was the only one being considered as a strategic planning tool and whether this item should first go to the Audit Subcommittee. Dr. Rice responded that Baldrige is one example of such tools. Mr. Holmes asked if this was really a task for the Audit Subcommittee or if it should be a shared task for the CEO and OC Chair.

CEO Roberts recommended that if the OC was going to participate in an "organizational excellence" program such as Total Quality Management or the Baldrige Program that it should be blended into the state required strategic plan. He advised the members that the state strategic planning process would begin soon. Mr. Roberts advised that any additional planning processes or programs for organizational excellence should accommodate and consider the time and resource demands of the statutorily required state strategic planning and budget system. He cautioned against shifting finite staff resources from CPRIT's programmatic policy operations towards administrative endeavors such as management programs and excellence pursuits.

Vice Chair Geren asked if the intent was to postpone the Baldrige Program discussion. It was agreed by consensus to postpone.

#### **B. Dashboard Metrics**

Dr. Rice stated that the Oversight Committee should consider whether to have agency staff report on baseline measures and operational metrics. Mr. Geren asked Mr. Roberts for his thoughts on the idea. Mr. Roberts responded that the planning and reporting tools could be useful when testifying at legislative committees; however, identifying completely satisfying measures can be difficult. For instance, inputs and outputs are easier to report than outcomes.

Vice Chair Geren asked if Dr. Rice and Mr. Roberts would develop a proposal for consideration at the next Oversight Committee Meeting on Feb. 19<sup>th</sup>.

A Motion was made by Dr. Rice to ask the CEO to produce a dashboard metrics template for consideration of the Oversight Committee. Seconded by Mr. Angelou.

**MOTION CARRIED UNANIMOUSLY**

**14. Process to Set Annual Program Priorities Pursuant to Health and Safety Code 102.107**

Dr. Rice led a discussion about the process to establish the priorities in awarding cancer research and prevention grants as mandated by a change to Health & Safety Code Section 102.107.

After considerable discussion, it was decided by consensus that Dr. Rice and CEO Roberts would develop a proposal for how to proceed with the Program Priorities Project for consideration and possible action at the February 19, 2014, Oversight Committee Meeting.

**16. Future Meeting Dates and Agenda Items**

The Chair advised that the next Oversight Committee meeting would be held February 19th. At this time, issues related to CPRIT's peer review and grant monitoring processes would be addressed. Chair Rice advised that the first grant awards under the new process established by SB 149 would be presented at the February meeting.

**17. Public Comment**

Annette Leslie, Chair of the Carson Leslie Foundation; Luke Gidden, a 14 year old childhood cancer survivor, and Elizabeth Trieu, board member of Quad W Foundation spoke on behalf of funding for childhood cancer.

**18. Adjourn**

As there was no further business, a motion to adjourn was made by Dr. Rosenfeld and seconded by Ms. Mitchell.

The meeting adjourned at 2:45 p.m.

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Signature

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Date



CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS

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MEMORANDUM

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**TO:** OVERSIGHT COMMITTEE MEMBERS  
**FROM:** WAYNE ROBERTS, CHIEF EXECUTIVE OFFICER  
**SUBJECT:** AGENDA ITEM 4: CHIEF EXECUTIVE OFFICER REPORT  
**DATE:** FEBRUARY 14, 2014

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As of this writing, the Chief Executive Officer Report for the February 19, 2014, Oversight Committee meeting includes the following.

**1. Status and summary of CPRIT staff vacancy postings**

- *Manager of Internal Audit* – The position description was modified and reposted until February 28 to expand the applicant pool by: 1) changing the title from “Internal Auditor”; 2) changing the licenses required to a Certified Public Accountant or a Certified Internal Auditor (previously both were required); 3) clarifying that the position is hired by and reports to the OC; and 4) increasing the salary cap from \$98,987 to \$112,288 per year. These changes were suggested by two internal auditors with extensive state experience. At the November 1, 2013, meeting the Oversight Committee decided that CPRIT staff will screen initial applicants and identify candidates to be interviewed by the Audit Subcommittee. The subcommittee will then recommend a finalist to the Oversight Committee for final approval.
- *Chief Product Development Officer* – The posting closed January 24. Interviewing for the position is underway.
- *Attorney* – The position reposting closes today (February 14). The posting was extended to increase the pool of applicants. As of this writing, 56 candidates have applied.
- *Procurement Specialist* – The position posting will close February 28, 2014.
- *Grant Specialists* – Position posting is close to being finalized.

**2. University Advisory Committee**

Based on interest expressed at the January 24 Oversight Committee meeting, I contacted the university officials that are designated by V.T.C.A., Health & Safety Code § 102.154 to appoint (or re-appoint) members to the University Advisory Committee. Input from the advisory committee may be useful as part of the Program Priorities Project. To date, the following individuals have been appointed by the chancellors/presidents of their respective institutions:

- David Cistola, M.D., Ph.D., Vice President for Research (University of North Texas Health Science Center)
- Dr. Michael Conn, Senior Vice President for Research and Associate Provost (Texas Tech University Health Sciences Center)
- C. Kent Osborne, M.D., Director of the Dan L. Duncan Cancer Center (Baylor College of Medicine)

- Dr. Mary Ann Ottinger, Associate Vice Chancellor for Research (University of Houston System)
- Dr. Cheryl Walker, Welch Professor and Director of the Institute of Biosciences and Technology at Texas A&M University (Texas A&M University System)

Texas State University System, Rice University, and The University of Texas System appointments are expected soon, and may be named by the time of the February 19 Oversight Committee meeting.

### 3. “Dashboard” Metrics

Based on the discussion from the January 24th Oversight Committee meeting, I assigned Heidi McConnell, Chief Operating Officer, to develop measures to be included for regular reporting of CPRIT operational indicators. The report is intended to provide the Oversight Committee, the Legislature, and the public with easy to understand operational indices on how CPRIT is meeting its constitutional and statutory mission of mitigating and preventing the consequences of cancer. The report also increases transparency related to CPRIT’s operations.

A draft report follows this memo. It is important to note that this is an iterative project that will evolve and expand over time. Some of the fields in the report are blank; these will be completed as data are mined. Other fields will be added as necessary.

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CPRIT has awarded **511** grants totaling **\$852.6 million**

- 115 prevention awards totaling \$96.7 million
- 396 research and product development awards totaling \$755.9 million

CPRIT has **3** open Requests for Applications (RFAs), all seeking proposals for the Prevention Program

Of the **144** grants affected by the moratorium:

- 94 award contracts have been executed (14 prevention and 80 research)
- 5 declined the award (research recruitments)

**CPRIT MANAGEMENT DASHBOARD  
FISCAL YEAR 2014**

	SEPT	OCT	NOV	DEC	JAN	FEB	MAR	APR	MAY	JUN	JUL	AUG	CUMULATIVE (ANNUAL)	CUMULATIVE (YTD)
<b>ACCOUNTABILITY</b>														
Announced Grant Awards			10		3								13	
New Grant Contracts Signed			31	1	28								60	
New Grant Contracts In Negotiation					44								44	
Grant Reimbursements Processed (#)	216	138	86	85	78								603	
Grant Reimbursements Processed (\$)	\$15,678,643	\$ 7,679,038	\$ 5,812,765	\$ 6,493,162	\$ 5,518,045								\$ 41,181,652	
Revenue Sharing Payments Received	\$ -	\$ 34,817			\$ 85,802								\$ 120,619	\$ 518,573
Total Grants Contracted (\$)			\$33,140,223	\$1,061,827	\$24,524,765								\$58,726,815	
Grants Awarded (#)/Applications Rec'd (#)	14%	14%	15%	15%	15%									
Debt Issued (\$)/Funding Awarded (\$)	40%	40%	46%	46%	45%									
Grantee Compliance Trainings/Monitoring Visits													0	
Awards with Delinquent Reimbursement Submission			5		52	20								
Awards with Delinquent Matching Funds Verification														
Awards with Delinquent Progress Report Submission			3		2	7								
IA Agency Operational Recommendations Implemented	0	0	0	0	0									
IA Agency Operational Recommendations In Progress	18	18	18	18	18									
IA Grantee Recommendations Implemented	0	0	0	0	0									
IA Grantee Recommendations In Progress	7	7	7	7	7									
Open RFAs				10	13	8								
Prevention Applications Received													0	439
Product Development Applications Received						43							43	176
Research Applications Received					12	584							596	3,332
Help Desk Calls/Emails	151	113	147	290	746								1,447	
<b>MISSION</b>														
<b>RESEARCH PROGRAM</b>														
Scientists Recruited (#)	54	54	54	54	54									
Published Articles on CPRIT-Funded Projects (#)													0	
Jobs Created & Maintained (#)													0	
Trainees in CPRIT-Funded Training Programs (#)													0	
Open Clinical Trials (#)													0	
Number of Patents Resulting from Research													0	

DRAFT

**CPRIT MANAGEMENT DASHBOARD  
FISCAL YEAR 2014**

	SEPT	OCT	NOV	DEC	JAN	FEB	MAR	APR	MAY	JUN	JUL	AUG	CUMULATIVE (ANNUAL)	CUMULATIVE (YTD)
<b><u>PRODUCT DEVELOPMENT PROGRAM</u></b>														
Life Science Companies Recruited (in TX)													0	1
Published Articles on CPRIT-Funded Projects													0	
Number of Jobs Created & Maintained													0	
<b><u>PREVENTION PROGRAM</u></b>														
People Served by CPRIT-Funded Prevention and Control Activities				1,454,705									1,454,705	
People Served through CPRIT-Funded Education and Training				840,096									840,096	
People Served through CPRIT-Funded Clinical Services				614,609									614,609	
<b><u>TRANSPARENCY</u></b>														
Total Website Hits	3,900	5,313	6,445	7,634	11,276								34,568	
Total Unique Visitors to Website	2,895	3,876	4,219	5,077	6,544								22,611	

DRAFT

# 111 Days



## Under Your Watch Since November 1:

- Hired CEO
- Elected presiding officers
- 4 Oversight Committee meetings
- 21 subcommittee meetings
- 55 new reviewers appointed
- 623 applications received
- 13 new awards
- 13 requests for applications
- 2013 Annual Report issued
- 43 of 51 SAO recommendations implemented
- 37 new or revised administrative rules adopted
- 1,002 pages in board packets reviewed
- 2,000 pages in grant award information reviewed



# DRAFT DASHBOARD METRICS



## Accountability

- Grant Applications & Awards
- Compliance
- Finance
- Audit Recommendations

## Mission

- Research Program
- Product Development Program
- Prevention Program

## Transparency

- Website

**Information for this item will be provided  
under separate cover.**



**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**

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**MEMORANDUM**

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**TO:** OVERSIGHT COMMITTEE  
**FROM:** MARGARET KRIPKE, PH.D., CHIEF SCIENTIFIC OFFICER  
**SUBJECT:** UPDATE OF RESEARCH ACTIVITIES  
**DATE:** FEBRUARY 19, 2014

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Progress since the last Oversight Committee meeting is as follows:

1. Applications for the continuation of 7 Research Training Awards and 5 Multi-investigator Research awards were evaluated by the Scientific Review Council (SRC) by teleconference. The recommended awards will be presented to the Oversight Committee at this meeting.
2. RFAs for new Individual Investigator Research Awards (IIRA) and High Impact High Risk (HIHR) Awards closed on February 3, 2014. A total of 584 applications have been distributed among the 7 peer review panels for review. Those recommended by the SRC will be presented at the August Oversight Committee meeting. These applications are the first to be reviewed by the newly reconstituted Scientific Review Panels. New members of these panels will be presented for consideration at this meeting.
3. New RFAs for Recruitment of First-time Faculty, Rising Starts, and Established Investigators were released on January 17, 2013. We anticipate that these will be reviewed by the SRC in time to be considered by the Oversight Committee at its May meeting.
4. We will be releasing another round of RFAs for research grants during the month of March, 2014. These will include an RFA for IIRAs (untargeted) and two targeted RFAs for IIRAs for studies on prevention and early detection research and for studies on cancer of children and adolescents.



CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS

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MEMORANDUM

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**TO:** OVERSIGHT COMMITTEE MEMBERS  
**FROM:** DAVID REISMAN, CHIEF COMPLIANCE OFFICER  
**SUBJECT:** COMPLIANCE CERTIFICATION – RESEARCH AWARDS SLATES  
**DATE:** FEBRUARY 14, 2014

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**Summary and Recommendation:**

As CPRIT's acting compliance officer, I am responsible for reporting to the Oversight Committee regarding the agency's compliance with applicable statutory and administrative rule requirements during the grant review process. I have reviewed the compliance pedigrees for the grant applications submitted to CPRIT for the:

Multi-Investigator Research Award (MIRA) Continuation Grants for Years 4 and 5 Award Slate; and

Research Training Award (RTA) Continuation Grants for Years 4 and 5 Award Slate

With regard to the *RTA Continuation Grants*, I have conferred with staff at CPRIT and SRA International (SRA), CPRIT's contracted third-party grant administrator, and studied the supporting grant review documentation, including third-party observer reports for the peer review meetings. I am satisfied that the application review process that resulted in the seven *RTA Continuation Grants*, recommended by the Chief Executive Officer for these two grant slates, followed applicable laws and agency administrative rules. I certify this award slate for the Oversight Committee's consideration.

With regard to the four *MIRA Continuation Grants*, I have conferred with staff at CPRIT and SRA International (SRA), CPRIT's contracted third-party grant administrator, and studied the supporting grant review documentation, including third-party observer reports for the peer review meetings. The applicable pedigrees and third-party observer reports support that the application review process was followed. However, a review of programmatic and fiscal reporting shows that all four MIRA Continuation grants had not submitted one or more required financial status report(s) (FSRs) in a time prescribed in the Request for Applications (RFA) for grants at issue.

Specifically, the RFA provides, “[a]n applicant institution that is delinquent in programmatic and/or fiscal reporting for its CPRIT MIRA grant at the time of the grant application deadline is not eligible for additional MIRA funding.” Item 6, p.11, CPRIT RFA R-14-MIRA-C-1. None of the four applicant institutions had provided the FSR(s) by the application deadline pursuant to the above stated requirement. After review of CPRIT reports and interviews with CPRIT employees, the reasons for the delinquencies vary among applicants. However, with regard to three of the applicants, the timing of

submission of approval of the matching funds verification with the submission of the FSR's appears to be a factor.

Since the four MIRA applicants did not provide the required FSR's by the application deadline, they were not in compliance with CPRIT's RFA requirements. These four MIRA applications do not qualify for compliance certification. These are addressed in the CEO's individual affidavits.

### **Background:**

Newly enacted statutory changes require that CPRIT employ a Chief Compliance Officer to report to the Oversight Committee regarding compliance with the statute and the agency's administrative rules. Among the Chief Compliance Officer's responsibilities is the obligation "to ensure that all grant proposals comply with this chapter and rules adopted under this chapter before the proposals are submitted to the oversight committee for approval." TEX HEALTH & SAFETY CODE §102.051(c) and (d).

Although the statutory requirement is new, CPRIT began using a compliance pedigree process to formally document compliance for the grant awards announced in December 2012. The compliance pedigree tracks the grant application as it moves through the review process and documents compliance with applicable laws and administrative rules. A compliance pedigree is created for each application; the information related to the procedural steps listed on the pedigree is entered and attested to by SRA employees and CPRIT employees. To the greatest extent possible, information reported in the compliance pedigree is imported directly from data contained in CPRIT's Application Receipt System (CARS), the grant application database managed by SRA. This is done to minimize the opportunity for error caused by manual data entry.

The compliance pedigree and supporting documentation is reviewed by the compliance officer as part of the award slate certification process. You have received a compliance pedigree for each of the applications recommended for a grant award through the grant portal and in the hard copy materials delivered to you. The compliance pedigree is divided into five categories that reflect the seven stages of review. A brief description of each category and information tracked in the category is provided below.

### **Pre-Receipt Compliance:**

The activities listed in pre-receipt stage cover the period beginning with CPRIT's issuance of the Request for Application (RFA) through the submission of grant applications. CPRIT's administrative rules require that RFAs be publicly posted in the *Texas Register*. The RFA specifies a deadline and mandates that only those applications submitted electronically through CARS are eligible for consideration. CARS blocks an application from being submitted once the deadline passes. Occasionally, an applicant may have technical difficulties that prevent the applicant from completing the application submission. When this occurs, the applicant may appeal to CPRIT (through the CPRIT Helpdesk that is managed by SRA) to allow for a submission after the deadline. The program officer considers any appeals and may approve a late filing for good cause. When a late filing request is

approved, the appellee is notified and CARS is reopened for a brief period – usually two to three hours – the next business day.

*For each research grant applicant, I reviewed the application pedigrees. All of the RFA's were posted in the Texas Register. All of the applicants registered through CARS and none of the applicants requested an extension.*

### **Receipt, Referral, and Assignment Compliance:**

Once research applications have been submitted through CARS, SRA staff reviews the applications for compliance with RFA directions. If an applicant does not comply with the directions, SRA notifies the program officer and the program officer makes the final decision to administratively withdraw the application. The program officer and the Review Council Chair assign applications to peer review panels and primary reviewers. Prior to distribution of the applications, reviewers are given summary information about the applicant, including the Project Director and collaborators. Reviewers must sign a conflict of interest agreement and confirm that they do not have a conflict of interest with the application before they are provided with the full application.

*At the time of the Research Applications at issue, a check for administrative or programmatic non-compliance was not required. The pedigrees attest that a conflict of interest statement was signed by each primary reviewer for each Grant Application.*

### **Peer Review:**

Primary reviewers (typically three) must submit written critiques for each of their assigned applications prior to the peer review meeting. After the peer review meetings, a final score report from the review committee is delivered to the Review Council for additional review. Following the peer review meetings, each participating peer reviewer must sign a post-review peer review statement certifying that the reviewer knew of and understood CPRIT's conflict of interest policy and followed the policy for this review process.

*I reviewed the peer reviewer critiques and supporting documentation, such as the sign-out sheets from the peer review panel meetings and post-review peer reviewer statements. Sign out sheets are used to document when a peer review panel member with a conflict of interest associated with a particular application leaves the room (or disengages from the conference call) during the discussion and scoring of the application. With regard to the four MIRA applications, in two peer reviews, a non-primary reviewer noted a conflict of interest and recused themselves. With regard to the RTA applications, in one peer review, one non-primary reviewer recused herself. I also reviewed and confirmed that the post review conflict of interest statements were signed by the peer reviewers.*

### **Programmatic Review:**

Programmatic review is conducted by the Scientific Review Council (SRC). The SRC creates the final list of grant applications it will recommend to the Program Integration Committee (PIC) for grant award slates.

*For these two slates before the Oversight Committee, I reviewed that the slates correspond to RFAs that have been released and that the pedigree reflects the date of the SRC meetings and that the applications were included on the slates of award recommendations.*

*I also reviewed the third party observer reports for these meetings. The third party observer reports document that the SRC members who indicated a conflict with an application recused themselves from the peer review meeting and left the discussion. The pedigrees for these applications, note that all SRC members participated in the final ranking of applications after the peer review meeting since the discussion did not influence scoring.*

### **Program Integration Committee Review:**

CPRIT rules require that, at the time the Program Integration Committee's final Grant Award recommendations are formally submitted to the Oversight Committee, the Chief Executive Officer shall prepare a written affidavit for each Grant Application recommended by the PIC containing relevant information related to the Grant Application recommendations. A review of the affidavits confirms that such affidavits were executed and provided for each Grant Application recommendation.

*For both slates, I reviewed the affidavits provided by Wayne Roberts, CEO. Mr. Roberts recommended ten of the eleven of the applications for grant awards. One application, a MIRA continuing grant application was recommended subject to certain actions occurring prior to the February 19, 2014 Oversight Committee meeting. I compared the list of grant applications submitted to the Oversight Committee by Mr. Roberts with the list of applications the SRC recommended for awards and confirmed that the recommendations are the same on both lists.*

### **Other Information Reviewed:**

Third-Party Observer Reports - In May 2012, CPRIT implemented the use of an independent third-party observer at peer review meetings to ensure that panel discussions are limited to the merits of the application and adhere to established evaluation criteria. In addition, the third-party observer reports whether CPRIT staff attending the peer review meeting participates in the discussion, scoring or vote on the grant application. CPRIT staff may attend peer review meetings, but may not participate in the review process other than to answer technical questions. The third-party reviewer is the agency's internal auditor, Grant Thornton. I have reviewed the third-party observer report for the peer review meetings for this cycle. Nothing unusual was reported. The report is attached.

Multi-Investigator Research Awards  
Continuation for Years 4 and 5



# CPRIT Scientific Review Council Observation Report

Panel Name: Scientific Review Council Meeting – Multi- Investigator  
Research Awards Continuation for Years 4 and 5

Panel Date: February 3, 2014

Report Date: February 3, 2014

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## **Background**

As part of CPRIT's on-going emphasis on continuous improvement in its grants review/management processes and to ensure that panel discussions are limited to the merits of the application and focused on the established evaluation criteria, CPRIT is implementing the use of a third-party observer at every in-person and telephone conference peer review meeting. CPRIT has authorized its out-sourced internal audit provider to function as a neutral third-party observer.

## **Introduction**

The subject of this report is the Scientific Review Council review of Multi-Investigator Research Awards Continuation for Years 4 and 5. The meeting was chaired by Richard Kolodner and held over the phone on February 3, 2014.

## **Panel Observation Objectives and Scope**

The third-party observation was limited to observing whether the following objectives were met:

- CPRIT's established procedures for panelists who have declared a conflict of interest are followed during the meeting (e.g., reviewers leave room or do not participate in the telephone conference if they have a conflict);
- CPRIT program staff participation is limited to offering general points of information when asked by peer review panel members;
- CPRIT program staff do not engage in the panel's discussion on the merits of applications;
- The peer review panel discussion is focused on the established scoring criteria.

## **Observation Results Summary**

Internal Audit participated in the Scientific Review Council meeting held telephonically and chaired by Richard Kolodner on February 3, 2014. The meeting was facilitated by SRA International, CPRIT's contracted third-party grant application administrator.

Internal Audit noted the following during our observation:

- Five research applications were discussed and evaluated by the Scientific Review Council to determine which grants would receive CPRIT funding.
- Eight council members, three CPRIT staff members, and two SRA employees were present for the Council meeting over the phone.

- Two conflicts of interest were identified prior to or during the call. The council members with conflicts of interest left the teleconference and did not participate in the review of the conflicted applications.
- CPRIT program staff participation was limited to answering procedural questions and clarifying policies.
- SRA program staff did not participate in the discussions around the merits of the applications.
- The Council members' discussions were limited to the application evaluation criteria.

**Disclaimer**

The third-party observation did not include the following:

- An evaluation of the appropriateness or rigor of the peer review panel's discussion of scientific, technical or programmatic aspects of the applications.

Internal Audit was not engaged to and did not conduct an examination or review, the objective of which would be the expression of an opinion or limited assurance on the accuracy of voting and scoring. Accordingly, we will not express such an opinion or limited assurance. Had we performed additional procedures, other matters might have come to our attention that would have been reported to you.

This report is intended solely for the information and use of CPRIT and its management and its Oversight Committee members and is not intended to be and should not be used by anyone other than these specified parties.

**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**  
**APPLICATION PEDIGREE**

**FY** 2014  
**CYCLE** 1  
**PROGRAM** Research  
**AWARD MECHANISM** Multi-Investigator Research Awards – Continuation (MIRA-C)  
**APPLICATION ID** RP140020  
**APPLICATION TITLE** Comparative Effectiveness Research on Cancer in Texas (CERCIT)  
**APPLICANT NAME** Goodwin, James  
**ORGANIZATION** The University of Texas Medical Branch at Galveston  
**PANEL NAME** MIRA-Continuation Review Panel

Category	Compliance Requirement	Information	Attestation Date	Attesting Party*
<b>1. Pre-Receipt</b>	RFA published in Texas Register	12/27/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) opened	12/19/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) closed	01/10/14	02/05/14	SRA International, Inc.
	Date application submitted	01/09/14	02/05/14	SRA International, Inc.
	Method of submission	CARS	02/05/14	SRA International, Inc.
	Within receipt period	YES	02/05/14	SRA International, Inc.
	Appeal to submit application after CARS closed	N/A	02/05/14	SRA International, Inc.
	Appeal for late application submission accepted	N/A	02/05/14	SRA International, Inc.
<b>2. Receipt, Referral, and Assignment</b>	Administrative non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Programmatic non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Assigned to primary reviewers	01/21/14	02/05/14	SRA International, Inc.
	Primary Reviewer 1 COI signed	12/06/13	02/05/14	SRA International, Inc.
	Primary Reviewer 2 COI signed	12/11/13	02/05/14	SRA International, Inc.
	Primary Reviewer 3 COI signed	01/07/14	02/05/14	SRA International, Inc.
<b>3. Peer Review</b>	Primary Reviewer 1 critique submitted	01/26/14	02/05/14	SRA International, Inc.
	Primary Reviewer 2 critique submitted	01/28/14	02/05/14	SRA International, Inc.
	Primary Reviewer 3 critique submitted	01/30/14	02/05/14	SRA International, Inc.
	COI indicated by non-primary reviewer	NONE	02/05/14	SRA International, Inc.
	COI recused from participation	N/A	02/05/14	SRA International, Inc.
	Peer Review: Teleconference	02/03/14	02/05/14	SRA International, Inc.
	Third Party Observer Report	02/03/14	02/10/14	SRA International, Inc.
	Post review statements signed	02/09/14	02/10/14	SRA International, Inc.
	Score report delivered to CPRIT	02/05/14	02/05/14	SRA International, Inc.
<b>4. Final SRC Recommendation</b>	COI indicated by SRC member	NONE	02/07/14	SRA International, Inc.
	COI recused from participation	N/A	02/07/14	SRA International, Inc.
	Third Party Observer Report	02/03/14	02/10/14	SRA International, Inc.
	SRC meeting	02/03/14	02/07/14	SRA International, Inc.
	Recommended for PIC review	02/03/14	02/07/14	SRA International, Inc.
	SRC Chair Notification to PIC and OC	02/05/14	02/07/14	SRA International, Inc.
<b>5. PIC Review</b>	PIC review meeting	02/06/14	02/07/14	SRA International, Inc.
	Approved by PIC	YES	02/07/14	SRA International, Inc.
	Follow-up PIC review meeting	02/11/14	02/12/14	SRA International, Inc.
	Approved by PIC	YES**	02/12/14	SRA International, Inc.
<b>6. Oversight Committee Ratification</b>	CEO Notification to Oversight Committee			
	COI indicated by Oversight Committee member			
	COI recused from participation			
	Presented to CPRIT Oversight Committee			
	Award Approved by Oversight Committee			
	Advance Funds Approved by Oversight Committee			

\*The identity of the attesting individual is retained by CPRIT

\*\*Subject to receiving the required FSRs by the OC meeting

**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**  
**APPLICATION PEDIGREE**

**FY** 2014  
**CYCLE** 1  
**PROGRAM** Research  
**AWARD MECHANISM** Multi-Investigator Research Awards – Continuation (MIRA-C)  
**APPLICATION ID** RP140021  
**APPLICATION TITLE** Novel MRI and MRS Methods for Imaging Cancer Metabolism  
**APPLICANT NAME** Sherry, Dean  
**ORGANIZATION** The University of Texas Southwestern Medical Center  
**PANEL NAME** MIRA-Continuation Review Panel

Category	Compliance Requirement	Information	Attestation Date	Attesting Party*
<b>1. Pre-Receipt</b>	RFA published in Texas Register	12/27/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) opened	12/19/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) closed	01/10/14	02/05/14	SRA International, Inc.
	Date application submitted	01/10/14	02/05/14	SRA International, Inc.
	Method of submission	CARS	02/05/14	SRA International, Inc.
	Within receipt period	YES	02/05/14	SRA International, Inc.
	Appeal to submit application after CARS closed	N/A	02/05/14	SRA International, Inc.
	Appeal for late application submission accepted	N/A	02/05/14	SRA International, Inc.
<b>2. Receipt, Referral, and Assignment</b>	Administrative non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Programmatic non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Assigned to primary reviewers	01/21/14	02/05/14	SRA International, Inc.
	Primary Reviewer 1 COI signed	01/17/14	02/05/14	SRA International, Inc.
	Primary Reviewer 2 COI signed	01/07/14	02/05/14	SRA International, Inc.
	Primary Reviewer 3 COI signed	01/09/14	02/05/14	SRA International, Inc.
<b>3. Peer Review</b>	Primary Reviewer 1 critique submitted	01/31/14	02/05/14	SRA International, Inc.
	Primary Reviewer 2 critique submitted	01/30/14	02/05/14	SRA International, Inc.
	Primary Reviewer 3 critique submitted	01/30/14	02/05/14	SRA International, Inc.
	COI indicated by non-primary reviewer	NONE	02/05/14	SRA International, Inc.
	COI recused from participation	N/A	02/05/14	SRA International, Inc.
	Peer Review: Teleconference	02/03/14	02/05/14	SRA International, Inc.
	Third Party Observer Report	02/03/14	02/10/14	SRA International, Inc.
	Post review statements signed	02/09/14	02/10/14	SRA International, Inc.
	Score report delivered to CPRIT	02/05/14	02/05/14	SRA International, Inc.
<b>4. Final SRC Recommendation</b>	COI indicated by SRC member	NONE	02/07/14	SRA International, Inc.
	COI recused from participation	N/A	02/07/14	SRA International, Inc.
	Third Party Observer Report	02/03/14	02/10/14	SRA International, Inc.
	SRC meeting	02/03/14	02/07/14	SRA International, Inc.
	Recommended for PIC review	02/03/14	02/07/14	SRA International, Inc.
	SRC Chair Notification to PIC and OC	02/05/14	02/07/14	SRA International, Inc.
<b>5. PIC Review</b>	PIC review meeting	02/06/14	02/07/14	SRA International, Inc.
	Approved by PIC	YES	02/07/14	SRA International, Inc.
	Follow-up PIC review meeting	02/11/14	02/12/14	SRA International, Inc.
	Approved by PIC	YES	02/12/14	SRA International, Inc.
<b>6. Oversight Committee Ratification</b>	CEO Notification to Oversight Committee			
	COI indicated by Oversight Committee member			
	COI recused from participation			
	Presented to CPRIT Oversight Committee			
	Award Approved by Oversight Committee			
	Advance Funds Approved by Oversight Committee			

\*The identity of the attesting individual is retained by CPRIT

**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**  
**APPLICATION PEDIGREE**

**FY** 2014  
**CYCLE** 1  
**PROGRAM** Research  
**AWARD MECHANISM** Multi-Investigator Research Awards – Continuation (MIRA-C)  
**APPLICATION ID** RP140022  
**APPLICATION TITLE** Targeted Therapies for Metastatic Osteosarcoma  
**APPLICANT NAME** Poplack, David  
**ORGANIZATION** Baylor College of Medicine  
**PANEL NAME** MIRA-Continuation Review Panel

Category	Compliance Requirement	Information	Attestation Date	Attesting Party*
<b>1. Pre-Receipt</b>	RFA published in Texas Register	12/27/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) opened	12/19/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) closed	01/10/14	02/05/14	SRA International, Inc.
	Date application submitted	01/10/14	02/05/14	SRA International, Inc.
	Method of submission	CARS	02/05/14	SRA International, Inc.
	Within receipt period	YES	02/05/14	SRA International, Inc.
	Appeal to submit application after CARS closed	N/A	02/05/14	SRA International, Inc.
	Appeal for late application submission accepted	N/A	02/05/14	SRA International, Inc.
<b>2. Receipt, Referral, and Assignment</b>	Administrative non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Programmatic non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Assigned to primary reviewers	01/21/14	02/05/14	SRA International, Inc.
	Primary Reviewer 1 COI signed	01/09/14	02/05/14	SRA International, Inc.
	Primary Reviewer 2 COI signed	01/08/14	02/05/14	SRA International, Inc.
	Primary Reviewer 3 COI signed	01/07/14	02/05/14	SRA International, Inc.
<b>3. Peer Review</b>	Primary Reviewer 1 critique submitted	01/30/14	02/05/14	SRA International, Inc.
	Primary Reviewer 2 critique submitted	01/30/14	02/05/14	SRA International, Inc.
	Primary Reviewer 3 critique submitted	01/30/14	02/05/14	SRA International, Inc.
	COI indicated by non-primary reviewer	Carol Prives	02/05/14	SRA International, Inc.
	COI recused from participation	YES	02/05/14	SRA International, Inc.
	Peer Review: Teleconference	02/03/14	02/05/14	SRA International, Inc.
	Third Party Observer Report	02/03/14	02/10/14	SRA International, Inc.
	Post review statements signed	02/09/14	02/10/14	SRA International, Inc.
	Score report delivered to CPRIT	02/05/14	02/05/14	SRA International, Inc.
<b>4. Final SRC Recommendation</b>	COI indicated by SRC member	**	02/07/14	SRA International, Inc.
	COI recused from participation	***	02/07/14	SRA International, Inc.
	Third Party Observer Report	02/03/14	02/10/14	SRA International, Inc.
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	SRC Chair Notification to PIC and OC	02/05/14	02/07/14	SRA International, Inc.
<b>5. PIC Review</b>	PIC review meeting	02/06/14	02/07/14	SRA International, Inc.
	Approved by PIC	YES	02/07/14	SRA International, Inc.
	Follow-up PIC review meeting	02/11/14	02/12/14	SRA International, Inc.
	Approved by PIC	YES	02/12/14	SRA International, Inc.
<b>6. Oversight Committee Ratification</b>	CEO Notification to Oversight Committee			
	COI indicated by Oversight Committee member			
	COI recused from participation			
	Presented to CPRIT Oversight Committee			
	Award Approved by Oversight Committee			
	Advance Funds Approved by Oversight Committee			

\*The identity of the attesting individual is retained by CPRIT

\*\*SRC members who indicated a COI with an application recused themselves from the peer review meeting and did not participate in the discussion, scoring, or funding recommendation(s) for these applications during this meeting.

\*\*\*All SRC members participated in the final ranking of applications after the peer review meetings. SRC members who had previously declared a COI did not recuse themselves from participation in the final ranking meeting since the discussion did not influence scoring.

**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**  
**APPLICATION PEDIGREE**

**FY** 2014  
**CYCLE** 1  
**PROGRAM** Research  
**AWARD MECHANISM** Multi-Investigator Research Awards – Continuation (MIRA-C)  
**APPLICATION ID** RP140024  
**APPLICATION TITLE** Texas Cancer Diagnostics Pipeline Consortium  
**APPLICANT NAME** McDevitt, John  
**ORGANIZATION** Rice University  
**PANEL NAME** MIRA-Continuation Review Panel

Category	Compliance Requirement	Information	Attestation Date	Attesting Party*
<b>1. Pre-Receipt</b>	RFA published in Texas Register	12/27/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) opened	12/19/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) closed	01/10/14	02/05/14	SRA International, Inc.
	Date application submitted	01/10/14	02/05/14	SRA International, Inc.
	Method of submission	CARS	02/05/14	SRA International, Inc.
	Within receipt period	YES	02/05/14	SRA International, Inc.
	Appeal to submit application after CARS closed	N/A	02/05/14	SRA International, Inc.
	Appeal for late application submission accepted	N/A	02/05/14	SRA International, Inc.
<b>2. Receipt, Referral, and Assignment</b>	Administrative non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Programmatic non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Assigned to primary reviewers	01/21/14	02/05/14	SRA International, Inc.
	Primary Reviewer 1 COI signed	01/08/14	02/05/14	SRA International, Inc.
	Primary Reviewer 2 COI signed	01/17/14	02/05/14	SRA International, Inc.
	Primary Reviewer 3 COI signed	12/11/13	02/05/14	SRA International, Inc.
<b>3. Peer Review</b>	Primary Reviewer 1 critique submitted	01/30/14	02/05/14	SRA International, Inc.
	Primary Reviewer 2 critique submitted	01/31/14	02/05/14	SRA International, Inc.
	Primary Reviewer 3 critique submitted	01/31/14	02/05/14	SRA International, Inc.
	COI indicated by non-primary reviewer	Richard O'Reilly	02/05/14	SRA International, Inc.
	COI recused from participation	YES	02/05/14	SRA International, Inc.
	Peer Review: Teleconference	02/03/14	02/05/14	SRA International, Inc.
	Third Party Observer Report	02/03/14	02/10/14	SRA International, Inc.
	Post review statements signed	02/09/14	02/10/14	SRA International, Inc.
	Score report delivered to CPRIT	02/05/14	02/05/14	SRA International, Inc.
<b>4. Final SRC Recommendation</b>	COI indicated by SRC member	**	02/07/14	SRA International, Inc.
	COI recused from participation	***	02/07/14	SRA International, Inc.
	Third Party Observer Report	02/03/14	02/10/14	SRA International, Inc.
	SRC meeting	02/03/14	02/07/14	SRA International, Inc.
	Recommended for PIC review	02/03/14	02/07/14	SRA International, Inc.
	SRC Chair Notification to PIC and OC	02/05/14	02/07/14	SRA International, Inc.
<b>5. PIC Review</b>	PIC review meeting	02/06/14	02/07/14	SRA International, Inc.
	Approved by PIC	YES	02/07/14	SRA International, Inc.
	Follow-up PIC review meeting	02/11/14	02/12/14	SRA International, Inc.
	Approved by PIC	YES	02/12/14	SRA International, Inc.
<b>6. Oversight Committee Ratification</b>	CEO Notification to Oversight Committee			
	COI indicated by Oversight Committee member			
	COI recused from participation			
	Presented to CPRIT Oversight Committee			
	Award Approved by Oversight Committee			
	Advance Funds Approved by Oversight Committee			

\*The identity of the attesting individual is retained by CPRIT

\*\*SRC members who indicated a COI with an application recused themselves from the peer review meeting and did not participate in the discussion, scoring, or funding recommendation(s) for these applications during this meeting.

\*\*\*All SRC members participated in the final ranking of applications after the peer review meetings. SRC members who had previously declared a COI did not recuse themselves from participation in the final ranking meeting since the discussion did not influence scoring.

Research Training Awards  
Continuation for Years 4 and 5

# CPRIT Scientific Review Council Observation Report

Panel Name: Scientific Review Council Meeting – Research Training  
Awards Continuation for Years 4 and 5

Panel Date: January 31, 2014

Report Date: January 31, 2014

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## **Background**

As part of CPRIT's on-going emphasis on continuous improvement in its grants review/management processes and to ensure that panel discussions are limited to the merits of the application and focused on the established evaluation criteria, CPRIT is implementing the use of a third-party observer at every in-person and telephone conference peer review meeting. CPRIT has authorized its out-sourced internal audit provider to function as a neutral third-party observer.

## **Introduction**

The subject of this report is the Scientific Review Council review of Research Training Awards Continuation for Years 4 and 5. The meeting was chaired by Richard Kolodner and held over the phone on January 31, 2014.

## **Panel Observation Objectives and Scope**

The third-party observation was limited to observing whether the following objectives were met:

- CPRIT's established procedures for panelists who have declared a conflict of interest are followed during the meeting (e.g., reviewers leave room or do not participate in the telephone conference if they have a conflict);
- CPRIT program staff participation is limited to offering general points of information when asked by peer review panel members;
- CPRIT program staff do not engage in the panel's discussion on the merits of applications;
- The peer review panel discussion is focused on the established scoring criteria.

## **Observation Results Summary**

Internal Audit participated in the Scientific Review Council meeting held telephonically and chaired by Richard Kolodner on January 31, 2014. The meeting was facilitated by SRA International, CPRIT's contracted third-party grant application administrator.

Internal Audit noted the following during our observation:

- Seven research applications were discussed and evaluated by the Scientific Review Council to determine which grants would receive CPRIT funding.
- Eight council members, three CPRIT staff members, and two SRA employees were present for the Council meeting over the phone.



- One conflict of interest was identified prior to or during the call. The council member with the conflict of interest left the teleconference and did not participate in the review of the conflicted application.
- CPRIT program staff participation was limited to answering procedural questions and clarifying policies.
- SRA program staff did not participate in the discussions around the merits of the applications.
- The Council members' discussions were limited to the application evaluation criteria.

**Disclaimer**

The third-party observation did not include the following:

- An evaluation of the appropriateness or rigor of the peer review panel's discussion of scientific, technical or programmatic aspects of the applications.

Internal Audit was not engaged to and did not conduct an examination or review, the objective of which would be the expression of an opinion or limited assurance on the accuracy of voting and scoring. Accordingly, we will not express such an opinion or limited assurance. Had we performed additional procedures, other matters might have come to our attention that would have been reported to you.

This report is intended solely for the information and use of CPRIT and its management and its Oversight Committee members and is not intended to be and should not be used by anyone other than these specified parties.

**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**  
**APPLICATION PEDIGREE**

**FY** 2014  
**CYCLE** 1  
**PROGRAM** Research  
**AWARD MECHANISM** Research Training Awards - Continuation Awards (RTA-C)  
**APPLICATION ID** RP140102  
**APPLICATION TITLE** Baylor College of Medicine Comprehensive Cancer Training Program  
**APPLICANT NAME** Rosen, Jeffrey  
**ORGANIZATION** Baylor College of Medicine  
**PANEL NAME** RTA-Continuation Review Panel

Category	Compliance Requirement	Information	Attestation Date	Attesting Party*
<b>1. Pre-Receipt</b>	RFA published in Texas Register	12/27/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) opened	12/17/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) closed	01/03/14	02/05/14	SRA International, Inc.
	Date application submitted	12/26/13	02/05/14	SRA International, Inc.
	Method of submission	CARS	02/05/14	SRA International, Inc.
	Within receipt period	YES	02/05/14	SRA International, Inc.
	Appeal to submit application after CARS closed	N/A	02/05/14	SRA International, Inc.
	Appeal for late application submission accepted	N/A	02/05/14	SRA International, Inc.
<b>2. Receipt, Referral, and Assignment</b>	Administrative non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Programmatic non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Assigned to primary reviewers	01/10/14	02/05/14	SRA International, Inc.
	Primary Reviewer 1 COI signed	01/08/14	02/05/14	SRA International, Inc.
	Primary Reviewer 2 COI signed	12/11/13	02/05/14	SRA International, Inc.
	Primary Reviewer 3 COI signed	01/07/14	02/05/14	SRA International, Inc.
<b>3. Peer Review</b>	Primary Reviewer 1 critique submitted	01/24/14	02/05/14	SRA International, Inc.
	Primary Reviewer 2 critique submitted	01/26/14	02/05/14	SRA International, Inc.
	Primary Reviewer 3 critique submitted	01/27/14	02/05/14	SRA International, Inc.
	COI indicated by non-primary reviewer	Carol Prives	02/05/14	SRA International, Inc.
	COI recused from participation	YES	02/05/14	SRA International, Inc.
	Peer Review: Teleconference	01/31/14	02/05/14	SRA International, Inc.
	Third Party Observer Report	01/31/14	02/10/14	SRA International, Inc.
	Post review statements signed	02/06/14	02/07/14	SRA International, Inc.
	Score report delivered to CPRIT	02/05/14	02/05/14	SRA International, Inc.
<b>4. Final SRC Recommendation</b>	COI indicated by SRC member	**	02/07/14	SRA International, Inc.
	COI recused from participation	***	02/07/14	SRA International, Inc.
	Third Party Observer Report	01/31/14	02/10/14	SRA International, Inc.
	SRC meeting	01/31/14	02/10/14	SRA International, Inc.
	Recommended for PIC review	01/31/14	02/10/14	SRA International, Inc.
	SRC Chair Notification to PIC and OC	02/05/14	02/07/14	SRA International, Inc.
<b>5. PIC Review</b>	PIC review meeting	02/06/14	02/07/14	SRA International, Inc.
	Approved by PIC	YES	02/07/14	SRA International, Inc.
<b>6. Oversight Committee Ratification</b>	CEO Notification to Oversight Committee			
	COI indicated by Oversight Committee member			
	COI recused from participation			
	Presented to CPRIT Oversight Committee			
	Award Approved by Oversight Committee			
	Advance Funds Approved by Oversight Committee			

\*The identity of the attesting individual is retained by CPRIT

\*\*SRC members who indicated a COI with an application recused themselves from the peer review meeting and did not participate in the discussion, scoring, or funding recommendation(s) for these applications during this meeting.

\*\*\*All SRC members participated in the final ranking of applications after the peer review meetings. SRC members who had previously declared a COI did not recuse themselves from participation in the final ranking meeting since the discussion did not influence scoring.

**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**  
**APPLICATION PEDIGREE**

**FY** 2014  
**CYCLE** 1  
**PROGRAM** Research  
**AWARD MECHANISM** Research Training Awards - Continuation Awards (RTA-C)  
**APPLICATION ID** RP140103  
**APPLICATION TITLE** Collaborative Training of a New Cadre of Innovative Cancer Prevention Researchers  
**APPLICANT NAME** Ness, Roberta  
**ORGANIZATION** The University of Texas Health Science Center at Houston  
**PANEL NAME** RTA-Continuation Review Panel

Category	Compliance Requirement	Information	Attestation Date	Attesting Party*
<b>1. Pre-Receipt</b>	RFA published in Texas Register	12/27/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) opened	12/17/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) closed	01/03/14	02/05/14	SRA International, Inc.
	Date application submitted	01/03/14	02/05/14	SRA International, Inc.
	Method of submission	CARS	02/05/14	SRA International, Inc.
	Within receipt period	YES	02/05/14	SRA International, Inc.
	Appeal to submit application after CARS closed	N/A	02/05/14	SRA International, Inc.
	Appeal for late application submission accepted	N/A	02/05/14	SRA International, Inc.
<b>2. Receipt, Referral, and Assignment</b>	Administrative non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Programmatic non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Assigned to primary reviewers	01/10/14	02/05/14	SRA International, Inc.
	Primary Reviewer 1 COI signed	12/06/13	02/05/14	SRA International, Inc.
	Primary Reviewer 2 COI signed	01/07/14	02/05/14	SRA International, Inc.
	Primary Reviewer 3 COI signed	01/09/14	02/05/14	SRA International, Inc.
<b>3. Peer Review</b>	Primary Reviewer 1 critique submitted	01/20/14	02/05/14	SRA International, Inc.
	Primary Reviewer 2 critique submitted	01/27/14	02/05/14	SRA International, Inc.
	Primary Reviewer 3 critique submitted	01/14/14	02/05/14	SRA International, Inc.
	COI indicated by non-primary reviewer	NONE	02/05/14	SRA International, Inc.
	COI recused from participation	N/A	02/05/14	SRA International, Inc.
	Peer Review: Teleconference	01/31/14	02/05/14	SRA International, Inc.
	Third Party Observer Report	01/31/14	02/10/14	SRA International, Inc.
	Post review statements signed	02/06/14	02/07/14	SRA International, Inc.
	Score report delivered to CPRIT	02/05/14	02/05/14	SRA International, Inc.
<b>4. Final SRC Recommendation</b>	COI indicated by SRC member	NONE	02/07/14	SRA International, Inc.
	COI recused from participation	N/A	02/07/14	SRA International, Inc.
	Third Party Observer Report	01/31/14	02/10/14	SRA International, Inc.
	SRC meeting	01/31/14	02/10/14	SRA International, Inc.
	Recommended for PIC review	01/31/14	02/10/14	SRA International, Inc.
	SRC Chair Notification to PIC and OC	02/05/14	02/07/14	SRA International, Inc.
<b>5. PIC Review</b>	PIC review meeting	02/06/14	02/07/14	SRA International, Inc.
	Approved by PIC	YES	02/07/14	SRA International, Inc.
<b>6. Oversight Committee Ratification</b>	CEO Notification to Oversight Committee			
	COI indicated by Oversight Committee member			
	COI recused from participation			
	Presented to CPRIT Oversight Committee			
	Award Approved by Oversight Committee			
	Advance Funds Approved by Oversight Committee			

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**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**  
**APPLICATION PEDIGREE**

**FY** 2014  
**CYCLE** 1  
**PROGRAM** Research  
**AWARD MECHANISM** Research Training Awards - Continuation Awards (RTA-C)  
**APPLICATION ID** RP140105  
**APPLICATION TITLE** Cancer Research Training Grant  
**APPLICANT NAME** Oyajobi, Babatunde  
**ORGANIZATION** The University of Texas Health Science Center at San Antonio  
**PANEL NAME** RTA-Continuation Review Panel

Category	Compliance Requirement	Information	Attestation Date	Attesting Party*
<b>1. Pre-Receipt</b>	RFA published in Texas Register	12/27/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) opened	12/17/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) closed	01/03/14	02/05/14	SRA International, Inc.
	Date application submitted	01/03/14	02/05/14	SRA International, Inc.
	Method of submission	CARS	02/05/14	SRA International, Inc.
	Within receipt period	YES	02/05/14	SRA International, Inc.
	Appeal to submit application after CARS closed	N/A	02/05/14	SRA International, Inc.
	Appeal for late application submission accepted	N/A	02/05/14	SRA International, Inc.
<b>2. Receipt, Referral, and Assignment</b>	Administrative non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Programmatic non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Assigned to primary reviewers	01/10/14	02/05/14	SRA International, Inc.
	Primary Reviewer 1 COI signed	01/09/14	02/05/14	SRA International, Inc.
	Primary Reviewer 2 COI signed	01/09/14	02/05/14	SRA International, Inc.
	Primary Reviewer 3 COI signed	01/08/14	02/05/14	SRA International, Inc.
<b>3. Peer Review</b>	Primary Reviewer 1 critique submitted	01/20/14	02/05/14	SRA International, Inc.
	Primary Reviewer 2 critique submitted	01/27/14	02/05/14	SRA International, Inc.
	Primary Reviewer 3 critique submitted	01/24/14	02/05/14	SRA International, Inc.
	COI indicated by non-primary reviewer	NONE	02/05/14	SRA International, Inc.
	COI recused from participation	N/A	02/05/14	SRA International, Inc.
	Peer Review: Teleconference	01/31/14	02/05/14	SRA International, Inc.
	Third Party Observer Report	01/31/14	02/10/14	SRA International, Inc.
	Post review statements signed	02/06/14	02/07/14	SRA International, Inc.
	Score report delivered to CPRIT	02/05/14	02/05/14	SRA International, Inc.
<b>4. Final SRC Recommendation</b>	COI indicated by SRC member	NONE	02/07/14	SRA International, Inc.
	COI recused from participation	N/A	02/07/14	SRA International, Inc.
	Third Party Observer Report	01/31/14	02/10/14	SRA International, Inc.
	SRC meeting	01/31/14	02/10/14	SRA International, Inc.
	Recommended for PIC review	01/31/14	02/10/14	SRA International, Inc.
	SRC Chair Notification to PIC and OC	02/05/14	02/07/14	SRA International, Inc.
<b>5. PIC Review</b>	PIC review meeting	02/06/14	02/07/14	SRA International, Inc.
	Approved by PIC	YES	02/07/14	SRA International, Inc.
<b>6. Oversight Committee Ratification</b>	CEO Notification to Oversight Committee			
	COI indicated by Oversight Committee member			
	COI recused from participation			
	Presented to CPRIT Oversight Committee			
	Award Approved by Oversight Committee			
	Advance Funds Approved by Oversight Committee			

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**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**  
**APPLICATION PEDIGREE**

**FY** 2014  
**CYCLE** 1  
**PROGRAM** Research  
**AWARD MECHANISM** Research Training Awards - Continuation Awards (RTA-C)  
**APPLICATION ID** RP140106  
**APPLICATION TITLE** The Future of Cancer Research: Training Program for Basic and Translational Scientists  
**APPLICANT NAME** Watowich, Stephanie  
**ORGANIZATION** The University of Texas M. D. Anderson Cancer Center  
**PANEL NAME** RTA-Continuation Review Panel

Category	Compliance Requirement	Information	Attestation Date	Attesting Party*
<b>1. Pre-Receipt</b>	RFA published in Texas Register	12/27/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) opened	12/17/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) closed	01/03/14	02/05/14	SRA International, Inc.
	Date application submitted	12/31/13	02/05/14	SRA International, Inc.
	Method of submission	CARS	02/05/14	SRA International, Inc.
	Within receipt period	YES	02/05/14	SRA International, Inc.
	Appeal to submit application after CARS closed	N/A	02/05/14	SRA International, Inc.
	Appeal for late application submission accepted	N/A	02/05/14	SRA International, Inc.
<b>2. Receipt, Referral, and Assignment</b>	Administrative non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Programmatic non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Assigned to primary reviewers	01/10/14	02/05/14	SRA International, Inc.
	Primary Reviewer 1 COI signed	12/11/13	02/05/14	SRA International, Inc.
	Primary Reviewer 2 COI signed	12/06/13	02/05/14	SRA International, Inc.
	Primary Reviewer 3 COI signed	01/09/14	02/05/14	SRA International, Inc.
<b>3. Peer Review</b>	Primary Reviewer 1 critique submitted	01/25/14	02/05/14	SRA International, Inc.
	Primary Reviewer 2 critique submitted	01/20/14	02/05/14	SRA International, Inc.
	Primary Reviewer 3 critique submitted	01/27/14	02/05/14	SRA International, Inc.
	COI indicated by non-primary reviewer	NONE	02/05/14	SRA International, Inc.
	COI recused from participation	N/A	02/05/14	SRA International, Inc.
	Peer Review: Teleconference	01/31/14	02/05/14	SRA International, Inc.
	Third Party Observer Report	01/31/14	02/10/14	SRA International, Inc.
	Post review statements signed	02/06/14	02/07/14	SRA International, Inc.
	Score report delivered to CPRIT	02/05/14	02/05/14	SRA International, Inc.
<b>4. Final SRC Recommendation</b>	COI indicated by SRC member	NONE	02/07/14	SRA International, Inc.
	COI recused from participation	N/A	02/07/14	SRA International, Inc.
	Third Party Observer Report	01/31/14	02/10/14	SRA International, Inc.
	SRC meeting	01/31/14	02/10/14	SRA International, Inc.
	Recommended for PIC review	01/31/14	02/10/14	SRA International, Inc.
	SRC Chair Notification to PIC and OC	02/05/14	02/07/14	SRA International, Inc.
<b>5. PIC Review</b>	PIC review meeting	02/06/14	02/07/14	SRA International, Inc.
	Approved by PIC	YES	02/07/14	SRA International, Inc.
<b>6. Oversight Committee Ratification</b>	CEO Notification to Oversight Committee			
	COI indicated by Oversight Committee member			
	COI recused from participation			
	Presented to CPRIT Oversight Committee			
	Award Approved by Oversight Committee			
	Advance Funds Approved by Oversight Committee			

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**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**  
**APPLICATION PEDIGREE**

**FY** 2014  
**CYCLE** 1  
**PROGRAM** Research  
**AWARD MECHANISM** Research Training Awards - Continuation Awards (RTA-C)  
**APPLICATION ID** RP140108  
**APPLICATION TITLE** Molecularly-Targeted Approaches to Cancer Therapeutics, Diagnostics and Prevention  
**APPLICANT NAME** Sessler, Jonathan  
**ORGANIZATION** The University of Texas at Austin  
**PANEL NAME** RTA-Continuation Review Panel

Category	Compliance Requirement	Information	Attestation Date	Attesting Party*
<b>1. Pre-Receipt</b>	RFA published in Texas Register	12/27/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) opened	12/17/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) closed	01/03/14	02/05/14	SRA International, Inc.
	Date application submitted	01/03/14	02/05/14	SRA International, Inc.
	Method of submission	CARS	02/05/14	SRA International, Inc.
	Within receipt period	YES	02/05/14	SRA International, Inc.
	Appeal to submit application after CARS closed	N/A	02/05/14	SRA International, Inc.
	Appeal for late application submission accepted	N/A	02/05/14	SRA International, Inc.
<b>2. Receipt, Referral, and Assignment</b>	Administrative non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Programmatic non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Assigned to primary reviewers	01/10/14	02/05/14	SRA International, Inc.
	Primary Reviewer 1 COI signed	01/07/14	02/05/14	SRA International, Inc.
	Primary Reviewer 2 COI signed	01/17/14	02/05/14	SRA International, Inc.
	Primary Reviewer 3 COI signed	12/06/13	02/05/14	SRA International, Inc.
<b>3. Peer Review</b>	Primary Reviewer 1 critique submitted	01/27/14	02/05/14	SRA International, Inc.
	Primary Reviewer 2 critique submitted	01/28/14	02/05/14	SRA International, Inc.
	Primary Reviewer 3 critique submitted	01/20/14	02/05/14	SRA International, Inc.
	COI indicated by non-primary reviewer	NONE	02/05/14	SRA International, Inc.
	COI recused from participation	N/A	02/05/14	SRA International, Inc.
	Peer Review: Teleconference	01/31/14	02/05/14	SRA International, Inc.
	Third Party Observer Report	01/31/14	02/10/14	SRA International, Inc.
	Post review statements signed	02/06/14	02/07/14	SRA International, Inc.
	Score report delivered to CPRIT	02/05/14	02/05/14	SRA International, Inc.
<b>4. Final SRC Recommendation</b>	COI indicated by SRC member	NONE	02/07/14	SRA International, Inc.
	COI recused from participation	N/A	02/07/14	SRA International, Inc.
	Third Party Observer Report	01/31/14	02/10/14	SRA International, Inc.
	SRC meeting	01/31/14	02/10/14	SRA International, Inc.
	Recommended for PIC review	01/31/14	02/10/14	SRA International, Inc.
	SRC Chair Notification to PIC and OC	02/05/14	02/07/14	SRA International, Inc.
<b>5. PIC Review</b>	PIC review meeting	02/06/14	02/07/14	SRA International, Inc.
	Approved by PIC	YES	02/07/14	SRA International, Inc.
<b>6. Oversight Committee Ratification</b>	CEO Notification to Oversight Committee			
	COI indicated by Oversight Committee member			
	COI recused from participation			
	Presented to CPRIT Oversight Committee			
	Award Approved by Oversight Committee			
	Advance Funds Approved by Oversight Committee			

\*The identity of the attesting individual is retained by CPRIT



**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**  
**APPLICATION PEDIGREE**

**FY** 2014  
**CYCLE** 1  
**PROGRAM** Research  
**AWARD MECHANISM** Research Training Awards - Continuation Awards (RTA-C)  
**APPLICATION ID** RP140110  
**APPLICATION TITLE** Cancer Intervention and Prevention Discoveries Program  
**APPLICANT NAME** White, Michael  
**ORGANIZATION** The University of Texas Southwestern Medical Center  
**PANEL NAME** RTA-Continuation Review Panel

Category	Compliance Requirement	Information	Attestation Date	Attesting Party*
<b>1. Pre-Receipt</b>	RFA published in Texas Register	12/27/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) opened	12/17/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) closed	01/03/14	02/05/14	SRA International, Inc.
	Date application submitted	01/02/14	02/05/14	SRA International, Inc.
	Method of submission	CARS	02/05/14	SRA International, Inc.
	Within receipt period	YES	02/05/14	SRA International, Inc.
	Appeal to submit application after CARS closed	N/A	02/05/14	SRA International, Inc.
	Appeal for late application submission accepted	N/A	02/05/14	SRA International, Inc.
<b>2. Receipt, Referral, and Assignment</b>	Administrative non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Programmatic non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Assigned to primary reviewers	01/10/14	02/05/14	SRA International, Inc.
	Primary Reviewer 1 COI signed	01/09/14	02/05/14	SRA International, Inc.
	Primary Reviewer 2 COI signed	01/08/14	02/05/14	SRA International, Inc.
	Primary Reviewer 3 COI signed	01/17/14	02/05/14	SRA International, Inc.
<b>3. Peer Review</b>	Primary Reviewer 1 critique submitted	01/28/14	02/05/14	SRA International, Inc.
	Primary Reviewer 2 critique submitted	01/24/14	02/05/14	SRA International, Inc.
	Primary Reviewer 3 critique submitted	01/28/14	02/05/14	SRA International, Inc.
	COI indicated by non-primary reviewer	NONE	02/05/14	SRA International, Inc.
	COI recused from participation	N/A	02/05/14	SRA International, Inc.
	Peer Review: Teleconference	01/31/14	02/05/14	SRA International, Inc.
	Third Party Observer Report	01/31/14	02/10/14	SRA International, Inc.
	Post review statements signed	02/06/14	02/07/14	SRA International, Inc.
	Score report delivered to CPRIT	02/05/14	02/05/14	SRA International, Inc.
<b>4. Final SRC Recommendation</b>	COI indicated by SRC member	NONE	02/07/14	SRA International, Inc.
	COI recused from participation	N/A	02/07/14	SRA International, Inc.
	Third Party Observer Report	01/31/14	02/10/14	SRA International, Inc.
	SRC meeting	01/31/14	02/10/14	SRA International, Inc.
	Recommended for PIC review	01/31/14	02/10/14	SRA International, Inc.
	SRC Chair Notification to PIC and OC	02/05/14	02/07/14	SRA International, Inc.
<b>5. PIC Review</b>	PIC review meeting	02/06/14	02/07/14	SRA International, Inc.
	Approved by PIC	YES	02/07/14	SRA International, Inc.
<b>6. Oversight Committee Ratification</b>	CEO Notification to Oversight Committee			
	COI indicated by Oversight Committee member			
	COI recused from participation			
	Presented to CPRIT Oversight Committee			
	Award Approved by Oversight Committee			
	Advance Funds Approved by Oversight Committee			

\*The identity of the attesting individual is retained by CPRIT

**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**  
**APPLICATION PEDIGREE**

**FY** 2014  
**CYCLE** 1  
**PROGRAM** Research  
**AWARD MECHANISM** Research Training Awards - Continuation Awards (RTA-C)  
**APPLICATION ID** RP140113  
**APPLICATION TITLE** Continuation of Computational Cancer Biomedicine Training Program  
**APPLICANT NAME** Bose, Rathindra  
**ORGANIZATION** University of Houston  
**PANEL NAME** RTA-Continuation Review Panel

Category	Compliance Requirement	Information	Attestation Date	Attesting Party*
<b>1. Pre-Receipt</b>	RFA published in Texas Register	12/27/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) opened	12/17/13	02/05/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) closed	01/03/14	02/05/14	SRA International, Inc.
	Date application submitted	12/23/13	02/05/14	SRA International, Inc.
	Method of submission	CARS	02/05/14	SRA International, Inc.
	Within receipt period	YES	02/05/14	SRA International, Inc.
	Appeal to submit application after CARS closed	N/A	02/05/14	SRA International, Inc.
	Appeal for late application submission accepted	N/A	02/05/14	SRA International, Inc.
<b>2. Receipt, Referral, and Assignment</b>	Administrative non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Programmatic non-compliance notification	N/A	02/05/14	SRA International, Inc.
	Assigned to primary reviewers	01/10/14	02/05/14	SRA International, Inc.
	Primary Reviewer 1 COI signed	01/17/14	02/05/14	SRA International, Inc.
	Primary Reviewer 2 COI signed	01/09/14	02/05/14	SRA International, Inc.
	Primary Reviewer 3 COI signed	12/11/13	02/05/14	SRA International, Inc.
<b>3. Peer Review</b>	Primary Reviewer 1 critique submitted	01/28/14	02/05/14	SRA International, Inc.
	Primary Reviewer 2 critique submitted	01/20/14	02/05/14	SRA International, Inc.
	Primary Reviewer 3 critique submitted	01/26/14	02/05/14	SRA International, Inc.
	COI indicated by non-primary reviewer	NONE	02/05/14	SRA International, Inc.
	COI recused from participation	N/A	02/05/14	SRA International, Inc.
	Peer Review: Teleconference	01/31/14	02/05/14	SRA International, Inc.
	Third Party Observer Report	01/31/14	02/10/14	SRA International, Inc.
	Post review statements signed	02/06/14	02/07/14	SRA International, Inc.
	Score report delivered to CPRIT	02/05/14	02/05/14	SRA International, Inc.
<b>4. Final SRC Recommendation</b>	COI indicated by SRC member	NONE	02/07/14	SRA International, Inc.
	COI recused from participation	N/A	02/07/14	SRA International, Inc.
	Third Party Observer Report	01/31/14	02/10/14	SRA International, Inc.
	SRC meeting	01/31/14	02/10/14	SRA International, Inc.
	Recommended for PIC review	01/31/14	02/10/14	SRA International, Inc.
	SRC Chair Notification to PIC and OC	02/05/14	02/07/14	SRA International, Inc.
<b>5. PIC Review</b>	PIC review meeting	02/06/14	02/07/14	SRA International, Inc.
	Approved by PIC	YES	02/07/14	SRA International, Inc.
<b>6. Oversight Committee Ratification</b>	CEO Notification to Oversight Committee			
	COI indicated by Oversight Committee member			
	COI recused from participation			
	Presented to CPRIT Oversight Committee			
	Award Approved by Oversight Committee			
	Advance Funds Approved by Oversight Committee			

\*The identity of the attesting individual is retained by CPRIT





## CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS

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### MEMORANDUM

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**TO:** OVERSIGHT COMMITTEE MEMBERS  
**FROM:** KRISTEN PAULING DOYLE, GENERAL COUNSEL  
**SUBJECT:** CONTINUATION RESEARCH GRANT AWARDS  
**DATE:** FEBRUARY 6, 2014

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#### Summary

The research continuation grant recommendations are the first grant applications to be considered under the “new” review process set out by SB 149. This memo describes the review process for the continuation grants, highlighting changes and new requirements for the Oversight Committee.

#### SRC Review for Continuation Grants

CPRIT released requests for applications (RFAs) in December for two continuation grants – the *Multi-Investigator Research Award Continuation Grants for Years 4 and 5* and the *Research Training Award Continuation Grants for Years 4 and 5*.

Pursuant to CPRIT’s administrative rules, CPRIT’s Scientific Review Council (SRC) is responsible for the review of continuation applications. SRC members assess the assigned applications individually prior to meeting together and provide an overall evaluation score that conveys the member’s impression of the progress made during the first three years of the project and whether continued funding is appropriate. After discussion, all SRC members score the application and the members’ individually assigned scores are averaged together for a final overall evaluation score for the grant application. The SRC met on January 31 and February 3 to discuss the continuation applications and assigned a numerical ranking score for the applications recommended for grant awards. Assigning a numerical ranking score for each grant recommendation is new to the review process.

The SRC prepared a list of recommended grant awards for the Program Integration Committee (PIC) and the Oversight Committee that includes the amount of funding suggested for each award. SRC Chair Dr. Richard Kolodner sent the transmittal letter on behalf of the SRC that explains how the recommended projects meet the SRC’s standards for continuation grants. The SRC’s decision not to recommend a grant application for an award is final, unless an undisclosed conflict of interest requires CPRIT to reevaluate the grant decision.

By law, the list of SRC recommendations must be provided to the PIC and the Oversight Committee at the same time. Providing the Review Council’s recommendations concurrently to the PIC and the Oversight Committee is a new step introduced by SB 149.

## **Program Integration Committee Recommendations**

The PIC meets today to review the SRC's recommendations and decide upon a final list of applications to recommend to the Oversight Committee for approval. The PIC's recommendations should be substantially based on the final list submitted by the SRC. The PIC must document the factors it considers in making grant award recommendations, including "priority funding" factors in the statute. The PIC also describes how the applications on its list meet the annual program priorities and affect the overall grant portfolio. The amount of funding, goals, timelines, or objectives for a recommended project may be changed by the PIC, but the PIC must provide a written explanation for the change(s). In the event that a grant application submitted by the SRC is *not* included on the PIC's list, the PIC must explain the reasons for excluding the application.

A majority vote of the PIC members is required to approve the final list of PIC recommendations. The decision to *not* recommend a grant application is final unless the PIC's vote is not unanimous. If the vote is not unanimous, then the dissenting PIC member(s) may submit an explanation to the Oversight Committee for voting against the PIC's list of recommendations and may provide an alternative list of recommendations. The Oversight Committee may consider the alternative list.

The PIC was created by SB 149 to replace the former process where the executive director created the final list of grant recommendations. Like the PIC, the executive director was statutorily required to create the list of recommendations substantially based on the Review Council's recommendations.

## **CEO Affidavits**

The CEO will provide an affidavit for every application recommended for funding at the time that the PIC's list of recommendations is sent to the Oversight Committee. The CEO's affidavit must include information on the peer review process, the peer review score, and due diligence (if applicable.) Like the Chief Compliance Officer's certification of the slates, the CEO affidavits are intended to document and affirm for the Oversight Committee that the recommended awards followed CPRIT's review process. The CEO affidavits will be available to the Oversight Committee through the grant portal and be made publicly available once the Oversight Committee votes to approve the grant recommendations. The CEO affidavit is a new step in the review process. It was originally recommended by the State Auditor and incorporated in SB 149 changes

## **Restriction on Communication**

State law prohibits a member of the PIC from discussing a grant application recommendation with an Oversight Committee member until the PIC has submitted its final list of grant recommendations, along with the CEO affidavits, to the Oversight Committee. The restriction on communication is a new statutory requirement.

## **Affirmative Vote by the Oversight Committee to Approve Grant Awards**

SB 149 changed the way that grant recommendations are formally approved. Previously, if the slate of award recommendations was not rejected by a two-thirds vote of the Oversight Committee, then the recommendations were considered ratified. Now a vote by two-thirds of the Oversight Committee that are present and voting (*i.e.* not recused because of a conflict of interest) is required to approve each funding recommendation made by the PIC. If two-thirds of the Oversight Committee does not vote to approve an award recommendation, then a statement explaining the reason for not following the PIC's recommendation must be included in the meeting minutes.

# Cancer Prevention and Research Institute of Texas



## **Research Grant Recommendations**

**TAB 4**

# Multi-Investigator Research Award – Continuation (MIRA-C)



Appl. ID	Organization	Title	Principle Investigator	Recommended Budget
RP140022	Baylor College of Medicine	Targeted Therapies for Metastatic Osteosarcoma	David Poplack	\$2,220,472
RP140024	Rice University	Texas Cancer Diagnostics Pipeline Consortium	John McDevitt	\$3,912,225
RP140020	The University of Texas Medical Branch at Galveston	Comparative Effectiveness Research on Cancer in Texas (CERCIT)	James Goodwin	\$3,231,048
RP140021	The University of Texas Southwestern Medical Center	Novel MRI and MRS Methods for Imaging Cancer Metabolism	Dean Sherry	\$1,758,888
Total				\$11,122,633

# Research Training Awards - Continuation (RTA-C)

Appl. ID	Organization	Title	Principle Investigator	Recommended Budget
RP140102	Baylor College of Medicine	Baylor College of Medicine Comprehensive Cancer Training Program	Jeffrey Rosen	\$1,574,628
RP140108	The University of Texas at Austin	Molecularly-Targeted Approaches to Cancer Therapeutics, Diagnostics and Prevention	Jonathan Sessler	\$1,648,814
RP140103	The University of Texas Health Science Center at Houston	Collaborative Training of a New Cadre of Innovative Cancer Prevention Researchers	Roberta Ness	\$1,745,076
RP140105	The University of Texas Health Science Center at San Antonio	Cancer Research Training Grant	Babatunde Oyajobi	\$1,588,673
RP140106	The University of Texas M. D. Anderson Cancer Center	The Future of Cancer Research: Training Program for Basic and Translational Scientists	Stephanie Watowich	\$1,710,524
RP140110	The University of Texas Southwestern Medical Center	Cancer Intervention and Prevention Discoveries Program	Michael White	\$1,916,038
RP140113	University of Houston	Continuation of Computational Cancer Biomedicine Training Program	Rathindra Bose	\$1,591,594
<b>Total</b>				<b>\$11,775,347</b>

**Conflicts of Interest for Research Cycle 14.1 Applications**  
**(Research Cycle 14.1 Awards Announced at February 2014 Oversight Committee Meeting)**

The table below lists the conflicts of interest (COIs) identified by peer reviewers, Program Integration Committee (PIC) members, and Oversight Committee members on an application-by-application basis. All applications with at least one identified COI are listed below; applications with no COIs are not included. It should be noted that an individual is asked to identify COIs for only those applications that are to be considered by the individual at that particular stage in the review process. For example, Oversight Committee members identify COIs, if any, with only those applications that have been recommended for the grant awards by the PIC. COI information used for this table was collected by SRA International, CPRIT's third party grant administrator, and by CPRIT.

<b>Grant ID</b>	<b>Applicant</b>	<b>Institution</b>	<b>Conflict Noted</b>
<b>Applications considered by the PIC and Oversight Committee</b>			
<b>RP140020</b>	Goodwin, James	The University of Texas Medical Branch at Galveston	Mitchell, Amy
<b>RP140021</b>	Sherry, Dean	The University of Texas Southwestern Medical Center	Mitchell, Amy
<b>RP140022</b>	Poplock, David	Baylor College of Medicine	Prives, Carol; Mitchell, Amy
<b>RP140024</b>	McDevitt, John	Rice University	O'Reilly, Richard; Mitchell, Amy; Geistweidt, Gerry
<b>RP140102</b>	Rosen, Jeffrey	Baylor College of Medicine	Prives, Carol; Mitchell, Amy
<b>RP140103</b>	Ness, Roberta	The University of Texas Health Science Center at Houston	Mitchell, Amy
<b>RP140105</b>	Oyajobi, Babatunde	The University of Texas Health Science Center at San Antonio	Mitchell, Amy
<b>RP140106</b>	Watowich, Stephanie	The University of Texas M.D. Anderson Cancer Center	Mitchell, Amy
<b>RP140108</b>	Sessler, Jonathan	The University of Texas at Austin	Mitchell, Amy
<b>RP140110</b>	White, Michael	The University of Texas Southwestern Medical Center	Mitchell, Amy
<b>RP140113</b>	Bose, Rathindra	University of Houston	Mitchell, Amy



CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS

# **REQUEST FOR APPLICATIONS**

## **RFA R-15-IIRAP-1**

### **Individual Investigator Research Awards for Prevention and Early Detection**

## **1. RATIONALE**

A major opportunity for investment in cancer research is in the area of cancer prevention. Nowhere is there greater potential to reduce the burden of cancer than by reducing its incidence. This has the added advantage of sparing people and families from the psychological and emotional trauma of a cancer diagnosis, the often devastating physical consequences of cancer therapies, and the financial burdens associated with cancer treatment. Identification of causes of cancer, including environmental chemicals, microbial agents, and genetic susceptibilities is essential for reducing cancer incidence. In addition, intervening in the process at early stages of cancer development, before genetic instability becomes widespread, holds promise of successfully eliminating cells destined to become cancer cells. Basic research on the identification and control of premalignant cells, the role of the tumor cell microenvironment in tumor development, environmental drivers, and predictive markers of cancer progression may provide new avenues for intervening early in the process of cancer development. Early detection of cancer using biomarkers and early screening methods also can reduce morbidity and mortality from cancer. Although CPRIT is required to spend 10% of its budget on cancer prevention, CPRIT's Cancer Prevention Program focuses exclusively on the delivery of evidence-based interventions to underserved populations and does not fund prevention research. Thus, there is a unique opportunity for CPRIT's Research Program to fund research on adoption of cancer-preventing behaviors, effectiveness of various interventions, and how best to deliver prevention services that could eventually result in implementation through the Prevention Program.

## **2. RESEARCH OBJECTIVES**

This Request for Applications (RFA) solicits applications for innovative research projects addressing questions that will advance our knowledge of the causes, prevention, early-stage progression, and/or early detection of cancer. Applications may address any topic or issue related to cancer causation, prevention, early progression, or early detection. Research may be laboratory-, clinical-, or population- based, and may include behavioral/intervention, dissemination or health services/outcomes research to reduce cancer incidence or promote early detection. CPRIT expects the outcomes of activities supported by this mechanism to reduce the burden of cancer in the near or long term. CPRIT encourages applications that seek to apply or develop state-of-the-art technologies, tools, and/or resources for prevention or early detection of



cancer, including those with potential commercialization opportunities. Successful applicants should be working in a research environment capable of supporting potentially high-impact studies.

The subject of applications may include, but is not limited to:

- Environmental carcinogenesis, including high through-put methods for carcinogen detection and identification of carcinogens and their mechanisms of action
- Role of microbial agents in cancer causation
- Cancer epidemiology
- Identification of populations at high risk of developing cancer
- Cellular and molecular alterations leading to development of precancerous lesions
- Approaches to prevent progression of early lesions
- Methods for early detection of cancer
- Development and testing of intervention strategies to increase access to and improve recently endorsed screening technologies for cancer
- Cancer-focused health services/outcomes or patient-centered outcomes research
- Development and adaptation of novel interventions for effective and efficient delivery of cancer prevention and screening services

The *degree of relevance* to reducing the burden of cancer will be an important criterion for evaluation of projects for funding by CPRIT.

### **3. FUNDING INFORMATION**

Applicants may request a maximum of \$500,000 in total costs per year for up to 3 years for laboratory and clinical research, and up to \$1,000,000 in total costs per year for up to 3 years for population-based research.



CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS

# **REQUEST FOR APPLICATIONS**

## **RFA R-15-IIRACCA-1**

### **Individual Investigator Research Awards for Cancer in Children and Adolescents**

## **1. RATIONALE**

In recent decades, great strides have been made in reducing mortality from childhood cancers. Most of these gains have been realized in childhood leukemia and lymphoma. However, improvements in survival have been less robust in other types of childhood cancers, which make up more than 40% of total cancer cases in children and adolescents aged 0-19 years. Furthermore, the overall incidence of pediatric cancer has increased at an annual rate of 0.6% since 1975, with most of the increases being seen in acute lymphocytic leukemia, brain and central nervous system tumors, non-Hodgkin's lymphoma, and testicular germ cell tumors. Reasons for increases in these tumor types are unknown, indicating that information on the etiology of these cancers is urgently needed. Because of the high rates of survival for certain childhood and adolescent cancers, there are increasing numbers of survivors of such cancers living today. These individuals have a high rate of late effects from the cancer or its treatment, including the occurrence of additional cancers. Clearly, more effective, less toxic treatments are needed for these diseases. However, few new therapies have been developed in recent years. Several reasons account for the paucity of new treatments, including the lack of interest on the part of pharmaceutical companies in developing treatments for cancers that account for only 1% of all cancer cases and the difficulty of collecting sufficient numbers of tumors for laboratory studies. Because cancers in children and adolescents differ from those in adults with regard to genetic alterations and biological behavior, application of adult therapies to these cancers may not be successful. Therefore, this area of investigation represents an opportunity for CPRIT to deploy funding in an area of critical need that is not heavily represented in other funding portfolios.

## **2. RESEARCH OBJECTIVES**

This Request for Applications solicits applications from individual investigators for innovative research projects addressing questions that will advance our knowledge of the causes, prevention, progression, detection, or treatment of cancer in children and adolescents. Applications may address any topic related to these areas, as well as projects dealing with the causes or amelioration of late effects of cancer treatment. Laboratory, clinical, or population-based studies are all acceptable. CPRIT expects the outcome of the research to reduce the incidence, morbidity, or mortality from cancer in children and/or adolescents in the near or long

term. Applications that seek to apply or develop state-of-the-art approaches, technologies, tools, treatments, and/or resources are encouraged, particularly those with potential for commercialization. Successful applicants should be working in a research environment capable of supporting potentially high impact studies.

The subject of applications may include, but is not limited to the following:

- Causes of cancer in children and adolescents, including genetic factors or prenatal exposure to environmental agents;
- Identification of risk factors for cancer development;
- New methods for diagnosing cancers in children and/or adolescents
- Development of new therapies, including targeted therapies, immunotherapies, and new drugs;
- Identification of patients at risk of developing late effects of cancer treatment;
- Improvements in quality of life for survivors of childhood and adolescent cancers.

The degree of relevance to reducing the burden of cancer in these populations will be an important criterion for evaluation of projects for funding by CPRIT.

### **3. FUNDING INFORMATION**

Applicants may request a maximum of \$500,000 per year for a period of up to 4 years.



CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS

# REQUEST FOR APPLICATIONS

## RFA R-15-IIRA-1

### Individual Investigator Research Awards

Please also refer to the Instructions for Applicants document,  
which will be posted **Date Year**

Application Receipt Opening Date: **Date Year**  
Application Receipt Closing Date: **Date Year**

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**RFA VERSION HISTORY**

Rev DATE     RFA release



## **1. ABOUT CPRIT**

The State of Texas has established the Cancer Prevention and Research Institute of Texas (CPRIT), which may issue up to \$3 billion in general obligation bonds to fund grants for cancer research and prevention.

CPRIT is charged by the Texas Legislature and the citizens of Texas to:

- Create and expedite innovation in the area of cancer research and product or service development, thereby enhancing the potential for a medical or scientific breakthrough in the prevention, treatment, and possible cures for cancer;
- Attract, create, or expand research capabilities of public or private institutions of higher education and other public or private entities that will promote a substantial increase in cancer research and in the creation of high-quality new jobs in the State of Texas; and
- Continue to develop and implement the Texas Cancer Plan by promoting the development and coordination of effective and efficient statewide public and private policies, programs, and services related to cancer and by encouraging cooperative, comprehensive, and complementary planning among the public, private, and volunteer sectors involved in cancer prevention, detection, treatment, and research.

CPRIT furthers cancer research in Texas by providing financial support for a wide variety of projects relevant to cancer research.

## **2. RATIONALE**

The goals of the CPRIT Research Grants Program are to support the discovery of new information about cancer that can lead to prevention, early detection, and cures, and to translate new and existing discoveries into practical advances in cancer diagnosis and treatment. CPRIT encourages applications that seek new fundamental knowledge about cancer and cancer development, as well as those attempting to develop state-of-the-art technologies, tools, computational models, and/or resources for cancer research, including those with potential commercialization opportunities. This award allows experienced or early career-stage cancer researchers the opportunity to explore new methods and approaches for investigating a question of importance that has been inadequately addressed or for which there may be an absence of an established paradigm or technical framework. CPRIT will look with special favor on new

approaches to be taken or new areas of investigation to be explored by established investigators and on supporting the research programs of the most promising investigators at the beginning of their research careers. Applicants need not be trained specifically in cancer research. Indeed, CPRIT strongly encourages investigators from other fields, including the mathematical and computational modeling, physical, chemical, and engineering sciences, to bring their expertise to bear on the exceptionally challenging problems posed by cancer. CPRIT expects outcomes of supported activities to directly and indirectly benefit subsequent cancer research efforts, cancer public health policy, or the continuum of cancer care—from prevention to treatment and cure. To fulfill this vision, applications may address any topic or issue related to cancer, including cancer biology, computational modeling, and systems biology, causation, prevention, detection or screening, treatment, or cure. Successful applicants should be working in a research environment capable of supporting potentially high-impact studies. Access to a clinical environment and interaction with translational cancer physician-scientists are highly desirable.

### **3. RESEARCH OBJECTIVES**

CPRIT will foster cancer research in Texas by providing financial support for a wide variety of projects relevant to cancer research. This Request for Applications (RFA) solicits applications for innovative research projects addressing critically important questions that will significantly advance knowledge of the causes, prevention, and/or treatment of cancer. The goal of awards made in response to this RFA is to fund exceptionally innovative research projects with great potential impact that are directed by a single investigator. Areas of interest include laboratory research, translational studies, and/or clinical investigations. Applications that include collaboration with computational modeling teams are welcomed. In that cancers arise from a large number of derangements of basic molecular and cellular functions and, in turn, cause many alterations in basic biological processes, almost any aspect of biology may be relevant to cancer research, more or less directly. The *degree of relevance* to cancer research will be an important criterion for evaluation of projects for funding by CPRIT ([Section 9.4.1](#)). For example, are alterations in the process in question *primarily* responsible for oncogenesis or secondary manifestations of malignant transformation? Will understanding the process or interfering with it offer selective and useful insight into prevention, diagnosis, or treatment of cancer? *Successful applicants for funding from CPRIT will have addressed these questions satisfactorily.*

## 4. FUNDING INFORMATION

Applicants may request a maximum of \$300,000 in total costs per year for up to 3 years for research. Exceptions to these limits may be requested if extremely well justified (see [Section 8.2.10](#)). Applications funded in this cycle will be eligible for competitive renewal. Funds may be used for salary and fringe benefits, research supplies, equipment, subject participation costs, and travel to scientific/technical meetings or collaborating institutions. Requests for funds to support construction and/or renovation will not be approved under this funding mechanism. State law limits the amount of award funding that may be spent on indirect costs to no more than 5 percent of the total award amount.

## 5. ELIGIBILITY

- The applicant must be a Texas-based entity. Any not-for-profit institution or organization that conducts research is eligible to apply for funding under this award mechanism. A public or private company is not eligible for funding under this award mechanism; these entities must use the appropriate award mechanism(s) under CPRIT's Product Development Program.
- The Principal Investigator (PI) must have a doctoral degree, including M.D., Ph.D., D.D.S., D.M.D., Dr.P.H., D.O., D.V.M., or equivalent, and must reside in Texas during the time the research that is the subject of the grant is conducted.
- A PI may submit only one new or resubmission application under this RFA during this funding cycle. If submitting a renewal application, a PI may submit both a new or resubmission application and a renewal application under this RFA during this funding cycle.
- Because this award mechanism is intended to support research directed by a single investigator, only one Co-PI may be included.
- Collaborations are permitted and encouraged, and collaborators may or may not reside in Texas. However, collaborators who do not reside in Texas are not eligible to receive CPRIT funds. Collaborators should have specific and well-defined roles. Subcontracting and collaborating organizations may include public, not-for-profit, and for-profit entities. Such entities may be located outside of the State of Texas, but non-Texas-based organizations are not eligible to receive CPRIT funds.

- An applicant is eligible to receive a grant award only if the applicant certifies that the applicant institution or organization, including the PI, any senior member or key personnel listed on the grant application, and any officer or director of the grant applicant's institution or organization (or any person related to one or more of these individuals within the second degree of consanguinity or affinity), have not made and will not make a contribution to CPRIT or to any foundation specifically created to benefit CPRIT.
- An applicant is not eligible to receive a CPRIT grant award if the applicant PI, any senior member or key personnel listed on the grant application, and any officer or director of the grant applicant's organization or institution is related to a CPRIT Oversight Committee member.
- The applicant must report whether the applicant institution or organization, the PI, or other individuals who contribute to the execution of the proposed project in a substantive, measurable way, whether or not those individuals are slated to receive salary or compensation under the grant award, are currently ineligible to receive Federal grant funds or have had a grant terminated for cause within 5 years prior to the submission date of the grant application.
- CPRIT grants will be awarded by contract to successful applicants. Certain contractual requirements are mandated by Texas law or by administrative rules. Although applicants need not demonstrate the ability to comply with these contractual requirements at the time the application is submitted, applicants should make themselves aware of these standards before submitting a grant application. Significant issues addressed by the CPRIT contract are listed in [Section 11](#) and [Section 12](#). All statutory provisions and relevant administrative rules can be found at [www.cprit.state.tx.us](http://www.cprit.state.tx.us).

## **6. RESUBMISSION POLICY**

An application previously submitted to CPRIT but not funded may be resubmitted once and must follow all resubmission guidelines. More than one resubmission is not permitted. This policy is in effect for all applications submitted to date. See [Section 8.2.5](#).

## 7. RENEWAL POLICY

An application funded by CPRIT under this mechanism may be submitted for a competitive renewal. This policy is in effect for all awards submitted to date. See [Section 8.2.6](#). Competitive renewals are not subject to preliminary evaluation. Renewal applications move directly to the full peer review phase. See Section 9.2.

## 8. RESPONDING TO THIS RFA

### 8.1. Application Submission Guidelines

Applications must be submitted via the CPRIT Application Receipt System (CARS) (<https://CPRITGrants.org>). **Only applications submitted through this portal will be considered eligible for evaluation.** The applicant is eligible solely for the grant mechanism specified by the RFA under which the grant application was submitted. The PI must create a user account in the system to start and submit an application. The Co-PI, if applicable, must also create a user account to participate in the application. Furthermore, the Authorized Signing Official (ASO) (a person authorized to sign and submit the application for the organization) and the Grants Contract/Office of Sponsored Projects Official (the individual who will manage the grant contract if an award is made) also must create a user account in CARS. Applications will be accepted beginning at 7 a.m. Central Time on **Date Year** and must be submitted by 3 p.m. Central Time on **Date Year**. **Submission of an application is considered an acceptance of the terms and conditions of the RFA.**

#### 8.1.1. Submission Deadline Extension

The submission deadline may be extended for one or more grant applications upon a showing of good cause. All requests for extension of the submission deadline must be submitted via e-mail to the CPRIT HelpDesk. Submission deadline extensions, including the reason for the extension, will be documented as part of the grant review process records.

### 8.2. Application Components

Applicants are advised to follow all instructions to ensure accurate and complete submission of all components of the application. Please refer to the *Instructions for Applicants* document for details that will be available when the application receipt system opens. Submissions that are missing one or more components or do not meet the eligibility requirements listed in [Section 5](#) will be administratively withdrawn without review.

### **8.2.1. Abstract and Significance (5,000 characters)**

Clearly explain the question or problem to be addressed and the approach to its answer or solution. The specific aims of the application must be obvious from the abstract, although they need not be restated verbatim from the Research Plan. Clearly address how the proposed project, if successful, will have a major impact on cancer. Summarize how the proposed research creates new paradigms or challenges existing ones. Indicate whether this research plan represents a new direction for the PI.

**Note:** It is the responsibility of the applicant to capture CPRIT's attention primarily with the Abstract and Significance statement alone. Therefore, applicants are advised to prepare this section wisely. Applicants should not waste this valuable space by stating obvious facts (e.g., that cancer is a significant problem; that better diagnostic and therapeutic approaches are needed urgently; that the type of cancer of interest to the PI is important, vexing, or deadly; etc.).

**Based on this statement (and the Budget and Justification and Biographical Sketches), applications that are judged to offer only modest contributions to the field of cancer research or that do not sufficiently capture the reviewers' interest may be excluded from further peer review (see [Section 9.1](#)).**

### **8.2.2. Layperson's Summary (2,000 characters)**

Provide a layperson's summary of the proposed work. Describe, in simple, nontechnical terms, the overall goals of the proposed work, the type(s) of cancer addressed, the potential significance of the results, and the impact of the work on advancing the field of cancer research, early diagnosis, prevention or treatment. The information provided in this summary will be made publicly available by CPRIT, particularly if the application is recommended for funding. Do not include any proprietary information in the Layperson's Summary. The Layperson's Summary will also be used by advocate reviewers ([Section 9.2](#)) in evaluating the significance and impact of the proposed work.

### **8.2.3. Goals and Objectives**

List specific goals and objectives for each year of the project. These goals and objectives will also be used during the submission and evaluation of progress reports and assessment of project success.

#### **8.2.4. Timeline (One page)**

Provide an outline of anticipated major milestones to be tracked. Timelines will be reviewed for reasonableness, and adherence to timelines will be a criterion for continued support of successful applications. If the application is approved for funding, this section will be included in the award contract. Applicants are advised not to include information that they consider confidential or proprietary when preparing this section.

#### **8.2.5. Resubmission Summary (One page)**

Applicants preparing a resubmission must describe the approach to the resubmission. If a summary statement was prepared for the original application review, applicants are advised to address all noted concerns.

**Note:** An application previously submitted to CPRIT but not funded may be resubmitted once after careful consideration of the reasons for lack of prior success. Applications that received overall numerical scores of 5 or higher are likely to need considerable attention. Applicants may prepare a fresh Research Plan or modify the original Research Plan and mark the changes. However, all resubmitted applications should be carefully reconstructed; a simple revision of the prior application with editorial or technical changes is not sufficient, and applicants are advised not to direct reviewers to such modest changes.

#### **8.2.6. Renewal Summary (Two pages)**

Applicants preparing a renewal must describe and demonstrate that appropriate/adequate progress has been made on the current funded award to warrant further funding. Publications and manuscripts in press that have resulted from work performed during the initial funded period should be listed in the renewal summary.

#### **8.2.7. Research Plan (Ten pages)**

**Background:** Present the rationale behind the proposed project, emphasizing the pressing problem in cancer research that will be addressed.

**Hypothesis and Specific Aims:** Concisely state the hypothesis and/or specific aims to be tested or addressed by the research described in the application.

**Research Strategy:** Describe the experimental design, including methods, anticipated results, potential problems or pitfalls, and alternative approaches. Preliminary data that support the proposed hypothesis are encouraged but not required.

#### **8.2.8. Vertebrate Animals and/or Human Subjects (One page)**

If vertebrate animals will be used, provide an outline of the appropriate protocols that will be followed. If human subjects or human biological samples will be used, provide a plan for recruitment of subjects or acquisition of samples that will meet the time constraints of this award mechanism.

#### **8.2.9. Publications/References**

Provide a concise and relevant list of publications/references cited for the application.

#### **8.2.10. Budget and Justification**

Provide a compelling justification of the budget for the entire proposed period of support, including salaries and benefits, supplies, equipment, patient care costs, animal care costs, and other expenses. Applicants are advised not to interpret the maximum allowable request under this award as a suggestion that they should expand their anticipated budget to this level. Reasonable budgets clearly work in favor of the applicant.

However, if there is a highly specific and defensible need to request more than the maximum amount in any year(s) of the proposed budget, include a special and clearly labeled section in the budget justification that explains the request. Poorly justified requests of this type will likely have a negative impact on the overall evaluation of the application.

In preparing the requested budget, applicants should be aware of the following:

- Equipment having a useful life of more than 1 year and an acquisition cost of \$5,000 or more per unit must be specifically approved by CPRIT. An applicant does not need to seek this approval prior to submitting the application.
- Texas law limits the amount of grant funds that may be spent on indirect costs to no more than 5 percent of the total award amount (5.263 percent of the direct costs). Guidance regarding indirect cost recovery can be found in CPRIT's Administrative Rules, which are available at [www.cpritis.state.tx.us](http://www.cpritis.state.tx.us). So-called grants management and facilities fees (e.g., sponsored programs fees; grants and contracts fees; electricity, gas and water;



custodial fees; maintenance fees; etc.) may not be requested. Applications that include such budgetary items will be rejected administratively and returned without review.

- The annual salary (also referred to as direct salary or institutional base salary) that an individual may receive under a CPRIT award for FY 2014 and FY 2015 is \$200,000; CPRIT FY 2014 is from September 1, 2013 through August 31, 2014 and FY 2015 is from September 1, 2014 through August 31, 2015. Salary does not include fringe benefits and/or facilities and administrative (F&A) costs, also referred to as indirect costs. An individual's institutional base salary is the annual compensation that the applicant organization pays for an individual's appointment, whether that individual's time is spent on research, teaching, patient care, or other activities. Base salary excludes any income that an individual may be permitted to earn outside of his or her duties to the applicant organization.

#### **8.2.11. Biographical Sketches (Two pages each)**

Applicants should provide a biographical sketch that describes their education and training, professional experience, awards and honors, and publications relevant to cancer research.

A biographical sketch must be provided for the PI and, if applicable, the Co-PI (as required by the online application receipt system). Up to two additional biographical sketches for key personnel may be provided. Each biographical sketch must not exceed two pages.

#### **8.2.12. Current and Pending Support**

Describe the funding source and duration of all current and pending support for all personnel who have included a biographical sketch with the application. For each award, provide the title, a two-line summary of the goal of the project and, if relevant, a statement of overlap with the current application. At a minimum, current and pending support of the PI and, if applicable, the Co-PI must be provided.

#### **8.2.13. Institutional/Collaborator Support and/or Other Certification (Four pages)**

Applicants may provide letters of institutional support, collaborator support, and/or other certification documentation relevant to the proposed project. A maximum of four pages may be provided.

#### **8.2.14. Previous Summary Statement**

If the application is being resubmitted, the summary statement of the original application review, if previously prepared, will be automatically appended to the resubmission. The applicant is not responsible for providing this document.

**Applications that are missing one or more of these components, exceed the specified page, word, or budget limits, or that do not meet the eligibility requirements listed above will be administratively rejected without review.**

## **9. APPLICATION REVIEW**

### **9.1. Preliminary Evaluation**

To ensure the timely and thorough review of only the most innovative and cutting-edge research with the greatest potential for advancement of cancer research, all eligible applications may be preliminarily evaluated by CPRIT Scientific Research Program panel members for scientific merit and impact.

**This preliminary evaluation will be based on a subset of material presented in the application—namely Abstract and Significance, Budget and Justification, and Biographical Sketches. Applications that do not sufficiently capture the reviewers’ interest at this stage will not be considered for further review. Such applications will have been judged to offer only modest contributions to the field of cancer research and will be excluded from further peer review.**

The applicant will be notified of the decision to disapprove the application after the preliminary evaluation stage has concluded. Due to the volume of applications to be reviewed, comments made by reviewers at the preliminary evaluation stage may not be provided to applicants. The preliminary evaluation process will be used only when the number of applications exceeds the capacity of the review panels to conduct a full peer review of all received applications.

### **9.2. Full Peer Review**

Applications that pass preliminary evaluation will undergo further review using a two-stage peer review process: (1) Full peer review, and (2) prioritization of grant applications by the CPRIT Scientific Review Council. In the first stage, applications will be evaluated by an independent peer review panel consisting of scientific experts as well as advocate reviewers using the criteria

listed below. In the second stage, applications judged to be most meritorious by the peer review panels will be evaluated and recommended for funding by the CPRIT Scientific Review Council based on comparisons with applications from all of the peer review panels and programmatic priorities. Applications approved by Scientific Review Council will be forwarded to the CPRIT Program Integration Committee (PIC) for review. The PIC will consider factors including program priorities set by the Oversight Committee, portfolio balance across programs, and available funding. The CPRIT Oversight Committee will vote to approve each grant award recommendation made by the PIC. The grant award recommendations will be presented at an open meeting of the Oversight Committee and must be approved by two-thirds of the Oversight Committee members present and eligible to vote. The review process is described more fully in CPRIT's Administrative Rules, Chapter 703, Sections 703.6–703.8.

### **9.3. Confidentiality of Review**

Each stage of application review is conducted confidentially, and all CPRIT Scientific Peer Review Panel members, Scientific Review Council members, Program Integration Committee members, CPRIT employees, and Oversight Committee members with access to grant application information are required to sign nondisclosure statements regarding the contents of the applications. All technological and scientific information included in the application is protected from public disclosure pursuant to Health and Safety Code §102.262(b).

Individuals directly involved with the review process operate under strict conflict of interest prohibitions. All CPRIT Scientific Peer Review Panel members, and Scientific Review Council members are non-Texas residents.

An applicant will be notified regarding the peer review panel assigned to review the grant application. Peer review panel members are listed by panel on CPRIT's Web site. **By submitting a grant application, the applicant agrees and understands that the only basis for reconsideration of a grant application is limited to an undisclosed Conflict of Interest as set forth in CPRIT's Administrative Rules, Chapter 703, Section 703.9.**

Communication regarding the substance of a pending application is prohibited between the grant applicant (or someone on the grant applicant's behalf) and the following individuals: an Oversight Committee Member, a Program Integration Committee Member, a Scientific Review Panel member, or a Scientific Review Council member. Applicants should note that the CPRIT

Program Integration Committee is comprised of the CPRIT Chief Executive Officer, the Chief Scientific Officer, the Chief Prevention Officer, the Chief Product Development Officer, and the Commissioner of State Health Services. The prohibition on communication begins on the first day that grant applications for the particular grant mechanism are accepted by CPRIT and extends until the grant applicant receives notice regarding a final decision on the grant application. The prohibition on communication does not apply to the time period when pre-applications or letters of interest are accepted. Intentional, serious, or frequent violations of this rule may result in the disqualification of the grant application from further consideration for a grant award.

#### **9.4. Review Criteria**

Full peer review of applications will be based on primary scored criteria and secondary unscored criteria, listed below. Review committees will evaluate and score each primary criterion and subsequently assign a global score that reflects an overall assessment of the application. **The overall assessment will not be an average of the scores of individual criteria; rather, it will reflect the reviewers' overall impression of the application. Evaluation of the scientific merit of each application is within the sole discretion of the peer reviewers.**

##### **9.4.1. Primary Criteria**

Primary criteria will evaluate the scientific merit and potential impact of the proposed work contained in the application. Concerns with any of these criteria potentially indicate a major flaw in the significance and/or design of the proposed study. Primary criteria include:

**Significance and Impact:** Will the results of this research, if successful, significantly change the research of others or the opportunities for better cancer prevention, diagnosis or treatment for patients? Is the application innovative? Does the applicant propose new paradigms or challenge existing ones? Does the project develop state-of-the-art technologies, methods, tools, or resources for cancer research or address important under- or unexplored areas? If the research project is successful, will it lead to truly substantial advances in the field rather than add modest increments of insight? Projects that modestly extend current lines of research will not be considered for this award. Projects that represent straightforward extensions of ongoing work, especially work traditionally funded by other mechanisms, will not be competitive.

**Research Plan:** Is the proposed work presented as a self-contained research project? Does the proposed research have a clearly defined hypothesis or goal that is supported by sufficient preliminary data and/or scientific rationale? Are the methods appropriate, and are potential experimental obstacles and unexpected results discussed?

**Applicant Investigator:** Does the applicant investigator demonstrate the required creativity and expertise to make a significant contribution to the research? Applicants' credentials will be evaluated in a career stage-specific fashion. Have early career stage investigators received excellent training, and do their accomplishments to date offer great promise for a successful career? Has the applicant devoted a sufficient amount of his or her time (percentage effort) to this project?

**Relevance:** Does the proposed research have a high degree of relevance to cancer research? This will be an important criterion for evaluation of projects for CPRIT support.

#### **9.4.2. Secondary Criteria**

Secondary criteria contribute to the global score assigned to the application. Concerns with these criteria potentially question the feasibility of the proposed research.

Secondary criteria include:

**Research Environment:** Does the research team have the needed expertise, facilities, and resources to accomplish all aspects of the proposed research? Are the levels of effort of the key personnel appropriate? Is there evidence of institutional support of the research team and the project?

**Vertebrate Animals and/or Human Subjects:** If vertebrate animals and/or human subjects are included in the proposed research, certification of approval by the institutional IACUC and/or IRB, as appropriate, will be required before funding can occur.

**Budget:** Is the budget appropriate for the proposed work?

**Duration:** Is the stated duration appropriate for the proposed work?

# RFA

## Date Year

Online application opens **Date Year**, 7 a.m. Central Time

Application due **Date Year**, 3 p.m. Central Time

Application review	Date	Year
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Award notification	Date	Year
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Anticipated start date	Date	Year
12/1/2023	12/1/2023	2023

Texas law requires that CPRIT grant awards be made by contract between the applicant and CPRIT. CPRIT grant awards are made to institutions or organizations, not to individuals. Award contract negotiation and execution will commence once the CPRIT Oversight Committee has approved an application for a grant award. CPRIT may require, as a condition of receiving a grant award, that the grant recipient use CPRIT's electronic Grant Management System to exchange, execute, and verify legally binding grant contract documents and grant award reports. Such use shall be in accordance with CPRIT's electronic signature policy as set forth in Chapter 701, Section 701.25.

Texas law specifies several components that must be addressed by the award contract, including needed compliance and assurance documentation, budgetary review, progress and fiscal monitoring, and terms relating to revenue sharing and intellectual property rights. These contract provisions are specified in CPRIT's Administrative Rules, which are available at [www.cprit.state.tx.us](http://www.cprit.state.tx.us). Applicants are advised to review CPRIT's administrative rules related to contractual requirements associated with CPRIT grant awards and limitations related to the use of CPRIT grant awards as set forth in Chapter 703, Sections 703.10, 703.12.

Prior to disbursement of grant award funds, the grant recipient organization must demonstrate that it has adopted and enforces a tobacco-free workplace policy consistent with the requirements set forth in CPRIT's Administrative Rules, Chapter 703, Section 703.20.

CPRIT requires award recipients to submit an annual progress report. These reports summarize the progress made toward the research goals and address plans for the upcoming year. In addition, fiscal reporting, human studies reporting, and vertebrate animal use reporting will be required as appropriate. Continuation of funding is contingent upon the timely receipt of these reports. Failure to provide timely and complete reports may waive reimbursement of grant award costs, and may result in the termination of award contract. Forms and instructions will be made available at [www.cprit.state.tx.us](http://www.cprit.state.tx.us).

## **12. REQUIREMENT TO DEMONSTRATE AVAILABLE FUNDS**

Texas law requires that prior to disbursement of CPRIT grant funds, the award recipient must demonstrate that it has an amount of funds equal to one-half of the CPRIT funding dedicated to the research that is the subject of the award. The demonstration of available matching funds must be made at the time the award contract is executed, and annually thereafter, not when the application is submitted. Grant applicants are advised to consult CPRIT's Administrative Rules, Chapter 703, Section 703.11, for specific requirements regarding demonstration of available funding.

## **13. CONTACT INFORMATION**

### **13.1. HelpDesk**

HelpDesk support is available for questions regarding user registration and online submission of applications. Queries submitted via e-mail will be answered within 1 business day. HelpDesk staff are not in a position to answer questions regarding scientific aspects of applications.

**Dates of operation:** December 9, 2013 – February 3, 2014 (excluding public holidays)

**Hours of operation:** Monday, Tuesday, Thursday, Friday, 7 a.m. to 4 p.m. Central Time  
Wednesday, 8 a.m. to 4 p.m. Central Time

**Tel:** 866-941-7146

**E-mail:** [Help@CPRITGrants.org](mailto:Help@CPRITGrants.org)

### **13.2. Scientific and Programmatic Questions**

Questions regarding the CPRIT program, including questions regarding this or any other funding opportunity, should be directed to the CPRIT Research Program Director.

**Tel:** 512-305-8491

**E-mail:** [Help@CPRITGrants.org](mailto:Help@CPRITGrants.org)

**Web site:** [www.cprit.state.tx.us](http://www.cprit.state.tx.us)





## CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS

### CPRIT's Product Development Program

CPRIT's Product Development Program was created to accelerate the progression of new cancer drugs, diagnostics, and therapies from the laboratory into clinical practice.

The objectives of CPRIT's Product Development Program are:

- To improve patient care through expedited innovation and product development;
- To foster economic development in Texas' emerging life sciences industry and the creation of high quality new jobs in this state; and
- To provide a direct return, through intellectual property and revenue sharing, on the investments made by the people of Texas.

CPRIT funds scientifically-meritorious product development projects based on the potential for translating research discoveries into products that can help cancer patients. In addition to the scientific peer review process used by all CPRIT initiatives, product development proposals undergo a due-diligence analysis to evaluate the company's regulatory plan and business operations capacity. The Oversight Committee's Economic Development Subcommittee issued a report for the commercialization program. The report is included in the appendix as Attachment 1.

CPRIT relies primarily on three grant award mechanisms to fulfill its objectives:

- **Established Company Awards** support Texas-based companies that have undertaken at least one round of professional institutional investment in developing marketable oncology products or services.
- **New Company Awards** assist start-up companies, with no previous rounds of professional institutional investment, seeking to develop marketable oncology products or services. Companies that are not already based in Texas must relocate to the state before receiving CPRIT funding.
- **Company Relocation Awards** target companies based outside of the state that have conducted at least one round of professional institutional investment. These companies must relocate to Texas to develop commercially oriented oncology products or services with CPRIT funding.

CPRIT's Requests for Applications for FY2014 Cycle 1 are included in the appendix as Attachment 2.

## ***CPRIT's Product Development Portfolio***

CPRIT's Product Development portfolio includes 13 CPRIT-funded company projects totaling \$98 million. The funded projects include promising drugs, diagnostics, and devices targeting a variety of cancers, including cancers of the blood, colon and rectum, esophagus, stomach, lung, liver, and prostate. Together with the companies' required matching funds, total investment in research and development for the 13 CPRIT-funded company projects will exceed \$150 million.

In addition to creating new and improved tools and treatments for fighting cancer, CPRIT's investments are helping to build Texas' life-science industry. While bringing a product to market can take time, jobs and economic activity are generated throughout the process. Projects funded by CPRIT are expected to create approximately 140 direct jobs — highly skilled, high-wage positions in life sciences — in Texas over the three-year term of CPRIT's grant awards.

Every CPRIT award includes an intellectual property agreement that specifies a revenue return to the State of Texas from the successful development of CPRIT-funded drugs, devices, diagnostics, or services. These revenue-sharing standards provide a fair return on Texas' grant funds without impeding the ability of the company to attract future investment. Like any interested investor, CPRIT is an engaged partner and holds award recipients accountable for their efforts to bring products to market.

As of August 31, 2013, ten CPRIT-funded company projects were active.<sup>1</sup>

### ***Representative Product Development Program Grants***

#### **New Company/Company Formation**

- Pulmotect (CP120014) *Expanding the Market and Success Rates for Myeloablative Cancer Treatments Using PUL-042, an Innate Immune Stimulant*

Pulmotect's technology is designed to boost the immune system of the lungs. For cancer patients, this could help prevent and treat pneumonia, a leading cause of death during periods of immunosuppression. In addition, cancer treatments may be improved by reducing the risk of respiratory infections. Multiple indications exist beyond the use for immunosuppressed patients, such as asthmatics, pandemic threats and bioterrorism. In its first year of CPRIT funding, the company is on track with milestones to complete pre-clinical activities to support the upcoming clinical trials. This has included manufacturing the drug for human use, conducting final animal testing, and conducting regulatory meetings with the FDA. According to Pulmotect, "CPRIT funding has allowed us the ability to focus on developing the technology and advancing it to the clinic

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<sup>1</sup> One project, Apollo Endosurgery, completed its work on the CPRIT-funded project prior to FY2013. One project, Kalon Biotherapeutics, did not begin in FY2013 due to the moratorium. The third project, Peloton Therapeutics, was frozen during FY2013 awaiting review of its application.

as quickly as possible to help save lives.” Since receiving the CPRIT award, Pulmotect has raised an additional \$3.5 million in grants and equity investments.

#### Established Company Award

- Bellicum (RP110508) *Clinical Development of CaspaCIDE, a Cell Therapy Safety Switch*

Bellicum is developing a combination product for adult and pediatric leukemia or lymphoma patients undergoing hematopoietic stem cell transplantation (HSCT). The product under development contains Bellicum’s CaspaCIDE safety switch, which allows rapid resolution of Graft versus Host Disease should it occur. This approach promises to reduce transplant related morbidity and mortality for patients who have a matched donor, and make HSCT a viable option for the 50% of transplant candidates who are currently ineligible because they do not have a matched donor. During the past year, Bellicum has initiated a Phase 1/2 clinical trial. Six US clinical trial sites open for enrollment, including two in Texas, with a parallel trial at a third Texas site in negotiation. The company reports that the diligence conducted by CPRIT and the funding award allowed the company to attract new investors for its second round of professional institutional fundraising totaling \$34 million.

- Asuragen (CP120017) *Clinically Actionable Mutation Profiling for Cancer Personalized Medicine using Scalable, Ultra-deep Next Generation Sequencing*

Next-generation sequencing is revolutionizing diagnostic and therapeutic approaches in oncology. Asuragen’s CPRIT-funded project for next-generation sequencing technology pinpoints molecular changes in cancer patients that can guide clinical care and help accelerate targeted drug development through clinical trials. By commercializing assays that identify “druggable” mutations in cancer genes, Asuragen’s innovations can improve the selection of patients likely to respond in cutting-edge clinical trials for emerging therapies, as well as improve surgical decisions or individualize treatments of existing anti-cancer drugs that can increase survival, reduce morbidity, and optimize quality of life. In its first year of funding, Asuragen launched SuraSeq® 500 in Asuragen’s CAP-accredited CLIA laboratory, a targeted next-generation sequencing clinical test that identifies >500 known cancer mutations from low inputs of DNA from patient cancer biopsies. This launch satisfied the most significant milestone for the company’s year 1 activities. The company also commercialized QFI®-PCR, a next-generating sequencing-based sample quality control tool, and SuraSight®, an optimized bioinformatic pipeline and user-friendly graphical interface for analyzing and interpreting sequencing data. The first revenues from the commercialization project were generated, and the first royalty payment to CPRIT was made. The company characterizes the CPRIT funding award as “critical to expanding Asuragen’s pharmacogenomics services business through

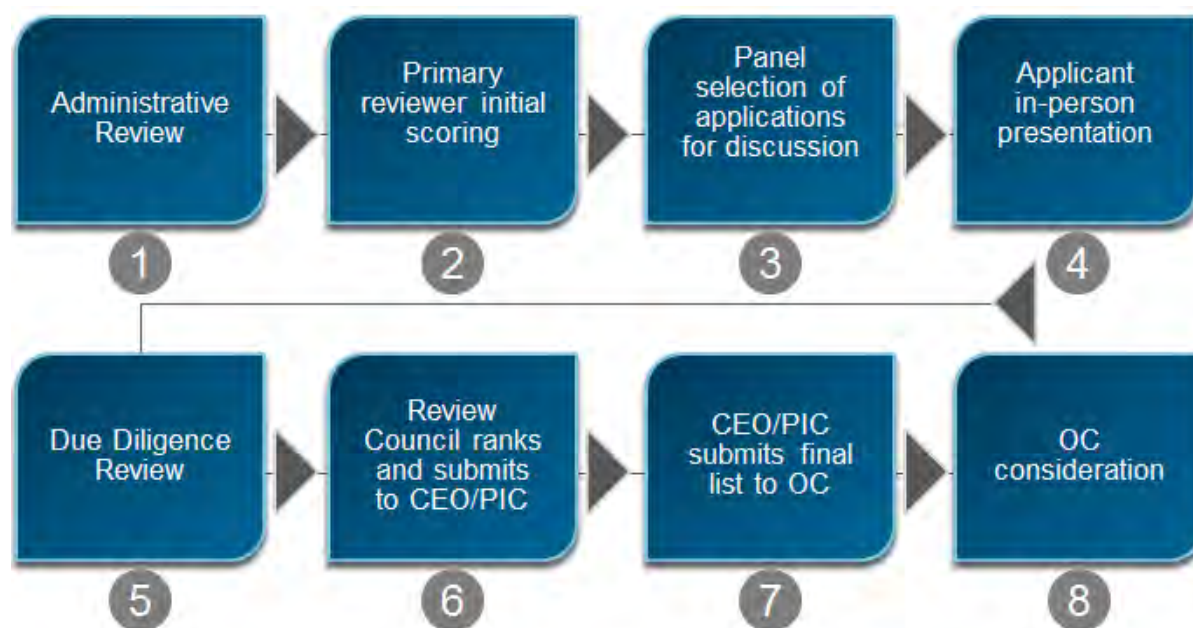
technology innovations for clinical research, and the development of comprehensive procedures, quality metrics, and controls that can support both diagnostic services and product development. Next-generation sequencing is the fastest growing category of Asuragen services, and CPRIT-funded resources have been integral to sequencing-based diagnostic assay development, hiring and retaining top talent, recruiting additional capital, and supporting commercialization opportunities that can advance personalized medicine in oncology.” Since receiving the CPRIT award, Asuragen has raised \$15 million in additional capital.

## CPRIT Company Portfolio

Company	CPRIT Investment	Funded Project	Follow-on Capital (Post Award)
Apollo Endosurgery, Inc. <i>Austin</i>	\$5,001,063	Flexible endoscopic surgical tools for better removal of flat polyps	\$130 million
Asuragen, Inc. <i>Austin</i>	\$6,837,265	Diagnostic gene-sequencing clinical test to identify known cancer mutations	\$15 million
Bellicum Pharmaceuticals, Inc. <i>Houston</i>	\$5,680,310	Combination product to rapidly resolve Graft vs. Host Disease	\$34 million
Caliber Biotherapeutics, Inc <i>College Station</i>	\$12,808,151	Monoclonal antibody therapy	\$10.4 million
Cell Medica, Inc. <i>Houston</i>	\$15,571,303	Cellular immunotherapy therapies for cancers associated with the Epstein Barr virus and for CMV infections following bone marrow transplants	\$11 million
InGeneron, Inc. <i>Houston</i>	\$198,111	Portable imaging device to visualize cell markers	\$2.4 million
Kalon Biotherapeutics, LLC <i>College Station</i>	\$7,901,420	Drug manufacturing technology capability for Phase I and II clinical trials	\$3.95 million
Mirna Therapeutics, Inc. <i>Austin</i>	\$10,297,454	Drug development to treat liver cancer	\$36.9 million
Molecular Templates, Inc. <i>Georgetown</i>	\$10,600,000	Drug development to treat Non-Hodgkin's Lymphoma	\$10 million
Peloton Therapeutics, Inc. <i>Dallas</i>	\$11,044,931	Drug development	\$30.6 million
Pulmotect, Inc. <i>Houston</i>	\$7,126,398	Boost immune system of lungs, helping to prevent and treat pneumonia	\$3.56 million
Rules-Based Medicine, Inc. <i>Austin</i>	\$3,024,432	Diagnostic tool identifying cancer biomarkers	\$81 million (includes acquisition)
Visualase, Inc. <i>Houston</i>	\$2,151,776	Laser technology to precisely target prostate tumors	\$5.2 million

### ***CPRIT's Product Development Review Process***

CPRIT uses a multi-step peer review process to evaluate Product Development applications. The peer reviewers are a group of qualified experts from the areas of academia, clinical medicine, investments and management with experience relevant to cancer. All reviewers live and work outside of Texas. Working as a team, the reviewers jointly evaluate applications in their respective areas of expertise and experience. In order to alternate the workload, there are currently two Product Development Review Panels, each conducting approximately two review cycles per year. The application submission and peer review processes are administered by SRA, International a CPRIT contractor with extensive experience in grants review.



#### **Step 1 - Administrative Review**

The first step in the application review process is administrative and is conducted by the SRA staff. The administrative review takes about a week to complete. This initial administrative review confirms that an application has all the elements requested in the RFA and that all other submission requirements are met. Applications that pass administrative review are forwarded to the Chair of the Product Development Review Panel and move into the second stage of evaluation.

#### **Steps 2 and 3 - Individual Evaluation and Teleconference Review**

In the second stage of evaluation, Review Panel members are provided abstracts for all applications that passed administrative review. Each Product Development reviewer advises the Product Development Review Panel Chair which applications fall within their area of experience, expertise and interest. Any potential conflicts of interest are also reported at this time so that the reviewer is excluded from any evaluation, discussion and vote related to the identified application(s). Using this information, the

Chairman assigns three to four primary reviewers to each application. Each reviewer is given multiple applications to review. Reviewers evaluate each assigned application through an online system, by submitting a written critique and numeric scores based on review criteria specified in the RFA. These scores range from 1 (excellent) to 9 (extremely poor). An overall score is also assigned to each application.

Once all applications have been initially scored (approximately five weeks after the RFAs are closed), the Product Development reviewers and the Chair meet via teleconference for approximately one-half day to compare notes, discuss the applications, assigned scores, and make additional comments. The assigned reviewers may adjust scores based on these discussions. The entire review panel decides which applications move to the next stage of evaluation. The conference call is set up and moderated by SRA staff to ensure that conflict of interest procedural requirements are followed. The Product Development Officer and other CPRIT staff may also attend the teleconference. However, CPRIT staff participation is limited to answering procedural questions. CPRIT staff do not participate in the substantive discussion, scoring or vote for any applications. All applicants are provided reviewer feedback on their application, regardless of their score.

#### **Step 4 - In-Person Presentations and Evaluation**

The applicants with sufficiently positive scores as determined by the Product Development reviewers move into the third stage of evaluation and are invited to present their proposal, in-person, to the entire Product Development Review Panel approximately three to four weeks later. Applicants that are invited to make in-person presentations are provided a list of questions that the Product Development reviewers want to have specifically addressed regarding their application.

At the in-person meeting, each applicant is given twenty minutes to make their presentation to the review panel. This is followed by twenty minutes for reviewer questions. At the conclusion of the Q&A session, the applicant is excused from the room. The reviewers discuss the application and all reviewers individually submit an overall score for the application. The reviewers' discussion and scoring may take up to 45 minutes. Regardless of score, all applicants receive feedback from this stage of evaluation. Patient advocate reviewers participate in the in-person presentations and scoring.

#### **Step 5 - Due Diligence Review**

The applications that the reviewers feel score sufficiently well after the in-person presentation move into the fourth stage of evaluation -- due diligence review. Due diligence is conducted by outside contractors hired by CPRIT and is overseen by the Chief Product Development Officer. These contractors conduct in-depth evaluations in the areas of intellectual property, clinical trial design, regulatory affairs, manufacturability of product, marketing, etc. Due diligence review takes 45 - 60 days to complete. A draft report is sent to the applicant for comment. Once the applicant comments are received, a final report is sent to the Primary Reviewers and the Product Development Review Council (the four senior members of the Review Panels) for their consideration.



## **Step 6 - Review Council Recommendations**

During the fifth and final stage of evaluation by the review panel, the Product Development Review Council and the primary reviewers hold a teleconference to discuss the due diligence results. A final recommendation is then made by the reviewers regarding whether the application should be recommended for CPRIT grant funding. All Product Development applications recommended for grant funding are ranked by the reviewers and submitted by the Product Development Chair to CPRIT's CEO (for applications submitted prior to June, 2013) or the Program Integration Committee (for applications submitted after June, 2013.)

## **Review Process Pre and Post SB149**

The review process for product development grants described above remains essentially unchanged for soliciting the applications, submitting the applications to CPRIT, documenting the recusal of reviewers with conflicts of interest, evaluating the applications, scoring the applications, and creating a list of grant applications that the Review Council recommends for CPRIT awards. Keep in mind that the intense legislative focus and criticism levied at CPRIT centered on past grant award decisions when CPRIT failed to follow its established processes, not that the processes were flawed or inadequate. There is not a lower or less rigorous standard of peer review for the applications that will be considered on February 19, 2014, by the Oversight Committee compared to the applications solicited after the passage of SB149.

This may be hard to reconcile with the 100 pages of new rules and rule changes that the Oversight Committee adopted on January 24, 2014. Some new issues, prohibitions, and requirements are being introduced for the first time in the administrative rules. The creation of the PIC is a good example. However, for the most part, the "new" rules codify existing CPRIT practices and procedures CPRIT is already fully implementing - particularly for the grant review process. For example, CPRIT has used third party observer reports to document compliance with CPRIT's conflict of interest requirements beginning in early 2012, even though third party observer reports are explicitly mentioned for the first time in the new rules. Similarly, CPRIT's compliance officer has publicly certified the grant award recommendations prior to Oversight Committee action since December 2012; however, the requirement for compliance certification was not introduced in CPRIT's statute or CPRIT administrative rules until 2013. In fact, the "new" conflict of interest requirements that are now part of CPRIT's statute following the last legislative session were lifted, almost verbatim, from CPRIT's administrative rules that were adopted in early 2010.



## ***Company Award Contract Negotiation and Monitoring***

Once the Product Development grants are approved, negotiations begin between the company and CPRIT (typically led by the Chief Product Development Officer, with assistance from legal counsel and the Product Development Review Council if necessary) regarding the revenue-sharing terms to be included in the grant funding contract. It is possible that a project may be approved by the Oversight Committee for CPRIT grant funding, but the grant is never awarded (and grant funds are not disbursed) because the applicant cannot agree to CPRIT's contractual terms. If no agreement is reached, the contract is not executed and grant funds set aside for the project are released.

For product development grant projects, CPRIT funding is typically provided in stages, often referred to as “tranches,” and tied to the achievement of specific milestones. Each slice of funding, commonly known as a tranche, and its associated objective or deliverable are negotiated and included in the award contract. Tranching adds complexity, both to contract negotiation and to contract monitoring, but it is an effective way to limit CPRIT's risk exposure. Although the total award amount for the project must be ratified by the Oversight Committee, the grantee receives only enough grant funds to accomplish the specified milestones within the particular tranche. The company must demonstrate successful completion through a written report detailing how the company has achieved the goals tied to a specific tranche in order to access the next amount of grant funding. Expert reviewers assess the work done by the company and approve the release of the next tranche of funding or recommend that funding be terminated.

Tranches for the grant project are developed using deal-specific documentation, including:

- Information supplied by the applicant. Applicants are asked to provide specific goals and associated timelines for the proposed project in the application. The aims and timeline are evaluated during the review process and the reviewers may indicate a change to be included in the contract or an issue to be negotiated.
- Information from the due diligence review. Icon, the company that performs due diligence reviews of CPRIT's product development applications, provides guidance on appropriate milestones to be achieved during the course of the project.
- Information from the intellectual property review. IP counsel may provide recommendations regarding specific steps to be taken regarding protecting IP, ensuring freedom to operate, or cleaning up problematic licensing agreements.

An issue may be identified by the Review Council or during the due diligence review that if not corrected or adequately addressed, could be a reason for CPRIT not executing the contract for an otherwise worthy project. Although it is not technically a tranche recommendation, this information impacts the contract negotiations. For example, the IP and licensing review may identify an issue with the license agreement for the underlying technology that, if not resolved, is a deal breaker. CPRIT will direct the company to fix the underlying licensing issue (usually

through renegotiation of the underlying licensing agreement) before contract negotiation with CPRIT can begin.

Another important negotiation point is the agreed form, amount, and timing of revenue sharing payments to CPRIT. CPRIT's return on investment can be in the form of equity, royalties, or a combination of both. Like the funding tranches, revenue sharing terms are deal specific and are negotiated to address the particular project.

Whether or not an applicant sufficiently achieves contract milestones to continue funding is monitored by the Chief Product Development Officer and determined by Product Development reviewers and the Product Development Review Council. Failure to meet required milestones will result in contract termination and will be reported to the Oversight Committee.

# Appendix

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# Memo

Date: June 15, 2009

To: CPRIT Oversight Committee

From: CPRIT Economic Development & Commercialization Subcommittee

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The Economic Development & Commercialization Subcommittee has been working over the past six months to develop a set of recommendations to the Oversight Committee regarding both what the commercial activities of CPRIT should constitute as well as how the Institute should administrate and execute on these activities.

The Subcommittee has been guided by the Institute's enabling Legislation as well as CPRIT's Mission Statement, both of which specifically provide for commercialization activities. The rationale behind providing for these activities is that research will only enhance patient outcomes if it leads to commercial products and processes to cure cancer or prolong the lives of patients and improve the quality of their lives while being treated, regardless of the patient's ultimate outcome. Behind Tab 1 are contained CPRIT's Mission Statement (Section A) and a **DRAFT** Mission Statement to serve as a framework for CPRIT's commercialization activities (Section B).

In developing its recommendations, the Subcommittee, through a series of meetings, has attempted to solicit input from a wide range of relevant constituencies, both in-state as well as out-of-state. A listing of meeting dates and participants is located in Tab 2.

The Oversight Committee needs to always keep in mind the challenge it faces in using funding to achieve successful commercial outcomes. It's the same challenge CPRIT faces with respect to the research initiatives it supports. The magnitude of this challenge is amply demonstrated in the charts contained under Tab 3.

The recommended scope of CPRIT's commercial activities is contained under Tab 4, Section A. The scope of these commercial activities extends from translational research through Phase 2, and into Phase 3 on an exception basis, as well as selected infrastructure investments. It is important to note that, in all cases, these recommended activities will require both a scientific and commercial review and, in some cases, a legal review of the quality of the underlying intellectual property. It is further recommended CPRIT not use an RFP process for translational and commercial proposals as it is difficult to predict innovation.

To assist the Oversight Committee, and the constituencies it serves, the Subcommittee has begun the process of defining commonly used life science terminology contained in this report (see Appendix). Having accepted definitions will simplify the Institute's future commercial activities, as currently multiple definitions exist within the industry for these frequently used terms. Expanding on and refining these definitions will constitute an ongoing process.

The rationale for the breadth of these commercial activities is the same as those that led to the creation in 2005 of the Texas Emerging Technology Fund ("ETF"). The underlying premise for the ETF is that, unlike the East and West Coasts, Texas lacks sufficient angel investors and venture capitalists to supply the necessary capital to fund these critical commercialization activities and, therefore, this "gap" needs to be provided by taxpayer support until our biomedical/device industry reaches critical mass. Tabs 5A&B lay out this "gap" schematically. Unfortunately, the underlying rationale for the ETF is even more true today than it was four years ago, as recent events in capital markets and the economy have adversely affected angel investors' balance sheets and the flow of institutional funding to life science venture capital firms, and the public IPO market is closed to early stage life science companies.

While the ETF is still open for business, the Subcommittee recommends that CPRIT also make funding available for these commercial activities, albeit for a broader range of programs than the ETF supports. Additionally, it is the expectation of the Subcommittee that CPRIT will receive a substantially greater volume of cancer-related commercial applications across the spectrum of these activities when their availability becomes known. Further, oncology commercial development has characteristics unique to the disease and requires specialized skills, particularly as it relates to demonstration of efficacy and approval of products. If approved, these commercial activities will require CPRIT to coordinate with those of the ETF.

As mentioned above, the Subcommittee has also addressed issues related to how CPRIT should administrate and execute on its commercial activities and has developed three major recommendations in this regard. The first addresses how the commercial programs should be administrated and the other two relate to strategic programs the Subcommittee believes will greatly enhance the process in Texas by which research discoveries are commercialized.

#### **Recommendation 1:**

CPRIT should utilize the Texas Life Science Center (TLSC) to conduct all "commercial" reviews which would include translational research, proof of concept, company and commercial infrastructure proposals. A flow chart laying out CPRIT's commercial review process utilizing the TLSC and a description of this process is contained in Tab 6.

#### **Rationale:**

The TLSC Board is uniquely positioned to assist CPRIT in reviewing promising oncology products and infrastructure.

1. Regardless of compelling scientific and/or clinical rationale, commercial proposals designed to ultimately provide a product for the marketplace require a commercial review consistent with "commercial standards". This review includes end user considerations that drive commercial viability, FDA exacting regulatory and development plans, manufacturing feasibility and an understanding of the competitive landscape.
2. Utilizing the TLSC for CPRIT commercial reviews will allow CPRIT to leverage the process the TLSC has developed and refined over the past four years acting in a similar capacity for the ETF.

3. The TLSC 24 member statewide Board would be difficult, if not impossible, to duplicate in the state. The Board consists of venture capitalists and private equity investors, university technology commercialization professionals, life science entrepreneurs, professors of medicine, academic researchers, and executives in early stage life science companies who possess diverse and complementary expertise in life science commercialization.

**Recommendations from the TLSC to CPRIT will be strictly advisory. Approval of all funding proposals will remain the exclusive prerogative of CPRIT.** The process by which proposals are reviewed by CPRIT and the TLSC would be multi-staged and structured to provide input from various constituencies, including the Scientific Research and Prevention Programs Committee.

#### **Budget:**

If the Oversight Committee approves utilizing the TLSC to assist in administering CPRIT's commercial activities, the Institute will be able to share costs associated with TLSC's activities with the ETF. Based upon TLSC's history, the oncology-related component of the ETF's total life science activities is approximately 40%. Based upon the TLSC's current budget, CPRIT's 40% share would total approximately \$200,000 for the first year.

#### **Strategic Initiatives:**

The two remaining Subcommittee recommendations attempt to address major shortcomings in the State that currently impact the process by which research discoveries lead into commercial products and services. The underlying rationale for these recommendations deserves elaboration.

Life science businesses need the support of committed, sophisticated investors and venture capital ("VC") partners in order to achieve successful commercialization. Texas is not creating enough "investor ready" companies. Experienced leadership at an early stage is a vital part of the commercialization process. Often, Texas companies make poor decisions in the early stages of their development that turn out to be fatal, causing the technology to "die on the vine". Examples of poor decisions fall into a variety of categories; a clinical trial not designed robustly enough to derive definitive answers, preclinical work that fails to give experienced investors adequate results, capitalization structures that do not fit the accepted model, and naïve intellectual property decisions. These mistakes are only a sampling of the poor decisions that can be made in early stage life science companies and are not meant to be a complete list. Tab 7 schematically sets forth these critical decision points

Poor decision-making frequently occurs in the State due to the lack of experienced management talent the East and West Coasts have in abundance. It is not simply a lack of CEOs or entrepreneurs, it is a lack of experience at all levels of middle and upper management. In Texas, we often have "enthusiastic inexperienced management" making decisions based upon consultation with people who possess limited experience, yet (in this region) are looked on as experts. These people are well meaning and can be useful; however, their advice results repeatedly in poor decisions by the State's young companies. This is not meant as a blanket indictment of local life science talent, as there are a number of talented individuals and good support companies. In summary, all too often our region's universities and research institutions' breakthrough research does not translate into approved products.

The State can wait and hope that in the same 30 years it took San Diego to build its cluster, we can successfully build ours - through natural evolution. Alternatively, CPRIT can implement novel approaches to fill this experience gap.

The Subcommittee recommends two strategic programs focused on adding value to the Texas life science ecosystem. The focus of these programs over the next 5 years will be the creation of investor ready companies. These programs target the creation and development of early-stage companies. The goal is to measurably impact the start-up, growth, and syndication of 20 cancer-related life science companies in the state over the next five years.

The plan is a bifurcated one, based on establishing two new programs:

- An Entrepreneur in Residence program at Texas based venture capital firms; and
- A Virtual Management Company which will be the umbrella program to provide more comprehensive guidance for company development to prevent the common pitfalls currently experienced by the state's early-stage life science companies.

#### **Recommendation 2:**

Establish an Entrepreneur in Residence (EIR) program in partnership with Texas venture capital firms to recruit experienced entrepreneurs to establish and build a variety of promising oncology product companies in Texas.

#### **Rationale:**

Ideas, technology, money, and institutions do not start companies – people do, specifically, entrepreneurs. We must attract experienced life science executives to Texas who have the ability to capitalize on the large number of opportunities generated by research breakthroughs. For years, national venture capital firms have embraced the concept of Entrepreneurs in Residence (EIR). VCs bring experienced entrepreneurs on staff, at below market compensation, for 12 -18 months while the entrepreneur searches for a technology(ies) around which to create their next portfolio company. This effort is done in tandem with the VC firm whereby when the company is born, a lead investor and CEO are already in place.

Rather than “warehouse” life science entrepreneurs at CPRIT, or a central location, EIRs should be placed in venture firms for numerous reasons:

- Total costs of the program will be substantially reduced.
- VC firms have the greatest access to deal flow and funding; either self-generated or through relationships with other worldwide VCs.



- Through the extensive experience of their VC partners and advisors, EIRs in venture capital firms will be ideally positioned to quickly identify the most promising Texas and non-Texas cancer technologies for further due diligence.
- Easy access to market research, expertise and experience provided by VC firms will allow EIRs to effectively due diligence the pipeline of potential technologies and identify those most promising.
- Texas VC firms have already expressed a willingness to invest in the proposed EIR program, allowing CPRIT to leverage its investments into a greater number of EIRs.
- The EIR concept is well established in the venture community as the best way to create new companies and investment opportunities.

Additionally, Texas research superiority funds have supported the recruitment of world-renowned researchers to Texas institutions. Research superiority funding has been a significant component of the ETF and is under consideration as a component program for CPRIT. Texas researchers create highly promising discoveries with strong commercial potential. The EIR program will build on these Texas investments by bringing experienced and talented commercial executives to Texas to work with venture firms to complete the value chain.

Finally, EIRs do not just bring their capabilities; they bring a vetted network of contacts and experts (and ultimately other experienced employees) that can be deployed to support the growth of all future oncology companies in the state.

**Concept:**

The EIR program will initially recruit five entrepreneurs/managers to Texas and place them in venture capital firms, either headquartered or with offices in Texas. A formal networking process will be established to allow the EIRs to support each other, share ideas and work closely with the Virtual Management Company (see discussion below).

The EIRs will be jointly funded on a 50/50 basis with the Texas venture capital firms selected to participate in the program. An executive search firm, experienced in life science executive recruiting, will conduct a worldwide search to identify experienced entrepreneurs/managers who live outside of Texas to be relocated to the state. CPRIT will fund the search and relocation of these executives. Once placed in a venture firm, CPRIT will support 50% of the EIR's salary and benefits. All other administrative costs will be paid by the VC firms which will exercise day-to-day control over the EIRs' efforts to identify new companies. It is planned that each EIR will identify, establish and lead a new oncology company within 18 months of arrival in Texas.

The program is budgeted for approximately \$800,000 in the first year of operation and \$1.3 million each year thereafter. This funding will support CPRIT's portion of the salary for five EIRs, recruiting and relocation expenses and administrative support for the program. It is assumed, as EIRs form new companies and leave the program, that new EIRs will be recruited and hired to replace these vacated positions.

### **Recommendation 3:**

Develop a network of capabilities through the establishment of a Virtual Management Company ("VMC") to leverage skills of commercial experts throughout the world to support the creation and growth of successful Texas based oncology product companies. An organization chart for the VMC and position descriptions are contained in Tab 8.

#### **Rationale:**

As discussed above, Texas must remedy our commercial expertise deficit. Commercial experts and capabilities are mobile and can be deployed to support our emerging oncology companies on a "virtual" basis. The majority of new emerging companies are being built "virtually" today. The VMC will organize a network of worldwide experts and serve as a clearinghouse to our state companies and institutions. The VMC will ensure that new projects or companies do not have to "reinvent the wheel" every time expertise needs to be sourced to solve specific problems. The establishment of a virtual management infrastructure can and will also work to incentivize experts to build a long term presence in Texas to support the development of all therapeutic areas, whether oncology or non-oncology.

#### **Concept:**

The key components of the VMC hub and spoke model are:

- Creating a virtual consulting network to provide a specialized support infrastructure;
- Building a human capital database of life science professionals to match with companies;
- Providing business tools and research for early stage life science companies; and
- Match-making (with investors) and business development (for R&D partnerships).

The VMC will work with CPRIT and the TLSC to vet both translational research, proof of concept, company and infrastructure related proposals. Once these companies/programs have been approved by CPRIT, VMC management will work with the selected companies/programs to develop a specific consulting needs plan and then retain a mix of consultants to work directly with the company/inventor. The VMC will manage the consulting process to ensure the needs of the companies and consultants are met. In addition, the VMC will report to CPRIT and the TLSC on a scheduled basis regarding the status of ongoing projects.

The VMC will work closely with institutional technology transfer offices and local management to transfer knowledge and provide advice at the earliest stage of development. Initial advice will be focused around product/business feasibility and the creation of sound development/regulatory/clinical plans such as:

- Turning a discovery into a product with clear commercial rationale;
- Identifying product development hurdles from first identification of a 'commercial' idea;
- Identifying the most efficient process to get to IND/IDE with FDA approval in mind;
- Sound pre-clinical and clinical development plans; and
- Future Chemistry, Manufacturing and Controls (CMC) requirements

This expert network will close the knowledge gap identified previously, and ensure ideas developed in the region receive the best advice possible, thereby allowing the greatest number of "investor ready" companies to be created.

The virtual management company is a novel concept not duplicated anywhere else in the United States. The Subcommittee believes this unique approach is a realistic and supportable way to close the talent and experience gaps that prevent Texas from effectively capitalizing on its research. It has the further benefit of bringing expertise into the region that can mentor the hundreds of educated scientists and business graduates from Texas institutions who want to pursue commercial careers in the life sciences.

The services of the VMC can be provided to participating academic/research institutions, their spin-off companies and shared with all communities in the state to support oncology and non-oncology life science product development efforts. For projects/companies that will not be CPRIT funded, the consulting services of the VMC industry experts can be made available through a fee-based structure.

The VMC budget will be built primarily around expert consultants, and a staff of project managers to work with the companies and consultants to ensure a smooth process. The VMC will also build three additional capabilities internally. First, a market research capability to provide input into business plans and help support the commercial rationale of projects to outside investors. This capability will include a mix of outside market research experts and internal materials (e.g., reports and databases) specifically focused on oncology markets. Second, the VMC will retain an internal Human Resources expert to support the development of the network of consultants and EIRs. Ultimately, the database created will support all emerging and developing companies as they seek experts and employees. Finally, the VMC will include a business development function to organize an outreach program to bring promising technologies into the state.

The total budget planned for the VMC is \$1.7 million in the first year increasing to \$5 million based upon the estimated number of companies and translational research projects in year 5.

**Summary:**

Taken together, the Subcommittee recommendations regarding CPRIT's commercial activities address both the funding and capability gaps inherent in the state which impede the many cancer-related research discoveries in the state translating into commercial products and services to improve cancer patient outcomes. Tab 9 sets forth a comprehensive schematic of how these recommendations come into play along this continuum from discovery to the marketplace.

# **CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**

## **Mission**

**The Cancer Prevention and Research Institute of Texas is established to:**

- **Create and expedite cancer research innovation and breakthroughs in cancer prevention and cures for cancer**
- **Attract, create, or expand research capabilities of public or private institutions of higher education and other public or private entities that will promote a substantial increase in cancer research and in the creation of high-quality new jobs in Texas**
- **Develop and implement the Texas Cancer Plan**

# **CPRIT ECONOMIC DEVELOPMENT AND COMMERCIALIZATION SUBCOMMITTEE**

## **Mission**

**CPRIT's Economic Development and Commercialization activities are defined as those which:**

- **Enhance opportunities for breakthrough cancer-related technologies**
- **Lower cost barriers to personalized treatment and clinical practice for treating cancer**
- **Create the critical mass necessary and infrastructure capabilities required for a sustainable cancer industry in Texas**

# Economic Development & Commercialization Subcommittee Process

Charles Tate, Jimmy Mansour, and Malcolm Gillis

## February 11, 2009: Academic & Research Institutions

- The Academy of Medicine, Engineering and Science of Texas
- Baylor College of Medicine
- The Methodist Hospital Research Institute
- Mary Crowley Cancer Research Centers
- Rice University
- Texas A&M University System Texas
- Texas State University – San Marcos
- Texas Tech University
- University of Houston
- University of North Texas
- The University of Texas System
  - UT Austin
  - UT Health Science Center San Antonio
  - UT Health Science Center Houston
  - MD Anderson Cancer Center
  - UT Southwestern

## March 6, 2009: Management & Venture Capital

- Bay City Capital
- Bruce Given Consulting
- Castle Biosciences, Inc.
- DFJ Mercury
- Forward Ventures
- Introgen Therapeutics
- Santé Ventures
- 30+ Executives and VCs in attendance

## April 7, 2009: Commercialization Working Group

- BioHouston, J. Northcut
- Bruce Given Consulting, B. Given
- Introgen Therapeutics, D. Enloe
- Mary Crowley Cancer Research Center, D. Shanahan, S. Cagnina, and J. Neumenitis
- Santé Ventures, J. Cunningham
- Texas Life Science Center, A. Nat

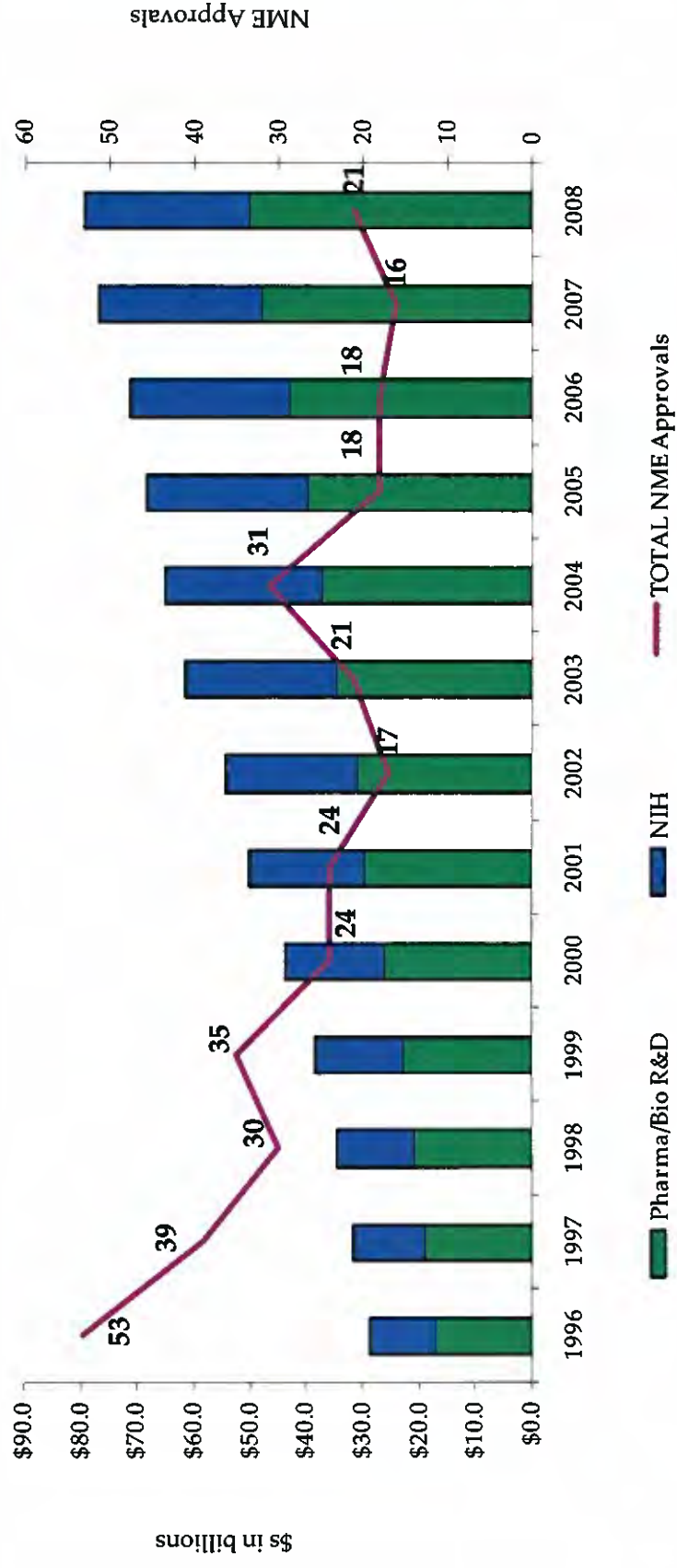
## April 15, 2009: Commercialization Working Group

- CPRIT, Bill Gimson
- Jimmy Mansour
- Governor's Office, H. McConnell
- Texas Life Science Center, A. Nat
- BioHouston, J. Northcut
- Charles Tate

## April 16, 2009: Management Meeting

- Agennix, Inc., A. Varadhachary and R. Barsky
- BioHouston, J. Northcut
- Lexicon Pharmaceuticals, A. Sands

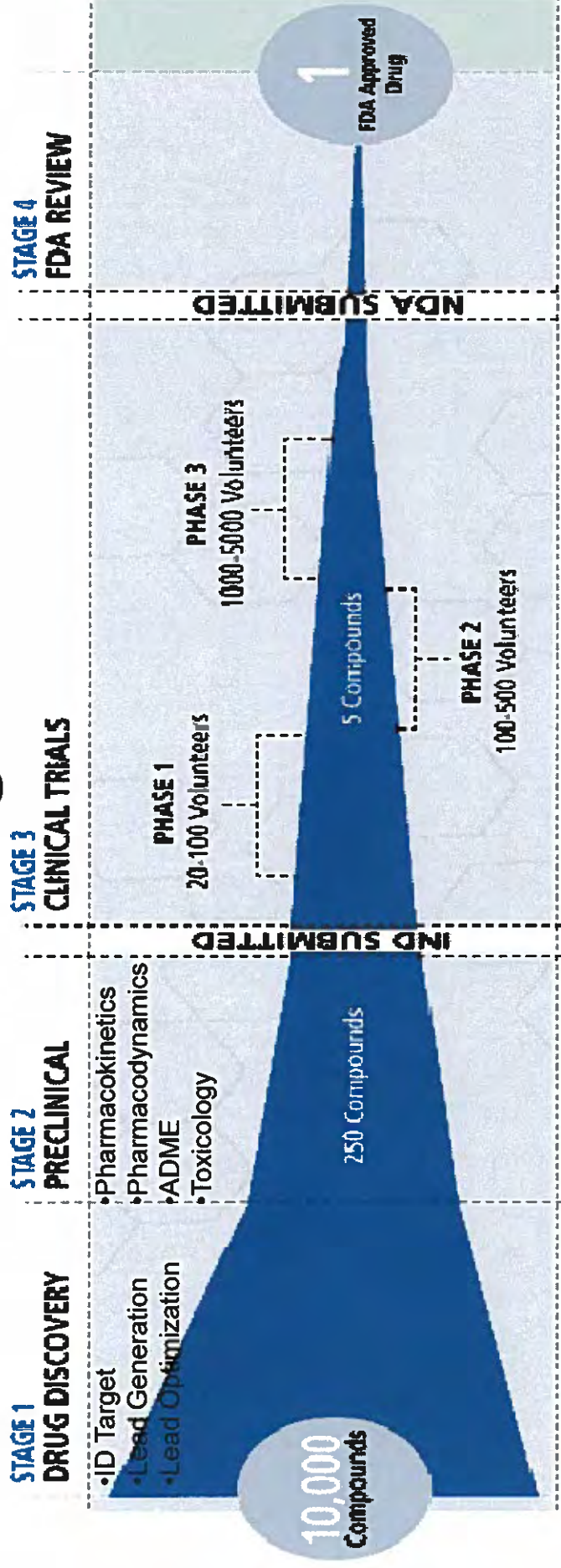
# R&D Spending Outpaces Results



US Drug Approval Trends and Yearbook 2008/2009; FDA; Paraxel Bio/Pharmaceutical R&D Sourcebook 2008/2009; Pharmaceutical Research and Manufacturers of America (PhRMA) Annual Membership Survey, 2009; L.E.K.



# Drug Development Product Diagram



*Oncology Drug Development Products Have Their Own Unique Characteristics*

# **CPRIT ECONOMIC DEVELOPMENT AND COMMERCIALIZATION PROGRAMS**

## **INCLUDED**

### **I. TYPES**

#### **A. Research Related**

- Translational Research**
- Proof of Concept**
- Pre-Clinical**

#### **B. Commercial**

- Early to Mid-Stage Cancer-Related Life Science Companies**
- Cancer-Related Infrastructure Companies/Facilities**

#### **C. Industry/Regulatory Outreach Programs**

- Pharmaceutical/Biotech**
- FDA/NCI**

### **II. ELIGIBLE PRODUCTS/SERVICES**

#### **A. Therapeutics**

- Small Molecule**
- Biologics**

#### **B. Diagnostics**

#### **C. Devices**

#### **D. Potential Breakthrough Technologies**

- Software**
- Research/Discovery Techniques**
- ?**

## **INCLUDED (Continued)**

### **III. ELIGIBLE STAGE OF DEVELOPMENT**

- Translational Research**
- Proof of Concept**
- Pre-Clinical**
- Clinical**
- Phase I**
- Phase II**
  - Limited, including Clinical Trials**

### **IV. ELIGIBLE APPLICANTS**

- A. Research Institutions**
- B. Texas Based Companies**
- C. Non-Texas Based Companies willing to move into Texas**

### **V. FORMS OF CPRIT ECONOMIC CONSIDERATION**

- A. Royalty/Revenue Participation**
- B. Common/Preferred Stock/Warrants**

**CPRIT ECONOMIC DEVELOPMENT AND  
COMMERCIALIZATION PROGRAMS**

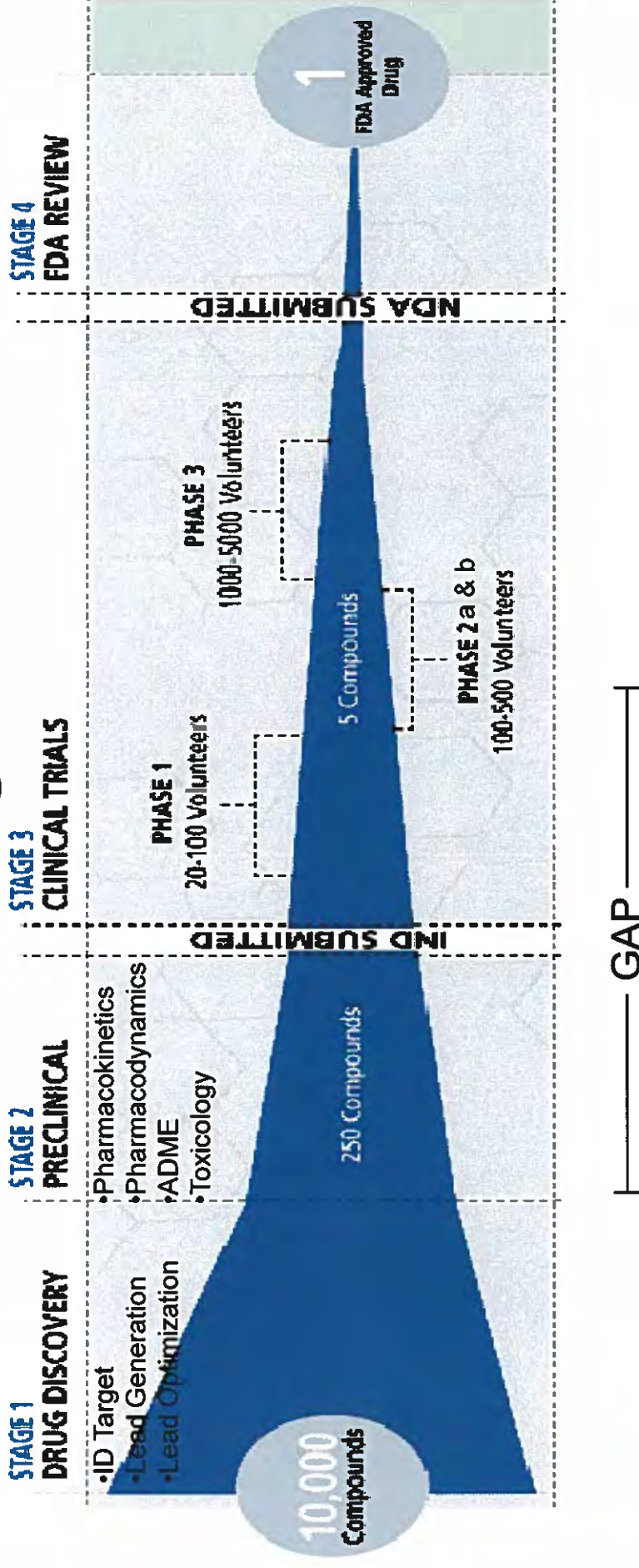
**EXCLUDED**

**-BASIC RESEARCH GRANTS**

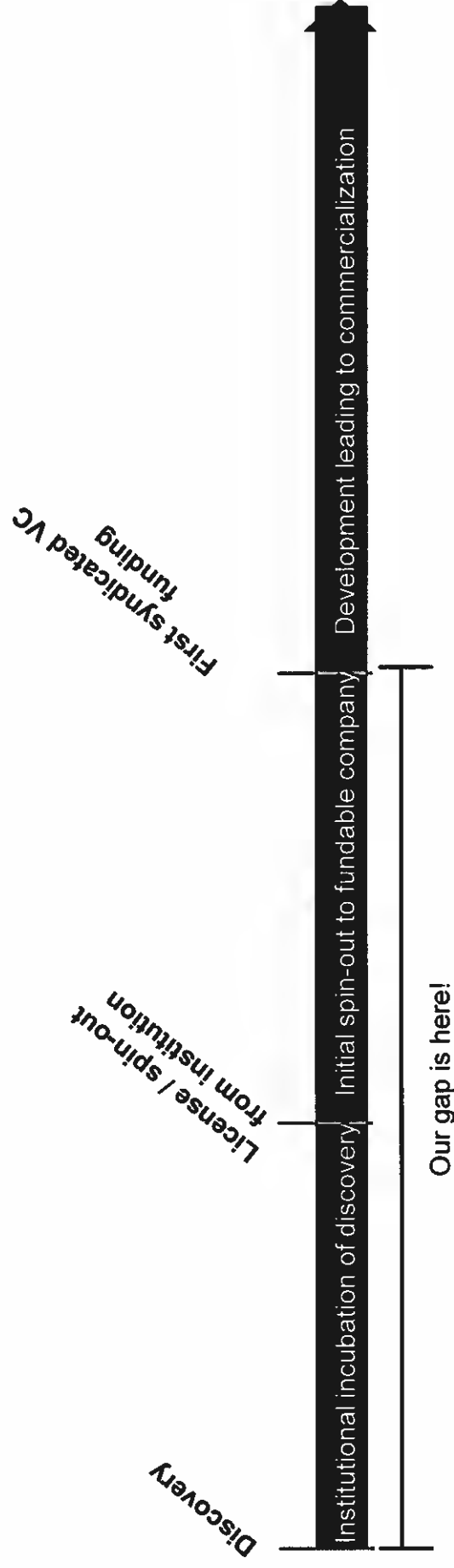
**-PREVENTION PROGRAMS**

**-RESEARCH SUPERIORITY GRANTS**

# Drug Development Product Diagram

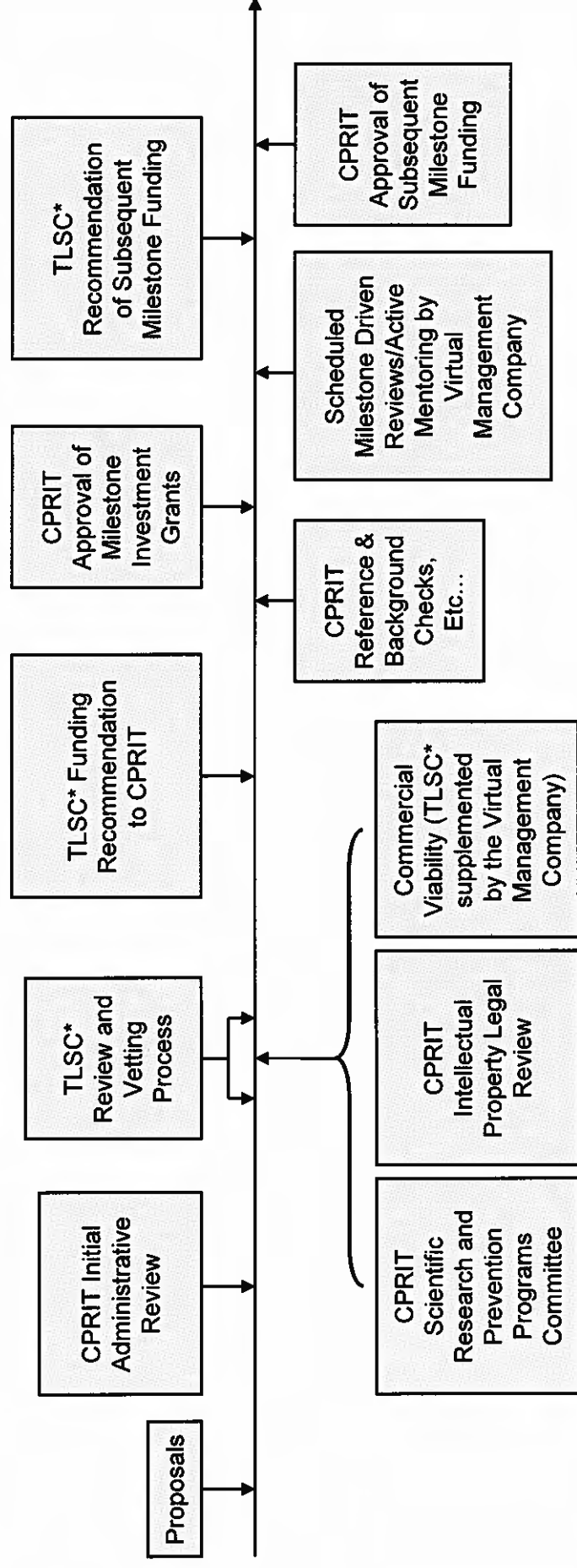


# Early Lifecycle of Typical Life Science Company



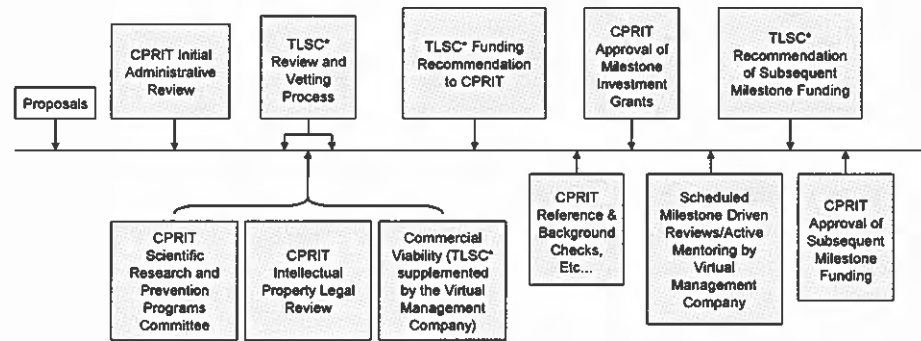
# CPRIT Commercial Review Process

## Due Diligence Steps



# CPRIT Commercial Review Process

## Due Diligence Steps



\*Texas Life Science Center (TLSC)

- Proposals for commercial funding will be submitted quarterly to CPRIT. An administrative review will be performed by CPRIT to ensure all applications are complete.
- Completed proposals will be submitted to the TLSC. There are multiple components to the vetting process including:
  1. Scientific due diligence by the Scientific Research and Prevention Programs Committee (SRPPC),
  2. Intellectual property legal review by an outside law firm, and
  3. Commercial viability by the members of the TLSC supplemented by the Virtual Management Company as needed.

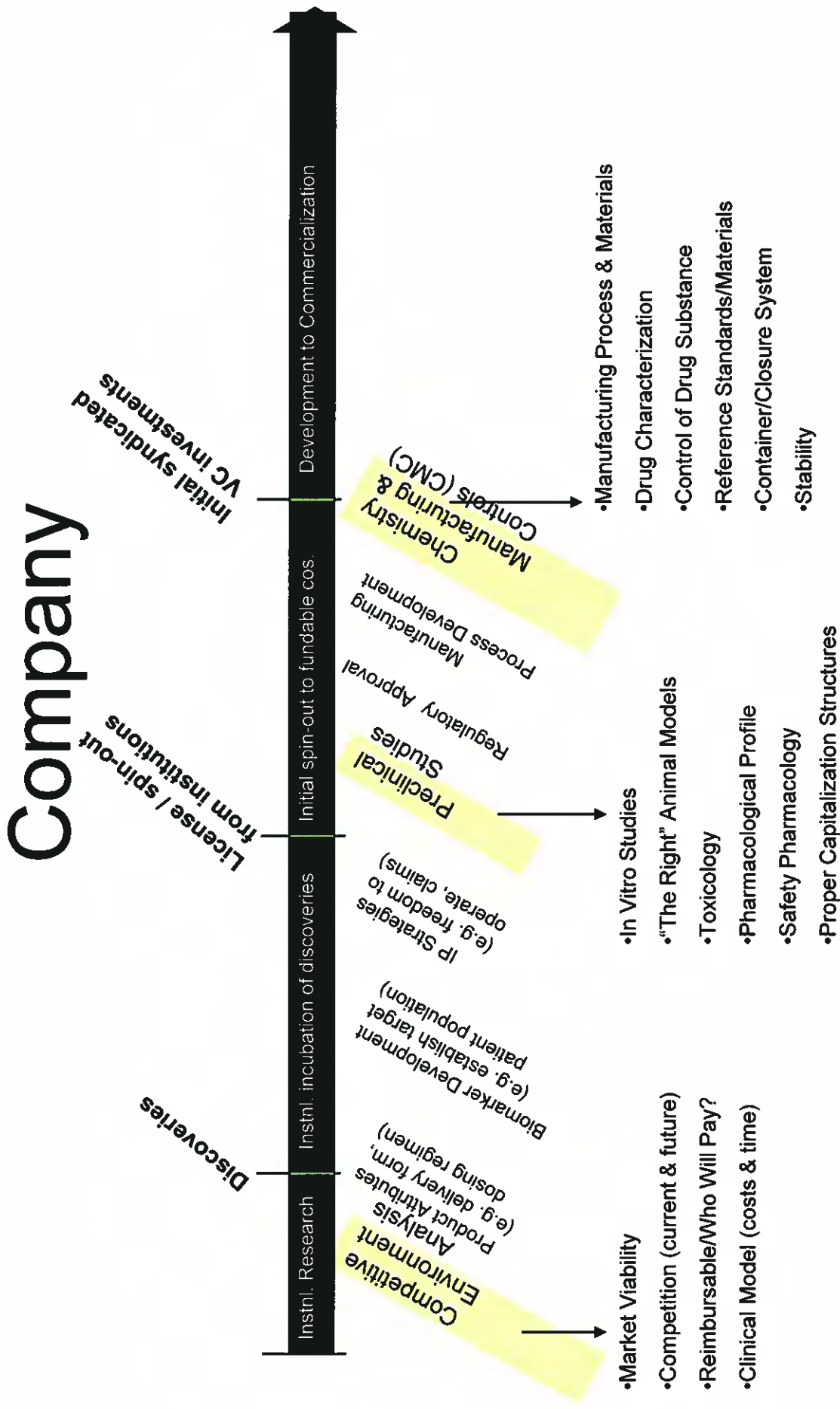
Additional components of the due diligence process include: site visits and formal company presentations to the TLSC.

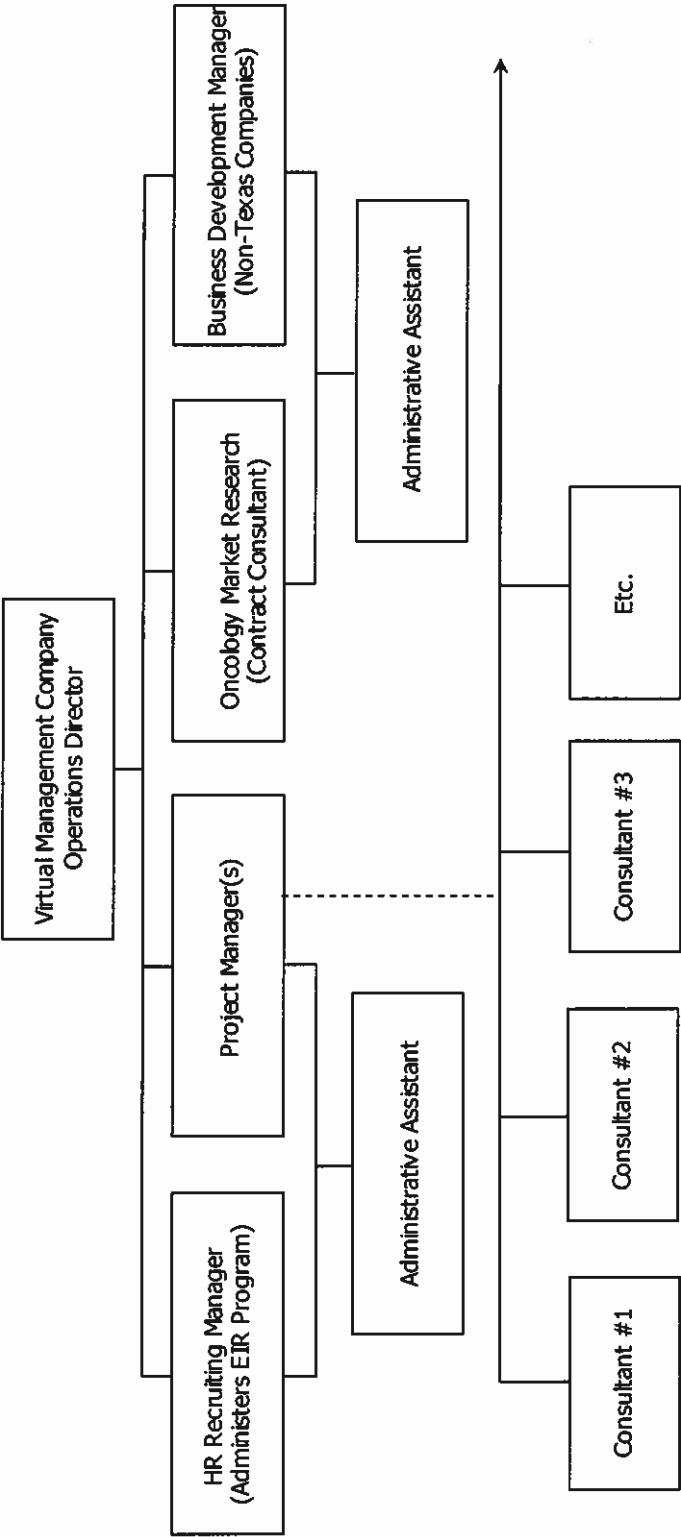
- After due diligence steps are completed the TLSC will make funding recommendations to the CPRIT Economic Development and Commercialization Subcommittee. The recommendations will include specific funding milestones.
- Company management references, customer references, and background checks will then be performed by CPRIT.
- Applicants will be submitted to the CPRIT Oversight Committee for ultimate funding decisions.

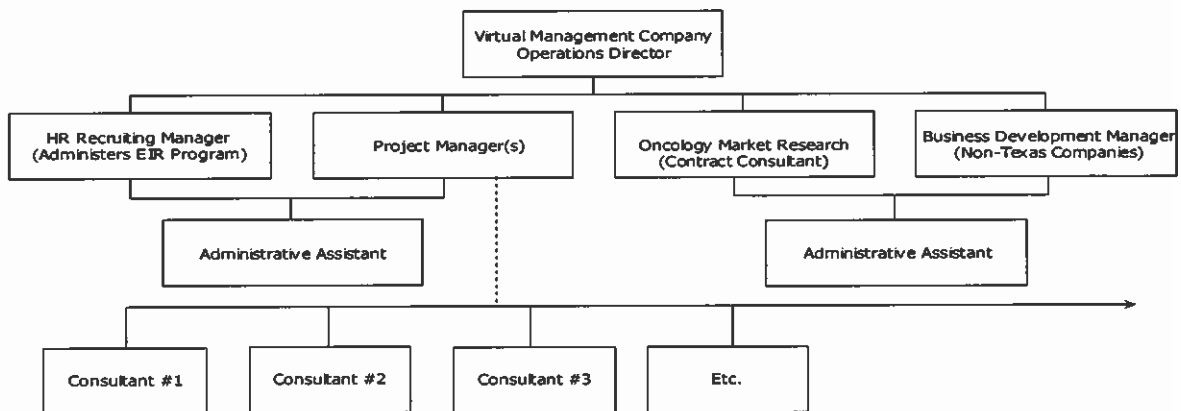


- The Virtual Management Company will actively mentor companies and researchers to reach commercial and funding milestones.
- The Virtual Management Company will report milestone progress to the TLSC.
- The TLSC will make recommendations for subsequent milestone driven funding to CPRIT.
- Milestone funding decisions will be made by CPRIT.
- Companies that are not recommended for funding will be given feedback from the TLSC on action items required to be reconsidered.
- Note: The type and pricing of the security issued will be determined based on matching funds. Anti-dilution, liquidation preferences, and other rights should be based on current market conditions.

# Examples of Critical Decision Points in a Typical Therapeutics Company







#### VIRTUAL MANAGEMENT COMPANY ORGANIZATION CHART DETAIL:

##### **Operations Director:**

##### **Description of Responsibilities:**

Direct the operations of the Virtual Management Company on a daily basis. Receive direction from CPRIT and the TLSC on Texas oncology companies to support and create a virtual management program for all companies supported. Build infrastructure to retain knowledge, assess the results of consulting engagements and report to CPRIT and/or the TLSC on a scheduled basis.

##### **Target Profile:**

Experience as an executive in a broad range of life science companies, with a focus on Oncology preferred. Demonstrated ability to work in complex matrix environments.

##### **Project Manager(s):**

##### **Description of Responsibilities:**

Project managers serve as a prime point of integration for all virtual management engagements, coordinate development of consulting projects, monitor the implementation of the projects, report on status of projects and ensure that knowledge

gained from the engagement is input into organizational knowledge retention systems. Each project manager will support approximately 5-7 companies, depending on the level of support required. The first project manager will have responsibility for building the support systems and process to conduct the engagements.

#### Target Profile

Initially, an MBA with life science company experience. Subsequently, BS level with right experience would be acceptable.

#### **HR Recruiting Manager:**

##### Description of Responsibilities:

Work with director and project manager to build EIR and virtual management company resource data bank. Later, maintain a data base that will link Texas life science companies and individuals interested in employment. Maintain databases to provide compensation advice to early stage companies.

##### Target Profile:

BS education level manager with experience recruiting in executive recruiting firms or large life science companies.

#### **Business Development Manager:**

##### Description of Responsibilities:

Market statewide oncology capabilities developed in all programs (CPRIT, EIR, VMC, research institutions, etc.) to promising non-Texas companies or researchers willing to develop or relocate oncology companies in or to Texas. Work closely with statewide economic development organizations to maximize marketing efforts and avoid duplication of spending.

##### Target profile:

BS and MBA education level with previous business development experience in a large life science company

#### **Program Coordinators:**

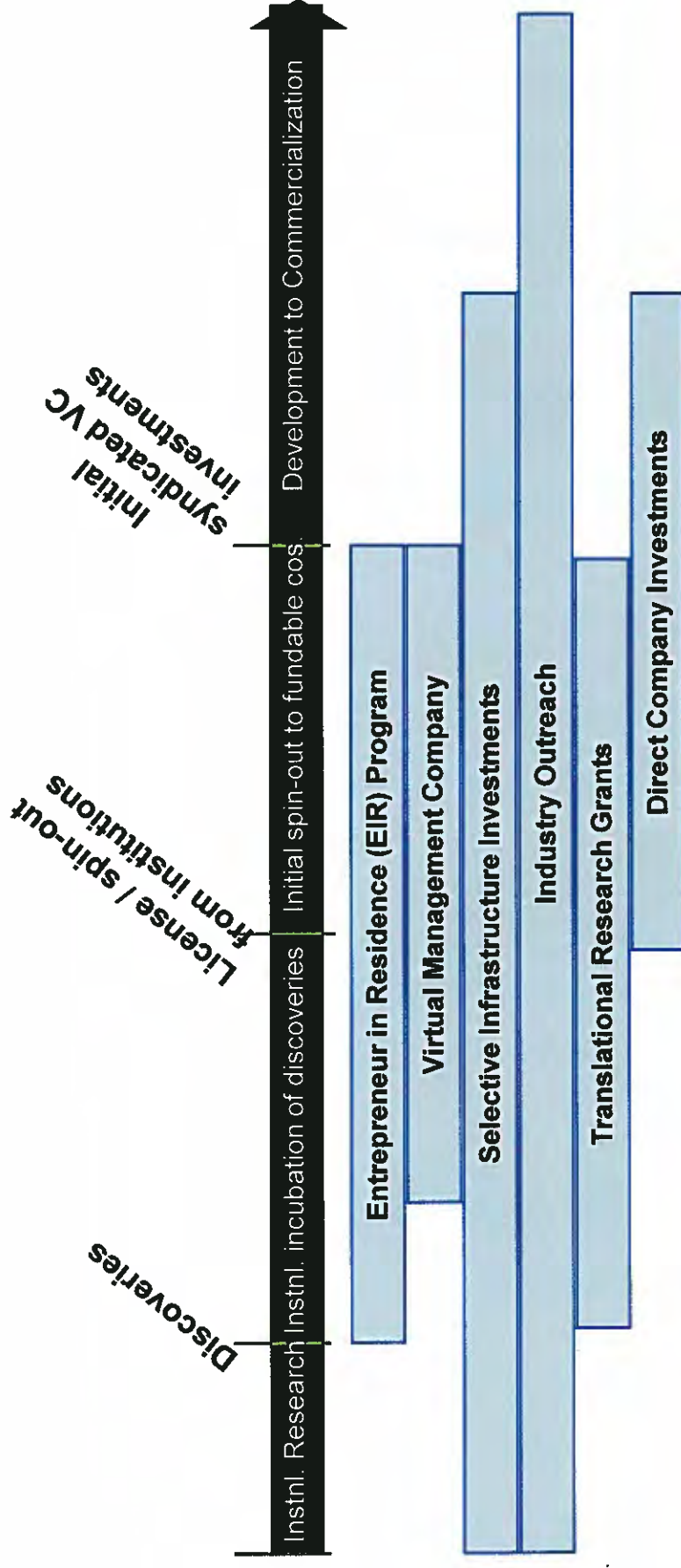
##### Description of Responsibilities:

Primarily support the VMC managerial staff administratively, but be capable of independent operation to assist in the development of portions of specific project engagements. Primary points of contact for Market Research support to companies. Cross train to provide backup to other coordinator.

Target profile:

BA or BS education level with previous life science company experience, in a market research capacity preferred for one of these positions. Demonstrated ability and willingness to continually assume new responsibilities and

# Building “Venture Capital Ready” Companies



*By taking a broader commercial approach to moving oncology research forward in Texas, CPRIT addresses both funding and capability gaps inherent in the sector today.*

## **APPENDIX**

### **Cancer Prevention and Research Institute of Texas**

#### **DEFINITIONS**

##### **Translational Research**

The concept of translational research has received very strong focus in the biomedical community over the last few years, as a new way of thinking about and conducting life sciences research to accelerate healthcare outcomes. Global pharmaceutical companies and the National Institutes of Health (NIH) have been investing billions of dollars into life sciences basic research without realizing a return on investment. With its focus on removing barriers to multi-disciplinary collaboration, translational research has the potential to drive the advancement of molecular-based medicine. By enabling physicians and pharmacologists to leverage systems biology technologies, translational research can enable early detection of cancer and other diseases, increase efficiency in drug development, improve drug efficacy and enable personalized medicine.

To qualify as translational research from a grant perspective, all five of the following criteria must be satisfied:

- The application is laboratory based, either a dry or wet (laboratory) or community based.
- The application is a clinical trial or an intervention or is observational research aimed at informing policy.
- The application demonstrates sustained engagement of stakeholders/end-users from the outset, e.g. patient or community.
- The proposal has the intent of application or uptake, i.e. demonstrated translatability. This needs to be clearly stated and identified within the research proposal.
- Timeliness - the research is likely to be translated into a commercial product in a short to medium time frame.

##### **Proof of concept**

Proof of concept is the demonstration of the feasibility of a therapeutic drug, device or diagnostic. Proof of concept is usually considered a milestone on the way to a fully



approved life science product. Proof of concept has a broad range of definitions, in many cases based upon the perspective of the end user. For a therapeutic, proof of concept definitions can range from positive animal data to conclusion of Phase II trials. For a device, definitions can range from creation of a working prototype to successful use in multiple clinical subjects.

### **Pre-clinical development**

Pre-clinical development is a stage of research that begins before clinical trials (testing in humans) can begin, and during which important feasibility, iterative testing and safety (also known as Good Laboratory Practice or "GLP") data is collected.

The main goals of pre-clinical studies (also named preclinical studies and nonclinical studies) are to determine a product's ultimate safety profile. Products may include new or iterated or like-kind medical devices, drugs, gene therapy solutions, etc. Each class of product may undergo different types of preclinical research. For instance, drugs may undergo pharmacodynamics (PD), pharmacokinetics (PK), absorption, distribution, metabolism and excretion (ADME), and toxicity testing through animal testing. Typically, both *in vitro* and *in vivo* tests will be performed. Studies of a drug's toxicity include which organs are targeted by that drug, as well as if there are any long-term carcinogenic effects or toxic effects on mammalian reproduction. This data allows researchers to allometrically estimate a safe starting dose of the drug for clinical trials in humans. Medical devices that do not have a drug attached will not undergo these additional tests and may go directly to GLP testing for safety of the device and its components. Some medical devices will also undergo biocompatibility testing which helps to show whether a component of the device or all components are sustainable in a living model. Most pre-clinical studies must adhere to Good Laboratory Practices (GLP) in International Conference on Harmonization (ICH) Guidelines to be acceptable for submission to regulatory agencies such as the Food & Drug Administration (FDA) in the United States.

### **Clinical**

In drug development, a covered clinical study refers to a clinical study, submitted to the Food and Drug Administration (FDA), as part of a marketing application (for example, as part of a New Drug Approval (NDA)). The FDA defines a covered study as "...any study of a drug, biological product or device in humans submitted in a marketing application or reclassification petition that the applicant or FDA relies on to establish that the product is effective (including studies that show equivalence to an effective product) or any study in which a single investigator makes a significant contribution to the demonstration of safety."

In health care, clinical trials are conducted to allow safety and efficacy data to be collected for new drugs or devices. These trials can only take place once satisfactory information has been gathered on the quality of the product and its non-clinical safety,

and Health Authority/Ethics Committee approval is granted in the country where the trial is taking place.

Depending on the type of product and the stage of its development, investigators enroll healthy volunteers and/or patients into small pilot studies initially, followed by larger scale studies in patients that often compare the new product with the currently prescribed treatment. As positive safety and efficacy data are gathered, the number of patients is typically increased. Clinical trials can vary in size from a single center in one country to multicenter trials in multiple countries.

Due to the sizable cost a full series of clinical trials may incur, the burden of paying for all the necessary people and services is usually borne by the sponsor who may be the pharmaceutical or biotechnology company that developed the agent under study. Since the diversity of roles may exceed resources of the sponsor, often a clinical trial is managed by an outsourced partner such as a contract research organization (CRO).

### **Infrastructure Investments**

Infrastructure investments will consists of life science product capabilities needed to build a successful oncology commercial industry in Texas. These proposals would require a rigorous business plan demonstrating demand and self sustainability. CPRIT should not duplicate complex, expensive and highly competitive capabilities already adequately provided by existing commercial markets inside and outside the state. CPRIT has to be extremely careful to invest only in high value capabilities not easily accessed by companies that would provide a unique differentiator for companies developing oncology products in Texas. Examples of these infrastructure programs could include a statewide commercially oriented tissue bank, a statewide clinical trials network, computational biology, and advanced imaging capabilities.

### **Development to commercial standards**

When CPRIT funds a company or project designed to ultimately provide a product for the marketplace, all work should be performed to "commercial standards" to eliminate the need to repeat work and to enhance its ability to attract future funding. Two examples of work that routinely has to be repeated are 1) investigator INDs that ultimately do not meet FDA standards required for an NDA filing and 2) preclinical or early clinical work designed insufficiently to satisfy investors due diligence.

In the great majority of cases, it is unlikely CPRIT funding will be sufficient to take a product all the way to market. Hence, there will need to be further investment by sophisticated investors such as VCs, pharmaceutical and/or large device companies. These sophisticated investors perform exacting diligence, including the following “commercial standards” considerations:

1. Funded work should conform with FDA approval standards. The FDA will often allow academics to conduct pilot therapeutic Phase I human studies or early stage device studies based on non-commercial standards. Depending upon the study, this could include less rigorous toxicology, manufacturing, validation and numerous other process areas. However, for work aimed at an eventual approval for a new drug or device, the FDA sets a higher standard. If the work done under the CPRIT grant is not up to this standard, the product has diminished value because some of the funded work may need to be repeated. Therefore, the value of investigator INDs is reduced and in a worst case scenario the project may come to a premature end as it fails diligence from future funding entities.
2. Venture capitalists and potential licensing companies want to see compelling indications of potential success in early stage development work before considering an investment. For example, they demand that the right animal models are chosen, the clinical study plans are designed robustly and that conditions of the testing are up-to-date, validated, compliant and include appropriate go/no go decision criteria. For instance, failing to construct and execute commercially driven pre-clinical models can make a great project “un-fundable”. These models can be extremely complex and unique due to the wide variety of molecules or diseases being studied. CPRIT companies that heed the advice of commercial experts will have a higher probability of future funding success and ultimately product approval.
3. Early experience from the Emerging Technology Fund indicates that young Texas companies often do not adequately consider critical end user considerations that drive commercial viability. This is also often the case with investigator sponsored INDs. Examples include delivery methods, dosing regimen, potency, ease of formulation and manufacturability. With devices, investigators do not usually focus on ease of use, degree that the device disrupts current clinical practice or other marketability criteria. However, these aspects and others, especially the competitive landscape, will have a great deal of influence on whether a product will be commercially viable and fundable, even if the scientific and/or clinical rationale is strong.



CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS

# **REQUEST FOR APPLICATIONS**

## **RFA C-14-ESTCO-1**

### **Established Company Product Development Awards**

**Please also refer to the Instructions for Applicants document,  
which will be posted December 23, 2013**

**Application Receipt Opening Date:** December 23, 2013

**Application Receipt Closing Date:** January 31, 2014

**FY 2014**

Fiscal Year Award Period

September 1, 2013–August 31, 2014

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## **RFA VERSION HISTORY**

Rev 12/9/13 RFA release

## 1. KEY POINTS

This Established Company Product Development Award mechanism is governed by the following restrictions:

- Company applicants must be Texas-based companies that have already received at least one round of professional institutional investment (i.e., Series A financing or a substantive equivalent). Applicants that have not yet received a round of professional institutional investment should apply under the New Company Product Development Awards mechanism.
- Recipient companies must currently have or must commit to the following: Headquarters in Texas, the majority of staff residing in or relocated to Texas, and use of Texas-based subcontractors and suppliers unless adequate justification is provided for the use of out-of-State entities.
- Of the total program budget, the Cancer Prevention and Research Institute of Texas (CPRIT) will contribute \$2.00 for every \$1.00 contributed in matching funds by the company. The demonstration of available matching funds must be made prior to the distribution of CPRIT grant funds, not at the time the application is submitted. CPRIT funds must, whenever possible, be spent in Texas. A company's matching funds must be designated for the CPRIT-funded project but may be spent outside of Texas.
- Funding may be tranching and will be tied to the achievement of contract-specified milestones.
- Funding award contracts will include a revenue-sharing agreement or equity to be negotiated at contract execution and will require CPRIT to have input on any future patents, agreements, or other financial arrangements related to the products, services, or infrastructure supported by the CPRIT investment. These contract provisions are specified in CPRIT's Administrative Rules, which are available at [www.cprit.state.tx.us](http://www.cprit.state.tx.us).
- Renewal applications will be accepted (see [Section 9.3](#) and [Section 11.4.5](#)).



## **2. ABOUT CPRIT**

The State of Texas established CPRIT, which may issue up to \$3 billion in general obligation bonds to fund grants for cancer research and prevention.

CPRIT is charged by the Texas Legislature to:

- Create and expedite innovation in the area of cancer research and product or service development, thereby enhancing the potential for a medical or scientific breakthrough in the prevention, treatment, and possible cures for cancer;
- Attract, create, or expand research capabilities of public or private institutions of higher education and other public or private entities that will promote a substantial increase in cancer research and in the creation of high-quality new jobs in the State of Texas; and
- Continue to develop and implement the Texas Cancer Plan by promoting the development and coordination of effective and efficient statewide public and private policies, programs, and services related to cancer and by encouraging cooperative, comprehensive, and complementary planning among the public, private, and volunteer sectors involved in cancer prevention, detection, treatment, and research.

CPRIT furthers cancer research in Texas by providing financial support for a wide variety of projects relevant to cancer research.

## **3. APPLICATION SURVEY**

CPRIT will be administering a survey to determine the operational aspects of peer review. Company representatives that anticipate submitting an application are requested to complete the survey as soon as possible, but no later than January 13, 2014. Company representatives should provide the following information: applicant name, name of company, telephone number, email address, estimated award amount, and award mechanism. Please select only one award mechanism as only one application can be submitted per funding cycle. This information will be used for planning purposes only, and will not be used for evaluation of the application. The survey is available [here](#).

#### **4. EXECUTIVE SUMMARY**

CPRIT will foster cancer research as well as product and service development in Texas by providing financial support for a wide variety of projects relevant to cancer. This Request for Applications (RFA) solicits applications for the research and development of innovative products addressing critically important needs related to diagnosis, prevention, and/or treatment of cancer and the product development infrastructure needed to support these efforts. CPRIT encourages applicants who seek to apply or develop state-of-the-art products, services (e.g., contract research organization services), technologies, tools, and/or resources for cancer research, prevention, or treatment. CPRIT expects outcomes of supported activities to directly and indirectly benefit subsequent cancer research efforts, cancer public health policy, or the continuum of cancer care—from prevention to treatment and cure. To fulfill this vision, applications may address any topic or issue related to cancer biology, causation, prevention, detection or screening, treatment, or cure.

#### **5. MECHANISM OF SUPPORT**

The goal of the Established Company Product Development Award is to finance the research and development of innovative products, services, and infrastructure with significant potential impact on patient care. These investments will provide companies or limited partnerships located and headquartered in Texas with the opportunity to further the research and development of new products for the diagnosis, treatment, supportive care, or prevention of cancer; to establish infrastructure that is critical to the development of a robust industry; or to fill a treatment, industry, or research gap. This award is intended to support companies that will be staffed with a majority of Texas-based employees, including C-level executives.

#### **6. OBJECTIVES**

The long-term objective of this award is to support commercially oriented therapeutic and medical technology products, diagnostic- or treatment-oriented information technology products, diagnostics, tools, services, and infrastructure projects. Common to all applications under this RFA (with the exception of infrastructure applications) should be

the intent to further the research and development of products that would eventually be approved for marketing for the diagnosis, prevention, and/or treatment of cancer. Eligible products or services include—but are not limited to—therapeutics (e.g., small molecules and biologics), diagnostics, devices, and potential breakthrough technologies, including software and research discovery techniques. Eligible stages of research and development include translational research, proof-of-concept studies, preclinical studies, and Phase I or Phase II clinical trials. By exception, Phase III clinical trials and later stage product development projects will be considered where circumstances warrant CPRIT investment.

## **7. FUNDING INFORMATION**

This is a 3-year funding program. Financial support will be awarded based upon the breadth and nature of the research and development program proposed. While requested funds must be well justified, there is no limit on the amount that may be requested. Funding will be milestone driven.

Funds may be used for salary and fringe benefits, research supplies, equipment, clinical trial expenses, intellectual property protection, external consultants and service providers, and other appropriate research and development costs, subject to certain limitations set forth by Texas State law. If a company is working on multiple projects, care should be taken to ensure that CPRIT funds are used to support activities directly related to the specific project being funded. Requests for funds to support construction and/or renovation may be considered under compelling circumstances for projects that require facilities that do not already exist in the State of Texas. Texas State law limits the amount of awarded funds that may be spent on indirect costs to no more than 5 percent of the total award amount (5.263 percent of the direct costs).

Consistent with statutory mandate, of the total program budget, CPRIT will contribute \$2.00 for every \$1.00 contributed in matching funds by the company. The demonstration of available matching funds must be made prior to the distribution of CPRIT funds, not at the time the application is submitted. The matching funds commitment may be made on a year-by-year basis.

## 8. KEY DATES

<b>RFA release</b>	December 9, 2013
<b>Online application opens</b>	December 23, 2013, 7 a.m. Central Time
<b>Applications due</b>	January 31, 2014, 3 p.m. Central Time
<b>Invitations to present sent</b>	March 2014
<b>Notifications sent if not invited</b>	March 2014
<b>Presentations to CPRIT*</b>	April 2014

\*All applicants who wish to be considered are requested to reserve these presentation dates until notified. Information on the timing of subsequent steps will be provided to applicants later in the process.

## 9. ELIGIBILITY

### 9.1. New Applications

- Company applicants must be Texas-based companies that have already received at least one round of professional institutional investment (i.e., Series A financing or a substantive equivalent). Applicants that have not yet received a round of professional institutional investment should apply under the New Company Product Development Award mechanism.
- Recipient companies must currently have or must commit to the following: Headquarters in Texas, the majority of staff residing in or relocated to Texas, and Texas-based subcontractors and suppliers unless adequate justification is provided for the use of out-of-State entities. To the extent that Texas-based subcontractors or collaborators are not available, non-Texas-based collaborators and subcontractors may be used. However, non-Texas-based collaborators and subcontractors are not eligible to receive funds from CPRIT unless exceptional circumstances are demonstrated and approved by CPRIT.

- An applicant may submit only one application under this RFA during this funding cycle.
- Only one co-applicant may be included on the application. Co-applicants should have specific and well-defined roles.
- A company applicant is eligible to receive a grant award only if the applicant certifies that the company, including the company representative, any senior member or key personnel listed on the application, any company officer or director (or any person related to one or more of these individual within the second degree of consanguinity or affinity) have not made and will not make a contribution to CPRIT or to any foundation specifically created to benefit CPRIT.
- A company applicant is not eligible to receive CPRIT funding if the company representative, any senior member or key personnel listed on the application, and any company officer or director is related to a CPRIT Oversight Committee member.
- The company applicant must report whether the company, company representative, or other individuals who contribute to the execution of the proposed project in a substantive, measurable way, whether or not those individuals are slated to receive salary or compensation under the grant award, are currently ineligible to receive Federal grant funds or have had a grant terminated for cause within 5 years prior to the submission date of the grant application.
- CPRIT grants will be awarded by contract to successful company applicants. Certain contractual requirements are mandated by Texas State law or by administrative rules. Although the company applicant need not demonstrate the ability to comply with these contractual requirements at the time the application is submitted, applicants should familiarize themselves with these standards before submitting a grant application. Significant issues addressed by the CPRIT contract are listed in [Section 12](#) and [Section 13](#). All statutory provisions and relevant administrative rules can be found at [www.cprit.state.tx.us](http://www.cprit.state.tx.us).

## 9.2. Resubmission Policy

An application previously submitted to CPRIT but not funded may be resubmitted once and must follow all resubmission guidelines (see [Section 11.4.4](#)). More than one resubmission is not permitted. Applicants who choose to resubmit should carefully consider the reasons for lack of prior success. Applications that received overall numerical scores of 5 or higher are likely to need considerable attention. All resubmitted applications should be carefully reconstructed; a simple revision of the prior application with editorial or technical changes is not sufficient, and applicants are advised not to direct reviewers to such modest changes. A one-page summary of the approach to the resubmission should be included. Resubmitted applications may be assigned to reviewers who did not review the original submission. Reviewers of resubmissions are asked to assess whether the resubmission adequately addresses critiques from the previous review. **Applicants should note that addressing previous critiques is advisable; however, it does not guarantee the success of the resubmission.** All resubmitted applications must conform to the structure and guidelines outlined in this RFA.

## 9.3. Renewal Policy

A grant recipient that has previously been awarded grant funding from CPRIT may submit an application under this mechanism to be considered for a competitive renewal. The eligibility criteria described in [Section 9.1](#) also apply to renewal applications. In addition:

- Applicants must have received a CPRIT award, either a Company Commercialization Award (this mechanism was called Company Investment in FY 2010), a Company Formation Award, a Company Relocation Award, an Individual Investigator Award with a commercialization component, or a High Impact/High Risk Award with a commercialization component.
- Before submitting a renewal application, applicants must consult with the Product Development Programmatic Office (see [Section 14.2](#)) to determine whether it is appropriate for their company to seek renewal funding at this time.

## 10. APPLICATION REVIEW

### 10.1. Overview

Applications will be assessed based on evaluation of the quality of the company and the potential for continued product development. CPRIT requires the submission of a comprehensive scientific plan (see [Section 11.4.8](#)) and a detailed business plan (see [Section 11.4.9](#)). The review will address the commercial viability, product feasibility, scientific merit, and therapeutic impact as detailed in the company's business and scientific plans. The plans will be reviewed by an integrated panel of individuals with biotechnology expertise and experience in translational and clinical research as well as in the business development/regulatory approval processes for therapeutics, devices, and diagnostics. In addition, advocate reviewers will participate in the review process.

Funding decisions are made by the review process described below.

### 10.2. Review Process

- 1. Product Development and Scientific Review:** Applications that pass initial administrative compliance review are assigned to independent CPRIT Product Development Peer Review Panel members for evaluation using the criteria listed below. Based on the initial evaluation and discussion by the Product Development Review Panel, a subset of company applicants may be invited to deliver in-person presentations to the review panel.
- 2. Due Diligence Review:** Following the in-person presentations, a subset of applications judged to be most meritorious by the Product Development Review Panels will be referred for additional in-depth due diligence, including—but not limited to—intellectual property, management, regulatory, manufacturing, and market assessments. Following the due diligence review, applications will be recommended for funding by the CPRIT Product Development Review Council based on the information set forth in the due diligence and intellectual property reviews, comparisons with applications from the Product Development Review Panels, and programmatic priorities.

3. **Program Integration Committee Review:** Applications recommended by the Product Development Review Council will be forwarded to the CPRIT Program Integration Committee (PIC) for review. The PIC will consider factors including program priorities set by the Oversight Committee, portfolio balance across programs, and available funding.
4. **Oversight Committee Approval:** The CPRIT Oversight Committee will vote to approve each grant award recommendation made by the PIC. The grant award recommendations will be presented at an open meeting of the Oversight Committee and must be approved by two-thirds of the Oversight Committee members present and eligible to vote.

The review process is described more fully in CPRIT's Administrative Rules, Chapter 703, Sections 703.6–703.8.

#### **10.2.1. Confidentiality of Review**

Each stage of application review is conducted confidentially, and all CPRIT Product Development Panel members, Product Development Review Council members, Program Integration Committee members, CPRIT employees, and Oversight Committee members with access to grant application information are required to sign nondisclosure statements regarding the contents of the applications. All technological and scientific information included in the application is protected from public disclosure pursuant to Health and Safety Code §102.262(b).

Individuals directly involved with the review process operate under strict conflict of interest prohibitions. All CPRIT Product Development Peer Review Panel members and Product Development Review Council members are non-Texas residents.

An applicant will be notified regarding the peer review panel assigned to review the grant application. Peer review panel members are listed by panel on CPRIT's Web site. **By submitting a grant application, the applicant agrees and understands that the only basis for reconsideration of a grant application is limited to an undisclosed Conflict**



**of Interest as set forth in CPRIT's Administrative Rules, Chapter 703,  
Section 703.9.**

Communication regarding the substance of a pending application is prohibited between the company applicant (or someone on the grant applicant's behalf) and the following individuals: an Oversight Committee member, a Program Integration Committee (PIC) member, a Product Development Review Panel member, or a Product Development Review Council member. Applicants should note that the CPRIT (PIC) is comprised of the CPRIT Chief Executive Officer, the Chief Scientific Officer, the Chief Prevention Officer, the Chief Product Development Officer, and the Commissioner of State Health Services. The prohibition on communication begins on the first day that grant applications for the particular grant mechanism are accepted by CPRIT and extends until the grant applicant receives notice regarding a final decision on the grant application. Intentional, serious, or frequent violations of this rule may result in the disqualification of the grant applicant from further consideration for a grant award.

### **10.3. Review Criteria**

Full peer review of applications will be based on primary scored criteria and secondary unscored criteria, listed below. Review committees will evaluate and score each primary criterion and subsequently assign a global score that reflects an overall assessment of the application. **The overall assessment will not be an average of the scores of the individual criteria; rather, it will reflect the reviewers' overall impression of the application. Evaluation of the scientific merit of each application is within the sole discretion of the peer reviewers.**

#### **10.3.1. Primary Criteria**

Primary criteria will evaluate the scientific merit and potential impact of the proposed work contained in the application. Concerns with any of these criteria potentially indicate a major flaw in the significance and/or design of the proposed study.

Primary criteria include:

**Significance and Impact:** Will the outcomes of this CPRIT-funded work result in the development of innovative products with significant product development potential? Will

the outcome substantially impact the diagnosis, treatment, prevention of cancer, or supportive care for patients with cancer? How would competing products or services affect the value of the proposed offering?

**Product:** Is there demonstrated proof of relevance, and does the product fulfill a clear, unmet medical or infrastructure need? Has work been conducted that supports the advancement of the proposed product, service, or technology? Can the product be produced or manufactured in a commercially viable fashion? Is there an appropriate basis for a reimbursement strategy?

**Market Plan:** Is there a realistic assessment of the market size and expected penetration? Has management adequately assessed potential competitors and described how the company's offering will successfully compete with them?

**Development Plan and/or Regulatory Path:** Is the development plan and/or regulatory path well characterized and appropriate? Is the plan milestone driven, and does it address both a positive and a negative outcome? Does the budget appropriately support the plan?

**Scientific Plan:** Is the proposed product, service, and/or infrastructure based on a feasible research framework, hypothesis, and/or goal? Are the methods appropriate, and are potential research and developmental obstacles and unexpected outcomes discussed?

**Management and Staffing:** Does the applicant have the appropriate level of management experience to execute the stated strategy? Does the team have the needed experience or access to experienced external assistance, facilities, and resources to accomplish all aspects of the proposed plan?

### **10.3.2. Secondary Criteria**

Secondary criteria contribute to the global score assigned to the application. Concerns with these criteria potentially question the feasibility of the proposed research and development activities.

Secondary criteria include:

**Budget and Duration of Support:** Are the budget and duration appropriate for the proposed work? Will the amount requested enable the applicant to reach appropriate milestones? Is the use of the funds requested in line with the stated objectives of the applicant and CPRIT? Is it clear how funds will be used? Does the proposed investment fund the research and development of the proposed product, service, or technology to a point where, if the results are positive, it is likely that the project will be able to attract further financial support outside of CPRIT?

## **11. SUBMISSION GUIDELINES**

Applicants are advised to carefully review all instructions in this section to ensure the accurate and complete submission of all components of the application. Please refer to the *Instructions for Applicants* document for details that will be available when the application receipt system opens. Applications that are missing one or more components, exceed the specified page or word limits, or that do not meet the eligibility requirements listed above will be administratively withdrawn without review.

### **11.1. Online Application Receipt System and Application Submission Deadline**

Applications must be submitted via the CPRIT Application Receipt System (CARS) (<https://CPRITGrants.org>). **Only applications submitted through this portal will be considered eligible for evaluation.** The applicant is eligible solely for the grant mechanism specified by the RFA under which the grant application was submitted. The company applicant must create a user account in the system to start and submit an application. The co-applicant, if applicable, must also create a user account to participate in the application. Furthermore, the Authorized Signing Official (ASO) (an individual authorized to sign and submit an application on behalf of the company applicant) must also create a user account in CARS. An application may not be submitted without ASO approval. Only the ASO is authorized to officially submit the application to CPRIT. Applications will be accepted beginning at 7 a.m. Central Time on December 23, 2013 and must be submitted by 3 p.m. Central Time on January 31, 2014. **Submission of an application is considered an acceptance of the terms and conditions of the RFA.**

## **11.2. Submission Deadline Extension**

The submission deadline may be extended for one or more grant applications upon a showing of good cause. All requests for extension of the submission deadline must be submitted via e-mail to the CPRIT HelpDesk. Submission deadline extensions, including the reason for the extension, will be documented as part of the grant review process records.

## **11.3. Product Development Review Fee**

All applicants must submit a fee of \$1,000 for product development review. Payment should be made by check or money order payable to CPRIT; electronic and credit card payments are not acceptable. The application ID and the name of the submitter must be indicated on the payment. All payments must be postmarked by the application submission deadline and mailed to:

Cancer Prevention and Research Institute of Texas  
P.O. Box 12097  
Austin, TX 78711

## **11.4. Application Components**

### **11.4.1. Layperson's Summary (1,500 characters)**

Provide an abbreviated summary for a lay audience using clear, nontechnical terms. Describe specifically how the proposed project would support CPRIT's mission (see [Section 2](#)). Would it fill a needed gap in patient care or in the development of a sustainable oncology industry in Texas? Would it synergize with Texas-based resources? Describe the overall goals of the work, the type(s) of cancer addressed, the potential significance of the results, and the impact of the work on advancing the fields of diagnosis, treatment, or prevention of cancer. Clearly address how the company's work, if successful, will have a major impact on the care of patients with cancer. The information provided in this summary will be made publicly available by CPRIT, particularly if the application is recommended for funding. The Layperson's Summary will be also used by advocate reviewers in evaluating the significance and impact of the proposed work. Do not include any proprietary information in this section.

#### **11.4.2. Goals and Objectives**

List specific goals and objectives for each year of the project. These goals and objectives will also be used during the submission and evaluation of progress reports and assessment of project success, if the award is made.

#### **11.4.3. Timeline (One page)**

Provide an outline of anticipated major milestones to be tracked. Timelines will be reviewed for reasonableness, and adherence to timelines will be a criterion for continued support of successful applications. If the application is approved for funding, this section will be included in the award contract. Applicants are advised not to include information that they consider confidential or proprietary when preparing this section.

#### **11.4.4. Resubmission Summary (One page)**

If this is a resubmission, upload a summary of the approach, including a summary of the applicant's response to previous feedback. Clearly indicate to reviewers how the application has been improved in response to the critiques. Refer the reviewers to specific sections of other documents in the application where further detail on the points in question may be found. When a resubmission is evaluated, responsiveness to previous critiques is assessed. If this is not a resubmission, then no summary is required.

**Note:** An application is a resubmission only if the previous application was finalized and submitted to CPRIT. However, an application that was submitted to CPRIT to be considered for FY2013 Cycle 3 awards and was returned by CPRIT due to the moratorium is not considered to be a resubmission.

#### **11.4.5. Renewal Justification Summary (One page)**

If this is a renewal, upload a summary that briefly outlines the progress made with the initial CPRIT award and outlines the proposed use of renewal funding and the resulting value for Texas. Clearly indicate whether (1) the technological/scientific underpinning is the same as that evaluated during review of the company's originally funded CPRIT application, or (2) whether funding is sought for the research and development of a new product or service not previously reviewed by CPRIT, or represents a significant

modification of the original product or service reviewed by CPRIT. (Either option is acceptable.) If this is not a renewal, no summary is required.

#### **11.4.6. Executive Summary (One page)**

Provide an executive summary that clearly explains the product, service, technology, or infrastructure proposed; competition; market need and size; development or implementation plans; regulatory path; reimbursement strategy; and funding needs. Applicants must clearly describe the existing or proposed company infrastructure and personnel located in Texas for this endeavor.

#### **11.4.7. Slide Presentation (Ten pages)**

Provide a slide presentation summarizing the application. The presentation should be submitted in PDF format, with one slide filling each landscape-orientation page. The slides should succinctly capture all essential elements of the application and should stand alone.

#### **11.4.8. Scientific Plan (Ten pages)**

Present the rationale behind the proposed product or service, emphasizing the pressing problem in cancer care that will be addressed. Summarize the evidence gathered to date in support of the company's ideas. Describe the label claims that the company ultimately hopes to make, and describe the plan to gather evidence to support these claims. Outline the steps to be taken during the proposed period of the award, including the design of the translational or clinical research, methods, and anticipated results. Describe potential problems or pitfalls and alternative approaches. If clinical research is proposed, present a realistic plan to accrue a sufficient number of human subjects meeting the inclusion criteria within the proposed time period.

**The scientific plan submitted must be of sufficient depth and quality to pass rigorous scrutiny by the highly qualified group of reviewers. To the extent possible, the scientific plan should be driven by data. In the past, applications that have been scored poorly have been criticized for assuming that assertions could be taken on faith. Convincing data are much preferred.**

#### **11.4.9. Business Plan (Fifteen pages)**

Provide a business plan covering all of the topics below in the order shown. Successful applicants will make thoughtful, careful, and economical use of the limited space. Note that if the company is selected to undergo due diligence, information to support a full intellectual property review will be requested at that time. Established Company Product Development Award applicants will be evaluated based not only on the current status of the components of the business plan, but also on whether current weaknesses and gaps are acknowledged and whether plans to address them are outlined.

- A. Introduction:** Describe the label claims that the company ultimately hopes to make, and briefly describe the plan to gather evidence to support these claims. Include the minimum level of detail required to provide a context for the rest of the business plan. Cross-reference sections in the scientific plan where further details may be found.
- B. Products and Markets:** Provide a brief description of the envisioned product and how the product will be administered to patients. Describe the initial market that will be targeted and how the envisioned product will fit within the standard of care.
- C. Regulatory Plans:** Provide a detailed regulatory plan, including preclinical and clinical activities, driven by interactions with the FDA, if possible. Summarize all interactions to date with the FDA.
- D. Risk Analysis:** Describe the specific risks inherent to the product plan and how they would be mitigated.
- E. Current and Pending Support:** Describe all funding sources. Provide a complete and detailed capitalization table, which should include all parties who have investments, stock, or rights in the company. The identities of all parties must be listed. It is not appropriate to list any funding source as anonymous.
- F. Financial Projections:** Provide a detailed source and use analysis of the development plan, focusing on the achievement of specific milestones.

- G. Resources Requested:** Include resources needed for research and product development and for any relocation expenses. The matching funds amount should be included in this section; however, this is the only section of the business plan that does not deal exclusively with CPRIT-requested funds.
- H. Scope of Work and Milestones:** Outline the specific goals of the project. Provide an outline of anticipated major milestones to be tracked. Timelines will be reviewed for reasonableness, and adherence to timelines will be a criterion for continued support of successful applications. If the application is approved for funding, this section will be included in the award contract.
- I. Key Personnel:** For each member of the senior management and scientific team, provide a paragraph briefly summarizing his or her present title and position, prior industry experience, education, and any other information considered essential for evaluation of qualifications.
- J. Organizational Commitment to Texas:** Describe how CPRIT funding of the applicant's company would benefit the State of Texas. For example, describe how the company would create high-quality new jobs in the State and/or recruit out-of-State talent, and mention any Texas-based subcontractors and suppliers that would be used and any other unique, Texas-based resources that would be leveraged.

#### **11.4.10. Biographical Sketches of Key Scientific Personnel (Eight pages)**

Provide a biographical sketch for up to four key scientific personnel that describes their education and training, professional experience, awards and honors, and publications relevant to cancer research. Each biographical sketch must not exceed two pages and must use the "Product Development Programs: Biographical Sketch" template.

(In addition, information on the members of the senior management and scientific team should be included in the "Key Personnel" section of the Business Plan

[see [Section 11.4.9](#)]).



#### **11.4.11. Budget and Justification**

Provide a compelling justification of the budget for the entire proposed period of support, including salaries and benefits, supplies, equipment, patient care costs, animal care costs, and other expenses. The budget must be aligned with the proposed milestones.

In preparing the requested budget, applicants should be aware of the following:

- Equipment having a useful life of more than 1 year and an acquisition cost of \$5,000 or more per unit must be specifically approved by CPRIT. An applicant does not need to seek this approval prior to submitting the application.
- Texas State law limits the amount of grant funds that may be spent on indirect costs to no more than 5 percent of the total award amount (5.263 percent of the direct costs). Guidance regarding indirect cost recovery can be found in CPRIT's Administrative Rules, which are available at [www.cprit.state.tx.us](http://www.cprit.state.tx.us).
- The annual salary that an individual may receive under a CPRIT award for FY 2014 is \$200,000. In other words, an individual may request salary proportional to the percentage effort up to a maximum of \$200,000. Salary does not include fringe benefits. CPRIT FY 2014 is from September 1, 2013, through August 31, 2014.

## **12. AWARD ADMINISTRATION**

Texas law requires that CPRIT awards be made by contract between the applicant and CPRIT. CPRIT grant awards are made to entities, not to individuals. Award contract negotiation and execution will commence once the CPRIT Oversight Committee has approved an application for a grant award. CPRIT may require, as a condition of receiving a grant award, that the grant recipient use CPRIT's electronic Grant Management System to exchange, execute, and verify legally binding grant contract documents and grant award reports. Such use shall be in accordance with CPRIT's electronic signature policy as set forth in Chapter 701, Section 701.25.

Texas law specifies several components that must be addressed by the award contract, including needed compliance and assurance documentation, budgetary review, progress and fiscal monitoring, and terms relating to revenue sharing and intellectual property

rights. These contract provisions are specified in CPRIT's Administrative Rules, which are available at [www.cprit.state.tx.us](http://www.cprit.state.tx.us). Applicants are advised to review CPRIT's Administrative Rules related to contractual requirements associated with CPRIT grant awards and limitations related to the use of CPRIT grant awards as set forth in Chapter 703, Sections 703.10 - 703.12.

Prior to disbursement of grant award funds, the grant recipient organization must demonstrate that it has adopted and enforces a tobacco-free workplace policy consistent with the requirements set forth in CPRIT's Administrative Rules, Chapter 703, Section 703.20.

CPRIT requires award recipients to submit an annual progress report. These reports summarize the progress made toward the research goals and address plans for the upcoming year. In addition, fiscal reporting, human studies reporting, and vertebrate animal use reporting will be required as appropriate. Continuation of funding is contingent upon the timely receipt of these reports. Failure to provide timely and complete reports may waive reimbursement of grant award costs, and may result in the termination of award contract. Forms and instructions will be made available at [www.cprit.state.tx.us](http://www.cprit.state.tx.us).

**Project Economics Sharing:** Recipients should also be aware that the funding award contract will include a revenue-sharing agreement and will require CPRIT to have input on any future patents, agreements, or other financial arrangements related to the products, services, or infrastructure supported by the CPRIT investment. These contract provisions are specified in CPRIT's Administrative Rules, which are available at [www.cprit.state.tx.us](http://www.cprit.state.tx.us).

### **13. REQUIREMENT TO DEMONSTRATE AVAILABLE FUNDS**

Texas State law requires that prior to disbursement of CPRIT grant funds, the award recipient demonstrate that it has \$1.00 in matching funds for every \$2.00 from CPRIT. Matching funds need not be in hand when the application is submitted. However, matching funds must be obtained before CPRIT funds will be released for use. CPRIT

funds must, whenever possible, be spent in Texas. A company's matching funds must be targeted for the CPRIT-funded project but may be spent outside of Texas. Grant applicants are advised to consult CPRIT's Administrative Rules, Chapter 703, Section 703.11 for specific requirements associated with the requirement to demonstrate available funds.

## **14. CONTACT INFORMATION**

### **14.1. HelpDesk**

HelpDesk support is available for questions regarding user registration and online submission of applications. Queries submitted via e-mail will be answered within 1 business day. HelpDesk staff are not in a position to answer questions regarding scientific and product development aspects of applications. **Before contacting the HelpDesk, please refer to the "Instructions for Applicants" document, which provides a step-by-step guide on using the Application Receipt System.**

**Dates of operation:** December 23, 2013 to January 31, 2014 (excluding public holidays)

**Hours of operation:** Monday, Tuesday, Thursday, Friday, 7 a.m. to 4 p.m. Central Time  
Wednesday, 8 a.m. to 4 p.m. Central Time

**Tel:** 866-941-7146

**E-mail:** [Help@CPRITGrants.org](mailto:Help@CPRITGrants.org)

### **14.2. Programmatic Questions**

Questions regarding the CPRIT Program, including questions regarding this or any other funding opportunity, should be directed to the CPRIT Product Development Program Director.

**Tel:** 512-305-8486

**E-mail:** [Help@CPRITGrants.org](mailto:Help@CPRITGrants.org)

**Web site:** [www.cprit.state.tx.us](http://www.cprit.state.tx.us)



**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**

# **REQUEST FOR APPLICATIONS**

## **RFA C-14-NEWCO-1**

### **New Company Product Development Awards**

**Please also refer to the Instructions for Applicants document,  
which will be posted December 23, 2013**

**Application Receipt Opening Date:** December 23, 2013

**Application Receipt Closing Date:** January 31, 2014

**FY 2014**

Fiscal Year Award Period

September 1, 2013–August 31, 2014

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## **RFA VERSION HISTORY**

Rev 12/9/13     RFA release

## 1. KEY POINTS

This New Company Product Development Award mechanism is governed by the following restrictions:

- Company applicants must be early-stage startup companies with no previous round of professional institutional investment (i.e., those that have not yet received Series A financing or a substantive equivalent). Companies at this early stage that are not currently located in Texas but intend to relocate to Texas should apply under this mechanism rather than the Company Relocation Awards mechanism.
- Recipient companies must currently have or must commit to the following: Headquarters or substantial business functions of the company in Texas; personnel sufficient to operate the Texas-based research and/or development activities of the company, along with appropriate management, relocated to or hired from within Texas.
- Of the total program budget, the Cancer Prevention and Research Institute of Texas (CPRIT) will contribute \$2.00 for every \$1.00 contributed, in matching funds, by the company. The demonstration of available matching funds must be made prior to the distribution of CPRIT grant funds, not at the time the application is submitted. CPRIT funds must, whenever possible, be spent in Texas. A company's matching funds must be targeted for the CPRIT-funded project but may be spent outside of Texas.
- Funding may be tranching and will be tied to the achievement of contract-specified milestones.
- Funding award contracts will include a revenue-sharing agreement or equity to be negotiated at contract execution and will require CPRIT to have input on any future patents, agreements, or other financial arrangements related to the products, services, services, or infrastructure supported by the CPRIT investment. These contract provisions are specified in CPRIT's Administrative Rules, which are available at [www.cpritch.state.tx.us](http://www.cpritch.state.tx.us).



## **2. ABOUT CPRIT**

The State of Texas has established CPRIT, which may issue up to \$3 billion in general obligation bonds to fund grants for cancer research and prevention.

CPRIT is charged by the Texas Legislature to:

- Create and expedite innovation in the area of cancer research and product or service development, thereby enhancing the potential for a medical or scientific breakthrough in the prevention, treatment, and possible cures for cancer;
- Attract, create, or expand research capabilities of public or private institutions of higher education and other public or private entities that will promote a substantial increase in cancer research and in the creation of high-quality new jobs in the State of Texas; and
- Continue to develop and implement the Texas Cancer Plan by promoting the development and coordination of effective and efficient statewide public and private policies, programs, and services related to cancer and by encouraging cooperative, comprehensive, and complementary planning among the public, private, and volunteer sectors involved in cancer prevention, detection, treatment, and research.

CPRIT furthers cancer research in Texas by providing financial support for a wide variety of projects relevant to cancer research.

## **3. APPLICATION SURVEY**

CPRIT will be administering a survey to determine the operational aspects of peer review. Company representatives that anticipate submitting an application are requested to complete the survey as soon as possible, but no later than January 13, 2014. Company representatives should provide the following information: applicant name, name of company, telephone number, email address, estimated award amount, and award mechanism. Please select only one award mechanism as only one application can be submitted per funding cycle. This information will be used for planning purposes only, and will not be used for evaluation of the application. The survey is available [here](#).

## **4. EXECUTIVE SUMMARY**

CPRIT will foster the creation of high-quality new jobs in Texas by providing financial support for a wide variety of projects relevant to cancer. This Request for Applications (RFA) is designed to support the formation of oncology-focused companies in Texas. CPRIT expects outcomes of supported activities to directly and indirectly benefit subsequent cancer research efforts, cancer public health policy, or the continuum of cancer care—from prevention to treatment and cure. To fulfill this vision, applications may address any product development topic or issue related to cancer biology, causation, prevention, detection or screening, treatment, or cure. The overall goal of this award program is to improve outcomes of patients with cancer by increasing the availability of Food and Drug Administration (FDA)-approved therapeutic interventions with a primary focus on Texas-centric programs.

## **5. MECHANISM OF SUPPORT**

The goal of the New Company Product Development Awards is to finance the research and development of innovative products, services, and infrastructure with significant potential impact on patient care. These investments will assist early-stage startup companies by providing the opportunity to further the research and development of new products for the diagnosis, treatment, supportive care, or prevention of cancer; to establish infrastructure that is critical to the development of a robust industry; or to fill a treatment, industry, or research gap. This award mechanism will support companies that intend to undertake product research and development in Texas with a strong presence of Texas-based employees. In determining eligibility for this award, CPRIT will evaluate whether applicants have a significant presence in Texas or are willing to relocate to Texas.

## **6. OBJECTIVES**

The State of Texas seeks to attract industry partners in the field of cancer care to advance economic development and cancer care efforts in the State. The goal of this award mechanism is to support the formation and establishment of new startup companies in Texas that will develop products to significantly impact cancer care. These companies

must be Texas based or have personnel sufficient to operate the Texas-based research and/or development activities of the company, along with appropriate management, who are willing to relocate to or be hired and remain in Texas for a specified period after funding. Eligible products or services include—but are not limited to—therapeutics (e.g., small molecules and biologics), diagnostics, devices, and potential breakthrough technologies, including software and research discovery techniques. Eligible stages of research and development include translational research, proof-of-concept studies, preclinical studies, and Phase I or Phase II clinical trials. By exception, Phase III clinical trials and later stage product development projects will be considered where circumstances warrant CPRIT investment.

## **7. FUNDING INFORMATION**

This is a 3-year funding program. Financial support will be awarded based upon the breadth and nature of the research and development program proposed. While requested funds must be well justified, there is no limit on the amount that may be requested. Funding will be milestone driven.

Funds may be used for salary and fringe benefits, research supplies, equipment, clinical trial expenses, intellectual property protection, external consultants and service providers, and other appropriate research and development costs, subject to certain limitations set forth by Texas State law. If a company is working on multiple projects, care should be taken to ensure that CPRIT funds are used to support activities directly related to the specific project being funded. Requests for funds to support construction and/or renovation may be considered under compelling circumstances for projects that require facilities that do not already exist in the State of Texas. Texas State law limits the amount of awarded funds that may be spent on indirect costs to no more than 5 percent of the total award amount (5.263 percent of the direct costs).

Consistent with statutory mandate, of the total program budget, CPRIT will contribute \$2.00 for every \$1.00 contributed, in matching funds, by the company. The demonstration of available matching funds must be made prior to the distribution of

CRPTI funds, not at the time the application is submitted. The matching funds commitment may be made on a year-by-year basis.

## **8. KEY DATES**

<b>RFA release</b>	December 9, 2013
<b>Online application opens</b>	December 23, 2013, 7 a.m. Central Time
<b>Applications due</b>	January 31, 2014, 3 p.m. Central Time
<b>Invitations to present sent</b>	March 2014
<b>Notifications sent if not invited</b>	March 2014
<b>Presentations to CPRIT*</b>	April 2014

\*All applicants who wish to be considered are requested to reserve these presentation dates until notified. Information on the timing of subsequent steps will be provided to applicants later in the process.

## **9. ELIGIBILITY**

### **9.1. New Applications**

- Early-stage startup companies are eligible. Such companies may have received seed funding from family, friends, and/or angel investors. However, only applicants with no previous round of professional institutional investment (i.e., those that have not yet received Series A financing or a substantive equivalent) are eligible. The inclusion of a complete and detailed capitalization table is required for assessment of eligibility.
- Recipient companies must commit to the following: Headquarters or substantial functions of the company in Texas; personnel sufficient to operate the Texas-based research and/or development activities of the company, along with appropriate management, relocated to or hired from within Texas who will remain in Texas for a specified period after funding; and use of Texas-based subcontractors and suppliers unless adequate justification is provided for the use of out-of-State entities. To the extent that Texas-based subcontractors or collaborators are not

available, non-Texas-based collaborators and subcontractors may be used.

However, non-Texas-based collaborators and subcontractors are not eligible to receive funds from CPRIT unless exceptional circumstances are demonstrated and approved by CPRIT.

- In general, a greater extent of commitment to establishing research and/or development functions in Texas will be viewed more favorably by CPRIT. However, it is left to the applicant's judgment to make a case for what they consider to be a sufficient extent of commitment to Texas.
- An applicant may submit only one application under this RFA during this funding cycle.
- A company applicant is eligible to receive a grant award only if the applicant certifies that the company, including the company representative, any senior member or key personnel listed on the application, any company officer or director (or any person related to one or more of these individuals within the second degree of consanguinity or affinity) have not made and will not make a contribution to CPRIT or to any foundation specifically created to benefit CPRIT.
- A company applicant is not eligible to receive CPRIT funding if the company representative, any senior member or key personnel listed on the application, and any company officer or director is related to a CPRIT Oversight Committee member.
- The company applicant must report whether the company, company representative, or other individuals who contribute to the execution of the proposed project in a substantive, measurable way, whether or not those individuals are slated to receive salary or compensation under the grant award, are currently ineligible to receive Federal grant funds, or have had a grant terminated for cause within 5 years prior to the submission date of the grant application.
- CPRIT grants will be awarded by contract to successful company applicants. Certain contractual requirements are mandated by Texas State law or by administrative rules. Although the company applicant need not demonstrate the ability to comply with these contractual requirements at the time the application is submitted, applicants should familiarize themselves with these standards before

submitting a grant application. Significant issues addressed by the CPRIT contract are listed in [Section 12](#) and [Section 13](#). All statutory provisions and relevant administrative rules can be found at [www.cprit.state.tx.us](http://www.cprit.state.tx.us).

## **9.2. Resubmission Policy**

An application previously submitted to CPRIT but not funded may be resubmitted once and must follow all resubmission guidelines (see [Section 11.4.4](#)). More than one resubmission is not permitted. Applicants who choose to resubmit should carefully consider the reasons for lack of prior success. Applications that received overall numerical scores of 5 or higher are likely to need considerable attention. All resubmitted applications should be carefully reconstructed; a simple revision of the prior application with editorial or technical changes is not sufficient, and applicants are advised not to direct reviewers to such modest changes. A one-page summary of the approach to the resubmission should be included. Resubmitted applications may be assigned to reviewers who did not review the original submission. Reviewers of resubmissions are asked to assess whether the resubmission adequately addresses critiques from the previous review. **Applicants should note that addressing previous critiques is advisable; however, it does not guarantee the success of the resubmission.** All resubmitted applications must conform to the structure and guidelines outlined in this RFA.

## **9.3. Renewal Policy**

Grant recipients that have previously received CPRIT grant funding may submit an application for competitive renewal under the Established Company Product Development Award RFA. Before submitting a renewal application, applicants must consult with the Product Development Programmatic Office (see [Section 14.2](#)) to determine whether it is appropriate for their company to seek renewal funding at this time.

# **10. APPLICATION REVIEW**

## **10.1. Overview**

Applications will be assessed based on evaluation of the quality of the company and the potential for continued product development. CPRIT requires the submission of a

comprehensive scientific plan (see [Section 11.4.7](#)) and a detailed business plan (see [Section 11.4.8](#)). The review will address the commercial viability, product feasibility, scientific merit, and therapeutic impact as detailed in the company's business and scientific plans. The plans will be reviewed by an integrated panel of individuals with biotechnology expertise and experience in translational and clinical research as well as in the business development/regulatory approval processes for therapeutics, devices, and diagnostics. In addition, advocate reviewers will participate in the review process.

Funding decisions are made by the review process described below.

## **10.2. Review Process**

- 1. Product Development and Scientific Review:** Applications that pass initial administrative compliance review are assigned to independent CPRIT Product Development Peer Review Panel members for evaluation using the criteria listed below. Based on the initial evaluation and discussion by the Product Development Review Panel, a subset of company applicants may be invited to deliver in-person presentations to the review panel.
- 2. Due Diligence Review:** Following the in-person presentations, a subset of applications judged to be most meritorious by the Product Development Review Panels will be referred for additional in-depth due diligence, including—but not limited to—intellectual property, management, regulatory, manufacturing, and market assessments. Following the due diligence review, applications will be recommended for funding by the CPRIT Product Development Review Council based on the information set forth in the due diligence and intellectual property reviews, comparisons with applications from the Product Development Review Panels and programmatic priorities.
- 3. Program Integration Committee Review:** Applications recommended by the Product Development Review Council will be forwarded to the CPRIT Program Integration Committee (PIC) for review. The PIC will consider factors including program priorities set by the Oversight Committee, portfolio balance across programs, and available funding.

4. **Oversight Committee Approval:** The CPRIT Oversight Committee will vote to approve each grant award recommendation made by the PIC. The grant award recommendations will be presented at an open meeting of the Oversight Committee and must be approved by two-thirds of the Oversight Committee members present and eligible to vote.

The review process is described more fully in CPRIT's Administrative Rules, Chapter 703, Sections 703.6–703.8.

#### **10.2.1. Confidentiality of Review**

Each stage of application review is conducted confidentially, and all CPRIT Product Development Peer Review Panel members, Product Development Review Council members, Program Integration Committee members, CPRIT employees, and Oversight Committee members with access to grant application information are required to sign nondisclosure statements regarding the contents of the applications. All technological and scientific information included in the application is protected from public disclosure pursuant to Health and Safety Code §102.262(b).

Individuals directly involved with the review process operate under strict conflict of interest prohibitions. All CPRIT Product Development Peer Review Panel members and Product Development Review Council members are non-Texas residents.

An applicant will be notified regarding the peer review panel assigned to review the grant application. Peer review panel members are listed by panel on CPRIT's Web site. **By submitting a grant application, the applicant agrees and understands that the only basis for reconsideration of a grant application is limited to an undisclosed Conflict of Interest as set forth in CPRIT's Administrative Rules, Chapter 703, Section 703.9.**

Communication regarding the substance of a pending application is prohibited between the company applicant (or someone on the applicant's behalf) and the following individuals: an Oversight Committee member, a Program Integration Committee (PIC) member, a Product Development Review Panel member, or a Product Development



Review Council member. Applicants should note that the CPRIT PIC is comprised of the CPRIT Chief Executive Officer, the Chief Scientific Officer, the Chief Prevention Officer, the Chief Product Development Officer, and the Commissioner of State Health Services. The prohibition on communication begins on the first day that grant applications for the particular grant mechanism are accepted by CPRIT and extends until the grant applicant receives notice regarding a final decision on the grant application. Intentional, serious, or frequent violations of this rule may result in the disqualification of the grant application from further consideration for a grant award.

### 10.3. Review Criteria

Full peer review of applications will be based on primary scored criteria and secondary unscored criteria, listed below. Review committees will evaluate and score each primary criterion and subsequently assign a global score that reflects an overall assessment of the application. **The overall assessment will not be an average of the scores of the individual criteria; rather, it will reflect the reviewers' overall impression of the application. Evaluation of the scientific merit of each application is within the sole discretion of the peer reviewers.**

#### 10.3.1. Primary Criteria

Primary criteria will evaluate the scientific merit and potential impact of the proposed work contained in the application. Concerns with any of these criteria potentially indicate a major flaw in the significance and/or design of the proposed study.

Primary criteria include:

**Significance and Impact:** Will the outcomes of this CPRIT-funded work result in the development of innovative products with significant product development potential? Will the outcome substantially impact the diagnosis, treatment, prevention of cancer, or supportive care for patients with cancer? How would competing products or services affect the value of the proposed offering?

**Product:** Is there demonstrated proof of relevance, and does the product fulfill a clear, unmet medical or infrastructure need? Has work been conducted that supports the advancement of the proposed product, service, or technology? Can the product be

produced or manufactured in a commercially viable fashion? Is there an appropriate basis for a reimbursement strategy?

**Market Plan:** Is there a realistic assessment of the market size and expected penetration? Has management adequately assessed potential competitors and described how the company's offering will successfully compete with them?

**Development Plan and/or Regulatory Path:** Is the development plan and/or regulatory path well characterized and appropriate? Is the plan milestone driven, and does it address both a positive and a negative outcome? Does the budget appropriately support the plan?

**Scientific Plan:** Is the proposed product, service, and/or infrastructure based on a feasible research framework, hypothesis, and/or goal? Are the methods appropriate, and are potential research and developmental obstacles and unexpected outcomes discussed?

**Management and Staffing:** Does the applicant have the appropriate level of management experience to execute the stated strategy in Texas, especially if the headquarters of the company are not in Texas? Would the proposed team have the needed experience or access to experienced external assistance, facilities, and resources to accomplish all aspects of the proposed plan?

### **10.3.2. Secondary Criteria**

Secondary criteria contribute to the global score assigned to the application. Concerns with these criteria potentially question the feasibility of the proposed research and development activities.

Secondary criteria include:

**Budget and Duration of Support:** Are the budget and duration appropriate for the proposed work? Will the amount requested enable the applicant to reach appropriate milestones? Is the use of the funds requested in line with the stated objectives of the applicant and CPRIT? Is it clear how funds will be used? (For example: Is it clear that no CPRIT funds will be used outside of Texas without specific authorization by CPRIT? Is it clear that no CPRIT funds will be sent to the corporate headquarters if those headquarters remain outside of Texas?) Does the proposed investment fund the research and

development of the proposed product, service, or technology to a point where, if the results are positive, it is likely that the project will be able to attract further financial support outside of CPRIT?

## **11. SUBMISSION GUIDELINES**

Applicants are advised to carefully review all instructions in this section to ensure the accurate and complete submission of all components of the application. Please refer to the *Instructions for Applicants* document for details that will be available when the application receipt system opens. Applications that are missing one or more components, exceed the specified page or word limits, or that do not meet the eligibility requirements listed above will be administratively withdrawn without review.

### **11.1. Online Application Receipt System and Application Submission Deadline**

Applications must be submitted via the CPRIT Application Receipt System (CARS) (<https://CPRITGrants.org>). **Only applications submitted through this portal will be considered eligible for evaluation.** The applicant is eligible solely for the grant mechanism specified by the RFA under which the grant application was submitted. The company applicant must create a user account in the system to start and submit an application. The co-applicant, if applicable, must also create a user account to participate in the application. Furthermore, the Authorized Signing Official (ASO) (an individual authorized to sign and submit an application on behalf of the company applicant) must also create a user account in CARS. An application may not be submitted without ASO approval. Only the ASO is authorized to officially submit the application to CPRIT. Applications will be accepted beginning at 7 a.m. Central Time on December 23, 2013 and must be submitted by 3 p.m. Central Time on January 31, 2014. **Submission of an application is considered an acceptance of the terms and conditions of the RFA.**

### **11.2. Submission Deadline Extension**

The submission deadline may be extended for one or more grant applications upon a showing of good cause. All requests for extension of the submission deadline must be submitted via e-mail to the CPRIT HelpDesk. Submission deadline extensions, including

the reason for the extension, will be documented as part of the grant review process records.

### **11.3. Product Development Review Fee**

All applicants must submit a fee of \$1,000 for product development review. Payment should be made by check or money order payable to CPRIT; electronic and credit card payments are not acceptable. The application ID and the name of the submitter must be indicated on the payment. All payments must be postmarked by the application submission deadline and mailed to:

Cancer Prevention and Research Institute of Texas  
P.O. Box 12097  
Austin, TX 78711

### **11.4. Application Components**

#### **11.4.1. Layperson's Summary (1,500 characters)**

Provide an abbreviated summary for a lay audience using clear, nontechnical terms. Describe specifically how the proposed project would support CPRIT's mission (see [Section 2](#)). Would it fill a needed gap in patient care or in the development of a sustainable oncology industry in Texas? Would it synergize with Texas-based resources? Describe the overall goals of the work, the type(s) of cancer addressed, the potential significance of the results, and the impact of the work on advancing the fields of diagnosis, treatment, or prevention of cancer. Clearly address how the company's work, if successful, will have a major impact on the care of patients with cancer. The information provided in this summary will be made publicly available by CPRIT, particularly if the application is recommended for funding. The Layperson's Summary will be also used by advocate reviewers in evaluating the significance and impact of the proposed work. Do not include any proprietary information in this section.

#### **11.4.2. Goals and Objectives**

List specific goals and objectives for each year of the project. These goals and objectives will also be used during the submission and evaluation of progress reports and assessment of project success.

#### **11.4.3. Timeline (One page)**

Provide an outline of anticipated major milestones to be tracked. Timelines will be reviewed for reasonableness, and adherence to timelines will be a criterion for continued support of successful applications. If the application is approved for funding, this section will be included in the award contract. Applicants are advised not to include information that they consider confidential or proprietary when preparing this section.

#### **11.4.4. Resubmission Summary (One page)**

If this is a resubmission, upload a summary of the approach, including a summary of the applicant's response to previous feedback. Clearly indicate to reviewers how the application has been improved in response to the critiques. Refer the reviewers to specific sections of other documents in the application where further detail on the points in question may be found. When a resubmission is evaluated, responsiveness to previous critiques is assessed. If this is not a resubmission, then no summary is required.

**Note:** An application is a resubmission only if the previous application was finalized and submitted to CPRIT. However, an application that was submitted to CPRIT to be considered for FY2013 Cycle 3 awards and was returned by CPRIT due to the moratorium is not considered to be a resubmission.

#### **11.4.5. Executive Summary (One page)**

Provide an executive summary that clearly explains the product, service, technology, or infrastructure proposed; competition; market need and size; development or implementation plans; regulatory path; reimbursement strategy; and funding needs. Applicants must clearly describe the existing or proposed company infrastructure and personnel located in Texas for this endeavor.

#### **11.4.6. Slide Presentation (Ten pages)**

Provide a slide presentation summarizing the application. The presentation should be submitted in PDF format, with one slide filling each landscape-orientation page. The slides should succinctly capture all essential elements of the application and should stand alone.

#### **11.4.7. Scientific Plan (Ten pages)**

Present the rationale behind the proposed product or service, emphasizing the pressing problem in cancer care that will be addressed. Summarize the evidence gathered to date in support of the company's ideas. Describe the label claims that the company ultimately hopes to make, and describe the plan to gather evidence to support these claims. Outline the steps to be taken during the proposed period of the award, including the design of the translational or clinical research, methods, and anticipated results. Describe potential problems or pitfalls and alternative approaches. If clinical research is proposed, present a realistic plan to accrue a sufficient number of human subjects meeting the inclusion criteria within the proposed time period.

**The scientific plan submitted must be of sufficient depth and quality to pass rigorous scrutiny by the highly qualified group of reviewers. To the extent possible, the scientific plan should be driven by data. In the past, applications that have been scored poorly have been criticized for assuming that assertions could be taken on faith. Convincing data are much preferred.**

#### **11.4.8. Business Plan (Fifteen pages)**

Provide a business plan covering all of the topics below in the order shown. Successful applicants will make thoughtful, careful, and economical use of the limited space. Note that if the company is selected to undergo due diligence, information to support a full intellectual property review will be requested at that time. New Company Product Development Award applicants will be evaluated based not only on the current status of the components of the business plan but also on whether current weaknesses and gaps are acknowledged and whether plans to address them are outlined.

- A. Introduction:** Present the rationale behind the proposed project, emphasizing the pressing problem in cancer care that will be addressed. Describe the label claims that the company ultimately hopes to make, and briefly describe the plan to gather evidence to support these claims. Include the minimum level of detail required to provide a context for the rest of the business plan. Cross-reference sections in the scientific plan where further details may be found.

- B. Products and Markets:** Provide a brief description of the envisioned product and how the product will be administered to patients. Describe the initial market that will be targeted and how the envisioned product will fit within the standard of care.
- C. Regulatory Plans:** Provide a detailed regulatory plan, including preclinical and clinical activities, driven by interactions with the FDA, if possible. Summarize all interactions to date with the FDA.
- D. Risk Analysis:** Describe the specific risks inherent to the product plan and how they would be mitigated.
- E. Current and Pending Support:** Describe all funding sources. Provide a complete and detailed capitalization table, which should include all parties who have investments, stock, or rights in the company. The identities of all parties must be listed. It is not appropriate to list any funding source as anonymous.
- F. Financial Projections:** Provide a detailed source and use analysis of the development plan, focusing on the achievement of specific milestones.
- G. Resources Requested:** Include resources needed for research and product development and for any relocation expenses. The matching funds should be included in this section; however, this is the only section of the business plan that does not deal exclusively with CPRIT-requested funds.
- H. Scope of Work and Milestones:** Outline the specific goals of the project. Provide an outline of anticipated major milestones to be tracked. Timelines will be reviewed for reasonableness, and adherence to timelines will be a criterion for continued support of successful applications. If the application is approved for funding, this section will be included in the award contract.
- I. Key Personnel Located in Texas and Any Key Management Located Outside of Texas:** Present a plan for recruiting a senior management and scientific team, describing the types of expertise and skill sets that the project will require. For each key person currently on board, provide a paragraph briefly summarizing his or her

present title and position, prior industry experience, education, and any other information considered essential for evaluation of qualifications.

**J. Organizational Commitment to Texas:** Describe how CPRIT funding of the applicant's company would benefit the State of Texas. For example, describe how the company would create high-quality new jobs in the State and/or recruit out-of-State talent, and mention any Texas-based subcontractors and suppliers that would be used and any other unique, Texas-based resources that would be leveraged.

#### **11.4.9. Biographical Sketches of Key Scientific Personnel (Eight pages)**

Provide a biographical sketch for up to four key scientific personnel that describes their education and training, professional experience, awards and honors, and publications relevant to cancer research. Each biographical sketch must not exceed two pages and must use the "Product Development Programs: Biographical Sketch" template.

(In addition, information on the members of the senior management and scientific team should be included in the "Key Personnel" section of the Business Plan

[see [Section 11.4.8](#)]).

#### **11.4.10. Budget and Justification**

Provide a compelling justification of the budget for the entire proposed period of support, including salaries and benefits, supplies, equipment, patient care costs, animal care costs, and other expenses. The budget must be aligned with the proposed milestones. In preparing the requested budget, applicants should be aware of the following:

- Equipment having a useful life of more than 1 year and an acquisition cost of \$5,000 or more per unit must be specifically approved by CPRIT. An applicant does not need to seek this approval prior to submitting the application.
- Texas State law limits the amount of grant funds that may be spent on indirect costs to no more than 5 percent of the total award amount (5.263 percent of the direct costs). Guidance regarding indirect cost recovery can be found in CPRIT's Administrative Rules, which are available at [www.cprit.state.tx.us](http://www.cprit.state.tx.us).
- The annual salary that an individual may receive under a CPRIT award for FY 2014 is \$200,000. In other words, an individual may request salary proportional to the



percentage effort up to a maximum of \$200,000. Salary does not include fringe benefits. CPRIT FY 2014 is from September 1, 2013, through August 31, 2014.

## **12. AWARD ADMINISTRATION**

Texas law requires that CPRIT awards be made by contract between the applicant and CPRIT. CPRIT grant awards are made to entities, not to individuals. Award contract negotiation and execution will commence once the CPRIT Oversight Committee has approved an application for a grant award. CPRIT may require, as a condition of receiving a grant award, that the grant recipient use CPRIT's electronic Grant Management System to exchange, execute, and verify legally binding grant contract documents and grant award reports. Such use shall be in accordance with CPRIT's electronic signature policy as set forth in Chapter 701, Section 701.25.

Texas law specifies several components that must be addressed by the award contract, including needed compliance and assurance documentation, budgetary review, progress and fiscal monitoring, and terms relating to revenue sharing and intellectual property rights. These contract provisions are specified in CPRIT's Administrative Rules, which are available at [www.cprit.state.tx.us](http://www.cprit.state.tx.us). Applicants are advised to review CPRIT's Administrative Rules related to contractual requirements associated with CPRIT grant awards and limitations related to the use of CPRIT grant awards as set forth in Chapter 703, Sections 703.10 - 703.12.

Prior to disbursement of grant award funds, the grant recipient organization must demonstrate that it has adopted and enforces a tobacco-free workplace policy consistent with the requirements set forth in CPRIT's Administrative Rules, Chapter 703, Section 703.20.

CPRIT requires award recipients to submit an annual progress report. These reports summarize the progress made toward the research goals and address plans for the upcoming year. In addition, fiscal reporting, human studies reporting, and vertebrate animal use reporting will be required as appropriate. Continuation of funding is contingent upon the timely receipt of these reports. Failure to provide timely and

complete reports may waive reimbursement of grant award costs, and may result in the termination of award contract. Forms and instructions will be made available at [www.cpritis.state.tx.us](http://www.cpritis.state.tx.us).

**Project Economics Sharing:** Recipients should also be aware that the funding award contract will include a revenue-sharing agreement and will require CPRIT to have input on any future patents, agreements, or other financial arrangements related to the products, services, or infrastructure supported by the CPRIT investment. These contract provisions are specified in CPRIT's Administrative Rules, which are available at [www.cpritis.state.tx.us](http://www.cpritis.state.tx.us).

### **13. REQUIREMENT TO DEMONSTRATE AVAILABLE FUNDS**

Texas State law requires that prior to disbursement of CPRIT grant funds, the award recipient demonstrate that it has \$1.00 in matching funds for every \$2.00 from CPRIT. Matching funds need not be in hand when the application is submitted. However, matching funds must be obtained before CPRIT funds will be released for use. CPRIT funds must, whenever possible, be spent in Texas. A company's matching funds must be designated for the CPRIT-funded project but may be spent outside of Texas. Grant applicants are advised to consult CPRIT's Administrative Rules, Chapter 703, Section 703.11 for specific requirements associated with demonstration of available funds.

### **14. CONTACT INFORMATION**

#### **14.1. HelpDesk**

HelpDesk support is available for questions regarding user registration and online submission of applications. Queries submitted via e-mail will be answered within 1 business day. HelpDesk staff are not in a position to answer questions regarding scientific and commercialization aspects of applications. **Before contacting the HelpDesk, please refer to the "Instructions for Applicants" document, which provides a step-by-step guide on using the Application Receipt System.**

**Dates of operation:** December 23, 2013 to January 31, 2014 (excluding public holidays)

**Hours of operation:** Monday, Tuesday, Thursday, Friday, 7 a.m. to 4 p.m. Central Time  
Wednesday, 8 a.m. to 4 p.m. Central Time

**Tel:** 866-941-7146

**E-mail:** [Help@CPRITGrants.org](mailto:Help@CPRITGrants.org)

#### **14.2. Programmatic Questions**

Questions regarding the CPRIT Program, including questions regarding this or any other funding opportunity, should be directed to the CPRIT Product Development Program Director.

**Tel:** 512-305-8486

**E-mail:** [Help@CPRITGrants.org](mailto:Help@CPRITGrants.org)

**Web site:** [www.cprit.state.tx.us](http://www.cprit.state.tx.us)



**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**

# **REQUEST FOR APPLICATIONS**

## **RFA C-14-RELCO-1**

### **Company Relocation Product Development Awards**

**Please also refer to the Instructions for Applicants document,  
which will be posted December 23, 2013**

**Application Receipt Opening Date:** December 23, 2013

**Application Receipt Closing Date:** January 31, 2014

**FY 2014**

Fiscal Year Award Period

September 1, 2013–August 31, 2014

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## **RFA VERSION HISTORY**

Rev 12/9/13 RFA release

## 1. KEY POINTS

This Company Relocation Product Development Award mechanism is governed by the following restrictions:

- Company applicants must be currently based outside of Texas and must have already received at least one round of professional institutional investment (i.e., Series A financing or a substantive equivalent). Applicants that have not yet received a round of professional institutional investment should apply under the New Company Product Development Award mechanism.
- Headquarters or substantial business functions of the company in Texas; personnel sufficient to operate the Texas-based research and/or development activities of the company, along with appropriate management, relocated to or hired from within Texas; and use of Texas-based subcontractors and suppliers unless adequate justification is provided for the use of out-of-State entities.
- Of the total program budget, the Cancer Prevention and Research Institute of Texas (CPRIT) will contribute \$2.00 for every \$1.00 contributed in matching funds by the company. The demonstration of available matching funds must be prior to the distribution of CPRIT grant funds, not at the time the application is submitted. CPRIT funds must, whenever possible, be spent in Texas. A company's matching funds must be designated for the CPRIT-funded project but may be spent outside of Texas.
- Funding may be tranching and will be tied to the achievement of contract-specified milestones.
- Funding award contracts will include a revenue-sharing agreement or equity to be negotiated at contract execution and will require CPRIT to have input on any future patents, agreements, or other financial arrangements related to the products, services, or infrastructure supported by the CPRIT investment. These contract provisions are specified in CPRIT's Administrative Rules, which are available at [www.cprit.state.tx.us](http://www.cprit.state.tx.us).



## **2. ABOUT CPRIT**

The State of Texas has established CPRIT, which may issue up to \$3 billion in general obligation bonds to fund grants for cancer research and prevention.

CPRIT is charged by the Texas Legislature to:

- Create and expedite innovation in the area of cancer research and product or service development, thereby enhancing the potential for a medical or scientific breakthrough in the prevention, treatment, and possible cures for cancer;
- Attract, create, or expand research capabilities of public or private institutions of higher education and other public or private entities that will promote a substantial increase in cancer research and in the creation of high-quality new jobs in the State of Texas; and
- Continue to develop and implement the Texas Cancer Plan by promoting the development and coordination of effective and efficient statewide public and private policies, programs, and services related to cancer and by encouraging cooperative, comprehensive, and complementary planning among the public, private, and volunteer sectors involved in cancer prevention, detection, treatment, and research.

CPRIT furthers cancer research in Texas by providing financial support for a wide variety of projects relevant to cancer research.

## **3. APPLICATION SURVEY**

CPRIT will be administering a survey to determine the operational aspects of peer review. Company representatives that anticipate submitting an application are requested to complete the survey as soon as possible, but no later than January 13, 2014. Company representatives should provide the following information: applicant name, name of company, telephone number, email address, estimated award amount, and award mechanism. Please select only one award mechanism as only one application can be submitted per funding cycle. This information will only be used for planning purposes only, and will not be used for evaluation of the application. The survey is available [here](#).

#### **4. EXECUTIVE SUMMARY**

CPRIT will foster the creation of high-quality new jobs in Texas by providing financial support for a wide variety of projects relevant to cancer. The award mechanism described in this Request for Applications (RFA) is designed to encourage the relocation of existing oncology-focused companies or a substantial portion of their business to Texas. CPRIT expects outcomes of supported activities to directly and indirectly benefit subsequent cancer research efforts, cancer public health policy, or the continuum of cancer care—from prevention to treatment and cure. To fulfill this vision, applications may address any product development topic or issue related to cancer biology, causation, prevention, detection or screening, treatment, or cure. The overall goal of this award program is to improve outcomes of patients with cancer by increasing the availability of Food and Drug Administration (FDA)–approved therapeutic interventions with a primary focus on Texas-centric programs.

#### **5. MECHANISM OF SUPPORT**

The goal of the Company Relocation Product Development Award is to finance the research and development of innovative products, services, and infrastructure with significant potential impact on patient care. These investments will provide companies or limited partnerships that are willing to relocate all or a substantial portion of their business to Texas with the opportunity to further the development of new products for the diagnosis, treatment, supportive care, or prevention of cancer; to establish infrastructure that is critical to the development of a robust industry; or to fill a treatment, industry, or research gap. This award mechanism will support companies that intend to undertake product research and development in Texas with a strong presence of Texas-based employees.

#### **6. OBJECTIVES**

The State of Texas seeks to attract industry partners in the field of cancer care to advance economic development and cancer care efforts in the State. The goal of this award mechanism is to recruit to Texas companies with proven management teams who are focused on exceptional product opportunities to improve cancer care. These companies

must presently be domiciled outside of Texas, and have sufficient personnel to operate the Texas-based research and/or development activities of the company and, along with appropriate management, must be willing to relocate to or be hired and remain in Texas for a specified period after funding. Eligible products or services include—but are not limited to—therapeutics (e.g., small molecules and biologics), diagnostics, devices, and potential breakthrough technologies, including software and research discovery techniques. Eligible stages of research development include translational research, proof-of-concept studies, preclinical studies, and Phase I or Phase II clinical trials. By exception, Phase III clinical trials and later stage product development projects will be considered where circumstances warrant CPRIT investment.

## **7. FUNDING INFORMATION**

This is a 3-year funding program. Financial support will be awarded based upon the breadth and nature of the research and development program proposed. While requested funds must be well justified, there is no limit on the amount that may be requested. Funding will be milestone driven.

Funds may be used for salary and fringe benefits, research supplies, equipment, clinical trial expenses, intellectual property protection, external consultants and service providers, and other appropriate research and development costs, subject to certain limitations set forth by Texas State law. If a company is working on multiple projects, care should be taken to ensure that CPRIT funds are used to support activities directly related to the specific project being funded. Requests for funds to support construction and/or renovation may be considered under compelling circumstances for projects that require facilities that do not already exist in the State of Texas. Texas State law limits the amount of awarded funds that may be spent on indirect costs to no more than 5 percent of the total award amount (5.263 percent of the direct costs).

Consistent with statutory mandate, of the total program budget, CPRIT will contribute \$2.00 for every \$1.00 contributed in matching funds by the company. The demonstration of available matching funds must be made prior to the distribution of CPRTI funds, not at

the time the application is submitted. The matching funds commitment may be made on a year-by-year basis.

## **8. KEY DATES**

<b>RFA release</b>	December 9, 2013
<b>Online application opens</b>	December 23, 2013, 7 a.m. Central Time
<b>Applications due</b>	January 31, 2014, 3 p.m. Central Time
<b>Invitations to present sent</b>	March 2014
<b>Notifications sent if not invited</b>	March 2013
<b>Presentations to CPRIT*</b>	April 2013

\*All applicants who wish to be considered are requested to reserve these presentation dates until notified. Information on the timing of subsequent steps will be provided to applicants later in the process.

## **9. ELIGIBILITY**

### **9.1. New Applications**

- Company applicants must be currently based outside of Texas and must have already received at least one round of professional institutional investment (i.e., Series A financing or a substantive equivalent). Applicants that have not yet received a round of professional institutional investment should apply under the New Company Product Development Awards mechanism.
- Recipient companies must commit to the following: Headquarters or substantial functions of the Company in Texas; personnel sufficient to operate the Texas-based research and/or development activities of the Company, along with appropriate management, relocated to or hired from within Texas and remain in Texas for a specified period after funding; and use of Texas-based subcontractors and suppliers unless adequate justification is provided for the use of out-of-State entities. To the extent that Texas-based subcontractors or collaborators are not available, non-Texas-based collaborators and subcontractors may be used. However,

non-Texas-based collaborators and subcontractors are not eligible to receive funds from CPRIT unless exceptional circumstances are demonstrated and approved by CPRIT.

- In general, a greater extent of commitment to establishing research and/or development functions in Texas will be viewed more favorably by CPRIT. However, it is left to the applicant's judgment to make a case for what they consider to be a sufficient extent of commitment to Texas.
- An applicant may submit only one application under this RFA during this funding cycle.
- A company applicant is eligible to receive a grant award only if the applicant certifies that the company, including the company representative, any senior member or key personnel listed on the application, any company officer or director (or any person related to one or more of these individual within the second degree of consanguinity or affinity) have not made and will not make a contribution to CPRIT or to any foundation specifically created to benefit CPRIT.
- A company applicant is not eligible to receive CPRIT funding if the company representative, any senior member or key personnel listed on the application, and any Company officer or director is related to a CPRIT Oversight Committee member.
- The company applicant must report whether the company, company representative, or other individuals who contribute to the execution of the proposed project in a substantive, measurable way, whether or not those individuals are slated to receive salary or compensation under the grant award, are currently ineligible to receive Federal grant funds or have had a grant terminated for cause within 5 years prior to the submission date of the grant application.
- CPRIT grants will be awarded by contract to successful company applicants. Certain contractual requirements are mandated by Texas State law or by administrative rules. Although the company applicant need not demonstrate the ability to comply with these contractual requirements at the time the application is submitted, applicants should familiarize themselves with these standards before submitting a grant application. Significant issues addressed by the CPRIT contract

are listed in [Section 12](#) and [Section 13](#). All statutory provisions and relevant administrative rules can be found at [www.cpriti.state.tx.us](http://www.cpriti.state.tx.us).

## 9.2. Resubmission Policy

An application previously submitted to CPRIT but not funded may be resubmitted once and must follow all resubmission guidelines (see [Section 11.4.4](#)). More than one resubmission is not permitted. Applicants who choose to resubmit should carefully consider the reasons for lack of prior success. Applications that received overall numerical scores of 5 or higher are likely to need considerable attention. All resubmitted applications should be carefully reconstructed; a simple revision of the prior application with editorial or technical changes is not sufficient, and applicants are advised not to direct reviewers to such modest changes. A one-page summary of the approach to the resubmission should be included. Resubmitted applications may be assigned to reviewers who did not review the original submission. Reviewers of resubmissions are asked to assess whether the resubmission adequately addresses critiques from the previous review. **Applicants should note that addressing previous critiques is advisable; however, it does not guarantee the success of the resubmission.** All resubmitted applications must conform to the structure and guidelines outlined in this RFA.

## 9.3. Renewal Policy

Grant recipients that have previously received CPRIT grant funding may submit an application for competitive renewal under the Established Company Product Development Award. Before submitting a renewal application, applicants must consult with the Product Development Programmatic Office (see [Section 14.2](#)) to determine whether it is appropriate for their company to seek renewal funding at this time.

# 10. APPLICATION REVIEW

## 10.1. Overview

Applications will be assessed based on evaluation of the quality of the company and the potential for continued product development. CPRIT requires the submission of a comprehensive scientific plan (see [Section 11.4.7](#)) and a detailed business plan (see [Section 11.4.8](#)). The review will address the commercial viability, product

feasibility, scientific merit, and therapeutic impact as detailed in the company's business and scientific plans. The plans will be reviewed by an integrated panel of individuals with biotechnology expertise and experience in translational and clinical research as well as in the business development/regulatory approval processes for therapeutics, devices, and diagnostics. In addition, advocate reviewers will participate in the review process.

Funding decisions are made by the review process described below.

## **10.2. Review Process**

- 1. Product Development and Scientific Review:** Applications that pass initial administrative compliance review are assigned to independent CPRIT Product Development Peer Review Panel members for evaluation using the criteria listed below. Based on the initial evaluation and discussion by the Product Development Review Panel, a subset of company applicants may be invited to deliver in-person presentations to the review panel.
- 2. Due Diligence Review:** Following the in-person presentations, a subset of applications judged to be most meritorious by the Product Development Review Panel will be referred for additional in-depth due diligence, including—but not limited to—intellectual property, management, regulatory, manufacturing, and market assessments. Following the due diligence review, applications will be recommended for funding by the CPRIT Product Development Review Council based on the information set forth in the due diligence and intellectual property reviews, comparisons with applications from the Product Development Review Panel, and programmatic priorities.
- 3. Program Integration Committee Review:** Applications recommended by the Product Development Review Council will be forwarded to the CPRIT Program Integration Committee (PIC) for review. The PIC will consider factors including program priorities set by the Oversight Committee, portfolio balance across programs, and available funding.

4. **Oversight Committee Approval:** The CPRIT Oversight Committee will vote to approve each grant award recommendation made by the PIC. The grant award recommendations will be presented at an open meeting of the Oversight Committee and must be approved by two-thirds of the Oversight Committee members present and eligible to vote.

The review process is described more fully in CPRIT's Administrative Rules, Chapter 703, Sections 703.6–703.8.

#### **10.2.1. Confidentiality of Review**

Each stage of application review is conducted confidentially, and all CPRIT Product Development Peer Review Panel members, Product Development Review Council members, Program Integration Committee members, CPRIT employees, and Oversight Committee members with access to grant application information are required to sign nondisclosure statements regarding the contents of the applications. All technological and scientific information included in the application is protected from public disclosure pursuant to Health and Safety Code §102.262(b).

Individuals directly involved with the review process operate under strict conflict of interest prohibitions. All CPRIT Product Development Peer Review Panel members and Product Development Review Council members are non-Texas residents.

An applicant will be notified regarding the review panel assigned to review the grant application. Peer review panel members are listed by panel on CPRIT's Web site.

**By submitting a grant application, the applicant agrees and understands that the only basis for reconsideration of a grant application is limited to an undisclosed Conflict of Interest as set forth in CPRIT's Administrative Rules, Chapter 703, Section 703.9.**

Communication regarding the substance of a pending application is prohibited between the company applicant (or someone on the applicant's behalf) and the following individuals: an Oversight Committee member, a Program Integration Committee (PIC) member, a Product Development Review Panel member, or a Product Development



Review Council member. Applicants should note that the CPRIT (PIC) is comprised of the CPRIT Chief Executive Officer, the Chief Scientific Officer, the Chief Prevention Officer, the Chief Product Development Officer, and the Commissioner of State Health Services. The prohibition on communication begins on the first day that grant applications for the particular grant mechanism are accepted by CPRIT and extends until the grant applicant receives notice regarding a final decision on the grant application. The prohibition on communication does not apply to the time period when pre-applications or letters of interest are accepted. Intentional, serious, or frequent violations of this rule may result in the disqualification of the grant applicant from further consideration for a grant award.

### **10.3. Review Criteria**

Full peer review of applications will be based on primary scored criteria and secondary unscored criteria, listed below. Review committees will evaluate and score each primary criterion and subsequently assign a global score that reflects an overall assessment of the application. **The overall assessment will not be an average of the scores of the individual criteria; rather, it will reflect the reviewers' overall impression of the application. Evaluation of the scientific merit of each application is within the sole discretion of the peer reviewers.**

#### **10.3.1. Primary Criteria**

Primary criteria will evaluate the scientific merit and potential impact of the proposed work contained in the application. Concerns with any of these criteria potentially indicate a major flaw in the significance and/or design of the proposed study.

Primary criteria include:

**Significance and Impact:** Will the outcomes of this CPRIT-funded work result in the development of innovative products with significant product development potential? Will the outcome substantially impact the diagnosis, treatment, prevention of cancer, or supportive care for patients with cancer? How would competing products or services affect the value of the proposed offering?

**Product:** Is there demonstrated proof of relevance, and does the product fulfill a clear, unmet medical or infrastructure need? Has work been conducted that supports the advancement of the proposed product, service, or technology? Can the product be produced or manufactured in a commercially viable fashion? Is there an appropriate basis for a reimbursement strategy?

**Market Plan:** Is there a realistic assessment of the market size and expected penetration? Has management adequately assessed potential competitors and described how the company's offering will successfully compete with them?

**Development Plan and/or Regulatory Path:** Is the development plan and/or regulatory path well characterized and appropriate? Is the plan milestone driven, and does it address both a positive and a negative outcome? Does the budget appropriately support the plan?

**Scientific Plan:** Is the proposed product, service, and/or infrastructure based on a feasible research framework, hypothesis, and/or goal? Are the methods appropriate, and are potential research and developmental obstacles and unexpected outcomes discussed?

**Management and Staffing:** Does the applicant have the appropriate level of management experience to execute the stated strategy in Texas, especially if the headquarters of the company are not in Texas? Does the team have the needed experience or access to experienced external assistance, facilities, and resources to accomplish all aspects of the proposed plan?

### **10.3.2. Secondary Criteria**

Secondary criteria contribute to the global score assigned to the application. Concerns with these criteria potentially question the feasibility of the proposed research and development activities.

Secondary criteria include:

**Budget and Duration of Support:** Are the budget and duration appropriate for the proposed work? Will the amount requested enable the applicant to reach appropriate milestones? Is the use of the funds requested in line with the stated objectives of the applicant and CPRIT? Is it clear how funds will be used? (For example: Is it clear that no

CPRIT funds will be used outside of Texas without specific authorization by CPRIT? Is it clear that no CPRIT funds will be sent to the corporate headquarters if those headquarters remain outside of Texas?) Does the proposed investment fund the development of the proposed product, service, or technology to a point where, if the results are positive, it is likely that the project will be able to attract further financial support outside of CPRIT?

## **11. SUBMISSION GUIDELINES**

Applicants are advised to carefully review all instructions in this section to ensure the accurate and complete submission of all components of the application. Please refer to the *Instructions for Applicants* document for details that will be available when the application receipt system opens. Applications that are missing one or more components, exceed the specified page or word limits, or that do not meet the eligibility requirements listed above will be administratively withdrawn without review.

### **11.1. Online Application Receipt System and Application Submission Deadline**

Applications must be submitted via the CPRIT Application Receipt System (CARS) (<https://CPRITGrants.org>). **Only applications submitted through this portal will be considered eligible for evaluation.** The applicant is eligible solely for the grant mechanism specified by the RFA under which the grant application was submitted. The company applicant must create a user account in the system to start and submit an application. The co-applicant, if applicable, must also create a user account to participate in the application. Furthermore, the Authorized Signing Official (ASO) (an individual authorized to sign and submit an application on behalf of the company applicant) must also create a user account in CARS. An application may not be submitted without ASO approval. Only the ASO is authorized to officially submit the application to CPRIT. Applications will be accepted beginning at 7 a.m. Central Time on December 23, 2013 and must be submitted by 3 p.m. Central Time on January 31, 2014. **Submission of an application is considered an acceptance of the terms and conditions of the RFA.**

### **11.2. Submission Deadline Extension**

The submission deadline may be extended for one or more grant applications upon a showing of good cause. All requests for extension of the submission deadline must be

submitted via e-mail to the CPRIT HelpDesk. Submission deadline extensions, including the reason for the extension, will be documented as part of the grant review process records.

### **11.3. Product Development Review Fee**

All applicants must submit a fee of \$1,000 for product development review. Payment should be made by check or money order payable to CPRIT; electronic and credit card payments are not acceptable. The application ID and the name of the submitter must be indicated on the payment. All payments must be postmarked by the application submission deadline and mailed to:

Cancer Prevention and Research Institute of Texas  
P.O. Box 12097  
Austin, TX 78711

### **11.4. Application Components**

#### **11.4.1. Layperson's Summary (1,500 characters)**

Provide an abbreviated summary for a lay audience using clear, nontechnical terms. Describe specifically how the proposed project would support CPRIT's mission (see [Section 2](#)). Would it fill a needed gap in patient care or in the development of a sustainable oncology industry in Texas? Would it synergize with Texas-based resources? Describe the overall goals of the work, the type(s) of cancer addressed, the potential significance of the results, and the impact of the work on advancing the fields of diagnosis, treatment, or prevention of cancer. Clearly address how the company's work, if successful, will have a major impact on the care of patients with cancer. The information provided in this summary will be made publicly available by CPRIT, particularly if the application is recommended for funding. The Layperson's Summary will be also used by advocate reviewers in evaluating the significance and impact of the proposed work. Do not include any proprietary information in this section.

#### **11.4.2. Goals and Objectives**

List specific goals and objectives for each year of the project. These goals and objectives will also be used during the submission and evaluation of progress reports and assessment of project success.

#### **11.4.3. Timeline (One page)**

Provide an outline of anticipated major milestones to be tracked. Timelines will be reviewed for reasonableness, and adherence to timelines will be a criterion for continued support of successful applications. If the application is approved for funding, this section will be included in the award contract. Applicants are advised not to include information that they consider confidential or proprietary when preparing this section.

#### **11.4.4. Resubmission Summary (One page)**

If this is a resubmission, upload a summary of the approach, including a summary of the applicant's response to previous feedback. Clearly indicate to reviewers how the application has been improved in response to the critiques. Refer the reviewers to specific sections of other documents in the application where further detail on the points in question may be found. When a resubmission is evaluated, responsiveness to previous critiques is assessed. If this is not a resubmission, then no summary is required.

**Note:** An application is a resubmission only if the previous application was finalized and submitted to CPRIT. An application that was submitted to CPRIT to be considered for FY2013 Cycle 3 awards and was returned by CPRIT due to the moratorium is not considered to be a resubmission.

#### **11.4.5. Executive Summary (One page)**

Provide an executive summary that clearly explains the product, service, technology, or infrastructure proposed; competition; market need and size; development or implementation plans; regulatory path; reimbursement strategy; and funding needs. Applicants must clearly describe the existing or proposed company infrastructure and personnel located in Texas for this endeavor.

#### **11.4.6. Slide Presentation (Ten pages)**

Provide a slide presentation summarizing the application. The presentation should be submitted in PDF format, with one slide filling each landscape-orientation page. The slides should succinctly capture all essential elements of the application and should stand alone.

#### **11.4.7. Scientific Plan (Ten pages)**

Present the rationale behind the proposed product or service, emphasizing the pressing problem in cancer care that will be addressed. Summarize the evidence gathered to date in support of the company's ideas. Describe the label claims that the company ultimately hopes to make and describe the plan to gather evidence to support these claims. Outline the steps to be taken during the proposed period of the award, including the design of the translational or clinical research, methods, and anticipated results. Describe potential problems or pitfalls and alternative approaches. If clinical research is proposed, present a realistic plan to accrue a sufficient number of human subjects meeting the inclusion criteria within the proposed time period.

**The scientific plan submitted must be of sufficient depth and quality to pass rigorous scrutiny by the highly qualified group of reviewers. To the extent possible, the scientific plan should be driven by data. In the past, applications that have been scored poorly have been criticized for assuming that assertions could be taken on faith. Convincing data are much preferred.**

#### **11.4.8. Business Plan (Fifteen pages)**

Provide a business plan covering all of the topics below in the order shown. Successful applicants will make thoughtful, careful, and economical use of the limited space. Note that if the company is selected to undergo due diligence, information to support a full intellectual property review will be requested at that time. Company Relocation Product Development Award applicants will be evaluated based not only on the current status of the components of the business plan but also on whether current weaknesses and gaps are acknowledged and whether plans to address them are outlined.

**A. Introduction:** Present the rationale behind the proposed project, emphasizing the

pressing problem in cancer care that will be addressed. Describe the label claims that the company ultimately hopes to make, and briefly describe the plan to gather evidence to support these claims. Include the minimum level of detail required to provide a context for the rest of the business plan. Cross-reference sections in the scientific plan where further details may be found.

- B. Products and Markets:** Provide a brief description of the envisioned product and how the product will be administered to patients. Describe the initial market that will be targeted and how the envisioned product will fit within the standard of care.
- C. Regulatory Plans:** Provide a detailed regulatory plan, including preclinical and clinical activities, driven by interactions with the FDA, if possible. Summarize all interactions to date with the FDA.
- D. Risk Analysis:** Describe the specific risks inherent to the product plan and how they would be mitigated.
- E. Current and Pending Support:** Describe all funding sources. Provide a complete and detailed capitalization table, which should include all parties who have investments, stock, or rights in the company. The identities of all parties must be listed. It is not appropriate to list any funding source as anonymous.
- F. Financial Projections:** Provide a detailed source and use analysis of the development plan, focusing on the achievement of specific milestones.
- G. Resources Requested:** Include resources needed for research and product development and for any relocation expenses. The matching funds amount should be included in this section; however, this is the only section of the business plan that does not deal exclusively with CPRIT-requested funds.
- H. Scope of Work and Milestones:** Outline the specific goals of the project. Provide an outline of anticipated major milestones to be tracked. Timelines will be reviewed for reasonableness, and adherence to timelines will be a criterion for continued support of successful applications. If the application is approved for funding, this

section will be included in the award contract.

**I. Key Personnel Located in Texas and Any Key Management Located Outside of Texas:** For each member of the senior management and scientific team, provide a paragraph briefly summarizing his or her present title and position, prior industry experience, education, and any other information considered essential for evaluation of qualifications.

**J. Organizational Commitment to Texas:** Describe how CPRIT funding of the applicant's company would benefit the State of Texas. For example, describe how the company would create high-quality new jobs in the State and/or recruit out-of-State talent, and mention any Texas-based subcontractors and suppliers that would be used and any other unique, Texas-based resources that would be leveraged.

#### **11.4.9. Biographical Sketches of Key Scientific Personnel (Eight pages)**

Provide a biographical sketch for up to four key scientific personnel that describes their education and training, professional experience, awards and honors, and publications relevant to cancer research. Each biographical sketch must not exceed two pages and must use the "Product Development Programs: Biographical Sketch" template.

(In addition, information on the members of the senior management and scientific team should be included in the "Key Personnel" section of the Business Plan [see [Section 11.4.8](#)]).

#### **11.4.10. Budget and Justification**

Provide a compelling justification of the budget for the entire proposed period of support, including salaries and benefits, supplies, equipment, patient care costs, animal care costs, and other expenses. The budget must be aligned with the proposed milestones. In preparing the requested budget, Applicants should be aware of the following:

- Equipment having a useful life of more than 1 year and an acquisition cost of \$5,000 or more per unit must be specifically approved by CPRIT. An Applicant does not need to seek this approval prior to submitting the application.



- Texas State law limits the amount of grant funds that may be spent on indirect costs to no more than 5 percent of the total award amount (5.263 percent of the direct costs). Guidance regarding indirect cost recovery can be found in CPRIT's administrative rules, which are available at [www.cprit.state.tx.us](http://www.cprit.state.tx.us).
- The annual salary that an individual may receive under a CPRIT award for FY 2013 is \$200,000. In other words, an individual may request salary proportional to the percentage effort up to a maximum of \$200,000. Salary does not include fringe benefits. CPRIT FY 2013 is from September 1, 2012, through August 31, 2013.

## **12. AWARD ADMINISTRATION**

Texas law requires that CPRIT awards be made by contract between the applicant and CPRIT. CPRIT grant awards are made to entities, not to individuals. Award contract negotiation and execution will commence once the CPRIT Oversight Committee has approved an application for a grant award. CPRIT may require, as a condition of receiving a grant award, that the grant recipient use CPRIT's electronic Grant Management System to exchange, execute, and verify legally binding grant contract documents and grant award reports. Such use shall be in accordance with CPRIT's electronic signature policy as set forth in Chapter 701, Section 701.25.

Texas law specifies several components that must be addressed by the award contract, including needed compliance and assurance documentation, budgetary review, progress and fiscal monitoring, and terms relating to revenue sharing and intellectual property rights. These contract provisions are specified in CPRIT's Administrative Rules, which are available at [www.cprit.state.tx.us](http://www.cprit.state.tx.us). Applicants are advised to review CPRIT's Administrative Rules related to contractual requirements associated with CPRIT grant awards and limitations related to the use of CPRIT grant awards as set forth in Chapter 703, Sections 703.10 - 703.12.

Prior to disbursement of grant award funds, the grant recipient organization must demonstrate that it has adopted and enforces a tobacco-free workplace policy consistent with the requirements set forth in CPRIT's Administrative Rules, Chapter 703, Section 703.20.

CPRIT requires award recipients to submit an annual progress report. These reports summarize the progress made toward the research goals and address plans for the upcoming year. In addition, fiscal reporting, human studies reporting, and vertebrate animal use reporting will be required as appropriate. Continuation of funding is contingent upon the timely receipt of these reports. Failure to provide timely and complete reports may waive reimbursement of grant award costs, and may result in the termination of award contract. Forms and instructions will be made available at [www.cprit.state.tx.us](http://www.cprit.state.tx.us).

**Project Economics Sharing:** Recipients should also be aware that the funding award contract will include a revenue-sharing agreement and will require CPRIT to have input on any future patents, agreements, or other financial arrangements related to the products, services, or infrastructure supported by the CPRIT investment. These contract provisions are specified in CPRIT's Administrative Rules, which are available at [www.cprit.state.tx.us](http://www.cprit.state.tx.us).

### **13. REQUIREMENT TO DEMONSTRATE AVAILABLE FUNDS**

Texas State law requires that prior to disbursement of CPRIT grant funds, the award recipient demonstrate that it has \$1.00 in matching funds for every \$2.00 from CPRIT. Matching funds need not be in hand when the application is submitted. However, matching funds must be obtained before CPRIT funds will be released for use. CPRIT funds must, whenever possible, be spent in Texas. A company's matching funds must be designated for the CPRIT-funded project but may be spent outside of Texas. Grant applicants are advised to consult CPRIT's Administrative Rules, Chapter 703, Section 703.11 for specific requirements associated with the requirement to demonstrate available funds.

### **14. CONTACT INFORMATION**

#### **14.1. HelpDesk**

HelpDesk support is available for questions regarding user registration and online submission of applications. Queries submitted via e-mail will be answered within

1 business day. HelpDesk staff are not in a position to answer questions regarding scientific and commercialization aspects of applications. **Before contacting the HelpDesk, please refer to the “Instructions for Applicants” document, which provides a step-by-step guide on using the Application Receipt System.**

**Dates of operation:** December 23, 2013 to January 31, 2014 (excluding public holidays)

**Hours of operation:** Monday, Tuesday, Thursday, Friday, 7 a.m. to 4 p.m. Central Time  
Wednesday, 8 a.m. to 4 p.m. Central Time

**Tel:** 866-941-7146

**E-mail:** [Help@CPRITGrants.org](mailto:Help@CPRITGrants.org)

#### **14.2. Programmatic Questions**

Questions regarding the CPRIT Program, including questions regarding this or any other funding opportunity, should be directed to the CPRIT Research Program Director.

**Tel:** 512-305-8486

**E-mail:** [Help@CPRITGrants.org](mailto:Help@CPRITGrants.org)

**Web site:** [www.cprit.state.tx.us](http://www.cprit.state.tx.us)



**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**

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**MEMORANDUM**

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**TO:** OVERSIGHT COMMITTEE MEMBERS  
**FROM:** DAVID A. REISMAN, CHIEF COMPLIANCE OFFICER  
**SUBJECT:** COMPLIANCE CERTIFICATION:  
PRODUCT DEVELOPMENT AWARD SLATES  
**DATE:** FEBRUARY 10, 2014

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**Summary and Recommendation:**

As CPRIT's chief compliance officer, I am responsible for reporting to the Oversight Committee regarding the agency's compliance with applicable statutory and administrative rule requirements during the grant review process. I have reviewed the compliance pedigrees for the grant applications for Product Development awards. I have conferred with staff at CPRIT and SRA International (SRA), CPRIT's contracted third-party grant administrator, and studied the supporting grant review documentation, including third-party observer reports for the peer review meetings. I am satisfied that the application review process that resulted in three Product Development award slates recommended by the Chief Executive Officer, which included the Company Product Development Awards, the Company Formation Awards, and the Company Relocation Awards, all followed applicable laws and agency administrative rules. I certify these award slates for the Oversight Committee's consideration.

**Background:**

Newly enacted statutory changes require that CPRIT employ a Chief Compliance Officer to report to the Oversight Committee regarding compliance with the statute and the agency's administrative rules. Among the Chief Compliance Officer's responsibilities is the obligation "to ensure that all grant proposals comply with this chapter and rules adopted under this chapter before the proposals are submitted to the oversight committee for approval." TEX HEALTH & SAFETY CODE §102.051(c) and (d).

Although the statutory requirement is new, CPRIT began using a compliance pedigree process to formally document compliance for the grant awards announced in December 2012. The compliance pedigree tracks the grant application as it moves through the review process and documents compliance with applicable laws and administrative rules. A compliance pedigree is created for each application; the information related to the procedural steps listed on the pedigree is entered and attested to by SRA employees and CPRIT employees. To the greatest extent possible, information reported in the compliance pedigree is imported directly from data contained in CPRIT's Application Receipt System (CARS), the grant application database managed by SRA. This is done to minimize the opportunity for error caused by manual data entry.

The compliance pedigree and supporting documentation is reviewed by the compliance officer as part of the award slate certification process. You have received a compliance pedigree for each of the applications recommended for a grant award through the grant portal and in the hard copy materials delivered to you. The compliance pedigree for product development grants is divided into seven categories that reflect the seven stages of review. A brief description of each category and information tracked in the category is provided below.

### **Pre-Receipt Compliance:**

The activities listed in pre-receipt stage cover the period beginning with CPRIT's issuance of the Request for Application (RFA) through the submission of grant applications. CPRIT's administrative rules require that RFAs be publicly posted in the *Texas Register*. The RFA specifies a deadline and mandates that only those applications submitted electronically through CARS are eligible for consideration. CARS blocks an application from being submitted once the deadline passes. Occasionally an applicant may have technical difficulties that prevent the applicant from completing the application submission. When this occurs, the applicant may appeal to CPRIT (through the CPRIT Helpdesk that is managed by SRA) to allow for a submission after the deadline. The program officer considers any appeals and may approve a late filing for good cause. When a late filing request is approved, the appellee is notified and CARS is reopened for a brief period – usually two to three hours – the next business day.

*In this cycle, three of the applicants appealed and were granted permission to submit the applications on August 29, 2012, the day after the deadline. The applications were submitted and reviewed.*

### **Receipt, Referral, and Assignment Compliance:**

Once product development applications have been submitted through CARS, SRA staff reviews the applications for compliance with RFA directions. If an applicant does not comply with the directions, SRA notifies the program officer and the program officer makes the final decision to administratively withdraw the application. The program officer and the Review Council Chair assign applications to peer review panels and primary reviewers. Prior to distribution of the applications, reviewers are given summary information about the applicant, including the Project Director and collaborators. Reviewers must sign a conflict of interest agreement and confirm that they do not have a conflict of interest with the application before they are provided with the full application.

*In this cycle, the application pedigrees indicate that a conflict of interest agreement was signed by each reviewer for each of the applications. None of the applications were administratively withdrawn prior to review for non-compliance.*

**Peer Review Screening Conference:**

At this stage, the entire review panel meets to discuss the preliminary reviews and scores. The review panel determines which applicants should be invited to proceed to the next stage of the review. For applicants that are not invited to the in-person presentation, the application process ends here.

*The application pedigrees were reviewed and showed that a non-primary reviewer in four of the applicant peer reviews indicated a conflict of interest and were recused from participation. This was cross-checked and verified with the sign-out sheets with those perspective teleconferences. The application pedigrees reflect that all applicants were invited to proceed to the next stage of the process.*

**Peer Review On-site Meeting:**

At this stage, applicants that proceed past Peer Review Screening present the proposal to the entire review panel. Following the presentation, the panel discusses the application and each panel member provides an overall score for the application. The pedigree reflects the date(s) of the on-site meeting and whether the application was submitted for due diligence and intellectual property review.

*The application pedigrees were reviewed and showed that a non-primary reviewer in four of the applicant peer reviews indicated a conflict of interest and were recused from participation. This was cross-checked and verified with the sign-out sheets with those perspective teleconferences. The application pedigrees showed that all applicants were invited to advance to the due diligence and IP review stage of the process.*

**Due Diligence Review:**

Applications are reviewed by outside legal counsel to identify any intellectual property concerns. The applicant and the project also undergo management and company due diligence by a third party. These reports are provided to the review panel. The pedigree reflects the date of the intellectual property conflict check and the date the final intellectual property review was submitted.

*The applications were reviewed and cross-checked with the application pedigrees to verify that due diligence and an IP review were completed for each of the applications.*

**Final Product Development Review Council (PDRC) Recommendation:**

The PDRC considers the intellectual property and due diligence reports and creates a final list of applications that it recommends to CPRIT for funding. The Review Council's written recommendations include specific reasons for selecting applications, which are provided to both the Chief Executive Officer and the Oversight Committee. Once the awards are announced, the written recommendation is made public on CPRIT's website. The pedigree reflects the date of the PDRC meeting and whether the application was included on the slate of award recommendations.

*The pedigrees reflect that a PDRC member indicated a conflict of interest in two of the applications and was recused from participation. This was cross-checked with the sign out sheets for the teleconference. The pedigrees note that six applications were recommended for the slate.*

#### **Post Review:**

The statute in effect at the time that these grant applications were submitted requires the Product Development Review Council to submit the grant award recommendations to the Executive Director. The Executive Director then submits to the Oversight Committee the list of grant applications that is “substantially based” on the list submitted by the Review Council. The pedigree reflects the date that the Executive Director was notified of the grant award slates and whether the award was presented to the Oversight Committee.

*In this cycle, I reviewed the written notification submitted by Jack Geltosky, Chair, Product Development Review Council, to Wayne Roberts recommending six of the applications for grant awards. I compared the list of grant applications submitted to the Oversight Committee by Mr. Roberts with the list of applications the PDRC recommended for awards and confirmed that the recommendations are the same on both lists.*

#### **Other Information Reviewed:**

Oversight Committee Conflict of Interest Policy Statements - Prior to receiving grant applicant information, the Oversight Committee members reviewed, signed, and returned CPRIT’s conflict of interest policy statement to CPRIT. In addition, prior to viewing grant applicant information, the Oversight Committee members confirmed that they did not have a conflict of interest requiring recusal with any of the applications recommended for grant awards

Third-Party Observer Reports - In May 2012, CPRIT implemented the use of an independent third-party observer at peer review meetings to ensure that panel discussions are limited to the merits of the application and adhere to established evaluation criteria. In addition, the third-party observer reports whether CPRIT staff attending the peer review meeting participates in the discussion, scoring or vote on the grant application. CPRIT staff may attend peer review meetings, but may not participate in the review process other than to answer technical questions. The third-party reviewer is the agency’s internal auditor, Grant Thornton. I have reviewed the third-party observer reports for the peer review meetings for this cycle. Nothing unusual was reported. The reports are attached.



# CPRIT Peer Review Panel Observation Report

Panel Name: Commercialization Panel A, FY 13 Cycle 1 Screening

Panel Date: September 27, 2012

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Report Date: September 27, 2012

## **Background**

As part of CPRIT's on-going emphasis on continuous improvement in its grants review/management processes and to ensure that panel discussions are limited to the merits of the application and focused on the established evaluation criteria, CPRIT is implementing the use of a third-party observer at every in-person and telephone conference peer review meeting. CPRIT has authorized its out-sourced internal audit provider to function as a neutral third-party observer.

## **Introduction**

The subject of this report is the Commercialization Screening Peer Review Panel meeting chaired by Dr. Robert D. Ulrich and held via teleconference on September 27<sup>th</sup> 2012.

## **Panel Observation Objectives and Scope**

This internal audit follows the guidelines set forth by the Institute of Internal Auditors (IIA). The internal audit conforms to the Standards for the Professional Practice of Internal Auditing, the Code of Ethics contained in the Professional Practices Framework as promulgated by the Institute of Internal Auditors, and generally accepted government auditing standards.

The third-party observation was limited to observing whether the following objectives were met:

- CPRIT's established procedures for panelists who have declared a conflict of interest are followed during the meeting (e.g., reviewers leave room or do not participate in the telephone conference if they have a conflict);
- CPRIT program staff participation is limited to offering general points of information when asked by peer review panel members;
- CPRIT program staff do not engage in the panel's discussion on the merits of applications;





- The peer review panel discussion is focused on the established scoring criteria.

### **Observation Results Summary**

Internal Audit attended the Commercialization Screening Peer Review Panel meeting held via teleconference chaired by Dr. Robert D. Ulrich on September 27<sup>th</sup>, 2012. The meeting was facilitated by SRA International, CPRIT's contracted third-party grant application administrator.

Internal Audit noted the following during our observation:

- Twenty one commercialization applications were discussed and evaluated by the peer review panel over the course of three and a half hours.
- Sixteen attendees participated through conference call.
- There were six conflicts of interest identified for the applications. SRA asked the conflicted attendees to disconnect from the call during the discussion of conflicted applications.
- CPRIT program staff participation was limited to answering procedural questions and clarifying policies.
- SRA program staff did not participate in the discussions around the merits of the applications.
- The peer reviewers' discussions were limited to the application evaluation criteria.

### **Disclaimer**

The third-party observation did not include the following:

- An evaluation of the appropriateness or rigor of the peer review panel's discussion of scientific, technical or programmatic aspects of the applications.

Internal Audit was not engaged to and did not conduct an examination or review, the objective of which would be the expression of an opinion or limited assurance on the accuracy of voting and scoring. Accordingly, we will not express such an opinion or limited assurance. Had we performed additional procedures, other matters might have come to our attention that would have been reported to you.

This report is intended solely for the information and use of CPRIT and its management and its Oversight Committee members and is not intended to be and should not be used by anyone other than these specified parties.



# CPRIT Peer Review Panel Observation Report

Panel Name: CPRIT Commercialization Panel B, FY13 Cycle 2  
Screening Teleconference

Panel Date: December 3, 2012

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Report Date: December 3, 2012

## **Background**

As part of CPRIT's on-going emphasis on continuous improvement in its grants review/management processes and to ensure that panel discussions are limited to the merits of the application and focused on the established evaluation criteria, CPRIT is implementing the use of a third-party observer at every in-person and telephone conference peer review meeting. CPRIT has authorized its out-sourced internal audit provider to function as a neutral third-party observer.

## **Introduction**

The subject of this report is the Commercialization Screening Peer Review Panel meeting chaired by Dr. Jack Geltosky and held via teleconference on December 3<sup>rd</sup> 2012.

## **Panel Observation Objectives and Scope**

This internal audit follows the guidelines set forth by the Institute of Internal Auditors (IIA). The internal audit conforms to the Standards for the Professional Practice of Internal Auditing, the Code of Ethics contained in the Professional Practices Framework as promulgated by the Institute of Internal Auditors, and generally accepted government auditing standards.

The third-party observation was limited to observing whether the following objectives were met:

- CPRIT's established procedures for panelists who have declared a conflict of interest are followed during the meeting (e.g., reviewers leave room or do not participate in the telephone conference if they have a conflict);
- CPRIT program staff participation is limited to offering general points of information when asked by peer review panel members;



- CPRIT program staff do not engage in the panel's discussion on the merits of applications;
- The peer review panel discussion is focused on the established scoring criteria.

### **Observation Results Summary**

Internal Audit attended the Commercialization Screening Peer Review Panel meeting held via teleconference chaired by Dr. Jack Geltosky on December 3, 2012. The meeting was facilitated by SRA International, CPRIT's contracted third-party grant application administrator. The panel discussions focused on the applications that would be asked to present during the in-person review panels later on December 17, 2012.

Internal Audit noted the following during our observation:

- Nine commercialization applications were discussed and evaluated by the peer review panel over the course of two and a half hours.
- Nineteen attendees participated through conference call.
- There were two conflicts of interest identified for the applications. SRA moved one of the conflicted attendees into a separate conference call waiting room area during the discussion of the conflicted applications. The other conflicted reviewer was not moved into the conference call waiting room because no additional discussion took place for the application due to the high score of the application.
- CPRIT program staff participation was limited to answering procedural questions and clarifying policies.
- SRA program staff did not participate in the discussions around the merits of the applications.
- The peer reviewers' discussions were limited to the application evaluation criteria.

### **Disclaimer**

The third-party observation did not include the following:

- An evaluation of the appropriateness or rigor of the peer review panel's discussion of scientific, technical or programmatic aspects of the applications.

Internal Audit was not engaged to and did not conduct an examination or review, the objective of which would be the expression of an opinion or limited assurance on the accuracy of voting and scoring. Accordingly, we will not express such an opinion or limited assurance. Had we performed additional procedures, other matters might have come to our attention that would have been reported to you.

This report is intended solely for the information and use of CPRIT and its management and its Oversight Committee members and is not intended to be and should not be used by anyone other than these specified parties.



# CPRIT Peer Review Panel Observation Report

Panel Name: Commercialization Program Peer Review FY13 Cycle  
2

Panel Date: December 17, 2012

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Report Date: December 17, 2012

## **Background**

As part of CPRIT's on-going emphasis on continuous improvement in its grants review/management processes and to ensure that panel discussions are limited to the merits of the application and focused on the established evaluation criteria, CPRIT is implementing the use of a third-party observer at every in-person and telephone conference peer review meeting. CPRIT has authorized its out-sourced internal audit provider to function as a neutral third-party observer.

## **Introduction**

The subject of this report is the Commercialization Peer Review Panel meeting chaired by Jack Geltosky and held in person on December 17, 2012.

## **Panel Observation Objectives and Scope**

This internal audit follows the guidelines set forth by the Institute of Internal Auditors (IIA). The internal audit conforms to the Standards for the Professional Practice of Internal Auditing; the Code of Ethics contained in the Professional Practices Framework as promulgated by the Institute of Internal Auditors, and generally accepted government auditing standards.

The third-party observation was limited to observing whether the following objectives were met:

- CPRIT's established procedures for panelists who have declared a conflict of interest are followed during the meeting (e.g., reviewers leave room or do not participate in the telephone conference if they have a conflict);
- CPRIT program staff participation is limited to offering general points of information when asked by peer review panel members;
- CPRIT program staff do not engage in the panel's discussion on the merits of applications;



- The peer review panel discussion is focused on the established scoring criteria.

### **Observation Results Summary**

Internal Audit attended the Commercialization Peer Review Panel meeting held in person chaired by Jack Geltosky on December 17, 2012. The meeting was facilitated by SRA International, CPRIT's contracted third-party grant application administrator.

Internal Audit noted the following during our observation:

- Four commercialization applications were discussed and evaluated by the peer review panel in one day.
- Ten panelists attended and participated in person two others join by conference call.
- There were two conflicts of interest identified for the applications. SRA asked the conflicted attendee to step out during the discussion of conflicted application after signing the conflict of interest sign-out sheet. Internal Audit reviewed the sign-out sheet and verified that it was signed by the attendee.
- CPRIT program staff participation was limited to answering procedural questions and clarifying policies.
- SRA program staff did not participate in the discussions around the merits of the applications.
- The peer reviewers' discussions were limited to the application evaluation criteria.

### **Disclaimer**

The third-party observation did not include the following:

- An evaluation of the appropriateness or rigor of the peer review panel's discussion of scientific, technical or programmatic aspects of the applications.

Internal Audit was not engaged to and did not conduct an examination or review, the objective of which would be the expression of an opinion or limited assurance on the accuracy of voting and scoring. Accordingly, we will not express such an opinion or limited assurance. Had we performed additional procedures, other matters might have come to our attention that would have been reported to you.

This report is intended solely for the information and use of CPRIT and its management and its Oversight Committee members and is not intended to be and should not be used by anyone other than these specified parties.

# CPRIT Product Development Review Council Report

Panel Name: Product Development Review Council Meeting – Due Diligence (FY13.1 and FY13.2 Product Development applications)

Panel Date: January 13, 2014

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## **Background**

As part of CPRIT's on-going emphasis on continuous improvement in its grants review/management processes and to ensure that panel discussions are limited to the merits of the application and focused on the established evaluation criteria, CPRIT is implementing the use of a third-party observer at every in-person and telephone conference peer review meeting. CPRIT has authorized its out-sourced internal audit provider to function as a neutral third-party observer.

## **Introduction**

The subject of this report is the Product Development Review Council review of Due Diligence evaluations for FY13.1 and FY13.2 Product Development applications. The meeting was chaired by Jack Geltosky and held over the phone on January 13, 2014.

## **Panel Observation Objectives and Scope**

The third-party observation was limited to observing whether the following objectives were met:

- CPRIT's established procedures for panelists who have declared a conflict of interest are followed during the meeting (e.g., reviewers leave room or do not participate in the telephone conference if they have a conflict);
- CPRIT program staff participation is limited to offering general points of information when asked by peer review panel members;
- CPRIT program staff do not engage in the panel's discussion on the merits of applications;
- The peer review panel discussion is focused on the established scoring criteria.

## **Observation Results Summary**

Internal Audit participated in the Product Development Review Council meeting held telephonically and chaired by Jack Geltosky on January 13, 2014. The meeting was facilitated by SRA International, CPRIT's contracted third-party grant application administrator.

Internal Audit noted the following during our observation:

- Seven product development applications were discussed and evaluated by the Product Development Review Council to determine which grants would receive CPRIT funding.
- Four council members, three CPRIT staff members, and two SRA employees were present for the Council meeting over the phone.
- One conflict of interest was identified prior to or during the call. The council member with the conflict of interest left the teleconference and did not participate in the review of the conflicted applications.
- CPRIT program staff participation was limited to answering procedural questions and clarifying policies.
- SRA program staff did not participate in the discussions around the merits of the applications.
- The Council members' discussions were limited to the application evaluation criteria.

**Disclaimer**

The third-party observation did not include the following:

- An evaluation of the appropriateness or rigor of the peer review panel's discussion of scientific, technical or programmatic aspects of the applications.

Internal Audit was not engaged to and did not conduct an examination or review, the objective of which would be the expression of an opinion or limited assurance on the accuracy of voting and scoring. Accordingly, we will not express such an opinion or limited assurance. Had we performed additional procedures, other matters might have come to our attention that would have been reported to you.

This report is intended solely for the information and use of CPRIT and its management and its Oversight Committee members and is not intended to be and should not be used by anyone other than these specified parties.

**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**  
**APPLICATION PEDIGREE**

**FY** 2013  
**CYCLE** 1  
**PROGRAM** Product Development  
**AWARD MECHANISM** Company Commercialization Awards (COMP)  
**APPLICATION ID** CP130013  
  
**APPLICATION TITLE** Clinical Development and Commercialization of  
Oncolytic Adenovirus for Treating Malignant Glioma  
  
**APPLICANT NAME** Dr. Frank Tufaro  
**ORGANIZATION** DNAtrix, Inc.  
**PANEL NAME** Panel A-FY13-Cycle 1

Category	Compliance Requirement	Information	Attestation Date	Attesting Party*
<b>1. Pre-Receipt</b>	RFA posted in Texas Register	08/10/12	01/14/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) opened	08/02/12	01/09/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) closed	08/30/12	01/09/14	SRA International, Inc.
	Date application submitted	08/31/12	01/09/14	SRA International, Inc.
	Method of submission	CARS	01/09/14	SRA International, Inc.
	Within receipt period	NO	01/09/14	SRA International, Inc.
	Appeal to submit application after CARS closed	08/30/12	01/09/14	SRA International, Inc.
	Appeal for late application submission accepted	YES	01/09/14	SRA International, Inc.
<b>2. Receipt, Referral, and Assignment</b>	Administrative non-compliance notification	N/A	01/09/14	SRA International, Inc.
	Programmatic non-compliance notification	N/A	01/09/14	SRA International, Inc.
	Assigned to primary reviewers	09/09/12	01/09/14	SRA International, Inc.
	Primary Reviewer 1 COI signed	09/25/12	01/09/14	SRA International, Inc.
	Primary Reviewer 2 COI signed	09/11/12	01/09/14	SRA International, Inc.
	Primary Reviewer 3 COI signed	09/06/12	01/09/14	SRA International, Inc.
	Primary Reviewer 4 COI signed	09/06/12	01/09/14	SRA International, Inc.
<b>3. Peer Review: Screening Teleconference</b>	Primary Reviewer 1 critique submitted	10/06/12	01/09/14	SRA International, Inc.
	Primary Reviewer 2 critique submitted	10/03/12	01/09/14	SRA International, Inc.
	Primary Reviewer 3 critique submitted	10/03/12	01/09/14	SRA International, Inc.
	Primary Reviewer 4 critique submitted	10/03/12	01/09/14	SRA International, Inc.
	COI indicated by non-primary reviewer	NONE	01/09/14	SRA International, Inc.
	COI recused from participation	N/A	01/09/14	SRA International, Inc.
	Peer Review: Screening Teleconference	09/27/12	01/09/14	SRA International, Inc.
	Third Party Observer Report	09/27/12	01/15/14	CPRIT - K. Doyle
	Post review statements signed	10/10/12	01/09/14	SRA International, Inc.
<b>4. Peer Review: On-Site Meeting</b>	Recommended for On-Site Meeting	YES	01/09/14	SRA International, Inc.
	COI indicated by non-primary reviewer	NONE	01/09/14	SRA International, Inc.
	COI recused from participation	N/A	01/09/14	SRA International, Inc.
	Post review statements signed	10/30/12	01/09/14	SRA International, Inc.
	Third Party Observer Report	10/30/12	01/15/14	CPRIT - K. Doyle
	Peer Review: On-Site Meeting	10/29/12-10/30/12	01/09/14	SRA International, Inc.
<b>5. Moratorium</b>	Referred for due diligence and IP review	YES	01/09/14	SRA International, Inc.
	Updated information provided	11/04/13	01/09/14	SRA International, Inc.
<b>6. Due Diligence and IP Review</b>	Final due diligence review submitted	11/11/13	01/14/14	SRA International, Inc.
	Intellectual Property conflict check	11/13/12	01/15/14	CPRIT - K. Doyle
	Final intellectual property review submitted	01/09/14	01/15/14	CPRIT - K. Doyle
<b>7. Final PDRC Recommendation</b>	COI indicated by PDRC member	K. Dhingra	01/13/14	SRA International, Inc.
	COI recused from participation	YES	01/13/14	SRA International, Inc.
	Third Party Observer Report	01/13/14	01/16/14	CPRIT - K. Doyle
	PDRC Meeting	01/13/14	01/13/14	SRA International, Inc.
	Recommended for slate	YES	01/13/14	SRA International, Inc.
<b>8. CEO Recommendation</b>	Notification to CEO of PDRC recommendation	01/15/14	01/15/14	CPRIT - K. Doyle
	Recommended for slate by CEO	YES	01/15/14	CPRIT - K. Doyle
<b>9. Oversight Committee Ratification</b>	CEO Notification to Oversight Committee	01/16/14	01/16/14	CPRIT - K. Doyle
	COI indicated by Oversight Committee member	NAME or NONE	Pending	
	COI recused from participation	YES/NO or N/A	Pending	
	Presented to CPRIT Oversight Committee	DATE	Pending	
	Award Ratified by Oversight Committee	YES/NO	Pending	
	Advance Funds Approved by Oversight Committee	YES/NO or N/A	Pending	

\*The identity of the attesting individual is retained by CPRIT



**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**  
**APPLICATION PEDIGREE**

**FY** 2013  
**CYCLE** 1  
**PROGRAM** Product Development  
**AWARD MECHANISM** Company Relocation Awards (RELO)  
**APPLICATION ID** CP130020  
  
**APPLICATION TITLE** Androgen Receptor N-Terminus Blocker Program for Prostate Cancer: Relocation Grant Application  
  
**APPLICANT NAME** Mr. Robert W Rieder  
**ORGANIZATION** ESSA Pharma Inc.  
**PANEL NAME** Panel A-FY13-Cycle 1

Category	Compliance Requirement	Information	Attestation Date	Attesting Party*
<b>1. Pre-Receipt</b>	RFA posted in Texas Register	08/10/12	01/14/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) opened	08/02/12	01/09/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) closed	08/30/12	01/09/14	SRA International, Inc.
	Date application submitted	08/30/12	01/09/14	SRA International, Inc.
	Method of submission	CARS	01/09/14	SRA International, Inc.
	Within receipt period	YES	01/09/14	SRA International, Inc.
	Appeal to submit application after CARS closed	N/A	01/09/14	SRA International, Inc.
	Appeal for late application submission accepted	N/A	01/09/14	SRA International, Inc.
<b>2. Receipt, Referral, and Assignment</b>	Administrative non-compliance notification	N/A	01/09/14	SRA International, Inc.
	Programmatic non-compliance notification	N/A	01/09/14	SRA International, Inc.
	Assigned to primary reviewers	09/09/12	01/09/14	SRA International, Inc.
	Primary Reviewer 1 COI signed	09/07/12	01/09/14	SRA International, Inc.
	Primary Reviewer 2 COI signed	09/14/12	01/09/14	SRA International, Inc.
	Primary Reviewer 3 COI signed	09/07/12	01/09/14	SRA International, Inc.
	Primary Reviewer 4 COI signed	09/22/12	01/09/14	SRA International, Inc.
<b>3. Peer Review: Screening Teleconference</b>	Primary Reviewer 1 critique submitted	10/03/12	01/09/14	SRA International, Inc.
	Primary Reviewer 2 critique submitted	10/03/12	01/09/14	SRA International, Inc.
	Primary Reviewer 3 critique submitted	10/03/12	01/09/14	SRA International, Inc.
	Primary Reviewer 4 critique submitted	10/03/12	01/09/14	SRA International, Inc.
	COI indicated by non-primary reviewer	R. McCloskey	01/09/14	SRA International, Inc.
	COI recused from participation	YES	01/09/14	SRA International, Inc.
	Peer Review: Screening Teleconference	09/27/12	01/09/14	SRA International, Inc.
	Third Party Observer Report	09/27/12	01/15/14	CPRIT - K. Doyle
	Post review statements signed	10/10/12	01/09/14	SRA International, Inc.
<b>4. Peer Review: On-Site Meeting</b>	Recommended for On-Site Meeting	YES	01/09/14	SRA International, Inc.
	COI indicated by non-primary reviewer	R. McCloskey	01/09/14	SRA International, Inc.
	COI recused from participation	YES	01/09/14	SRA International, Inc.
	Post review statements signed	10/30/12	01/09/14	SRA International, Inc.
	Third Party Observer Report	10/30/12	01/15/14	CPRIT - K. Doyle
	Peer Review: On-Site Meeting	10/29/12-10/30/12	01/09/14	SRA International, Inc.
	Referred for due diligence and IP review	YES	01/09/14	SRA International, Inc.
<b>5. Moratorium</b>	Updated information provided	11/04/13	01/09/14	SRA International, Inc.
<b>6. Due Diligence and IP Review</b>	Final due diligence review submitted	11/11/13; 1/6/14	01/14/14	SRA International, Inc.
	Intellectual Property conflict check	11/13/12	01/15/14	CPRIT - K. Doyle
	Final intellectual property review submitted	01/09/14	01/15/14	CPRIT - K. Doyle
<b>7. Final PDRC Recommendation</b>	COI indicated by PDRC member	NONE	01/13/14	SRA International, Inc.
	COI recused from participation	N/A	01/13/14	SRA International, Inc.
	Third Party Observer Report	01/13/14	01/16/14	CPRIT - K. Doyle
	PDRC Meeting	01/13/14	01/13/14	SRA International, Inc.
	Recommended for slate	YES	01/13/14	SRA International, Inc.
<b>8. CEO Recommendation</b>	Notification to CEO of PDRC recommendation	01/15/14	01/15/14	CPRIT - K. Doyle
	Recommended for slate by CEO	YES	01/15/14	CPRIT - K. Doyle
<b>9. Oversight Committee Ratification</b>	CEO Notification to Oversight Committee	01/16/14	01/16/14	CPRIT - K. Doyle
	COI indicated by Oversight Committee member	NAME or NONE	Pending	
	COI recused from participation	YES/NO or N/A	Pending	
	Presented to CPRIT Oversight Committee	DATE	Pending	
	Award Ratified by Oversight Committee	YES/NO	Pending	
	Advance Funds Approved by Oversight Committee	YES/NO or N/A	Pending	

\*The identity of the attesting individual is retained by CPRIT

**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**  
**APPLICATION PEDIGREE**

**FY** 2013  
**CYCLE** 1  
**PROGRAM** Product Development  
**AWARD MECHANISM** Company Formation Awards (FORM)  
**APPLICATION ID** CP130023  
**APPLICATION TITLE** Novel Ceramide-modulating Therapeutics for Cancer Use  
**APPLICANT NAME** Mr. Richard Love  
**ORGANIZATION** CerRx, Inc.  
**PANEL NAME** Panel A-FY13-Cycle 1

Category	Compliance Requirement	Information	Attestation Date	Attesting Party*
<b>1. Pre-Receipt</b>	RFA posted in Texas Register	08/10/12	01/14/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) opened	08/02/12	01/09/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) closed	08/30/12	01/09/14	SRA International, Inc.
	Date application submitted	08/30/12	01/09/14	SRA International, Inc.
	Method of submission	CARS	01/09/14	SRA International, Inc.
	Within receipt period	YES	01/09/14	SRA International, Inc.
	Appeal to submit application after CARS closed	N/A	01/09/14	SRA International, Inc.
	Appeal for late application submission accepted	N/A	01/09/14	SRA International, Inc.
<b>2. Receipt, Referral, and Assignment</b>	Administrative non-compliance notification	N/A	01/09/14	SRA International, Inc.
	Programmatic non-compliance notification	N/A	01/09/14	SRA International, Inc.
	Assigned to primary reviewers	09/09/12	01/09/14	SRA International, Inc.
	Primary Reviewer 1 COI signed	09/12/12	01/09/14	SRA International, Inc.
	Primary Reviewer 2 COI signed	09/07/12	01/09/14	SRA International, Inc.
	Primary Reviewer 3 COI signed	09/11/12	01/09/14	SRA International, Inc.
<b>3. Peer Review: Screening Teleconference</b>	Primary Reviewer 4 COI signed	09/22/12	01/09/14	SRA International, Inc.
	Primary Reviewer 1 critique submitted	09/29/12	01/09/14	SRA International, Inc.
	Primary Reviewer 2 critique submitted	10/03/12	01/09/14	SRA International, Inc.
	Primary Reviewer 3 critique submitted	10/03/12	01/09/14	SRA International, Inc.
	Primary Reviewer 4 critique submitted	10/03/12	01/09/14	SRA International, Inc.
	COI indicated by non-primary reviewer	NONE	01/09/14	SRA International, Inc.
	COI recused from participation	N/A	01/09/14	SRA International, Inc.
	Peer Review: Screening Teleconference	09/27/12	01/09/14	SRA International, Inc.
	Third Party Observer Report	09/27/12	01/15/14	CPRIT - K. Doyle
	Post review statements signed	10/10/12	01/09/14	SRA International, Inc.
<b>4. Peer Review: On-Site Meeting</b>	Recommended for On-Site Meeting	YES	01/09/14	SRA International, Inc.
	COI indicated by non-primary reviewer	NONE	01/09/14	SRA International, Inc.
	COI recused from participation	N/A	01/09/14	SRA International, Inc.
	Post review statements signed	10/30/12	01/09/14	SRA International, Inc.
	Third Party Observer Report	10/30/12	01/15/14	CPRIT - K. Doyle
	Peer Review: On-Site Meeting	10/29/12-10/30/12	01/09/14	SRA International, Inc.
<b>5. Moratorium</b>	Referred for due diligence and IP review	YES	01/09/14	SRA International, Inc.
	Updated information provided	11/04/13	01/09/14	SRA International, Inc.
<b>6. Due Diligence and IP Review</b>	Final due diligence review submitted	11/11/13	01/14/14	SRA International, Inc.
	Intellectual Property conflict check	11/13/12	01/15/14	CPRIT - K. Doyle
	Final intellectual property review submitted	01/09/14	01/15/14	CPRIT - K. Doyle
<b>7. Final PDRC Recommendation</b>	COI indicated by PDRC member	K. Dhingra	01/13/14	SRA International, Inc.
	COI recused from participation	YES	01/13/14	SRA International, Inc.
	Third Party Observer Report	01/13/14	01/16/14	CPRIT - K. Doyle
	PDRC Meeting	01/13/14	01/13/14	SRA International, Inc.
	Recommended for slate	YES	01/13/14	SRA International, Inc.
<b>8. CEO Recommendation</b>	Notification to CEO of PDRC recommendation	01/15/14	01/15/14	CPRIT - K. Doyle
	Recommended for slate by CEO	YES	01/15/14	CPRIT - K. Doyle
<b>9. Oversight Committee Ratification</b>	CEO Notification to Oversight Committee	01/16/14	01/16/14	CPRIT - K. Doyle
	COI indicated by Oversight Committee member	NAME or NONE		
	COI recused from participation	YES/NO or N/A		
	Presented to CPRIT Oversight Committee	DATE		
	Award Ratified by Oversight Committee	YES/NO		
	Advance Funds Approved by Oversight Committee	YES/NO or N/A		

\*The identity of the attesting individual is retained by CPRIT

**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**  
**APPLICATION PEDIGREE**

**FY** 2013  
**CYCLE** 1  
**PROGRAM** Product Development  
**AWARD MECHANISM** Company Commercialization Awards (COMP)  
**APPLICATION ID** CP130050  
**APPLICATION TITLE** Real-time Nerve Identification for Robotic Surgery  
**APPLICANT NAME** Mr. James Stone  
**ORGANIZATION** ProPep Surgical  
**PANEL NAME** Panel A-FY13-Cycle 1

Category	Compliance Requirement	Information	Attestation Date	Attesting Party*
<b>1. Pre-Receipt</b>	RFA posted in Texas Register	08/10/12	01/14/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) opened	08/02/12	01/09/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) closed	08/30/12	01/09/14	SRA International, Inc.
	Date application submitted	08/31/12	01/09/14	SRA International, Inc.
	Method of submission	CARS	01/09/14	SRA International, Inc.
	Within receipt period	NO	01/09/14	SRA International, Inc.
	Appeal to submit application after CARS closed	08/30/12	01/09/14	SRA International, Inc.
	Appeal for late application submission accepted	YES	01/09/14	SRA International, Inc.
<b>2. Receipt, Referral, and Assignment</b>	Administrative non-compliance notification	N/A	01/09/14	SRA International, Inc.
	Programmatic non-compliance notification	N/A	01/09/14	SRA International, Inc.
	Assigned to primary reviewers	09/09/12	01/09/14	SRA International, Inc.
	Primary Reviewer 1 COI signed	09/16/12	01/09/14	SRA International, Inc.
	Primary Reviewer 2 COI signed	09/07/12	01/09/14	SRA International, Inc.
	Primary Reviewer 3 COI signed	09/25/12	01/09/14	SRA International, Inc.
	Primary Reviewer 4 COI signed	09/06/12	01/09/14	SRA International, Inc.
<b>3. Peer Review: Screening Teleconference</b>	Primary Reviewer 1 critique submitted	10/03/12	01/09/14	SRA International, Inc.
	Primary Reviewer 2 critique submitted	10/03/12	01/09/14	SRA International, Inc.
	Primary Reviewer 3 critique submitted	10/08/12	01/09/14	SRA International, Inc.
	Primary Reviewer 4 critique submitted	10/03/12	01/09/14	SRA International, Inc.
	COI indicated by non-primary reviewer	R. McCloskey	01/09/14	SRA International, Inc.
	COI recused from participation	YES	01/09/14	SRA International, Inc.
	Peer Review: Screening Teleconference	09/27/12	01/09/14	SRA International, Inc.
	Third Party Observer Report	09/27/12	01/15/14	CPRIT - K. Doyle
	Post review statements signed	10/10/12	01/09/14	SRA International, Inc.
<b>4. Peer Review: On-Site Meeting</b>	Recommended for On-Site Meeting	YES	01/09/14	SRA International, Inc.
	COI indicated by non-primary reviewer	R. McCloskey	01/09/14	SRA International, Inc.
	COI recused from participation	YES	01/09/14	SRA International, Inc.
	Post review statements signed	10/30/12	01/09/14	SRA International, Inc.
	Third Party Observer Report	10/30/12	01/15/14	CPRIT - K. Doyle
	Peer Review: On-Site Meeting	10/29/12-10/30/12	01/09/14	SRA International, Inc.
	Referred for due diligence and IP review	YES	01/09/14	SRA International, Inc.
<b>5. Moratorium</b>	Updated information provided	11/01/13	01/09/14	SRA International, Inc.
<b>6. Due Diligence and IP Review</b>	Final due diligence review submitted	11/11/13	01/14/14	SRA International, Inc.
	Intellectual Property conflict check	11/13/12	01/15/14	CPRIT - K. Doyle
	Final intellectual property review submitted	01/09/14	01/15/14	CPRIT - K. Doyle
<b>7. Final PDRC Recommendation</b>	COI indicated by PDRC member	NONE	01/13/14	SRA International, Inc.
	COI recused from participation	N/A	01/13/14	SRA International, Inc.
	Third Party Observer Report	01/13/14	01/16/14	CPRIT - K. Doyle
	PDRC Meeting	01/13/14	01/13/14	SRA International, Inc.
	Recommended for slate	YES	01/13/14	SRA International, Inc.
<b>8. CEO Recommendation</b>	Notification to CEO of PDRC recommendation	01/15/14	01/15/14	CPRIT - K. Doyle
	Recommended for slate by CEO	YES	01/15/14	CPRIT - K. Doyle
<b>9. Oversight Committee Ratification</b>	CEO Notification to Oversight Committee	01/16/14	01/16/14	CPRIT - K. Doyle
	COI indicated by Oversight Committee member	NAME or NONE		
	COI recused from participation	YES/NO or N/A		
	Presented to CPRIT Oversight Committee	DATE		
	Award Ratified by Oversight Committee	YES/NO		
	Advance Funds Approved by Oversight Committee	YES/NO or N/A		

\*The identity of the attesting individual is retained by CPRIT

**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**  
**APPLICATION PEDIGREE**

**FY** 2013  
**CYCLE** 2  
**PROGRAM** Product Development  
**AWARD MECHANISM** Company Formation Awards (FORM)  
**APPLICATION ID** CP130058  
**APPLICATION TITLE** Developing to Clinical Proof of Concept Drugs that Inhibit a Novel Cancer Cell Target  
**APPLICANT NAME** Mr. Jonathan Northrup  
**ORGANIZATION** Beta Cat Pharmaceuticals, LLC  
**PANEL NAME** Panel B-FY13-Cycle 2

Category	Compliance Requirement	Information	Attestation Date	Attesting Party*
<b>1. Pre-Receipt</b>	RFA posted in Texas Register	11/02/12	01/14/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) opened	10/25/12	01/09/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) closed	11/15/12	01/09/14	SRA International, Inc.
	Date application submitted	11/15/12	01/09/14	SRA International, Inc.
	Method of submission	CARS	01/09/14	SRA International, Inc.
	Within receipt period	YES	01/09/14	SRA International, Inc.
	Appeal to submit application after CARS closed	N/A	01/09/14	SRA International, Inc.
<b>2. Receipt, Referral, and Assignment</b>	Appeal for late application submission accepted	N/A	01/09/14	SRA International, Inc.
	Administrative non-compliance notification	N/A	01/09/14	SRA International, Inc.
	Programmatic non-compliance notification	N/A	01/09/14	SRA International, Inc.
	Assigned to primary reviewers	11/19/12	01/09/14	SRA International, Inc.
	Primary Reviewer 1 COI signed	09/12/12	01/09/14	SRA International, Inc.
	Primary Reviewer 2 COI signed	11/12/12	01/09/14	SRA International, Inc.
<b>3. Peer Review: Screening Teleconference</b>	Primary Reviewer 3 COI signed	11/19/12	01/09/14	SRA International, Inc.
	Primary Reviewer 1 critique submitted	12/04/12	01/09/14	SRA International, Inc.
	Primary Reviewer 2 critique submitted	12/03/12	01/09/14	SRA International, Inc.
	Primary Reviewer 3 critique submitted	12/02/12	01/09/14	SRA International, Inc.
	COI indicated by non-primary reviewer	NONE	01/09/14	SRA International, Inc.
	COI recused from participation	N/A	01/09/14	SRA International, Inc.
	Peer Review: Screening Teleconference	12/03/12	01/09/14	SRA International, Inc.
	Third Party Observer Report	12/03/12	01/15/14	CPRIT - K. Doyle
	Post review statements signed	12/10/12	01/09/14	SRA International, Inc.
<b>4. Peer Review: On-Site Meeting</b>	Recommended for On-Site Meeting	YES	01/09/14	SRA International, Inc.
	COI indicated by non-primary reviewer	NONE	01/09/14	SRA International, Inc.
	COI recused from participation	N/A	01/09/14	SRA International, Inc.
	Post review statements signed	12/20/12	01/09/14	SRA International, Inc.
	Third Party Observer Report	12/17/12	01/15/14	CPRIT - K. Doyle
	Peer Review: On-Site Meeting	12/17/12	01/09/14	SRA International, Inc.
<b>5. Moratorium</b>	Referred for due diligence and IP review	YES	01/09/14	SRA International, Inc.
	Updated information provided	11/04/13	01/09/14	SRA International, Inc.
<b>6. Due Diligence and IP Review</b>	Final due diligence review submitted	01/03/14	01/14/14	SRA International, Inc.
	Intellectual Property conflict check	12/02/13	01/15/14	CPRIT - K. Doyle
	Final intellectual property review submitted	01/09/14	01/15/14	CPRIT - K. Doyle
<b>7. Final PDRC Recommendation</b>	COI indicated by PDRC member	NONE	01/13/14	SRA International, Inc.
	COI recused from participation	N/A	01/13/14	SRA International, Inc.
	Third Party Observer Report	01/13/14	01/16/14	CPRIT - K. Doyle
	PDRC Meeting	01/13/14	01/13/14	SRA International, Inc.
	Recommended for slate	YES	01/13/14	SRA International, Inc.
<b>8. CEO Recommendation</b>	Notification to CEO of PDRC recommendation	01/15/14	01/15/14	CPRIT - K. Doyle
	Recommended for slate by CEO	YES	01/15/14	CPRIT - K. Doyle
<b>9. Oversight Committee Ratification</b>	CEO Notification to Oversight Committee	01/16/14	01/16/14	CPRIT - K. Doyle
	COI indicated by Oversight Committee member	NAME or NONE		
	COI recused from participation	YES/NO or N/A		
	Presented to CPRIT Oversight Committee	DATE		
	Award Ratified by Oversight Committee	YES/NO		
<b>9. Oversight Committee Ratification</b>	Advance Funds Approved by Oversight Committee	YES/NO or N/A		

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**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**  
**APPLICATION PEDIGREE**

**FY** 2013  
**CYCLE** 2  
**PROGRAM** Product Development  
**AWARD MECHANISM** Company Relocation Awards (RELO)  
**APPLICATION ID** CP130066  
**APPLICATION TITLE** Phase II Clinical Studies for a First in Class Bcl-2 Cancer Drug  
**APPLICANT NAME** Dr. Richard A Messmann  
**ORGANIZATION** ProNAi Therapeutics, Inc.  
**PANEL NAME** Panel B-FY13-Cycle 2

Category	Compliance Requirement	Information	Attestation Date	Attesting Party*
<b>1. Pre-Receipt</b>	RFA posted in Texas Register	11/02/12	01/14/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) opened	10/25/12	01/09/14	SRA International, Inc.
	CPRIT Application Receipt System (CARS) closed	11/15/12	01/09/14	SRA International, Inc.
	Date application submitted	11/16/12	01/09/14	SRA International, Inc.
	Method of submission	CARS	01/09/14	SRA International, Inc.
	Within receipt period	NO	01/09/14	SRA International, Inc.
	Appeal to submit application after CARS closed	11/15/12	01/09/14	SRA International, Inc.
	Appeal for late application submission accepted	YES	01/09/14	SRA International, Inc.
<b>2. Receipt, Referral, and Assignment</b>	Administrative non-compliance notification	N/A	01/09/14	SRA International, Inc.
	Programmatic non-compliance notification	N/A	01/09/14	SRA International, Inc.
	Assigned to primary reviewers	11/19/12	01/09/14	SRA International, Inc.
	Primary Reviewer 1 COI signed	11/19/12	01/09/14	SRA International, Inc.
	Primary Reviewer 2 COI signed	09/18/12	01/09/14	SRA International, Inc.
	Primary Reviewer 3 COI signed	11/19/12	01/09/14	SRA International, Inc.
<b>3. Peer Review: Screening Teleconference</b>	Primary Reviewer 1 critique submitted	12/02/12	01/09/14	SRA International, Inc.
	Primary Reviewer 2 critique submitted	11/27/12	01/09/14	SRA International, Inc.
	Primary Reviewer 3 critique submitted	11/28/12	01/09/14	SRA International, Inc.
	COI indicated by non-primary reviewer	N. Clendeninn	01/09/14	SRA International, Inc.
	COI recused from participation	YES	01/09/14	SRA International, Inc.
	Peer Review: Screening Teleconference	12/03/12	01/09/14	SRA International, Inc.
	Third Party Observer Report	12/03/12	01/15/14	CPRIT - K. Doyle
	Post review statements signed	12/10/12	01/09/14	SRA International, Inc.
<b>4. Peer Review: On-Site Meeting</b>	Recommended for On-Site Meeting	YES	01/09/14	SRA International, Inc.
	COI indicated by non-primary reviewer	N. Clendeninn	01/09/14	SRA International, Inc.
	COI recused from participation	YES	01/09/14	SRA International, Inc.
	Post review statements signed	12/20/12	01/09/14	SRA International, Inc.
	Third Party Observer Report	12/17/12	01/15/14	CPRIT - K. Doyle
	Peer Review: On-Site Meeting	12/17/12	01/09/14	SRA International, Inc.
<b>5. Moratorium</b>	Referred for due diligence and IP review	YES	01/09/14	SRA International, Inc.
	Updated information provided	11/04/13	01/09/14	SRA International, Inc.
<b>6. Due Diligence and IP Review</b>	Final due diligence review submitted	01/09/14	01/09/14	SRA International, Inc.
	Intellectual Property conflict check	11/27/13	01/15/14	CPRIT - K. Doyle
	Final intellectual property review submitted	01/07/14	01/15/14	CPRIT - K. Doyle
<b>7. Final PDRC Recommendation</b>	COI indicated by PDRC member	NONE	01/13/14	SRA International, Inc.
	COI recused from participation	N/A	01/13/14	SRA International, Inc.
	Third Party Observer Report	01/13/14	01/16/14	CPRIT - K. Doyle
	PDRC Meeting	01/13/14	01/13/14	SRA International, Inc.
	Recommended for slate	YES	01/13/14	SRA International, Inc.
<b>8. CEO Recommendation</b>	Notification to CEO of PDRC recommendation	01/15/14	01/15/14	CPRIT - K. Doyle
	Recommended for slate by CEO	YES	01/15/14	CPRIT - K. Doyle
<b>9. Oversight Committee Ratification</b>	CEO Notification to Oversight Committee	01/16/14	01/16/14	CPRIT - K. Doyle
	COI indicated by Oversight Committee member	NAME or NONE		
	COI recused from participation	YES/NO or N/A		
	Presented to CPRIT Oversight Committee	DATE		
	Award Ratified by Oversight Committee	YES/NO		
	Advance Funds Approved by Oversight Committee	YES/NO or N/A		

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## CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS

### PRODUCT DEVELOPMENT REVIEW PROCESS DESCRIPTION

CPRIT is authorized to award grants to support cancer research activities conducted by public or private entities in Texas. Research activities supported by CPRIT fall into two general categories: "basic" and "translational". Basic research leads to actual discoveries. Translational research advances basic science discoveries toward practical applications that improve patient outcomes. CPRIT's Product Development program supports research activities that span the translational research continuum, including preclinical studies, proof of concept, and Phase I and Phase II clinical trials. The end goal is improved cancer diagnostics, devices, therapeutics, and treatment protocols that are available to improve cancer patient care, quality of life, and longevity.

CPRIT has created a Product Development Review Process to identify opportunities that will benefit through CPRIT Product Development grant funding. It is expected that in addition to CPRIT's primary goals, this form of funding also benefits the state through job creation, financial return on investment and other economic benefits such as relocation of companies to Texas.

CPRIT utilizes a multi-step peer review process to evaluate Product Development applications. The peer reviewers are a group of qualified experts from the areas of academia, clinical medicine, investments and management with experience relevant to cancer. All reviewers live and work outside of Texas. Working as a team, the reviewers jointly evaluate applications in their respective areas of expertise and experience. In order to alternate the workload, there are currently two Product Development Review Panels, each conducting approximately two review cycles per year.

**Three to four times a year, requests for applications (RFAs) for Product Development cancer projects are posted on the CPRIT website.** RFAs are open for submission of applications for approximately one month. The application submission and peer review processes are administered by SRA, International, a CPRIT contractor with extensive experience in grants review

There are three categories of Product Development grant awards: "Company Formation" awards for startup companies that have not completed a professional round of fund-raising, "Company Product Development" awards for established companies that have completed at least one round of professional fundraising, and "Company Relocation" for startups or established companies that intend to relocate to Texas. Applicants choose which category of Product Development application is appropriate for their situation. These categories help the Product Development reviewers better focus on the relevant information unique to each category.

**The first application review is administrative** and is conducted by the SRA staff. The administrative review takes about a week to complete. This initial administrative review confirms that an application has



all the elements requested in the RFA and that all other submission requirements are met. Applications that pass administrative review are forwarded to the Chair of the Product Development Review Panel and move into the second stage of evaluation.

**In the second stage of evaluation, Review Panel members are provided abstracts for all applications that passed administrative review.** Each Product Development reviewer advises the Product Development Review Panel Chair which applications fall within their area of experience, expertise and interest. Any potential conflict of interest is also reported at this time so that the reviewer is excluded from any evaluation, discussion and vote related to the identified application(s). Using this information, the Chairman assigns three to four primary reviewers to each application. Each reviewer is given multiple applications to review. Reviewers evaluate each assigned application through an online system, by submitting a written critique and numeric scores based on review criteria specified in the RFA. These scores range from 1 (excellent) to 9 (extremely poor). An overall score is also assigned to each application.

Once all applications have been initially scored (approximately five to six weeks after the RFAs are closed), the Product Development reviewers and the Chair meet via teleconference for approximately one-half day to compare notes, discuss the applications, assigned scores, and make additional comments. The assigned reviewers may adjust scores based on these discussions. The entire review panel decides which applications move to the next stage of evaluation. The conference call is set up and moderated by SRA staff to ensure that conflict of interest procedural requirements are followed. The Product Development Officer and other CPRIT staff may also attend the teleconference. However, CPRIT staff participation is limited to answering procedural questions. CPRIT staff do not participate in the substantive discussion, scoring or vote for any applications.

All applicants are provided reviewer feedback on their application, regardless of their score. **Those applicants with sufficiently positive scores as determined by the Product Development reviewers move into the third stage of evaluation and are invited to present their proposal, in-person, to the entire Product Development Review Panel** approximately three to four weeks later. Applicants that are invited to make in-person presentations are provided a list of questions that the Product Development reviewers want to have specifically addressed regarding their application.

At the in-person meeting, each applicant is given twenty minutes to make their presentation to the review panel. This is followed by twenty minutes for reviewer questions. At the conclusion of the Q&A session, the applicant is excused from the room. The reviewers discuss the application and all reviewers individually submit an overall score for the application. The reviewers' discussion and scoring may take up to 45 minutes. Regardless of score, all applicants receive feedback from this stage of evaluation.

**Those applications that the reviewers feel score sufficiently well after the in-person presentation move into the fourth stage of evaluation -- due diligence review.** Due diligence is conducted by outside contractors hired by CPRIT and is overseen by the Chief Product Development Officer. These contractors conduct in-depth evaluations in the areas of intellectual property, clinical trial design, regulatory affairs, manufacturability of product, marketing, etc. Due diligence review takes 45 - 60 days to complete. A draft

report is sent to the applicant for comment. Once the applicant comments are received, a final report is sent to the Primary Reviewers and the Product Development Review Council (the four senior members of the Review Panels) for their consideration.

**During the fifth and final stage of evaluation by the review panel, the Product Development Review Council and the primary reviewers hold a teleconference to discuss the due diligence results.** A final recommendation is then made by the reviewers regarding whether the application should be recommended for CPRIT grant funding. All Product Development applications recommended for grant funding are ranked by the reviewers and submitted by the Product Development Chair to CPRIT's Program Integration Committee (PIC) for consideration.

**The PIC considers the Product Development grant recommendations and, after deliberation, presents a list of recommended grant awards to the CPRIT Oversight Committee for final approval.** The time between the Product Development Chair's submission of the grant recommendations to the PIC and the final approval or rejection by the Oversight Committee is dependent upon the meeting schedules for the PIC and the Oversight Committee.

Once the Product Development grants are approved, negotiations begin between the company and CPRIT's Chief Product Development Officer regarding the revenue-sharing terms to be included in the grant funding contract. CPRIT funding is typically provided in stages and tied to the achievement of specific milestones. CPRIT's return on investment can be in the form of equity, royalties, or a combination of both. Whether or not an applicant sufficiently achieves milestones to continue funding is monitored by the Chief Product Development Officer and determined by Product Development reviewers and the Product Development Review Council.





## CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS

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### MEMORANDUM

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**TO:** OVERSIGHT COMMITTEE MEMBERS  
**FROM:** KRISTEN PAULING DOYLE, GENERAL COUNSEL  
**SUBJECT:** AGENDA ITEM #6 – PRODUCT DEVELOPMENT AWARDS  
**DATE:** JANUARY 23, 2014

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#### Summary and Recommendation

Disbursement of grant funds requires ratification of the grant award recommendation by the Oversight Committee and a signed grant award contract. I recommend that the Oversight Committee delegate contract negotiation authority to the CEO and the general counsel for any product development grant recommendation ratified by the Oversight Committee. The delegation of authority to the CEO to execute the negotiated award contracts should be subject to the Oversight Committee approval of the project-specific contractual terms. To facilitate review and approval of the contract terms, the Oversight Committee may wish to task the Product Development subcommittee with the responsibility for recommending contract approval to the Oversight Committee.

#### Contract Process Overview

State law requires grant funding recommendations to be ratified by the Oversight Committee as the final step in the application review process. However, approval of the grant recommendation does not entitle an applicant to grant funds. The statute is clear that a grant is awarded by signing a written grant contract. Disbursement of grant funds is contingent upon a final contract.

The statutory bifurcation of the grant recommendation approval and award contract is meaningful. The statute lays out several issues that must be included and agreed to in the award contract, including revenue sharing terms. Therefore, it is possible that a project may be approved by the Oversight Committee for CPRIT grant funding, but the grant is never awarded (and grant funds are not disbursed) because the applicant cannot agree to CPRIT's contractual terms. If no agreement is reached, the contract is not executed and grant funds set aside for the project are released.

The statute directs that "the Oversight Committee shall negotiate on behalf of the state regarding awarding, by grant, money under this chapter." It has been the standard practice for the committee to approve a motion delegating contract negotiation authority to CPRIT's CEO and general counsel and authorizing the CEO to execute the award contract following the ratification of the grant recommendations by the Oversight Committee.

CPRIT makes an award contract available to the grantee via the electronic grants management system. CPRIT uses a template grant contract that encompasses general state contracting terms as well as CPRIT-specific requirements. CPRIT also incorporates attachments to the contract that are customizable by the grantee and/or by program. The contractual attachments are:

- Attachment A – Scope of Work, Goals, and Timelines (grantee specific)
- Attachment B – Approved Budget (grantee specific)
- Attachment C – Certifications (including the matching funds certification for scientific and product development research awards) (program specific)
- Attachment D – Intellectual Property and Revenue Sharing (grantee specific for product development awards, standard form for academic research and prevention grants)
- Attachment E – Reporting Requirements (program specific)
- Attachment F – Contract Amendments (contract specific)

Grantees are asked to complete Attachment A, Attachment B, and Attachment C (for matching funds certification). The attachments must be reviewed and approved by CPRIT programmatic and fiscal staff before the grant contract is executed by CPRIT's CEO.

### **Contract Process for Product Development**

For product development grant projects, CPRIT ties the disbursement of grant funds to the achievement of defined milestones that are specified in the grant contract in Attachment A. Each slice of funding, commonly known as a tranche, and its associated objective or deliverable are negotiated and included in the award contract.

Tranching adds complexity, both to contract negotiation and to contract monitoring, but it is an effective way to limit CPRIT's risk exposure. Although the total award amount for the project must be ratified by the Oversight Committee, the grantee receives only enough grant funds to accomplish the specified milestones within the particular tranche. The company must demonstrate successful completion through a written report detailing how the company has achieved the goals tied to a specific tranche in order to access the next amount of grant funding. Expert reviewers assess the work done by the company and approve the release of the next tranche of funding or recommend that funding be terminated.

Tranches for the grant project are developed using deal-specific documentation, including:

- Information supplied by the applicant. Applicants are asked to provide specific goals and associated timelines for the proposed project in the application. The aims and timeline are evaluated during the review process and the reviewers may indicate a change to be included in the contract or an issue to be negotiated.
- Information from the due diligence review. Icon, the company that performs due diligence reviews of CPRIT's product development applications, provides guidance on appropriate milestones to be achieved during the course of the project.
- Information from the intellectual property review. IP counsel may provide recommendations regarding specific steps to be taken regarding protecting IP, ensuring freedom to operate, or cleaning up problematic licensing agreements.

Icon, IP counsel, or the Review Council may identify an issue that if not corrected or adequately addressed prior to contract, could be a reason for CPRIT not executing the contract. Although this is not technically a tranche recommendation, this information impacts the contract negotiations. For example, the IP and licensing review may identify an issue with the license agreement for the underlying technology that, if not resolved, is a deal breaker. CPRIT will direct the company to fix the underlying licensing issue (usually through renegotiation of the underlying licensing agreement) before contract negotiation with CPRIT can begin.

Another important negotiation point is the agreed form, amount, and timing of revenue sharing payments to CPRIT. Like the funding tranches, revenue sharing terms are deal specific and are negotiated to address the particular project.

### **Oversight Committee Contract Review and Approval Prior to Final Execution**

I recommend that the Oversight Committee delegate contract negotiation authority to CPRIT's CEO and general counsel, and contract execution authority to the CEO contingent upon approval by the Oversight Committee of the final contract. This process is consistent with the statutory directive that the Oversight Committee negotiate award contracts. The CEO and general counsel may be assisted by outside IP counsel, consultants, and Product Development Review Council members for contract negotiation.

The Oversight Committee's review of the deal-specific terms can be led by the Product Development subcommittee. The subcommittee can assess the proposed terms negotiated by the CEO and general counsel and recommend Oversight Committee approval at the next Oversight Committee. The approval process may add some time to the contract execution, but it is not an unreasonable burden.



# **CPRIT** Product Development Portfolio

February 2014



CANCER PREVENTION & RESEARCH  
INSTITUTE OF TEXAS

# Why Product Development?

## Patient Impact

- Improve patient care
- Lower barriers to personalized treatment and clinical practice for treating cancer
- Enhance opportunities for breakthrough cancer-related technologies

## Economic Development

- Create high quality new jobs in Texas
- Generate the critical mass, infrastructure capabilities required for a sustainable cancer industry in Texas

## Direct Return on Investment

- State shares in the economic benefits of Investments
- Milestone Payments
- Royalty Payments
- Equity
- Other Mechanisms

# 90% of all drug development candidates fail to make it to market

## Time

- 15 years to develop lab discovery into drug patient can use
- Takes 6 months to 2 years to go through FDA regulatory approval process

## Money

- \$1 billion to bring one new therapy from the lab to the patient

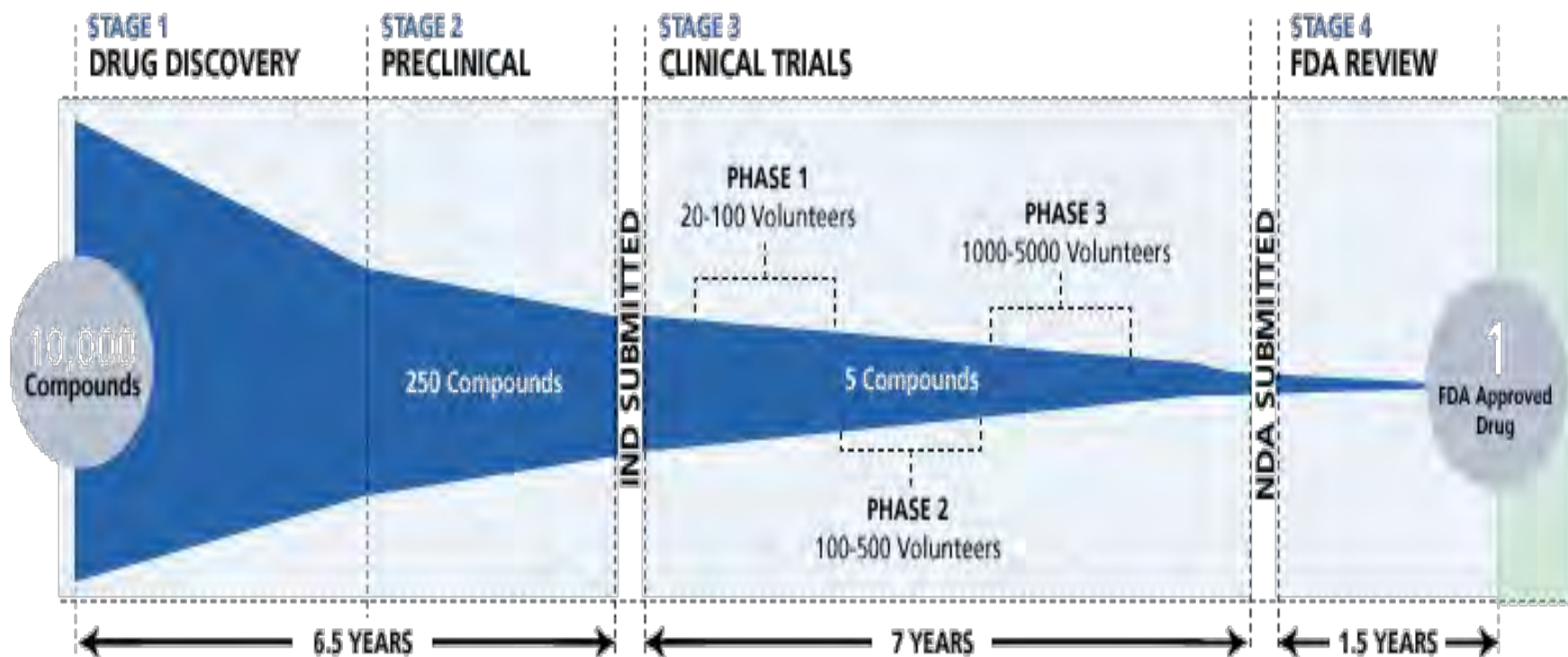
## Testing

- 80 – 90% of preclinical research fails before human testing
- 40% of cancer clinical trials don't accrue enough patients
- 80% of drugs fail in Phase I
- 50% of drugs in Phase III become drugs

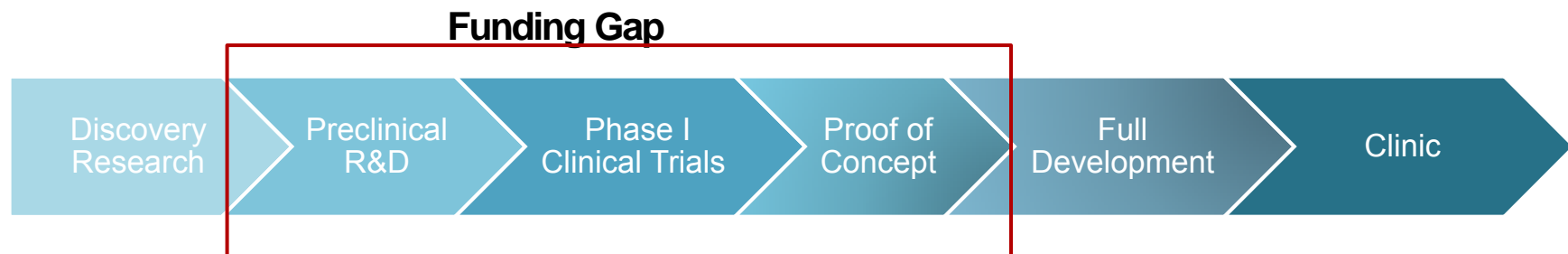


# Drug Development Continuum

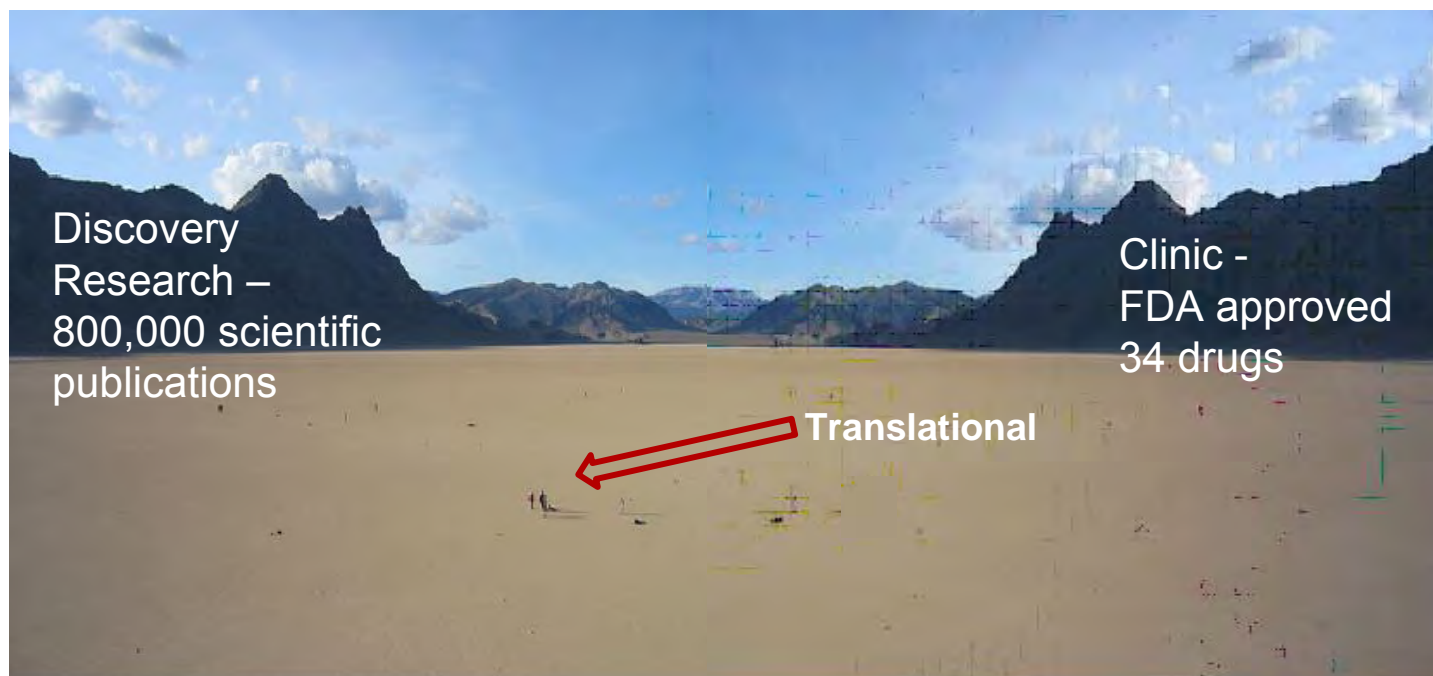
One approved drug  
for every 10,000 compounds that enter drug discovery



# The Gap CPRIT Can Address



CPRIT funding at critical stages supports data development to advance the program, attract more funds





# Product Development Candidates

## Eligibility

- Texas-based entity at time of contract
- Matching funds (50%) at funding
- Bulk of funds must be expended in Texas
- Investment award via negotiated contract with milestones and tranche funding

## Stages of Development

- Translational Research
- Proof of Concept-  
Proof of Relevance
- Pre-Clinical
- Clinical
- Phase I
- Phase II
- Phase III with limitations

## Products and Services

- Therapeutics (e.g. Small Molecules and Biologics)
- Diagnostics (e.g. Biomarkers, Personalized therapy)
- Devices
- Potential Breakthrough Technologies
  - Software
  - Research/Discovery Techniques
  - Other

# CPRIT Product Development Portfolio

Company	CPRIT Investment	Funded Project	Follow-on Capital
Apollo Endosurgery, Inc. (Austin)	\$ 5,001,063	Flexible endoscopic surgical tools for better removal of flat polyps	\$130 million
Asuragen, Inc. (Austin)	\$ 6,837,265	Diagnostic gene-sequencing clinical test to identify cancer mutations	\$ 15 million
Bellicum Pharmaceuticals, Inc. (Houston)	\$ 5,680,310	Combination product to rapidly resolve Graft vs. Host Disease	\$ 34 million
Caliber Biotherapeutics, Inc (College Station)	\$12,808,151	Monoclonal antibody therapy	\$10.4 million
Cell Medica, Inc. (Houston)	\$15,571,303	Cellular immunotherapy therapies for cancers associated w/ Epstein Barr virus and for CMV infections w/ bone marrow transplants	\$ 11 million
InGeneron, Inc. (Houston)	\$ 198,111	Portable imaging device to visualize cell markers	\$2.4 million
Kalon Biotherapeutics, LLC (College Station)	\$ 7,901,420	Drug manufacturing technology capability for Phase I and II clinical trials	\$3.95 million
Mirna Therapeutics, Inc. (Austin)	\$10,297,454	Drug development to treat liver cancer	\$36.9 million
Molecular Templates, Inc. (Georgetown)	\$10,600,000	Drug development to treat Non-Hodgkin's Lymphoma	\$10 million
Peloton Therapeutics, (Dallas)	\$11,044,931	Drug development	\$30.6 million
Pulmotect, Inc. (Houston)	\$ 7,126,398	Boost immune system of lungs, helping to prevent/treat pneumonia	\$3.56 million
Rules-Based Medicine (Austin)	\$ 3,024,432	Diagnostic tool identifying cancer biomarkers	\$81 million
Visualase, Inc. (Houston)	\$ 2,151,776	Laser technology to precisely target prostate tumors	\$5.2 million

# CPRIT Portfolio Performance

\$98 million

Total CPRIT Investment

\$374 million

Total Follow-on Capital

# Product Development Review Process

## **Product Development Review Council**

Jack Geltosky, PhD (Chair)

Roy Cosan, Kapil Dhingra, MD, and David Shoemaker, PhD

22 expert reviewers and 4 advocate reviewers for FY14 Cycle 1

CPRIT funds projects at critical stages to develop enough data to attract more funds to advance the program

- Funded project must be:
  - Well grounded in basic science
  - Based on a sensible product development plan
  - Managed by capable, experienced executives
  - Protected by solid intellectual property
- CPRIT funding fosters intelligent risk taking, which feeds innovation
- CPRIT diligence is well-regarded in the venture community
- Endorsement makes it much easier for companies to attract more money

# Product Development Applications: FY 2014 Cycle 1

43 applications seeking \$408,792,719

Submission Date: January 31

Review Dates: February 28, April 1

Due Diligence: May - June

10

Established  
Companies

6

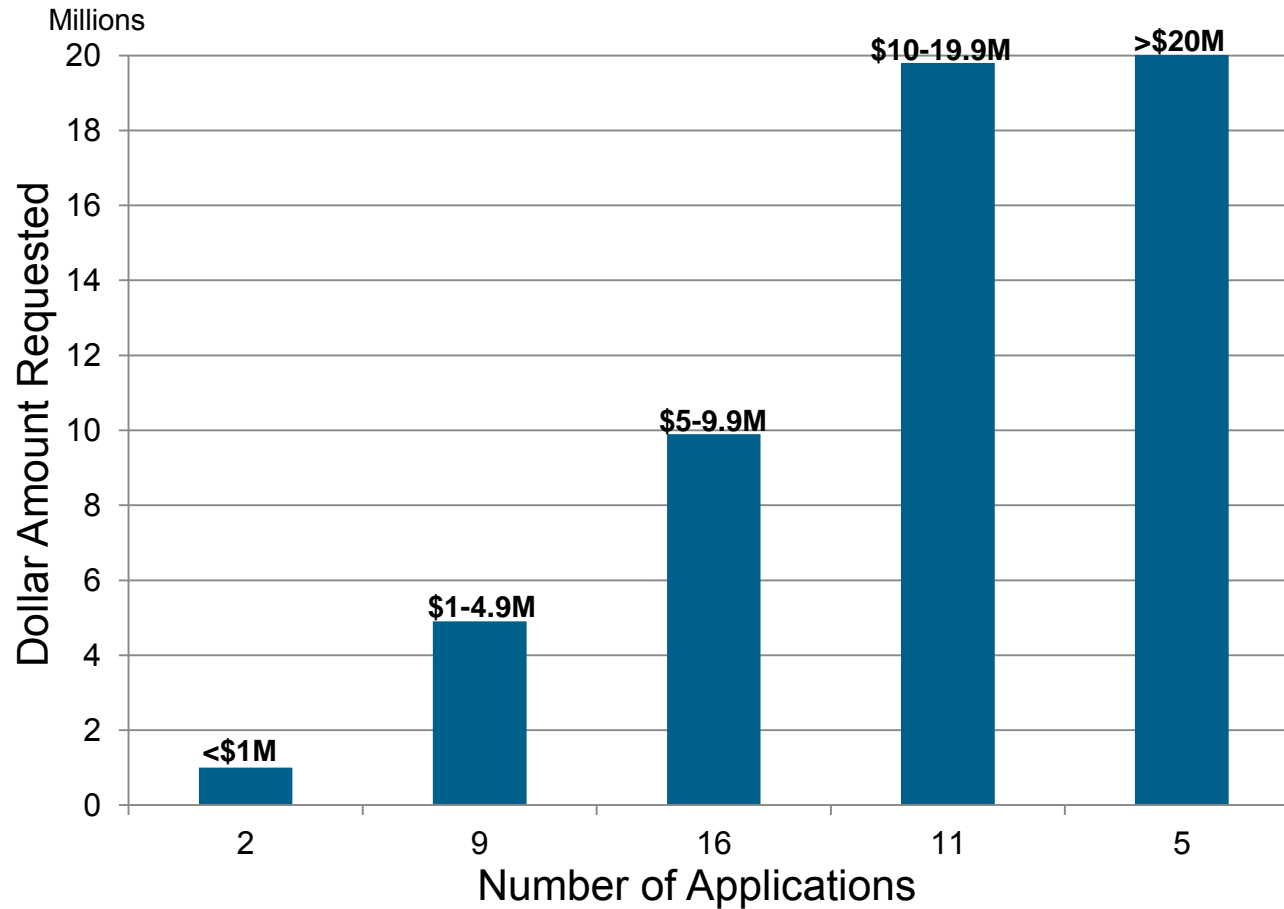
Company  
Relocations

27

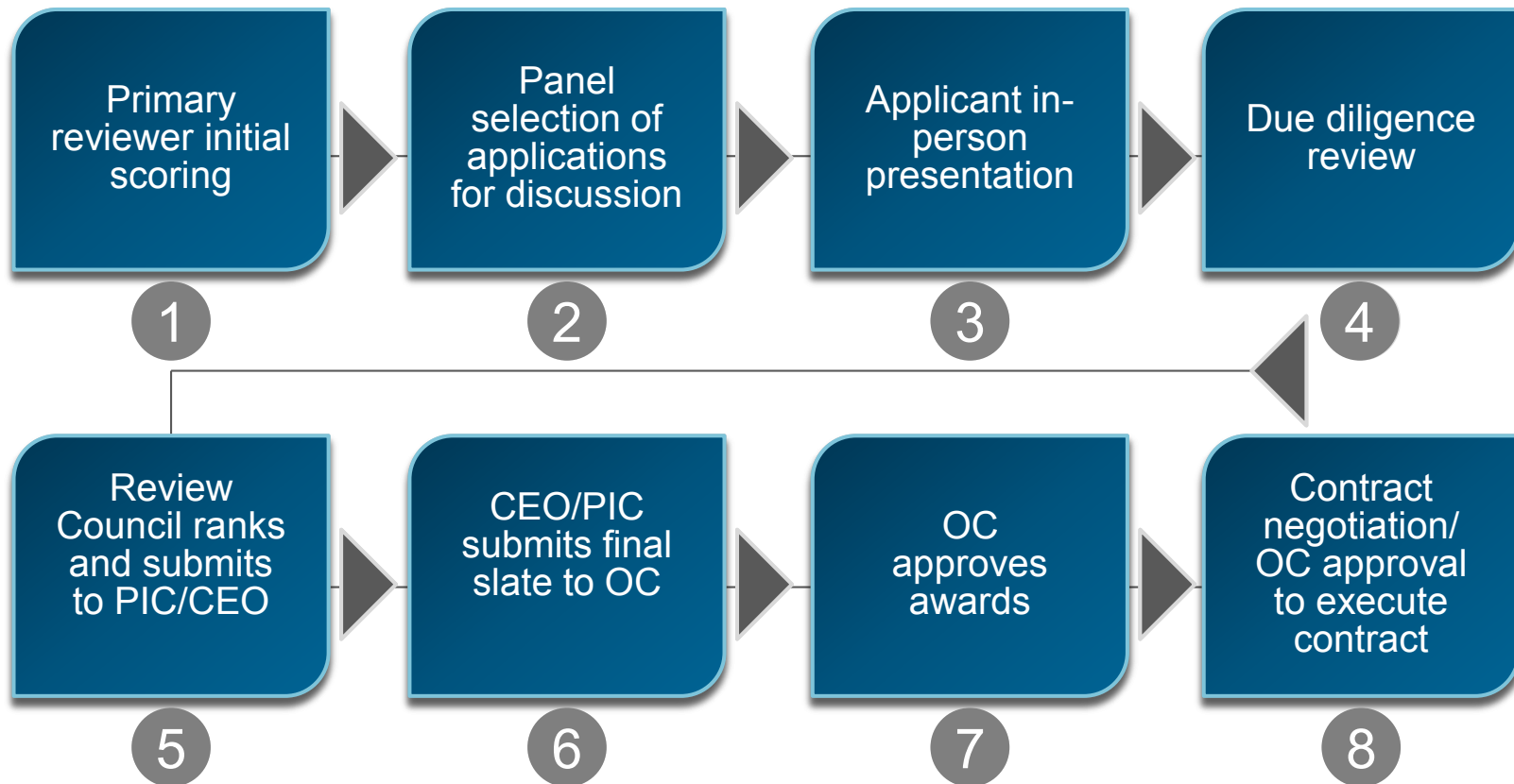
New  
Companies

16 companies from out-of-state

# FY 2014 Cycle 1 Funding Requests



# Product Development Review Process



# Contract Negotiation

## AWARD CONTRACT

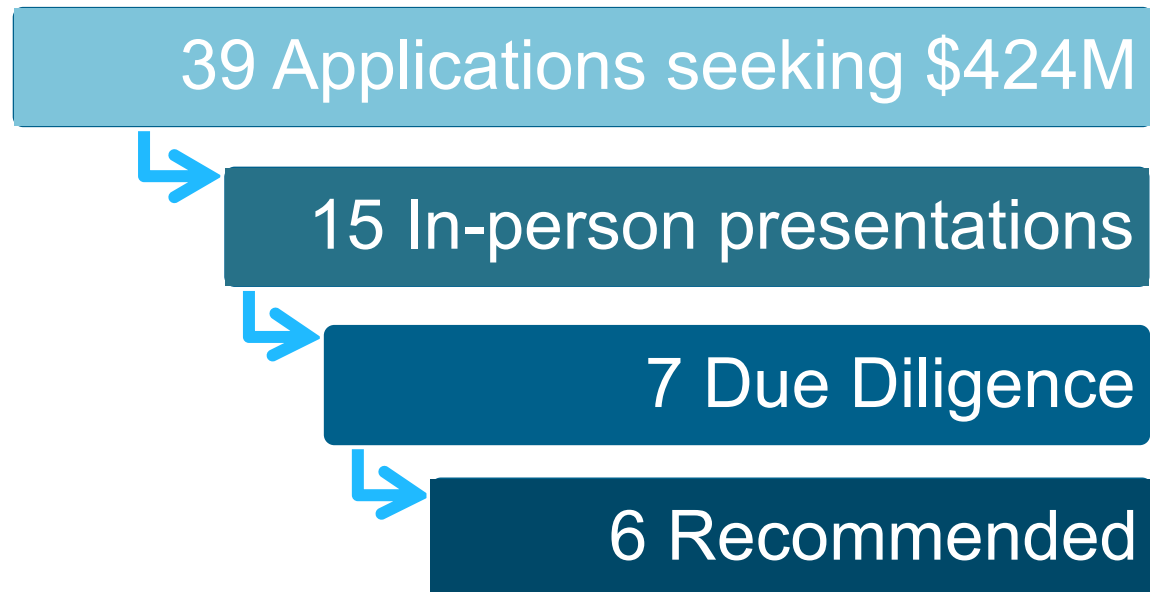
- Ratification of award recommendation does not entitle applicant to grant funds
- Award funds disbursed pursuant to executed award contract
- Terms to be negotiated in the award contract
  - Milestones – usually tied to funding tranches
  - Revenue Sharing Agreement (timing, royalties, equity, milestone payments)
- Tranche funding adds complexity to contract negotiation and monitoring, but limits CPRIT risk
- Grantee receives only enough award funds to accomplish specified milestones within tranche
- Must report detailed information about achievement of each milestone; approval required before next tranche of funds is released

## OVERSIGHT COMMITTEE ROLE

- Delegate contract negotiation authority to CEO, General Counsel
- CEO contract execution authority is contingent upon final approval of award contract by Oversight Committee; recommendation made by Product Dev Subcommittee



# FY 2013 Cycles 1 and 2



Submission Date: August 31, 2012 (Cycle 1)  
and November 15, 2012 (Cycle 2)

Updates following moratorium: November 11, 2013

# Company Commercialization Slate - \$15,249,480

Appl. ID	Title	Company Representative	Company	Total Recommended Budget
CP130013	Clinical Development and Commercialization of Oncolytic Adenovirus for Treating Malignant Glioma	Tufaro, Frank	DNatrix, Inc.	\$10,813,623
CP130050	Real-time Nerve Identification for Robotic Surgery	Stone, James	ProPep Surgical	\$ 4,435,857

# Company Formation Slate - \$26,633,085

Appl. ID	Title	Company Representative	Company	Total Recommended Budget
CP130023	Novel Ceramidemodulating Therapeutics for Cancer Use	Love, Richard	CerRx, Inc.	\$10,725,000
CP130058	Developing Two Clinical Proof of Concept Drugs that Inhibit a Novel Cancer Cell Target	Northrup, Jonathan	Beta Cat Pharmaceuticals, LLC (relocation from Gaithersburg, MD)	\$15,908,085

# Company Relocation Slate - \$26,000,000

Appl. ID	Title	Company Representative	Company	Total Recommended Budget
CP130020	Androgen Receptor N-Terminus Blocker Program for Prostate Cancer	Reider, Robert	ESSA Pharma Inc. (relocation from Vancouver, British Columbia)	\$12,000,000
CP130066	Phase II Clinical Studies for a First in Class Bcl-2 Cancer Drug	Messman, Richard	ProNAi Therapeutics, Inc. (relocation from Plymouth, MI)	\$14,000,000

# Cancer Prevention and Research Institute of Texas



## **Product Development Grant Recommendations**

**TAB 5**

# Company Commercialization

\$15,249,480



Appl. ID	Title	Company Representative	Company	Total Recommended Budget
CP130013	Clinical Development and Commercialization of Oncolytic Adenovirus for Treating Malignant Glioma	Tufaro, Frank	DNAtrix, Inc.	\$10,813,623
CP130050	Real-time Nerve Identification for Robotic Surgery	Stone, James	ProPep Surgical	\$ 4,435,857

# Company Formation

\$26,663,085



Appl. ID	Title	Company Representative	Company	Total Recommended Budget
CP130023	Novel Ceramidemodulating Therapeutics for Cancer Use	Love, Richard	CerRx, Inc.	\$10,725,000
CP130058	Developing Two Clinical Proof of Concept Drugs that Inhibit a Novel Cancer Cell Target	Northrup, Jonathan	Beta Cat Pharmaceuticals, LLC	\$15,938,085

# Company Relocation

\$26,000,000



Appl. ID	Title	Company Representative	Company	Total Recommended Budget
CP130020	Androgen Receptor N-Terminus Blocker Program for Prostate Cancer	Reider, Robert	ESSA Pharma Inc.	\$12,000,000
CP130066	Phase II Clinical Studies for a First in Class Bcl-2 Cancer Drug	Messman, Richard	ProNAi Therapeutics, Inc.	\$14,000,000



# Cancer Prevention and Research Institute of Texas



## **Product Development Grant Recommendations REVISED Company Formation Recommendation**

**TAB 5**

# Company Formation

\$21,938,085



Appl. ID	Title	Company Representative	Company	Total Recommended Budget
CP130023	Novel Ceramidemodulating Therapeutics for Cancer Use	Love, Richard	CerRx, Inc.	Not to exceed \$6,000,000*
CP130058	Developing Two Clinical Proof of Concept Drugs that Inhibit a Novel Cancer Cell Target	Northrup, Jonathan	Beta Cat Pharmaceuticals, LLC	\$15,938,085

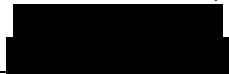
\* The revised award amount will fund the Phase 2a Proof of Concept trial in Peripheral T-Cell lymphoma

**Conflicts of Interest for Product Development Cycle 13.1 and 13.2 Applications**  
**(Product Development Cycle 13.1 and 13.2 Awards Announced at February 2014 Oversight**  
**Committee Meeting)**

The table below lists the conflicts of interest (COIs) identified by peer reviewers, Program Integration Committee (PIC) members, and Oversight Committee members on an application-by-application basis. All applications with at least one identified COI are listed below; applications with no COIs are not included. It should be noted that an individual is asked to identify COIs for only those applications that are to be considered by the individual at that particular stage in the review process. For example, Oversight Committee members identify COIs, if any, with only those applications that have been recommended for the grant awards by the PIC. COI information used for this table was collected by SRA International, CPRIT's third party grant administrator, and by CPRIT.

<b>Grant ID</b>	<b>Applicant</b>	<b>Company</b>	<b>Conflict Noted</b>
<b>Applications considered by the PIC and Oversight Committee</b>			
<b>CP130013</b>	Tufaro, Frank	DNAtrix, Inc.	Dhingra, Kapil; Mitchell, Amy
<b>CP130020</b>	Rieder, Robert	ESSA Pharma, Inc.	McCloskey, Richard; Mitchell, Amy
<b>CP130023</b>	Love, Richard	CerRx, Inc.	Dhingra, Kapil
<b>CP130050</b>	Stone, James	ProPep Surgical	McCloskey, Richard
<b>CP130066</b>	Messmann, Richard	ProNAi Therapeutics, Inc.	Clendeninn, Neil
<b>Applications Not Recommended for the PIC or Oversight Committee Consideration</b>			
<b>CP130014</b>	Heidel, Jeremy	PeptiMed	Lyerly, Kim
<b>CP130037</b>	Kirkpatrick, D. Lynn	Phusis Therapeutics, Inc.	Scheinberg, David
<b>CP130040</b>	Klemm, Steve	Leonardo BioSystems, Inc.	Cosan, Roy
<b>CP130045</b>	Matthews, William	Leuchemix, Inc.	Scheinberg, David
<b>CP130057</b>	Josey, John	Peloton Therapeutics, Inc.	Arkin, Michelle

**JACK GELTOSKY, PH.D.**



Home



jack



Cell



**SUMMARY**

Highly energized pharmaceutical licensing executive with a strong R&D background. Experience with numerous and diverse deal structures supporting research collaborations. Strong leader, excels at working in a matrix environment, highly successful problem solver, creative and passionate developer of alliances and an ability to attract and retain key personnel. Groups managed are characterized by strong performance, a high degree of teamwork, and genuine enthusiasm for the work.

**PROFESSIONAL EXPERIENCE**

**Current:**

**JEG and Associates, LLC**  
**Biotech and Pharmaceuticals Business Development Consulting**  
 Managing Director

**2008-current**

Provide licensing/business development advice to biotechs. This includes overall strategic input (including R&D activities); preparation of relevant marketing documents; introduction to key personnel in pharma; and negotiations. Current clients include those advancing protein agents and small molecules across a broad spectrum of therapeutic areas.

**JSB-Partners, LLC**  
 Senior Advisor

**2009-current**

As a member of the team, drive all business development /partnering activities, through to negotiations, of clinical stage assets (Phase II and beyond) on behalf of clients (usually small biotechs). Remit includes all therapeutic areas.

**Commercialization Review Council for Cancer Prevention and Research Institute of Texas (CPRIT)** **2010-current**  
 Member

Evaluate business plans submitted by Texas based biotechs focused on oncology diagnostics and drug development as part of the State of Texas' investment in attacking cancer on multiple fronts.

**Board Positions**

- **Protox Therapeutics**  
 Independent Director

**2008-current**

- **Enzon Pharmaceuticals**  
 Independent Director

**2008-2009**

**Past:****Arizona Technology Enterprises****2007-2011**

*Senior Vice President of Business Development, Life Science  
(Technology Transfer Office for Arizona State University)*

Working with ASU faculty, identify proprietary, breakthrough technologies with commercial potential for the life science sector. Develop business strategies for commercialization of the technologies, prepare marketing packages, identify potential licensees and negotiate licensing agreements. This includes straight outlicensing transactions and company formations.

**Bristol-Myers Squibb****2002 – 2007**

*Vice President External Science, Technology & Licensing  
Corporate and Business Development Department*

Directed all sourcing and evaluation activities of platform technologies and compounds in all stages of development (preclinical to phase III), across all therapeutic areas. Collaborated closely with R&D, marketing and business development to in and out- license compounds/products. Managed group of 15 licensing professionals. Reported to SVP, Worldwide Business Development and to President, Pharmaceutical Research Institute.

A Sampler of completed compound deals:

<b><u>DRUG</u></b>	<b><u>LICENSOR</u></b>	<b><u>THERAPEUTIC AREA</u></b>	<b><u>DEVELOP. STAGE</u></b>
Long acting insulin	Flamel	Metabolics	Phase 1
E2F Decoy	Corgentech	Cardiovascular	Phase 3
CB1 antagonist	Solvay	Obesity	Phase 1
Vinflunine	Pierre-Fabre	Oncology	Phase 3
Ipilumimab	Medarex	Oncology	Phase 2
Emsam	Somerset	CNS	Pre-registered
Triple uptake inh.	Albany	CNS	Preclinical
LXR	Exelixis	Cardiovascular	Preclinical
NNRTI	Medivir	HIV	Preclinical
Dabs	Domantis	Immunology	Preclinical
Folate receptor tx	Endocyte	Oncology	Preclinical
Adnexins	Adnexus	Oncology	Preclinical
PCSK inhibitor	Isis	Cardiovascular	Preclinical
Anxiolytic	U of Wisconsin	CNS	Preclinical
Oncology program	Exelixis	Oncology	Preclinical

A sampler of technology deals: Celera Diagnostics, Athersys, Sequenom, Lexicon, Iconix, Ambit, Genomics Health, and Albany Molecular.

- Supported collaborations with Merck - muraglitazar; AstraZeneca - DPP4 and SGLT2 inhibitors; Pfizer- Factor Xa inhibitor.
- Successfully out-licensed a CDK 2 inhibitor to Sunesis; a growth hormone secretagogue to Elixir; an aurora kinase inhibitor to Ambit; and a dual inhibitor anti-hypertensive to Pharmacopeia.
- Developed an innovative “Venture Capital” outreach program to enhance sourcing function; developed and orchestrated three highly successful “VC Days”.

**Message Pharmaceuticals****2001 – 2002**

*CEO*

Managed all fund raising and collaborations with pharmaceutical companies for this 35 person biotech start-up focused on discovering a novel class of drugs aimed at post-transcriptional regulation. Laid groundwork for closing a series C round and lined up a significant corporate collaboration.

### **Smithkline Beecham Pharmaceuticals**

**1995 - 2000**

*Vice President and Director, Scientific Licensing,  
Worldwide Business Development*

*1997 - 2000*

Managed the identification of all in-licensed compounds and technical due diligence: drove the in-licensing enterprise, maintained in-depth knowledge of external pipelines and provided timely and cogent analyses to senior management. Focused on compounds twelve months from an IND through phase IV. Therapeutic areas included neuroscience, cardio-pulmonary, anti-infectives, inflammation, and oncology. Managed a group of seven licensing professionals. Reported to Senior VP, Worldwide Business Development.

A sampler of completed compound deals:

<u>DRUG</u>	<u>LICENSOR</u>	<u>THERAPEUTIC AREA</u>	<u>DEVELOP.STAGE</u>
Argatroban	Texas Biotech	Cardiovascular	Phase 3
Bexxar	Coulter	Oncology	Phase 2
Tranilast	Kissei	Cardiovascular	Phase 1
Factive	LG Chemical	Anti-infectives	Phase 1
LF06.0337	Fournier	Cardiovascular	Phase 1
huC242	Immunogen	Oncology	Preclinical
IL 18	Hayashibara	Oncology	Preclinical
Beta 3 agonist	Asahi	Obesity	Preclinical
YH 1885	Yuhan	GI	Preclinical
TAS 106	Taiho	Oncology	Phase 1

### **Director, Scientific Licensing**

*Worldwide Business Development*

*1995 - 1997*

Identified and evaluated in-licensing opportunities across all therapeutic areas. Worked with R&D, business development, commercial, and legal personnel to bring forward selected opportunities for final approval. Reported to VP, Scientific Licensing. Gained upper management approval resulting in formal licensing offers being made on three projects during this twelve month period.

### **Consultant**

**1994 – 1995**

Major client was Johnson and Johnson. The goal was to out-license twelve biopharmaceutical technologies which J&J no longer had an interest in developing for strategic reasons. By the end of the period, concluded eight deals.

### **Molecumetics**

*Managing Director*

**1994 – 1995**

Developed and executed a strategic plan to extract commercial value from proprietary technology in the field of peptidomimetics as an enabling tool for drug discovery.

### **Johnson and Johnson**

**1984 – 1994**

*RW Johnson Pharmaceuticals, Research Institute – La Jolla, CA*

*Senior Director of Research*

*1989 – 1994*

As the technical liaison between J&J and the Scripps Research Institute, managed a group of 45 scientists (17 PhD's) whose role was to address proof of principle of technologies from Scripps. Negotiated research grants with Scripps. Ran the La Jolla facility for J&J.

- Raised the J & J-Scripps relationship to its most productive level ever. Eight new projects instituted in four years, each dependent on strong collaborations with Scripps inventors.
- Identified newly emerging technology from Scripps with commercial potential.
- Established preclinical proof of concept on four molecules and recommended for clinical development. Projects halted because of J&J's strategic decision to exit biopharm product development.

### **RW Johnson Pharmaceutical Research Institute**

*Director of Biosciences*

1989

Managed group of 60 scientists (18 PhD's) developing protein formulations and analytical R&D. Research aimed at growth factors, acute therapy Mabs, imaging agents, etc.

- Instituted development of new formulation for a more "user friendly" version of erythropoietin.
- Instituted efforts to develop a more stable formulation of OKT3. The recommended version is now incorporated into the current product.
- Department performed quality control (QC) of EPO for sale in Europe by Cilag.

**Ortho Biotech Imaging Products, Washington Crossing, NJ***Director, Technical Development*

1988 – 1989

Managed preclinical development in radiopharmaceuticals.

- Manufacture and QC of Macroscint, imaging agent for the localization of occult infections.
- Conducted proof of principle studies and filed IND for Macroscint, all in one year.
- Directed preparation of CMC section for Macroscint's IND.
- Managed two key external relationships, one that led to a novel second generation product.

**Ortho Diagnostics***Director of Infectious Disease Product Development*

1985 – 1988

Managed all of Ortho's product development activities in infectious disease diagnostics. Grew a team from 20 to 40 research scientists. Four products launched during this period. Managed several outside collaborators: Chiron, Enzo, Cambridge Bioscience.

**Johnson & Johnson Biotechnology Center, La Jolla, CA***Diagnostics Program Director*

1984 – 1985

Technical liaison between J&J and Scripps Research Institute. Responsible for identifying and executing proof of concept studies with an eight member group on technologies emerging from Scripps with *in vitro* diagnostics applications. Negotiated research contracts with Scripps scientists.

**E.I. DuPont**

1980-1984

*Research Scientist*

Introduced monoclonal antibody technology to the company. Developed a number of *in vitro* diagnostic assays.

- Sole inventor on key patents for use of the monoclonals to theophylline in diagnostic assays.

**EDUCATION**

**PhD**, Biochemistry, California Institute of Technology  
**BS**, Chemistry (*magna cum laude*), Memphis State University  
Training in Richard Lerner's Lab, Scripps Research Institute

**HONORS**

NIH Postdoctoral Fellowship  
American Cancer Society Senior Fellowship, California Division



**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**

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**MEMORANDUM**

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**TO:** OVERSIGHT COMMITTEE MEMBERS  
**FROM:** REBECCA GARCIA PH.D., CHIEF PREVENTION AND COMMUNICATIONS OFFICER  
**SUBJECT:** UPDATE OF PREVENTION PROGRAM ACTIVITIES  
**DATE:** FEBRUARY 19, 2014

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Progress since the last Oversight Committee meeting is as follows:

1. Requests for Applications (RFAs) for Competitive Continuation/Expansion Projects, Health Behavior Change through Public Education and Evidence Based Cancer Prevention Services will close on Feb. 27, 2014.
2. We conducted a webinar on January 29, 2014 to answer questions about the open RFAs and highlight changes due to the new administrative rules. One hundred and ten people attended the session.
3. Contracts for the 10 projects the Oversight Committee ratified in November have all been signed.
4. We will be releasing another round of RFAs for prevention awards during the month of March 2014.
5. A recommendation for a Strategic Communications Services contract was forwarded to the CEO and Audit Subcommittee.





**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**

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**MEMORANDUM**

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**TO:** CPRIT AUDIT COMMITTEE  
**FROM:** REBECCA GARCIA, PH.D., CHIEF PREVENTION AND COMMUNICATION OFFICER  
**SUBJECT:** STRATEGIC COMMUNICATIONS CONTRACT  
**DATE:** FEBRUARY 7, 2014

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**Summary and Recommendation:**

An effective, coordinated, strategic communications program is needed to inform the public, legislature, media, health professionals, and partner organizations about CPRIT's activities. After conducting an evaluation pursuant to the rules for procurement of services set forth by the Texas State Comptroller of Public Accounts, CPRIT staff recommends that \_\_\_\_\_ be awarded the contract for strategic communications services.

**Background:**

Created to make a difference in the lives of Texans affected by cancer, CPRIT has a responsibility to inform the public, legislature, media, health professionals, and partner organizations about its activities. An effective, coordinated, strategic communications program is needed to provide this service.

CPRIT has minimal staff to support the agency's communication needs, and historically has relied on contractors to assist with many of the communications activities. Since 2010, CPRIT has contracted with communications firms to provide strategy and support to ensure successful CPRIT communications. With increasing demand for immediate information in the ever-changing information age, providing a coordinated and comprehensive communications strategy could require 3-4 full time staff members.

Support from a professional communications firm is needed to assist CPRIT in providing an effective communications program and to further develop and implement a strategic communications plan. Some of the services outlined in the communications contract include but are not limited to:

1. Providing ongoing counsel and strategic direction, including daily media monitoring.

2. Drafting informational releases, talking points and other key communications pieces related to CPRIT and its initiatives.
3. Developing key messages and talking points for CPRIT staff and Oversight Committee members.
4. Ensuring consistent messaging and branding across all communications vehicles: annual report, website, social media, legislative requests, and advocacy groups.
5. Developing and implementing external communication strategies to promote the opportunities, work and successes of CPRIT and its funded initiatives not only in Texas but nationally.
6. Serving as a point of contact for media inquiries and managing interview preparation, messaging and execution.
7. Assisting staff in planning and execution of potential CPRIT conferences; e.g., conference overarching themes and brand, promotion, media relations, developing collateral materials (brochures, program books, etc.).
8. Performing environmental scanning to determine emerging communications opportunities and threats.

**Evaluation Process:**

In accordance with the rules for procurement of services set forth by the Texas State Comptroller of Public Accounts, CPRIT issued a Request for Proposals for a Strategic Communications Program. The estimated budget amount was up to \$125,000 through August 31, 2014 plus \$250,000 in fiscal year 2015. Renewal options may be exercised for an additional three years.

A proposal evaluation team met and discussed the eligible submissions and a recommendation to retain \_\_\_\_\_ was forwarded to the Chief Executive Officer.



**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**

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**MEMORANDUM**

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**TO:** OVERSIGHT COMMITTEE MEMBERS  
**FROM:** ANGELOS ANGELOU, AUDIT SUBCOMMITTEE CHAIR  
**SUBJECT:** STRATEGIC COMMUNICATIONS SERVICES CONTRACT  
**DATE:** FEBRUARY 17, 2014

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**Summary and Recommendation:**

Support from a professional communications firm is needed to assist CPRIT in providing an effective communications program and to further develop and implement a strategic communications plan. CPRIT conducted an evaluation to consider firms that submitted proposals pursuant to the rules for procurement of services set forth by the Texas State Comptroller of Public Accounts. The Audit Subcommittee recommends that the strategic communications contract be awarded as recommended by the CPRIT CEO.

**Discussion:**

Pursuant to Section 4.4 of the Oversight Committee Bylaws, the Audit Subcommittee is required to review and make recommendations to the Oversight Committee with respect to non-grant contracts exceeding \$100,000. In accordance with the rules for procurement of services set forth by the Texas State Comptroller of Public Accounts, CPRIT issued a Request for Proposals for a Strategic Communications Program. The estimated budget amount is up to \$125,000 through August 31, 2014 plus \$250,000 in fiscal year 2015. Renewal options may be exercised for an additional three years.

A proposal evaluation team met and discussed the eligible submissions and a recommendation was forwarded to the Chief Executive Officer.



## CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS

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**MEMORANDUM**

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**TO:** OVERSIGHT COMMITTEE MEMBERS  
**FROM:** BILL RICE, M.D., NOMINATIONS SUBCOMMITTEE ACTING CHAIR  
**SUBJECT:** AGENDA ITEM # 10 - INTENTION TO RECOMMEND APPROVAL OF THE CHIEF EXECUTIVE OFFICER'S APPOINTMENTS TO THE SCIENTIFIC RESEARCH AND PREVENTION PROGRAMS COMMITTEE  
**DATE:** FEBRUARY 18, 2014

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**Summary and Recommendation:**

The Chief Executive Officer has appointed 27 people to the CPRIT's Scientific Research and Prevention Programs Committee. The Nominations Subcommittee discussed these appointments at its meeting on February 17, 2014. CPRIT's statute requires the appointments to be approved by the Oversight Committee. The Nominations subcommittee recommends that the Oversight Committee vote to approve the Chief Executive Officer's appointments at the February 19, 2014, meeting.

**Discussion:**

Scientific Research and Prevention Programs committee members (also referred to as "peer reviewers") are responsible for reviewing grant applications and recommending grant awards for meritorious projects addressing cancer prevention and research (including product development) in Texas. Peer reviewers perform an important role for the state; all CPRIT grant awards must first be recommended by a Scientific Research and Prevention Programs committee. Therefore, the individuals appointed to serve as CPRIT's Scientific Research and Prevention Programs committee members must be exceptionally qualified, highly respected, well-established members of the cancer research, product development, and prevention communities.

Texas Health and Safety Code Section 102.151(a) directs the Chief Executive Officer to appoint members to the Scientific Research and Prevention Programs committees. The CEO's appointments are final once approved by a simple majority of the Oversight Committee. The Nominations Subcommittee charter assigns the subcommittee with the responsibility "to circulate to Oversight Committee members in advance of a public meeting written notification of the committee's intent to make the nomination, along with such information about the nominee as may be relevant."

The Nominations Subcommittee has considered the pending appointments and recommends Oversight Committee approval.



# **CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**

## **Oversight Committee Nominations Subcommittee**

### **Peer Review Panel Nominations**

## Scientific Research

Basic Cancer Research Panel 1  
Tom Curran, Ph.D./FRS, Chair

Peer Review Panel Members for Approval

1. Suzanne Baker, Ph.D.
2. Martin McMahon, Ph.D.
3. Avraham Raz, Ph.D.
4. Sara Sukumar, Ph.D.
5. Jean Wang, Ph.D.

**BIOGRAPHICAL SKETCH**

Provide the following information for the Senior/key personnel and other significant contributors in the order listed on Form Page 2.  
Follow this format for each person. **DO NOT EXCEED FOUR PAGES.**

NAME BAKER, Suzanne J	POSITION TITLE		
eRA COMMONS USER NAME (credential, e.g., agency login) SBAKER	Member		
EDUCATION/TRAINING <i>(Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable.)</i>			
INSTITUTION AND LOCATION	DEGREE <i>(if applicable)</i>	MM/YY	FIELD OF STUDY
Miami University, Oxford, Ohio	B.S.	05/86	Zoology
The Johns Hopkins University, Baltimore, Maryland	Ph.D.	05/91	Molecular Biology and Human Genetics

**A. Personal Statement.**

My research is directed towards understanding how disruption of key signaling pathways contributes to the development of high-grade gliomas, with a special focus on pediatric brain tumors. My laboratory employs the latest genomic technologies to identify the underlying molecular defects driving gliomagenesis in primary human tumors, and then develops *in vitro* and *in vivo* mouse model systems to determine the mechanisms through which such mutations contribute to cancer. I also serve as the Co-Leader of the Neurobiology and Brain Tumor Program at St. Jude. In this role, I work to facilitate productive interactions between basic and clinical researchers to translate basic research findings into new clinical trials for children with brain tumors.

**B. Positions and Honors****Positions and Employment**

1987-1991	Pre-Doctoral Trainee, Department of Oncology, The Johns Hopkins University School of Medicine, Baltimore, MD
1991-1995	Postdoctoral Fellow, Department of Molecular Oncology and Virology, Roche Institute of Molecular Biology, Nutley, NJ
1995-2001	Assistant Member, Department of Developmental Neurobiology, St. Jude Children's Research Hospital, Memphis, TN
2002-2010	Associate Member, Department of Developmental Neurobiology, St. Jude Children's Research Hospital, Memphis, TN
2004-2010	Affiliated Associate Professor, Department of Pathology University of Tennessee, Memphis, TN
2006 – 2011	Track Head, Cancer and Developmental Biology Track, UT/St Jude IPBS graduate program
2010 – present	Affiliated Professor, Department of Pathology University of Tennessee, Memphis, TN
2010-present	Member, Department of Developmental Neurobiology, St. Jude Children's Research Hospital, Memphis, TN
2011 – present	Co-leader, Neurobiology and Brain Tumor Program, St. Jude Children's Research Hospital Comprehensive Cancer Center

**Awards and Honors:**

1986	Elected Phi Beta Kappa
1990	Sciencewatch: Most highly cited paper in biomedical literature over 2.5 year period (Science 244:217-221, 1989.)
1991	David Israel Macht Award (outstanding Ph.D. Student Research, Johns Hopkins School of Medicine)

2007 LIMA International Award for Excellence in Pediatric Brain Tumor Research from the Children's Brain Tumor Foundation.  
 2009 Sydney Schlobohm Chair of Research, National Brain Tumor Society  
 2006-2010 Member, NIH study section Cancer Molecular Pathobiology  
 2011 V Foundation Translational Research Scholar  
 2012-present Co-Chair, Scientific Advisory Board, National Brain Tumor Society

**C. Selected peer-reviewed publications (from a total of 74).**

1. **Baker SJ**, Fearon ER, Nigro JM, Hamilton SR, Preisinger AC, Jessup JM, vanTuinen P, Ledbetter DH, Barker DF, Nakamura Y, White R, Vogelstein B. Chromosome 17 deletions and p53 gene mutations in colorectal carcinomas. *Science* 244:217-221, 1989.
2. Kwon CH, Zhu X, Zhang J, Knoop LL, Tharp R, Smeyne RJ, Eberhart CG, Burger PC, **Baker SJ**. Pten regulates neuronal soma size: a mouse model for Lhermitte-Duclos disease. *Nature Genet* 29:404-411, 2001.
3. Fraser, MM, Bayazitov, IT, Zakharenko, SS and **Baker, SJ**. Pten deficiency in brain causes defects in synaptic structure, transmission and plasticity, and myelination abnormalities. *Neuroscience* 151:476-488, 2008. PMCID: PMC2278004
4. Chalhoub, N and **Baker, SJ**. PTEN and the PI3K Signaling Pathway in Cancer. *Annu Rev Pathol.* 4:127-150, 2009. PMCID: N/A (Review)
5. Frappart, P-O, Lee, Y, Russell, HR, Chalhoub, N, Wang, Y-D, Orie, KE, Zhao, J, Kondo, N, **Baker, SJ** and McKinnon PJ. Genetic Alterations in medulloblastoma from mice with defective DNA double strand break repair. *Proc Natl Acad Sci USA* 106:1880-1885, 2009. PMCID: PMC2644132
6. Chalhoub, N, Zuo, G, Zuo X and **Baker SJ**. Cell-type specificity of PI3K signaling in Pdk1- and Pten-deficient brain. *Genes Dev.* 23:1619-1624, 2009. PMCID: PMC2714713
7. Paugh, BS, Qu, C, Jones, C, Liu, Z, Adamowicz-Brice, M, Zhang, J, Coyle, B, Barrow, J, Bax, DA, Hargrave, D, Lowe, J, Gajjar, A, Zhao, W, Broniscer, A, Ellison, DW, Grundy, R, **Baker, SJ**. Integrated molecular genetic profiling of pediatric high-grade gliomas reveals key differences with the adult disease. *J Clin Oncol* 28:3061-3068, 2010. PMCID: PMC2903336
8. Chow, LM, Endersby, R, Zhu, X, Rankin, S, Qu, C, Zhang, J, Broniscer, A, Ellison, DW, **Baker, SJ**. Cooperativity within and among Pten, p53 and Rb pathways induces high-grade astrocytoma in adult brain. *Cancer Cell*, 19:305-316, 2011. PMCID: PMC3060664
9. Endersby, R, Zhu X, Hay, N, Ellison, DW, **Baker, SJ**. Non-redundant functions for Akt isoforms in astrocyte growth and gliomagenesis in an orthotopic transplantation model. *Cancer Res* 71: 4106-4116, 2011. PMCID: PMC3118569
10. Paugh, BS, Broniscer, A, Qu, Ch, Miller, CP, Zhang, J, Tatevossian,, RG, Olson, JM, Geyer, JR, Chi, S, da Silva, NS, Onar-Thomas, A, Baker, JN, Gajjar, A, Ellison, DW and **Baker, SJ**. Genome-wide Analyses Identify Recurrent Amplifications of Receptor Tyrosine Kinases and Cell Cycle Regulatory Genes in Diffuse Intrinsic Pontine Glioma. *J Clin Oncol* 29:3999-4006, 2011. PMCID: PMC3209696
11. Zhu, G, Chow, LML, Bayazitov, IT, Tong, Y, Gilbertson, RJ, Zakharenko, S, Solecki, DJ, and **Baker, SJ**. Pten deletion causes mTorc1-dependent ectopic neuroblast differentiation without causing uniform migration defects. *Development* 139:3422-3431, 2012. PMID: 22874917 PMCID: PMC3424045
12. Wu, G, Broniscer, A, McEachron, T, Lu, C, Paugh, BS, Becksfort, J, Qu, C, Ding, L, Huether, R, Parker, M, Zhang, J, Gajjar, A, Dyer, MA, Mullighan, CG, Gilbertson, RJ, Mardis, ER, Wilson, RK, Downing, JR, Ellison, DW, Zhang, J, **Baker SJ**. Somatic histone H3 alterations in pediatric diffuse intrinsic pontine gliomas and non-brainstem glioblastomas. *Nat Genet* 44:251-253, 2012. PMCID: PMC3288377
13. Zhang, J, Wu, G, Miller, CP, Tatevossian, RG, Dalton, JD, Tang, B, Orisme, W, Punchihewa, C, Parker, M, Qaddoumi, I, Boop, FA, Lu, C, Kandoth, C, Ding, L, Lee, R, Huether, R, Chen, X, Hedlund, E, Nagahawatte, P, Rusch, M, Boggs, K, Cheng, J, Becksfort, J, Ma, J, Song, G, Li, Y, Wei, L, Wang, J,



## BIOGRAPHICAL SKETCH

Provide the following information for the Senior/key personnel and other significant contributors in the order listed on Form Page 2.  
Follow this format for each person. **DO NOT EXCEED FOUR PAGES.**

NAME Martin McMahon	POSITION TITLE Efim Guzik Distinguished Professor of Cancer Biology and Professor-In-Residence		
eRA COMMONS USER NAME (credential, e.g., agency login) mcmahon@cc.ucsf.edu			
EDUCATION/TRAINING (Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable.)			
INSTITUTION AND LOCATION	DEGREE (if applicable)	MM/YY	FIELD OF STUDY
University of Glasgow, UK	B.Sc. (Hons)	06/1981	Biochemistry
King's College, University of London, UK	Ph.D.	10/1985	Biochemistry
University of California, San Francisco	Postdoctoral	1985-1990	Molecular Oncology

### A. PERSONAL STATEMENT:

I conducted my graduate research with Drs. Ian Kerr & George Stark at the Imperial Cancer Research Fund in the UK and at Stanford University in the USA and post-doctoral training with Dr. J. Michael Bishop at the University of California, San Francisco. I have run my own independent cancer research program since 1991, first at the DNAX Research Institute and then as a faculty member of the University of California, San Francisco Comprehensive Cancer Center. I am currently the *Efim Guzik Distinguished Professor of Cancer Biology* and Professor-in-Residence in the UCSF Department of Cellular and Molecular Pharmacology. Since 1991 I have mentored over 30 post-doctoral fellows, 15 students, including 3 medical students, in my laboratory. Former postdoctoral fellows in my lab have gone on to highly successful careers in the University or the private sector. Most notably: Dr. Catrin Pritchard is Professor of Biochemistry at the University of Leicester; Dr. Steen Hansen is Associate Professor at Harvard University; Dr. Terunaga Nakagawa is Assistant Professor at UC San Diego; Dr. David Dankort is Assistant Professor at McGill University; Dr. Sean McCarthy is Chief Executive Office at CyTomix Inc and; Drs. Meghna Das Thakur, Natasha Aziz and Eleni Venetsanakos are Staff Scientists at Novartis Institute for Biomedical Research.

Previously, I served for five years as Co-Director of the UCSF Biomedical Sciences (BMS) graduate program that trains Ph.D. and M.D./Ph.D students in biological sciences related to normal human development, physiology and disease. I am currently the Assistant Director of Professional Education and the Co-Leader of the Developmental Therapeutics Program within the UCSF/Helen Diller Comprehensive Cancer Center, the mission of latter being to identify and develop new targets and potential therapeutics for cancer patients. I am currently the Chair of the NIH Basic Mechanisms of Cancer Therapeutics study section, the President for the Society for Melanoma Research and serve on study sections of various philanthropic cancer research funding agencies such as Cancer Research UK, the Lustgarten Foundation for Pancreatic Cancer Research and the Melanoma Research Alliance.

Since joining the UCSF faculty in 1998 my lab has generated mice carrying conditionally active alleles of mouse *BRaf* that allow induced expression  $BRAF^{V600E}$  in response to Cre recombinase. We, and others, have used *BRaf<sup>CA</sup>* mice to generate models of metastatic melanoma, glioma, lung, pancreatic, prostate and thyroid cancer and to test the anti-tumor effects of signal pathway-targeted experimental therapeutics in the pre-clinical setting. Consequently, my expertise fits within the broad remit of cancer research grants likely to be reviewed by the Cancer Prevention Research Institute of Texas.

### B. POSITIONS AND HONORS:

1977-1981	Imperial Chemical Industries (ICI) Undergraduate Educational Scholarship
1981	J. N. Davidson Memorial Prize in Biochemistry
1981-1985	Imperial Cancer Research Fund/King's College, London Graduate student
1981-1985	ICI Graduate Student Bursary and Postgraduate Educational Scholarship
1982	Visiting Graduate Student at Stanford University
1985-1990	Postdoctoral Fellow, Hooper Foundation, University of California, San Francisco, CA

1985-1987	Imperial Cancer Research Fund Postdoctoral Traveling Fellowship
1988-1990	American Cancer Society (California Division) Senior Postdoctoral Fellowship
1990-1991	Assistant Specialist, Hooper Foundation, University of California, San Francisco, CA.
1991-1997	Staff Scientist, Dept of Cell Signaling, DNAX Research Institute, Palo Alto, CA
1997-1998	Senior Staff Scientist, Dept. of Cell Signaling, DNAX Research Institute, Palo Alto, CA
1998	Associate Professor, University of California San Francisco, CA
2001	NIH/NCI Pancreatic Cancer Progress Review Group (Panc CA PRG)
2001	Served on the National Cancer Legislative Action Committee
2001	Appointed Efim Guzik Distinguished Professor of Cancer Biology
2001-2007	Co-Director of the UCSF Biomedical Sciences Graduate Program
2004-2006	Co-Leader of the UCSF Comprehensive Cancer Center Program in Liver Cancer
2006	Diana Ashby Award of the Melanoma Research Foundation
2006-Date	Professor-In-Residence, UCSF Comprehensive Cancer Center
2007-2012	Leader of the UCSF Comprehensive Cancer Center Program in Pancreas Cancer
2007-Date	Director of Professional Education, UCSF Comprehensive Cancer Center
2009-Date	Charter member of the NIH BMCT study section
2012-Date	Co-Leader of the HDFCCC Developmental Therapeutics Program
2013-2016	President, Society for Melanoma Research
2013-2015	Chair of the NIH BMCT study section

### **C. SELECTED RELEVANT PEER-REVIEWED PUBLICATIONS (16 OF 118):**

1. Woods, D., Parry, D., Cherwinski, H., Bosch, E., Lees E. & **McMahon M.** (1997) Raf-induced proliferation or cell cycle arrest is determined by the level of Raf activity with arrest mediated by p21<sup>Cip1</sup>. *Mol. Cell Biol.* 17: 5598-5611.
2. Zhu J., Woods, D., **McMahon M.** & Bishop, JM. (1998) Senescence of human fibroblasts induced by oncogenic Raf. *Genes. Dev.* 12: 2997-3007.
3. Dankort D, Filenova E, Collado M, Serrano M, Jones, K & **McMahon M.** (2007) A new mouse model to explore the initiation, progression and therapy of *BRAF*<sup>V600E</sup>-induced lung tumors *Genes & Development* 21: 379-384 PMID: 17299132
4. Dankort D, Curley DP, Cartlidge RA, Nelson B, You MJ, Karnezis AN, DePinho RA, **McMahon M**, Bosenberg M\*. (2009) *BRAF*<sup>V600E</sup> cooperates with *PTEN* silencing to induce metastatic melanoma. 2009 *Nature Genetics*, 41: 544-52 PMCID: PMC2705918 (\* Indicates co-senior author)
5. Charles R-P, Iezza G., Amendola E., Dankort D., **McMahon M.** (2011) Mutationally activated *BRAF*<sup>V600E</sup> elicits papillary thyroid cancer in the mouse *Cancer Research* 71: 3863-71 PMCID: PMC3107361
6. Collisson EA, Szeto C, Trejo C, Gu S, Korkola J, Karnesis AN, Heiser L, Zhu J, Charles R-P, Rabinovich BA, Hann B, Dankort D, Spellman PT, Philips W, Haussler D, Gray JW, **McMahon M.** (2012) A Central Role for RAF→MEK→ERK Signaling in the Genesis of Pancreatic Ductal Adenocarcinoma. *Cancer Discov.* (Published online Jun 1). PMCID: PMC3425446
7. Das Thakur M, Salangsang F, Sellers W, Pryer N, **McMahon M** & Stuart, D. (2013) A preclinical model of BRAF inhibitor resistance in melanoma reveals a novel approach to forestall drug resistance *Nature* 494: 251-5
8. Andreadi C., Cheung L-K., Giblett S., Patel B., Jin H., Mercer K., Kamata T., Lee P., Williams A., **McMahon M.**, Marais R & Pritchard C. (2012) The intermediate-activity *L597V* *BRAF* mutant acts as an epistatic modifier of oncogenic RAS by enhancing signaling through the RAF/MEK/ERK pathway. *Genes Dev.* 26: 1945-1958 PMCID: PMC3435497
9. Mitra D., Luo X., Morgan A., Lo J., Hoang MP., Lennerz JK, Mihm MC., Wargo JA., Robinson KC., Devi S., Vanover JC., D'Orazio JA., **McMahon M.**, Bosenberg MW., Haigis KM., Haber DA. & Fisher DE. (2012) Redheads and melanoma risk: pheomelanin synthesis is carcinogenic by a UV-independent mechanism. *Nature* 491: 449–453
10. Maertens O, Johnson B, Hollstein P, Frederick D, Cooper Z, Messiaen L, Bronson R, **McMahon M**, Granter S, Flaherty K, Wargo J, Marais R, Cichowski K. (2012) Elucidating distinct roles for NF1 in melanomagenesis *Cancer Discovery*, PMCID: PMC3595355

**BIOGRAPHICAL SKETCH**

Provide the following information for the key personnel and other significant contributors in the order listed on Form Page 2.  
Follow this format for each person. **DO NOT EXCEED FOUR PAGES.**

NAME Avraham Raz	POSITION TITLE Professor		
eRA COMMONS USER NAME avrahan			
EDUCATION/TRAINING <i>(Begin with baccalaureate or other initial professional education, such as nursing, and include postdoctoral training.)</i>			
INSTITUTION AND LOCATION	DEGREE <i>(if applicable)</i>	YEAR(s)	FIELD OF STUDY
Ben- Gurion Univ of the Negev, Beer Sheva	B.S.	1970	Biology
Ben- Gurion Univ of the Negev, Beer Sheva & The Hebrew Univ	M.S.	1972	Physiology
Weizmann Institute of Science	Ph.D.	1978	Membrane Biology

**A. Personal Statement**

I am the Paul Zuckerman Professor of Oncology and Pathology at the Barbara Ann Karmanos Cancer Institute Wayne State University, School of Medicine, and the recipient of the NCI Merit Award. I am the Past President of the International Metastasis Research Society and the co-Chief Editor of the esteemed Cancer metastasis Reviews (CMR). I have over 30 years of experience in cancer biology and have published numerous research articles and reviews summarizing my contributions to the field of tumor progression and metastasis. I have pioneered the field of galectins and cancer and was the first to identify and clone human galectin-1 and -3, generate the first immunological and molecular reagents to study galectin biology, an accomplishment for which I have received the "Merit Award" from the NCI/NIH. I was the first to generate the biological active galectin-3 antagonist to be tested in the clinic. In addition was the first to identify and clone Autocrine motility Factor (AMF) and its receptors (gp78 and Her2) and have established their functional role, molecular and biochemical pathways during cancer progressing and metastasis.

**B. Positions and Honors****Positions and Employment:**

1978-1980 Visiting Scientist, National Cancer Institutes, Frederick Cancer Research Facility, Frederick, MD  
 1980-1981 Research Fellow, Department of Cell Biology, the Weizmann Institute of Science, Rehovot, Israel  
 1981-1986 Senior Scientist, Department of Cell Biology, the Weizmann Institute of Science, Rehovot, Israel  
 1986-1988 Associate Professor, Department of Cell Biology, The Weizmann Institute of Science, Rehovot, Israel  
 1987-1995 Member and Director of Cancer Metastasis Research Program, Michigan Cancer Foundation, Detroit, MI  
 1988-present Professor, Department of Radiation Oncology, Wayne State Univ, Sch of Med, Detroit, MI  
 1989-present Scientific Director, RenSci Biotech, Ltd., Detroit, MI  
 1989-present Faculty, Graduate Program in Cancer Biology, Wayne State Univ, Sch of Med, Detroit, MI  
 1990-1997 Director, Tumor Progression and Metastasis, Karmanos Cancer Institute, Detroit, MI  
 1992-present Professor, Department of Pathology, Wayne State Univ, Sch of Med, Detroit, MI  
 1995-2000 Director, Division of Basic Research, Karmanos Cancer Institute, Detroit, MI  
 1997-present Professor, Oncology, Wayne State Univ, Sch of Med, Detroit, MI

**Honors and Awards:** 1985 - "H. Dudley Wright Research Award" in Membrane Research, Weizmann Institute of Science, 1987 - Incumbent of Paul Zuckerman Support Foundation for Cancer Research, Michigan Cancer Foundation, 1989 - Consultant Expertise, NCI, 1992 - Ad Hoc Member Pathology B Study Section, NCI, 1992 - Board of Directors, International Metastasis Society, 1994 - Ad Hoc Member, Study Section, ACS, 1994 - "The Maximilian-Nitze Award" - The German Urological Society, 1995 - Member Pathology B Study Section, NCI, 1995 - Outside Advisor, Biochemistry and Endocrinology Scientific Advisory Committee, ACS, 1995 - Ad Hoc Member, Special Emphasis Panel, Minority Pre doctoral Fellowship, NCI, 1995 - Ad Hoc Member, Diagnosis and Research, Chemical Exposures and Molecular Biology, Prostate, NCI, 1995 - Ad Hoc Member Cell Biology Study Section, ACS, 1995 - Ad Hoc Member RFA-CA95-006, National Institutes of Health, Bethesda, MD, 1995 - Scientific Advisory Board, Hipple Cancer Research Center, 1996 - Ad Hoc Member

Oncological Sciences Special Emphasis Panel, NCI, 1996 - Ad Hoc Reviewer, Biochemical Genetics Study Section, National Science Foundation, 1996 - Charter Member, The International Cancer Microenvironment Forum, 1996 - Ad Hoc Member, Experimental Immunology Study Section, NIH, 1997 - Member ZRG2 MEP (03) Biological Physiological Sci. Special Emphasis Panel Ctr. for Sci. Review, NIH, 1998 - Member SRA, MEP Study Section, Special Emphasis Section, NIH, Program Project, 1998 - Chairperson, AACR, Mini symposium, Cell and Tumor Biology 27, 1998 - President Elect, International Metastasis Research Society, 1998 - Member ZCA1-SRC(99) Special Emphasis Panel, National Institutes of Health, 2000 - President, International Metastasis Research Society, 2000 - "R.E. Bob Smith Lecture" Award - UTMD Anderson Cancer Center, 2001 - Visiting Professor - University of Pennsylvania Medical Center, 2001 - Plenary Speaker - Japanese Association of Metastasis Research, Japan, 2001 - Plenary Speaker - British Cancer Research Meeting, 2003 - Plenary Lecturer- Japan's Urological Society, 2004 - "Paget-Ewing" Award - International Metastasis Research Society, 2004 - Member- Center for Scientific Review, NIH Oncological Sciences Integrated Review Group Tumor Progression and Metastasis Study Section, 2004 - Member, Reviewer - Israel Cancer Research Fund, 2005 - Member, Reviewer - Center for Scientific Review, Oncological Sciences Integrated Review Group Tumor Progression and Metastasis, Study Section, 2005 - Member, Reviewer - Congressionally Directed Medical Research Program (CDMRP) 2005 Prostate Cancer Research Program Pathobiology-2 Panel, USARMRC, DOD, 2005 - "Featured Synopsis in Profiles and Legacies", Cancer Biology and Therapies, Sugar Recognition and Metastasis, From the Birth of a Research Field to the Clinic, Landes Bioscience, 2005 - Reviewer -North West Cancer Research Fund Scientific Committee (NWCRCF), Project Grant, London, UK, 2005, "MERIT Award", 2006, Division of Cancer Biology, National Institute of Health, National Cancer Institute, , Plenary Lecturer- Japan's 59th Broncho-Esophagological Society, November 1-2, 2007 The Role of Tumor Cell Surface Lectins in Metastasis, R37CA046120-19. Adhoc Committee Member, AACR Grants Sub-Committee for Basic Cancer Research, 2008; Reviewer, NIH, Special Emphasis Panel, ZRG 1CB-N 02(M), Cell Biology, Teleconference, 2008; Reviewer, NIH, Center for Scientific Review, Cell Biology Integrated Review Group, MIST Study Section, 2008 Reviewer, DOD, BCRP, Study Section, 2009, Reviewer, Special Emphasis Panel NIH CCNE, 2010, Reviewer, Prostate Cancer Charity Research Awards, The Cancer Research, UK, 2010, Division of Cancer Treatment and Diagnosis, Developmental Therapeutic Program, 2010, Reviewer, Department of Oncology, Grant Review, National Cancer Institute Grants, 2010, Reviewer, NIH, National Cancer Institute, K1C, 2011, Reuben Lotan Distinguished Lecture- University of Texas , MD Anderson Cancer center April 10- 1012; Cold Spring Harbor Asia / International Cancer Microenvironment Society Joint Conference on Tumor Microenvironment; Organizing Committee and Plenary Lecture - Suzhou, China November 13-17, 2012; Chair Person the 8th Annual Midwest Carbohydrate Research Symposium October 5-6, 2012 WSU, Reviewer, NIH/NCI Career Development Review Committee, Bethesda, MD, 2013, "Kales Award in Oncology", Karmanos Cancer Institute, 2013. "Outstanding Research Achievement Award", WSU, 2013, Visiting Professor, Northeast Normal University, Changchun, China, 2013. Fiirst- "Faculty Award for Career Achievement" Karmanos Cancer Institute, 2013.

**Editorial Activity:** 1982 - Editorial Board: Anti-Cancer Research, 1983 - Editorial Advisory Board: Cancer Metastasis Reviews, 1983 - Editorial Advisor: Revisiões Sobre Biologia Celular (RBC), 1985 - International Editorial Board, Excerpta Medica, 1993 - Editorial Academy of the International Journal of Oncology, 1994 - International Editorial Board, Pathology & Oncology Research, 1995 - Editorial Board, Clinical & Experimental Metastasis, 1996 - Editorial Board - Invasion & Metastasis, 2001-2010 - Associate Editor - Cancer Research, 2001-2010 - Editorial Board - Cancer Biology and Therapy, 2002- Managing Editorial Board - Frontiers in Science 2002 - Co- Editor in Chief, Cancer Metastasis Reviews, 2003 - Managing Editorial Board - Frontiers in Bioscience, 2005 - Editorial Board Member - Current Cancer Therapy Reviews, Editorial Board Member - Cancer Microenvironment, 2007; Editorial Board Member - Open Glycoscience, 2007, Editorial Board Member, Journal of Cancer Research & Therapy, 2012; Editorial Board Member -Journal of OncoBiomarkers, 2012 -; Editorial Board Member - Datasets Papers in Biology, 2012; Editorial Board Member - Journal of Cancer Research & Therapy, 2013 Editorial Board Member -Journal of Oncobiomarkers

**Professional Memberships:** American Society for Microbiology , American Association for Cancer Research, European Association for Cancer Research, International Metastasis Research Society, Southwest Oncology

**BIOGRAPHICAL SKETCH**

NAME Sukumar, Saraswati		POSITION TITLE Professor of Oncology/Pathology	
EDUCATION/TRAINING			
INSTITUTION AND LOCATION	DEGREE (if applicable)	MM/YY	FIELD OF STUDY
Nagpur University, Nagpur, India	M.S.	1969	Biochemistry
Atomic Energy Commission at Cancer Institute, Madras, India	Research Fellow	1971-76	Microbiology
Nagpur University, Nagpur, India	Ph.D.	1977	Biochemistry
National Cancer Institute, NIH, Bethesda, MD	Postdoc Fellow	1978-83	Immunology, Molecular Biology

**Positions and Employment**

1978-1983	Visiting Associate, Laboratory of Immunobiology, National Cancer Institute, Bethesda, MD
1983-1988	Scientist Associate, NCI and Bionetics Research Inc., NCI/Frederick Cancer Research Facility, Frederick, MD (Section Chief, Mariano Barbacid)
1989-1994	Assistant Professor, Molecular Biology of Breast Cancer Laboratory, The Salk Institute for Biological Studies, La Jolla, CA
1994-2001	Associate Professor of Oncology and
1996-2001	Associate Professor of Pathology, JHUSOM, Baltimore, MD
1996-2001	Associate Professor in Human Genetics, JHUSOM, Baltimore, MD
2000-2002	Associate Professor of Pathobiology, JHUSOM, Baltimore, MD
2002-present	Barbara B. Rubenstein Professor of Oncology and Pathology, Director of Basic Research; Breast Cancer Program, The Johns Hopkins University School of Medicine, Baltimore, MD
2002-2004	Soo Lin Professor and Head of Breast Cancer Laboratories, ORI, National University of Singapore
2002-present	Professor in Human Genetics, JHUSOM, Baltimore, MD
2002-present	Preceptor in Graduate Programs in- Pathobiology, Center for Molecular Medicine, JHUSOM, Baltimore, MD
2004-present	Professor of Pathology, JHUSOM, Baltimore, MD

**Other Experience and Professional Memberships**

1994-2005	Director of Basic Research, Breast Cancer Program, SKCCC at Johns Hopkins, Baltimore, MD
2005-present	Co-Director, Breast Cancer Program at Johns Hopkins, SKCCC, Baltimore, MD
2007-2010	Member of the Scientific Advisory Board, Susan G. Komen Foundation for the Cure
2010-2013	Member of the Scientific Advisory Council, Susan G. Komen Foundation for the Cure
2010-present	Senior Editor: Cancer Research, Cancer Biology and Therapy; Associate Editor: Journal of Molecular Medicine
2011-present	Member of the Steering Committee, CBCRP, CA

**Peer-reviewed relevant publications (in chronological order, selected out of 140).**

1. Almendro V, Kim H, Cheng YK, Gonen M, Itzkovitz S, Argani P, van Oudenaarden A, Sukumar S, Michor F, Polyak K. Genetic and phenotypic diversity in breast tumor metastases. Cancer Res. 2014 Jan 21. [Epub ahead of print] PubMed PMID: 24448237.
2. Horvath A, Pakala SB, Mudvari P, Reddy SD, Ohshiro K, Casimiro S, Pires R, Fuqua SA, Toi M, Costa L, Nair SS, Sukumar S, Kumar R. Novel Insights into Breast Cancer Genetic Variance through RNA Sequencing. Sci Rep. 2013 Jul 25;3:2256. doi: 10.1038/srep02256. PMCID: PMC3722564.
3. Shah N, Jin K, Cruz LA, Park S, Sadik H, Cho S, Goswami CP, Nakshatri H, Gupta R, Chang HY, Zhang Z, Cimino-Mathews A, Cope L, Umbricht C, Sukumar S. HOXB13 mediates tamoxifen resistance and invasiveness in human breast cancer by suppressing ER $\alpha$  and inducing IL-6 expression. Cancer Res. 2013 Jul 5. [Epub ahead of print] PubMed PMID: 23832664.
4. Choudhury S, Almendro V, Merino VF, Wu Z, Maruyama R, Su Y, Martins FC, Fackler MJ, Bessarabova M, Kowalczyk A, Conway T, Beresford-Smith B, Macintyre G, Cheng YK, Lopez-Bujanda Z, Kaspi A, Hu R, Robens J, Nikolskaya T, Haakensen VD, Schnitt SJ, Argani P, Ethington G, Panos L, Grant M, Clark J, Herlihy W, Lin SJ, Chew G, Thompson EW, Greene-Colozzi A, Richardson AL,

## BIOGRAPHICAL SKETCH

Provide the following information for the Senior/key personnel and other significant contributors in the order listed on Form Page 2.  
Follow this format for each person. **DO NOT EXCEED FOUR PAGES.**

NAME Jean Y. J. Wang	POSITION TITLE		
eRA COMMONS USER NAME (credential, e.g., agency login) REFLECTRUTH	Distinguished Professor of Medicine & Biology		
EDUCATION/TRAINING (Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable.)			
INSTITUTION AND LOCATION	DEGREE (if applicable)	MM/YY	FIELD OF STUDY
National Taiwan University	B.S.	1974	Biochemistry
University of California, Berkeley	Ph.D.	1980	Biochemistry
Massachusetts Institute of Technology	Postdoc	80-83	Cancer Biology

### A. Personal Statement

My research expertise is in the biology of cancer. My laboratory employs biochemistry, cell biology, molecular biology, mouse genetics and high throughput technologies to interrogate the functions of several cancer genes, in particular, ABL and RB, in the regulation of cancer cell response to radiation and chemotherapeutic drugs.

I cloned the *ABL* gene during my postdoc training and proved it to encode a tyrosine kinase. My lab discovered the BCR coiled-coil domain and its essential role in the constitutive activation of the tyrosine kinase and F-actin binding functions of ABL in the BCR-ABL fusion protein of chronic myelogenous leukemia (CML). We also discovered the nucleo-cytoplasmic shuttling of ABL and demonstrated its regulation by cell adhesion and DNA damage. We showed that activation of the nuclear ABL kinase by DNA damage stimulates the apoptotic response. We showed that nuclear entrapment of the BCR-ABL kinase converts it from a survival factor into a death inducer. We inactivated the three nuclear localization signals in the mouse *Ab1* gene and showed that loss of nuclear ABL reduced the renal apoptotic response to cisplatin. Using this mouse model, we have discovered that nuclear ABL is required for the *post-transcriptional* up-regulation of PUMA- $\alpha$  expression, and for the *processing* of pro-apoptotic microRNA, e.g., miR34c.

We have established a mouse model for CML in which we could identify, isolate, and propagate leukemic stem cells in *ex vivo* culture and in syngeneic mice. With this model, we have induced resistance to BCR-ABL tyrosine kinase inhibitors by triggering the formation of Bim-resistant mitochondria. In other words, we have identified a mitochondrial switch that can rapidly induce a drug-resistant state in CML cells. I have held two patents relating to the detection and treatment of CML.

My lab discovered the cell-cycle-regulated phosphorylation of the retinoblastoma protein RB. We also discovered the protein-scaffolding function of RB and showed that RB must be able to assemble protein complexes in order to suppress cell proliferation. Following our discovery that the growth suppression and the apoptosis suppression functions of RB can be separated by specific mutations, we have been focusing on the role of RB in the suppression of TNF-induced apoptosis. We discovered that RB is cleaved by caspase at a C-terminal site and generated a cleavage-resistant *Rb1* allele (*Rb-MI*) in mice. We showed that the *Rb-MI* intestinal epithelial cells are resistant to inflammation and TNF induced apoptosis, and that *Rb-MI* promotes colon cancer in combination with p53-loss.

My lab has recently developed methods to conduct functional screens of human genome-wide shRNA libraries in combination with massive parallel sequencing of shRNA amplicons recovered from pools of cells. Using these methods, we have identified novel regulators of cell adhesion and of the cell death response to deprivation of cell-matrix contact. Our approach can be applied to identify gene networks underlying the response of cancer cells to conventional or oncogene-targeted cancer drugs.

Our research projects are designed to gain knowledge on fundamental processes that are relevant to cancer biology and to train the next generation of investigators. Over the past 29 years as a faculty at UCSD, I have

served as the thesis advisor of sixteen Ph.D. students and as a member of the Ph.D. thesis committees of more than a hundred other graduate students. I have also mentored the postdoctoral research training of approximately 60 Ph.D. and M.D./Ph.D. fellows. In recent years, I have been actively involved in the recruitment and training of under-represented minority students in the Biological Sciences and the Biomedical Sciences graduate programs at UCSD. The majority of my former trainees are holding faculty positions at research universities and institutions in the United States, Europe, Japan, Taiwan and China. Other former trainees conduct research in the pharmaceutical and biotechnology industry, and are founders of start-up biotechnology companies.

## **B. Positions and Honors**

### Academic Positions:

1983-1988 Assistant Professor, Department of Biology, UC, San Diego, La Jolla, CA.

1988-1993 Associate Professor, Department of Biology, UC, San Diego, La Jolla, CA.

1993-2005 Professor, Division of Biological Sciences, UC, San Diego, La Jolla, CA.

1997-2014, Associate Director of Basic Research, Moores-UCSD Cancer Center, La Jolla, CA.

2005-2007 Professor, Div. of Hematology-Oncology, Dept. of Medicine, School of Medicine, UC, San Diego, La Jolla CA

2007-Present, Distinguished Professor of Medicine and Biology, UC, San Diego, La Jolla, CA

2008-2010, Chair, Biomedical Sciences Graduate Program, UC, San Diego, La Jolla, CA

### Editorial Positions (selected):

1995-Present, Editorial Board, Molecular Cancer Research; 1997-2005, Editor, Molecular and Cellular Biology

1999-2004, Editorial Board, Cell Death & Differentiation; 1999-2002, Editorial Board, Encyclopedia of Cancer, Second Edition; 2010-Present, Editorial Board, Cancer Research.

### International and National Committees (selected):

1986-1991, Scientific Advisory Committee on Cell and Developmental Biology, American Cancer Society; 1991-1995, Molecular Biology Study Section, National Institutes of Health; 1995-1996, Chair, Cornelius P. Rhoads Award Committee, American Association for Cancer Research; 1996-2000, Board of Scientific Counselors, National Cancer Institute; 1998-2001, Board of Directors, American Association for Cancer Research; 1999-2000, Chair, Career Development Award Committee, American Association for Cancer Research; 2000-2001, Chair, Gertrude B. Elion Award Committee, American Association for Cancer Research; 2001-2003, Selection Committee, Alfred P. Sloan Prize, General Motors Cancer Research Foundation; 2001-2005, Subcommittee A on Cancer Centers, National Cancer Institute; 2002-Present, External Scientific Advisory Board, University of New Mexico Cancer Research and Treatment Center; 2004, Kirk A. Landon-AACR Prize Committee for Basic Cancer Research; 2005-2006, Program Committee, AACR Annual Meeting; 2005-Present, Scientific Advisory Board, William Guy Forbeck Research Foundation; 2006-Present, External Scientific Advisory Board, Chao Family Comprehensive Cancer Center, UC Irvine; 2006-2011, Board of Scientific Advisors, NCI, NIH; 2007-Present, Scientific Advisory Board, Sanford/Burnham Cancer Center; 2009-2012, Member, Review Committee, Cancer Prevention and Research Institute of Texas; 2010-present, Scientific Advisory Board, Sanford/Burnham Institute for Medical Research.

### Honors and Awards (selected):

1970-1974, Taiwanese Ministry of Education Fellowship on Culture and Science; 1970-1974, Book Cupon Award, National Taiwan University; 1980-1983, Postdoctoral Fellow, Jane Coffin Childs Memorial Fund for Biomedical Research; 1983, Junior Faculty Award, The Camille and Henry Dreyfus Foundation; 1984, Searle Scholar; 1994, Outstanding Teacher Award, Earl Warren College, UC, San Diego; 1995, Chair, Gordon Research Conference on Cancer; 1995, Burroughs Wellcome Visiting Professorship; 1997, MERIT Award, National Cancer Institute; 1998, Co-organizer, Keystone Symposium on the Cell Cycle; 2000, Herbert Stern Endowed Chair; 2006, Fellow, The American Academy of Microbiology; 2008, Chair, Gordon Research Conference on Signal Transduction; 2011, Director's Service Award, National Cancer Institute, USA; 2011, Fellow, The American Academy of Arts and Sciences.

## **C. Selected Publications Relevant to the Proposed Research (from a total of 199)**

1. Vigneri, P. and **J. Y. J. Wang**. (2001). Induction of apoptosis in chronic myelogenous leukemia cells through nuclear entrapment of BCR-ABL tyrosine kinase. *Nature Med.* 7: 228-234. **PMID:11175855**
  2. Chau, B.N., H. Borges, T-T Chen, A. Masselli, I.C. Hunton, and **J. Y. J. Wang** (2002). Signal dependent protection from apoptosis in mice with caspase-resistant RB. *Nature Cell Biol.* 4: 757-765. **PMID:12360286**
-

Basic Cancer Research Panel 2  
Carol Prives, Ph.D., Chair

Peer Review Panel Members for Approval

1. Shelly Berger, Ph.D.
2. James Manley, Ph.D.
3. John Petrini, Ph.D.
4. Ali Shilatifard, Ph.D.
5. Nahum Sonenberg, Ph.D.



## BIOGRAPHICAL SKETCH

NAME Berger, Shelley L.	POSITION TITLE Professor		
eRA COMMONS USER NAME SBERGER			
EDUCATION/TRAINING <i>(Begin with baccalaureate or other initial professional education, such as nursing, and include postdoctoral)</i>			
INSTITUTION AND LOCATION	DEGREE <i>(if applicable)</i>	YEAR(s)	FIELD OF STUDY
University of Michigan, Ann Arbor, MI	Ph.D.	1987	Cellular & Molecular Bio
University of Michigan, Ann Arbor, MI	B.S.	1982	Biology

### A. Personal Statement

Shelley Berger, Ph.D., is the Daniel S. Och University Professor at University of Pennsylvania, and is a faculty member in the Cell & Developmental Biology Department and the Genetics Department in the Perelman School of Medicine, as well as the Biology Department in the School of Arts and Sciences. Dr. Berger also serves as Director of the Epigenetics Program in Penn School of Medicine. Dr. Berger earned her PhD from University of Michigan and was a post-doctoral fellow at Massachusetts Institute of Technology. She previously held the Hilary Koprowski Professorship at Wistar Institute in Philadelphia. Dr. Berger's research focuses on the role of histone and factor post-translations modifications in chromatin regulation and of the tumor suppressor p53; her lab investigates chromatin mechanisms and physiology during senescence and aging, gametogenesis, and underlying organismal level behavior. Dr. Berger has organized numerous international meetings on epigenetics and chromatin, has served as Senior Editor at *Molecular and Cellular Biology*, and participates on advisory committees for several research institutions and chromatin-focused biotechnology companies. She has served on international committees to establish nomenclature for histone modifying enzymes, and to help create the NIH-sponsored Human Epigenome Project. Dr. Berger is the recipient of an Ellison Foundation Senior Scholar Award and an HHMI Collaborative Research award. She is an elected Fellow of AAAS, and elected member of the American Academy of Arts and Sciences, and elected member of Institute of Medicine in the National Academies of Sciences.

### B. Positions and Honors:

2009-present	Director, Epigenetics Program, University of Pennsylvania School of Medicine
2009-present	Professor, Departments Cell and Developmental Biology, Genetics, and Biology, Penn
2002-2009	Professor, Wistar Institute
1998-2002	Associate Professor, Wistar Institute
1993-1997	Assistant Professor, Wistar Institute
1994-2008	Adjunct Professor, Departments of Biology and Genetics, Penn
1989-1992	Postdoctoral Fellow, Dept Biology, MIT (mentor: Leonard Guarente);
1988-1989	Postdoctoral Fellow, Dept Biochem and Molec Biol, Harvard (mentor: Matthew Meselson)
1982-1987	Graduate student, Dept. of Biochemistry, Univ of Michigan (mentor: William Folk)

Elected member of American Academy of Arts and Sciences (2013)

Elected member of Institute of Medicine of National Academies of Sciences (2012)

Elected fellow of AAAS (2012)

HHMI Collaborative Research Award (2008-2012; 2012-2016)

Daniel S. Och Endowed Chair, Penn (2009-present)

PIK Professorship ("Penn Integrates Knowledge"), Penn (2009-present)

Ellison Foundation Senior Scholar Award (2010)

Hilary Koprowski Endowed Professorship, The Wistar Institute (2003-2009)

Keynote lectures: NIH Director's series, Florence Mahoney Memorial lecture (14); Wellcome Trust Conference "Epigenetics and Disease", Baltimore (12); CRG Epigenetics Conference, Barcelona, Spain (11); WABI Bioinformatics Conference, Penn, Philadelphia (09); CSBMCB Chromatin Meeting, Banff (08); CMB symposium, Univ of Mich, Ann Arbor (06)

NIH Shannon Award (1998-99)  
ACS Junior Faculty Research Award (1994-97)  
Charles King Trust postdoctoral fellowship (1990-92)  
NIH postdoctoral fellowship (1988-90)  
Arthritis Foundation postdoctoral fellowship (1987-88)  
NIH predoctoral award (1982-85)

#### **Journal Editorial Boards, Conference Organization, Committee membership, and Consultancies**

Editorial Boards: *Aging Cell* (13-present); *Epigenetics and Chromatin* (08-present); *Epigenomics* (08-present)  
*Nature Scientific Reports* Editorial Advisory Panel (10-present)  
Senior Editor, *Molecular and Cellular Biology*, (03-08); Board Member, (99-03)  
Perelman School of Medicine Committee on Prestigious Awards and Honors (13-16)  
Conference Organization: CSH "Chromatin" (14); Keystone "Epigenetic Mechanisms" (14); CSH "Chromatin" (12); FASEB "Chromatin" (11); Banbury/CSH Lab meeting "Epigenetics: Mechanisms and Regulation (08); EMBL Transcription Meeting, Heidelberg (08); ESRF Schering Workshop, "Histone Modifications and Cancer", Berlin (05); Keystone Conference, "Histone Modification Pathways", Snowbird, Utah (05); Wistar Institute conference "Chromatin, Transcription and Epigenetics" (04); Keystone Conference, "Enzymology of Transcription and Chromatin", Sante Fe (03)  
Organization of five Epigenetic Symposia at Penn (10-14)  
Executive Committee, Institute of Regenerative Medicine, UPenn, Philadelphia (08-present)  
Member, AACR "Human Epigenome" AHEAD Task Force (06-10)  
Member, Nomenclature Committee for histone modifying enzymes, Chair of acetylase sub-committee (06-07)  
Member of "Faculty of 1000" on-line review  
Consultancies: Cell Centric (08-10); Lake Placid Biologicals (08); Smithkline Beecham Pharmaceutical (93-94)

#### **Grant and Manuscript Reviews, Departmental Reviews, and Scientific Advisory Boards**

Scientific Advisory Panel, Novartis, Cambridge, MA (2011)  
Scientific Advisory Board, Gladstone Institute, San Francisco (06-present)  
Scientific Advisory Board, IGBMC Institute, Strasbourg, France (05-08)  
Scientific Advisory Board, Chroma Inc., Cambridge, UK (00-08)  
St. Jude's Department of Biochemistry site review panel, Memphis, TN (04)  
Wellcome Trust/Cancer UK Institute, Cambridge UK, site visit (03)  
NIH: MGB grant Panel (12); NIH, Chair review P01 for NIA (10); CDF2 panel, Molecular Cytology, member (99-03); NIH NICHD site review panel (04; 08; 11)  
NSF, Bio/MCB panel, member (97-99)  
Reviewer for: *Science*, *Cell*, *Molecular Cell*, *Nature*, *Nature Genetics*, *Nature Cell Biology*, *Nature Reviews*, *Genes & Develop*, *Mol Cell Biol*, *EMBO*, *Genetics*, *Curr Biol*, *PNAS*

#### **Invited lectures and international conferences/symposia:** 1993-2014, total = 320 (attended only)

##### **In 2014**

NIH Director's Series, National Institute of Aging Florence Mahoney Memorial Lecture, Bethesda MD  
Harvard Medical School seminar, Biochemistry department, Boston MA  
Cornell University seminar, Genetics department, Ithaca, NY  
Cold Spring Harbor Transcription course, lecture, CSH NY  
IDIBELL Cancer Conference (ICC), "50 Years of Histone Acetylation - Barcelona", Barcelona, Spain  
Keystone Conference, "Chromatin Mechanisms and Physiology", Munich, Germany  
Gordon Conference, "Chromatin Structure and Function", Bentley College, Waltham MA  
EMBL Conference, "Transcription Mechanisms", Heidelberg, Germany  
Cold Spring Harbor Conference, Chromatin and Epigenetics, CSH NY  
NYU Medical School symposium, "Epigenetics", NY  
UNC Cancer Center symposium, Chapel Hill, NC

#### **C. Selected publications** (in reverse chronological order; total = 150)

1. Dang W, Sutphin, GL, Dorsey JA, Otte GL, Cao K, Perry RM, Wanat JJ, Saviolaki D, Murakami CJ, Tsuchiyama S, Robison B, Gregory BD, Vermeulen M, Shiekhatair R, Johnson FB, Kennedy BK,

## BIOGRAPHICAL SKETCH

Provide the following information for the key personnel and other significant contributors.  
Follow this format for each person. **DO NOT EXCEED FOUR PAGES.**

NAME <b>James L. Manley</b>	POSITION TITLE  <b>J. C. Levi Professor of Life Sciences</b>		
eRA COMMONS USER NAME <b>JLManley</b>			
EDUCATION/TRAINING <i>(Begin with baccalaureate or other initial professional education, such as nursing, and include postdoctoral training.)</i>			
INSTITUTION AND LOCATION	DEGREE <i>(if applicable)</i>	YEAR(s)	FIELD OF STUDY
Columbia University	B.S.	1971	Biology
State University of New York, Stony Brook, NY	Ph.D.	1976	Molecular Biology
Massachusetts Institute of Technology	Postdoc	1980	Gene Expression

### A. Personal Statement

My laboratory has studied the mechanisms and regulation of mRNA transcription, splicing and 3' end formation in eukaryotic cells for many years. We have also elucidated important links between these processes, and shown how they are integrated with other cellular events including cell cycle, DNA damage, differentiation and disease. Our studies relevant to cancer involve investigation of the role of splicing regulatory proteins in glioblastoma, and of mutations affecting components of the core splicing machinery in myelodysplastic syndrome and leukemia.

### B. Positions and Honors.

#### Professional Experience:

Chairman; Department of Biological Sciences, Columbia University 1995-2001  
 Julian Clarence Levi Professor of Life Sciences, Columbia University 1995-  
 Professor; Department of Biological Sciences, Columbia University 1987-  
 Associate Professor; Department of Biological Sciences, Columbia University 1985-1987.  
 Assistant Professor; Department of Biological Sciences, Columbia University 1980-1985.  
 Research Associate; Massachusetts Institute of Technology; Dr. M.L. Gefter, supervisor 1977-1980.  
 Graduate Research: Cold Spring Harbor Laboratory; Dr. R.F. Gesteland, supervisor 1972-1976.

#### Honors and Service:

1976-1977	Anna Fuller Fellowship	1983-1985	Editorial Board, Nucleic Acids Research
1996-2006	NIH MERIT Award (mRNA splicing)	1984-2001	Editorial Board, Mol. Cell. Biol.
2002-	Fellow, American Academy of Microbiology	1988-	Editorial Board, Genes and Dev.
2002-	Board of Directors, Cold Spring Harbor Alumni Association	1989-1992	Editorial Board, Techniques
2005-	ISI Highly Cited Researcher	1991-1995	Editorial Board, Mechanisms of Dev.
2006-2009	Senior Fellow, American Asthma Foundation	1991-	Associate Editor, Gene Expression
2006-	Fellow, American Academy of Arts and Sciences	1993-1998	Editorial Board, Journal of Virology
2007-	Edina High School Hall of Fame	1994-	Editorial Board, RNA
2008-	Fellow, American Association for the Advancement of Science	1997-	Editorial Board, Molecular Cell
2011-	Member, National Academy of Sciences	2001-	Editorial Advisor, BioMedCentral-Mol. Biol.
		2003-	Editor, Mol. Cell. Biol.
		2003-	Editorial Board, BioMedCentral-Biology
		2006-	Editorial Board, Recent Patents on DNA & Gene Sequences
		2010-	Editorial Board, Transcription
		2012-	Senior Editor, eLife
		2012-	Editorial Board, Methods
1989-2013	Co-organizer, RNA 3' End Formation Meetings, Oxford, England (7)		
1990, 1991	Co-organizer, RNA Processing Meeting, Cold Spring Harbor Laboratory		
1997, 1999	Co-organizer, Eukaryotic mRNA Processing Meeting, Cold Spring Harbor Laboratory		

1988-1991 Member, ACS Microbiology and Virology Committee  
1989-1993 Member, NIH Molecular Biology Study Section  
1999-2002 Member, NIH Molecular Cytology Study Section (CDF-2) (Chair, 2000-2002)

### C. Publications (since 2010):

266. Tan, A.Y. and Manley, J.L. (2010). TLS inhibits RNA polymerase III transcription. *Mol. Cell. Biol.* **30**, 186-196. PMID: PMC2798296.
267. Chen, L.S., Du-Cuny, L., Vethantham, V., Hawke, D.H., Manley, J.L., Zhang, S., Gandhi, V. (2010). Chain termination and inhibition of mammalian poly(A) polymerase by modified ATP analogues. *Biochem. Pharmacol.* **79**, 669-677. PMID: PMC2812641.
268. Mutsuddi, M. Mukherjee, A., Shen, B., Manley, J.L. and Nambu, J.R. (2010). Drosophila Pelle phosphorylates Dichaete protein and influences its subcellular distribution in developing oocytes. *Int. J. Dev. Biol.* **54**, 1309-15.
269. David, C.J., Chen, M., Assanah, M.C., Canoll, P. and Manley, J.L. (2010). HnRNP proteins controlled by c-Myc deregulate pyruvate kinase mRNA splicing in cancer. *Nature* **463**, 364-368. PMID: PMC2950088.
270. Pedrotti, S., Bielli, P., Paronetto, M.P., Ciccocanti, F., Fimia, G.M., Stamm, S., Manley, J.L. and Sette, C. (2010). The splicing regulator Sam68 binds to a novel exonic splicing silencer and functions in *SMN2* alternative splicing in Spinal Muscular Atrophy. *EMBO J.* **29**, 1235-1247. PMID: PMC2857462.
271. Rosonina, E. and Manley, J.L. (2010). Alternative polyadenylation blooms. *Dev. Cell* **18**, 172-174.
272. Chen, M., David, C.J. and Manley, J.L. (2010). Tumor metabolism: hnRNP proteins get in on the act. *Cell Cycle* **9**, 1863-1864.
273. Manley, J.L. and Krainer, A.R. (2010). A rational nomenclature for serine/arginine-rich protein splicing factors (SR proteins). *Genes Dev.* **24**, 1073-1074. PMID: PMC2878644.
274. Rosonina, E., Duncan, S.M. and Manley, J.L. (2010). SUMO functions in constitutive transcription and during activation of inducible genes in yeast. *Genes Dev.* **24**, 1242-1252. PMID: PMC2885660.
275. García, A., Rosonina, E., Manley, J.L. and Calvo, O. (2010). Sub1 globally regulates RNA polymerase II C-terminal domain phosphorylation. *Mol. Cell. Biol.* **30**, 5180-5193. PMID: PMC2953048.
276. Xiang, K., Nagaike, T., Xiang, S., Kilic, T., Beh, M.M., Manley, J.L. and Tong, L. (2010). Crystal structure of the human symplekin-Ssu72-CTD phosphopeptide complex. *Nature* **467**, 729-733. PMID: PMC3038789.
277. Chen, M., Zhang, J. and Manley, J.L. (2010). Turning on a fuel switch of cancer: hnRNP proteins regulate alternative splicing of pyruvate kinase mRNA. *Cancer Res.* **70**, 8977-8980. PMID: PMC2982937.
278. David, C.J. and Manley, J.L. (2010). Alternative pre-mRNA splicing regulation in cancer: Pathways and programs unhinged. *Genes Dev.* **24**, 2343-2364. PMID: PMC2964746.
279. Bai, Y., Srivastava, S.K., Chang, J.H., Manley, J.L. and Tong, L. (2011). Structural basis for dimerization and activity of human PAPD1, a noncanonical poly(A) polymerase. *Mol. Cell* **41**, 311-320. PMID: PMC3057501.
280. Chang, J.H., Xiang, S., Xiang, K., Manley, J.L. and Tong, L. (2011). Structural and biochemical studies of the 5'→3' exoribonuclease Xrn1. *Nat. Struct. Mol. Biol.* **18**, 270-276. PMID: PMC3075561.
281. Shi, Y., Nishida, K., Campigli Di Giammartino, D. and Manley, J.L. (2011). Heat shock-induced SRSF10 dephosphorylation displays thermotolerance mediated by Hsp27. *Mol. Cell. Biol.* **31**, 458-465. PMID: PMC3028621.
282. Nagaike, T., Logan, C., Hotta, I., Rozenblatt-Rosen, O., Meyerson, M. and Manley, J.L. (2011). Transcriptional activators enhance polyadenylation of mRNA precursors. *Mol. Cell* **41**, 409-418. PMID: PMC3060669.
283. David, C.J., Boyne, A.R., Millhouse, S.R. and Manley, J.L. (2011). The RNA polymerase II C-terminal domain promotes splicing activation through recruitment of a U2AF65-Prp19 complex. *Genes Dev.* **25**, 972-983. PMID: PMC3084030.
284. Nagaike, T. and Manley J.L. (2011). Transcriptional activators enhance polyadenylation of mRNA precursors. *RNA Biol.* **8**, 964-967. PMID: PMC3256420.
285. David, C.J. and Manley, J.L. (2011). The RNA polymerase C-terminal domain: A new role in spliceosome assembly. *Transcription* **2**, 221-225. PMID: PMC3265779.
286. Campigli Di Giammartino, D., Nishida, K. and Manley, J.L. (2011). Mechanisms and consequences of alternative polyadenylation. *Mol. Cell* **43**, 853-866. PMID: PMC3194005.

**BIOGRAPHICAL SKETCH**

Provide the following information for the Senior/key personnel and other significant contributors in the order listed on Form Page 2.  
Follow this format for each person. **DO NOT EXCEED FOUR PAGES.**

NAME <b>John H.J. Petrini</b>	POSITION TITLE <b>Professor and Member</b>		
eRA COMMONS USER NAME (credential, e.g., agency login) <b>petrinij</b>			
EDUCATION/TRAINING <i>(Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable.)</i>			
INSTITUTION AND LOCATION	DEGREE <i>(if applicable)</i>	MM/YY	FIELD OF STUDY
Kalamazoo College, Kalamazoo, MI	BA	1/82	Chemistry
University of Michigan Medical School Ann Arbor, MI	Ph.D.	1/88	CMB

**A. Personal Statement**

I have 18 years of experience as an independent investigator, and have been funded by NIGMS for the last 15 and years. Research in my laboratory comprises a multidisciplinary approach to understanding the mediators and mechanisms by which genomic integrity is maintained. The integration of genetic models in mice and budding yeast with cell biological and biochemical analyses has provided detailed mechanistic insight regarding DNA recombination, DNA damage signaling, and tumor suppression. The overarching goal of our research program is to define the mechanisms underlying the response to DNA double strand breaks (hereafter designated the DNA damage response (DDR)). The human Mre11 complex, consisting of Mre11, Rad50, and Nbs1 (Xrs2 replaces Nbs1 in the *S. cerevisiae* complex) was identified in our laboratory in 1996. Since that time, we have come to appreciate, and have shed light on the intimate relationship between the DNA damage response and cancer. This new grant is a manifestation of the evolving focus of the lab, and reflects the fact that spontaneous DSBs in S phase cells is a primary issue in the maintenance of genome integrity and the suppression of malignancy. We have assembled a diverse and unique set of tools to address this fundamentally important issue which has been illuminated by, and is complementary to our previous and ongoing work.

**B. Positions and Honors**

1988-1989 **Postdoctoral Associate**, Fox Chase Cancer Center.  
In the laboratory of Dr. M. J. Bosma.

1989-1994 **Postdoctoral Associate**, Dana-Farber Cancer Institute.  
In the laboratory of Dr. David Weaver.

1994 **Assistant Professor**, Department of Medical Genetics  
University of Wisconsin Medical School.

1999 **Associate Professor** (with tenure), Department of Medical Genetics  
University of Wisconsin Medical School.

2002 **Member**, Memorial Sloan Kettering Cancer Center.  
2002 Professor, Weill Graduate School of Medical Sciences at Cornell University.

**Honors**

1993-1996 Leukemia Society of America Special Fellowship

1996-1998 Basil O'Connor Scholar of The March of Dimes Foundation

1996-2000 Milwaukee Foundation Shaw Scientist Award

1999-2001 Michael Fry Award of The Radiation Research Society

2000 The Joel and Joan Smilow Initiative for Research in Genomic Integrity

2001 Paul A. Marks Chair in Molecular Cell Biology

#### Other Experience and Professional Memberships

2005-2010	Senior Editor, Molecular Cancer Research
2009-Present	Genome Integrity Joint Editor-in-Chief
2010-Present	Board of Review Editors, Science
2008-2011	Science Foundation of Ireland Principal Investigator Program, Executive Panel Member
2008-2012	Member of the Cancer Etiology Study Section
2009- 2012	Member of CPRIT Review Panel BRRC3
2003-Present	Founding Organizer, Genome Integrity Discussion Group New York Academy of Sciences
2012	Member of Executive Committee, American-Italian Cancer Foundation Scientific Advisory Board

#### **C. Selected Peer-reviewed Publications (of 87)**

1. Dolganov, G.M., Maser, R.S., Novikov, A., Tosto, L., Chong, S., Bressan, D.A., and **Petrini, J.H.** (1996). *Human Rad50 is physically associated with human Mre11: identification of a conserved multiprotein complex implicated in recombinational DNA repair.* Mol Cell Biol, **16**. 4832-4841.
2. Maser, R.S., Monsen, K.J., Nelms, B.E., and **Petrini, J.H.** (1997). *Mre11 and hRad50 nuclear foci are induced during the normal cellular response to DNA double-strand breaks.* Mol Cell Biol, **17**. 6087-6096.
3. Carney, J.P., Maser R.S., Olivares, H., Davis, E.M., Le Beau, M., Yates JR 3<sup>rd</sup>, Hays, L., Morgan W.F., and **Petrini, J.H.** (1998). *The hMre11/hRad50 protein complex and Nijmegen breakage syndrome: linkage of double-strand break repair to the cellular DNA damage response.* Cell, **93**. 477-486.
4. Nelms, B.E., Maser, R.S., MacKay, J.F., Lagally M.G., and **Petrini, J.H.** (1998). *In situ visualization of DNA double-strand break repair in human fibroblasts.* Science, **280**. 590-592.
5. Bressan, D.A., Baxter, B.K. and **Petrini, J.H.** (1999). *The Mre11-Rad50-Xrs2 protein complex facilitates homologous recombination-based double-strand break repair in Saccharomyces cerevisiae.* Mol Cell Biol, **19**. 7681-7687.
6. Luo, G., Yao, M.S. Bender, C.F., Mills, M., Bladl, A.R., Bradley, A. and **Petrini, J.H.**, (1999). *Disruption of mRad50 causes embryonic stem cell lethality, abnormal embryonic development, and sensitivity to ionizing radiation.* Proc Natl Acad Sci U S A, **96**. 7376-7381.
7. Stewart, G.S., Maser, R.S., Stankovic, T., Bressan, D.A., Kaplan, M.I., Jaspers, N.G., Raams, A., Byrd, P.J., **Petrini, J.H.**, and Taylor, A.M. (1999). *The DNA double-strand break repair gene hMRE11 is mutated in individuals with an ataxia-telangiectasia-like disorder.* Cell, **99**. 577-587.
8. Lim, D.S., Kim, S.T., Xu, B., Maser, R.S., Lin, J., **Petrini, J.H.**, and Kastan, M.B. (2000). *ATM phosphorylates p95/nbs1 in an S-phase checkpoint pathway.* Nature, **404**. 613-617.
9. Wu, X., **Petrini, J.H.**, Heine W.F., Weaver D.T., Livingston, D.M. and Chen, J. (2000). *Independence of R/M/N focus formation and the presence of intact BRCA1.* Science, **289**. 11.
10. Zhu, X.D., Kuster, B., Mann, M. **Petrini, J.H.**, and de Lange, T. (2000). *Cell-cycle-regulated association of RAD50/MRE11/NBS1 with TRF2 and human telomeres.* Nat Genet, **25**. 347-352.
11. Maser, R.S., Zinkel R., and **Petrini, J.H.** (2001). *An alternative mode of translation permits production of a variant NBS1 protein from the common Nijmegen breakage syndrome allele.* Nat Genet, **27**. 417-421.
12. Mirzoeva, O.K. and **Petrini, J.H.** (2001). *DNA damage-dependent nuclear dynamics of the Mre11 complex.* Mol Cell Biol, **21**. 281-288.
13. Usui, T., Ogawa H., and **Petrini, J.H.** (2001). *A DNA damage response pathway controlled by Tel1 and the Mre11 complex.* Mol Cell, **7**. 1255-1266.
14. Maser, R.S., Mirzoeva, O.K., Wells, J., Olivares, H., Williams, B.R., Zinkel, R.A., Farnham, P.J., and **Petrini, J.H.** (2001). *The MRE11 complex and DNA replication: linkage to E2F and sites of DNA synthesis.* Mol Cell Biol, **21**. 6006-6016.
15. Lee, S. E., Bressan, D. A., **Petrini, J.H.**, and Haber, J. E. (2002). *Complementation between N-terminal Saccharomyces cerevisiae mre11 alleles in DNA repair and telomere length maintenance.* DNA Repair, **1**. 27-40.
16. Bender, C. F., Sikes M. L., Sullivan R., Huye L.E., Le Beau M. M., Roth D. B., Mirzoeva, O.K., Oltz E. M., and **Petrini, J.H.** (2002). *Cancer predisposition and hematopoietic failure in Rad50S/S mice.* Genes Dev, **16**. 2237-2251.
17. Williams, B. R., Mirzoeva, O. K., Morgan, W. F., Lin, J., Dunnick, W. and **Petrini, J.H.** (2002). *A murine model of Nijmegen breakage syndrome.* Current Biology, **12**. 648-653.
18. Falck, J., **Petrini, J.H.**, Williams, B. R., Lukas, J., and Bartek, J. (2002). *The DNA damage-dependent intra-S phase checkpoint is regulated by parallel pathways.* Nat Genet, **30**. 290-294.

**BIOGRAPHICAL SKETCH**

Provide the following information for the Senior/key personnel and other significant contributors.  
Follow this format for each person. **DO NOT EXCEED FOUR PAGES.**

NAME Shilatifard, Ali	POSITION TITLE Investigator		
eRA COMMONS USER NAME (credential, e.g., agency login) shilatia			
EDUCATION/TRAINING (Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable.)			
INSTITUTION AND LOCATION	DEGREE (if applicable)	MM/YY	FIELD OF STUDY
Kennesaw State University	B.S.	06/89	Organic Chemistry
University of Georgia		07/92	Biochemistry
University of Oklahoma	Ph.D.	08/94	Biochemistry
Oklahoma Medical Research Foundation	Postdoctoral	11/97	Biochemistry

**A. Personal Statement**

Chromosomal translocations that fuse mixed lineage leukemia 1 (*MLL1*), a histone H3K4 methylase, to any one of a large number of translocation partners is indicative of a poor clinical outcome in acute leukemias. The *MLL* translocations are the most common translocations seen in infant leukemia. Most of the *MLL* translocation partners share little sequence or functional similarities and the mechanism by which they induce leukemia has been unclear until recently. I identified RNA polymerase II (Pol II) elongation factor ELL as the first translocation partner of *MLL* for which a function was determined. Based on this finding, ~17 years ago my laboratory proposed that the regulation of the rate of transcription elongation by Pol II could play a central role in *MLL*-based leukemogenesis. Recent studies from my laboratory have identified some of the most frequently occurring translocation partners of *MLL* as components of three newly discovered complexes: the ELL-containing Super Elongation Complex (SEC), the ELL-containing Little Elongation Complex (LEC) and DotCom, a histone methyltransferase complex. These findings suggest that *MLL* fusion proteins are recruited to *MLL* target genes within SEC or DotCom and license Pol II to elongate without the appropriate checkpoints, leading to an unregulated expression of *MLL* targets. Furthermore, studies from my lab have demonstrated that under normal developmental conditions, SEC functions in the regulation of rapid transcriptional induction via paused RNA Pol II and that LEC is required for RNA Pol II transcribed snRNA genes. Overall, these studies suggest the presence of specific classes of elongation factors for each class of genes transcribed by RNA polymerase II and that transcriptional elongation control is central for development and its misregulation can result in cancer pathogenesis

Based on the studies from my laboratory during the past seventeen years, I have presented over 250 invited lectures on our research at scientific meetings and universities in the U.S. and at international institutes and universities. Our studies have been continuously supported by the National Institute of Health. In addition to the ongoing research in my lab, I have served my community as a member of a NIH CDF1/MGB panel from 2001-2006, member of the Cancer Molecular Pathology study section from 2007-2012, as a member of the Editorial Board of *Journal of Biological Chemistry* (2002-2007) and currently serving as the Editor of *Molecular and Cellular Biology* since 2007-2017. I am committed to working very hard to discover information that can be used for the targeted treatment of leukemia and lymphomas caused as a result of mutations or translocations within transcription factors.

## B. Positions and Honors

### Positions

2007-present	Investigator, Stowers Institute for Medical Research
2007-present	Editor, <i>Molecular and Cellular Biology</i>
2004-present	Instructor, Cold Spring Harbor Laboratory, Gene Expression Course
2006	James B. and Joan C. Peters Endowed Chair (due to move to the Stowers Institute, did not accept this endowed position)
2005-2007	Professor of Biochemistry, Saint Louis University School of Medicine
2002-2007	Associate Director for Basic Sciences, Saint Louis University Cancer Center
2002-2005	Associate Professor of Biochemistry with tenure, Saint Louis University School of Medicine
1997-2002	Assistant Professor of Biochemistry, Saint Louis University School of Medicine
1995-1997	Jane Coffin Childs Postdoctoral Fellow, Program in Molecular Biology, Oklahoma Medical Research Foundation

### Other Experience and Professional Memberships

2014	Scientific Advisory Board, Keystone Symposia
2011-Present	Scientific Advisory Board, Israel Centers of Research Excellence program (I-CORE)
2011-Present	Scientific Advisory Board, Cell Signaling Technology
2011-Present	Fox Chase Cancer Center, Keystone External Advisory Board
2011	Novartis, Scientific Advisory Board
2011-Present	Scientific Advisory Board, Starr Cancer Consortium
2009-Present	Cancer Molecular Pathology (CAMP) study section member
2004-2007	Member, MGB/CDF1 study section National Institute of Health
2002-2007	Editorial Board member, <i>Journal of Biological Chemistry</i>
2003-2005	Member, Genetic Mechanisms in Cancer (GMC) study section, American Cancer Society
Presently	Ad hoc reviewer, <i>Cell</i> , <i>Science</i> , <i>Nature</i> , <i>Molecular Cell</i> , <i>Nature Cell Biology</i> , <i>Nature Structural and Molecular Biology</i> , <i>Genes and Development</i> , and <i>Proceedings of the National Academy of Sciences</i>

### Honors

2007	Innovation Award, Academy of Sciences, St. Louis
2006	ASBMB-AMGEN Award
2006	American Cancer Society Award of Excellence
2006	Stohlman Scholar, Leukemia & Lymphoma Society (Due to schedule conflict was not able to accept)
2002	Recipient of the Sword of the American Cancer Society
2001-2006	Scholar of the Leukemia and the Lymphoma Society
1999-2002	Edward Mallinckrodt, Jr. Young Investigator
1995-1997	Jane Coffin Childs Postdoctoral Fellow

## C. Selected Peer-reviewed Publications (Selected from over 130 peer-reviewed publications)

1. Thornton, J., Westfield, G. H., Takahashi, Y-H., Cook, M., Gao, X., Woodfin, A. R., Lee, J-S., Morgan, M. A., Jackson, J., Smith, E. R., Couture, J-F., Skiniotis, G., and **Shilatifard, A** (2014) Context Dependency of Set1/COMPASS Mediated H3 Lysine 4 Trimethylation. *Genes and Dev* In press
2. Hu, D., Gao, X., Morgan, M.A., Herz, H.M., Smith, E.R., and **Shilatifard, A.** (2013) The MLL3/MLL4 branch of the COMPASS family is a major H3K4 monomethylase at enhancers. *Mol Cell Biol* 33, 4745-4754.
3. Hu, D., Garruss, A.S., Gao, X., Morgan, M.A., Cook, M., Smith, E.R., and **Shilatifard, A.** (2013) The Mll2 branch of the COMPASS family regulates bivalent promoters in mouse embryonic stem cells. *Nature SMB* 20, 1093-1097.
4. Morgan M. A., and Shilatifard A. (2013) (Poly)Combing the Pediatric Cancer Genome for Answers *Science* 340, 823-824.
5. Lin, C., Garruss, A.S., Luo, Z., Guo, F., **Shilatifard, A.** (2013) The RNA polymerase II elongation factor Ell3 marks enhancers in embryonic stem cells and primes future gene activation. *Cell* 152, 144-156.



**BIOGRAPHICAL SKETCH**

Provide the following information for the Senior/key personnel and other significant contributors in the order listed on Form Page 2.  
Follow this format for each person. **DO NOT EXCEED FOUR PAGES.**

NAME Sonenberg, Nahum	POSITION TITLE		
eRA COMMONS USER NAME (credential, e.g., agency login) NAHUM.SONENBERG	James McGill Professor (Biochemistry)		
EDUCATION/TRAINING <i>(Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable.)</i>			
INSTITUTION AND LOCATION	DEGREE <i>(if applicable)</i>	MM/YY	FIELD OF STUDY
Tel-Aviv University, Israel Weizmann Institute of Science, Israel	BSc & MSc PhD	1970&1971 1976	Microbial & Immunol Biochemistry

**A. Personal Statement**

My expertise is in the field of translational control in physiological conditions and disease. A major interest is understanding the mechanisms by which signaling pathways modulate translation initiation in cancer.

**B. Professional Positions**

1976-1979 Postdoctoral Fellow, Roche Institute of Molecular Biology, Nutley, New Jersey, USA.  
1979-1982 Assistant Professor, Dept. of Biochem. & McGill Cancer Centre, McGill University, Montreal, QC  
1983-1987 Assoc. Professor, Dept. of Biochem. & McGill Cancer Centre, McGill University, Montreal, QC  
1985-1986 Visiting Professor, Whitehead Institute for Biomedical Research, Cambridge, MA, USA.  
1987- Professor, Dept. of Biochem & Goodman Cancer Research Centre, McGill University, Montreal, QC  
2010- Deputy Scientific Director, Goodman Cancer Research Centre, McGill University, Montreal, QC  
2011- Associate Member, Division of Experimental Medicine, Dept. of Medicine, McGill University, Mtl, QC

**Awards and Honors**

1975 EMBO Short Term Fellowship (Dr. Ebel, Strasbourg)  
1976-1978 Dr. Chaim Weizmann Postdoctoral Fellowship for Scientific Research  
1982-1985 Terry Fox Cancer Research Scientist Award, NCI Canada  
1985-1986 Medical Research Council (Canada) retraining award, Sabbatical leave.  
1986-1991 Medical Research Council (Canada) Scientist Award  
1992 Fellow of the Royal Society of Canada  
1993 Cancer Research Campaign (U.K.) 70th Anniversary Lecturer  
1996-2001 Medical Research Council of Canada Distinguished Scientist Award  
1997-2002 Howard Hughes International Scholar  
2001-2006 Canadian Institutes of Health Research Distinguished Investigator  
2002- James McGill Professor Award  
2002-2006 Howard Hughes International Scholar  
2002 Robert L. Noble Prize, National Cancer Institute of Canada  
2005 Killam Prize for Health Sciences  
2006 Elected member of the American Academy of Arts and Sciences  
2006 Elected member of the Royal Society, UK  
2007 Katharine Berkan Judd Award from Memorial Sloan-Kettering Cancer Center  
2007 Roche Diagnostics Award, CSBMB  
2008 Gairdner International Award  
2009 CIHR Health Researcher of the Year Award in Biomedical and Clinical Research  
2010 Officer, Order of Canada  
2011 Centenary Award, Biochemical Society (UK)  
2012 The Lewis S. Rosenstiel Award for Distinguished Work in Basic Medical Science  
2013 Queen Elizabeth II Diamond Jubilee Medal, Governor General of Canada  
2013 McLaughlin Medal, Royal Society of Canada  
2014 Wolf Prize in Medicine, Wolf Foundation.

Cancer Biology  
Peter Jones, Ph.D., Chair

Peer Review Panel Members for Approval

1. Karen Knudsen, Ph.D.

## BIOGRAPHICAL SKETCH

Provide the following information for the key personnel and other significant contributors in the order listed on Form Page 2.

NAME Karen E. Knudsen	POSITION TITLE Professor of Cancer Biology, Urology, and Radiation Oncology		
eRA COMMONS USER NAME KKNUDSEN			
EDUCATION/TRAINING <i>(Begin with baccalaureate or other initial professional education, such as nursing,</i>			
INSTITUTION AND LOCATION	DEGREE <i>(if applicable)</i>	YEAR(s)	FIELD OF STUDY
The George Washington University	B.S.	1990	Biology
University of California San Diego, La Jolla CA	Ph.D.	1996	Molecular Biology
Ludwig Institute for Cancer Research	Post-doc	1997-99	AR & cell cycle control in prostate cancer

### A. Personal Statement.

My laboratory is dedicated to discerning molecular basis of hormone-dependent cancer development and progression, with a focus on cell cycle and transcriptional control in advanced prostate cancer. I serve as the Hilary Koprowski Professor of Cancer Biology at the Thomas Jefferson University, with joint appointments in the Departments of Urology, Radiation Oncology, and Medical Oncology. For the NCI-designated Kimmel Cancer Center, I serve as Deputy Director (Basic Science) and also as Leader of the Biology of Prostate Cancer Program. I am also Vice Chair (Strategic Planning) for the Department of Cancer Biology, and Director of Research for the Department of Medical Oncology.

My research interests are dedicated to understanding the mechanisms by which hormone receptor and cell cycle deregulation lead to prostate cancer progression and therapeutic bypass. The overall goal of our research is to utilize this information for successful development of precision medicine, to improve therapeutic outcome through rational therapy delivery. Our studies identifying tumor suppressor and hormone receptor alterations have uncovered new targets for treating advanced disease, and led to development of biomarker-driven clinical trials.

### B. Positions and Honors:

#### FACULTY APPOINTMENTS:

2000-2005 Assistant Professor, Department of Cell & Cancer Biology  
University of Cincinnati College of Medicine

2005-2007 Associate Professor (with tenure), Department of Cell & Cancer Biology  
University of Cincinnati, College of Medicine

2007-2010 Associate Professor (with tenure), Departments of Cancer Biology (primary) and Urology (secondary appointment), Thomas Jefferson University

2007-present Member, Kimmel Cancer Center

2010-present **Professor**, Departments of Cancer Biology (primary), Urology, Radiation Oncology, and Medical Oncology, Thomas Jefferson University

2012-present **Vice Chair** (Strategic Planning), Department of Cancer Biology

2012-present **Hilary Koprowski Endowed Professor, Department of Cancer Biology**

2012-present **Program Leader**, Kimmel Cancer Center, Biology of Prostate Cancer Program

2012-present **Deputy Director (Basic Science)**, Kimmel Cancer Center

2013- present **Director of Research**, Department of Medical Oncology

#### Editorial Positions:

2006-present **Editorial Board**, *Molecular Cancer Therapeutics*

2007-present **Associate Editor**, *Endocrine Related Cancer*

2007-present **Senior Editor**, *Cancer Research*

2008-present Editorial Board, *The Prostate*

2008-present Editorial Board, *American Journal of Pathology*

2009-present Editorial Board, *Environmental Health Perspectives*

2010-present Editorial Board, *Molecular Endocrinology*

2010-present Editorial Board, *Oncogene*

2013-present **Editor-in-Chief**, *Molecular Cancer Research*

#### Grant Review (current only)

2011-present FASEB Excellence in Science Award Committee (2011-present)  
 2012-13 Stand Up to Cancer  
 2010-present Prostate Cancer Foundation Creativity Award study section  
 2012-present Prostate Cancer Foundation Mazzone Challenge Award study section, member  
 2012-present Chair, AACR Millennium Fellowship in Prostate Cancer Research Review Committee (2012-14)  
 2012-present National Institutes of Health (NIH) Molecular Endocrinology (MCE) study section member  
 2013 Chair, NIH ZRG1 OBT M (SEP) study section

#### **AWARDS, HONORS**

1989-present Omicron Delta Kappa (National Leadership Honor Society)  
 1990 Undergraduate Fellowship, ABL-Basic Research Program, NIH-Frederick Cancer Research Center  
 1991-93 NIH Cell and Molecular Biology Training Grant CM07313  
 1993 Powell Foundation Fellowship Award  
 1994-96 NIH/NCI Basic Biochemical and Biological Mechanisms in Cancer Training Grant CA09345  
 1998 Postdoctoral Scholar Research Forum, Best presentation award  
 1998-2000 NIH/NCI National Research Service Award CA82034  
 2005 YWCA Rising Star Award (given by YWCA Academy of Career Women of Achievement)  
 2006 Society of Basic Urologic Research, Young Investigator Award  
 2009 Endocrine Society Laureate Award: Richard E. Weitzman Award  
 2011 Ron Ross Award, Pacific Rim Breast & Prostate Cancer Foundation  
 2012 Creighton University Distinguished Lecture  
 2012 Excellence in Mentoring Award, Thomas Jefferson University  
 2013 Wesibach Lectureship, University of Michigan

#### **Invited Major International Conference Lectures and Leadership** (selected from 2012-present)

2012 Keystone Symposium, Nuclear Receptors  
 2012 Oslo Prostate Cancer Symposium (Norway)  
 2012 AACR Special Conference: Prostate Cancer (Speaker, Session Chair, Scientific Advisory Committee)  
 2012 AACR Annual Meeting (**\*Speaker and Session Chair**)  
 2012 American Society of Andrology  
 2012 Endocrine Society, Annual Meeting  
 2012 FASEB meeting, Integration of Genomic & Non-Genomic Steroid Receptor Actions  
 2012 Endocrine Society of Australia Annual Meeting  
 2102 Reproductive Cancers Meeting, Australia  
 2012 Pro-Nest Conference, EAU Section of Urological Research Conference, France  
 2012 Cold Spring Harbor Nuclear Receptor Conference  
 2013 Gordon Conference on Hormone Action (**\*Chair**)  
 2013 FEBS Nuclear Receptor Workshop, Greece  
 2013 GU-ASCO Annual Meeting  
 2013 Prouts Neck/Prostate Cancer Foundation Meeting on Prostate Cancer  
 2013 Cold Spring Harbor Banbury Conference on the INK4/ARF Locus  
 2013 Spanish Oncology Genito-Urinary Group annual meeting, Madrid  
 2014 Keystone Nuclear Receptor Meeting  
 2014 AACR-PCF Conference on Advances in Prostate Cancer Research  
 2014 1st Annual Summit on Practical and Emerging Agents in Prostate Cancer  
 2014 Cold Spring Harbor Laboratories Symposium on PARP  
 2014 PCF-University of Oslo Prostate Cancer Conference: From Bench to Clinic  
 2014 Pezcoller Foundation Symposium on Cancer, Trento, Italy  
 2014 56<sup>th</sup> Annual ASTRO Meeting  
 2014 Cold Spring Harbor Laboratories Symposium: Nuclear Receptors & Disease (**\*Co-Chair**)

#### **C. Peer-Reviewed Publications:** *last of >95 peer-reviewed peer reviewed publications*

**Knudsen, KE** (2012) Cyclin D1 goes metabolic: dual functions of Cyclin D1 in regulating lipogenesis. *Cell Cycle*, Oct 1;11(19):3533-4. doi: 10.4161/cc.22039.

Schiewer, MJ, Goodwin, JF, Han, S, Brenner, JC, Augello, MA, Dean, JL, Liu, F, Planck, JL, Ravindranathan, P, Chinnaiyan, A, McCue, P, Gomella, LG, Raj, GV, Dicker, AP, Brody, JR, Pascal, JM, Centenera, MM, Butler, LM, Tilley, WD, Feng, FY, and **Knudsen, KE** (2012)

Cancer Prevention Research  
Thomas Sellers, Ph.D./M.P.H., Chair

Peer Review Panel Members for Approval

1. Nagi Kumar, Ph.D.
2. Christopher Li, M.D./Ph.D.
3. Elena Martinez, Ph.D./M.P.H.
4. Alexander Parker, Ph.D.

## BIOGRAPHICAL SKETCH

Provide the following information for the Senior/key personnel and other significant contributors.  
Follow this format for each person. **DO NOT EXCEED FOUR PAGES.**

NAME <b>Nagi B. Kumar, Ph.D.</b>	POSITION TITLE Director, Cancer Chemoprevention/Senior Member, Moffitt Cancer Center, Professor, Department of Oncologic Sciences, University of South Florida		
eRA COMMONS USER NAME (credential, e.g., agency login) <b>nkumar</b>			
EDUCATION/TRAINING (Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable.)			
INSTITUTION AND LOCATION	DEGREE (if applicable)	MM/YY	FIELD OF STUDY
Madras University, India	B.S.	1976	Nutrition and Dietetics
City Hospital center at Elmhurst, Mt. Sinai School of Medicine, New York	RD	1982	Dietetics
University of South Florida, Tampa, FL	Internship		
	M.A.	1984	Adult Education
			Public Health Nutrition & Applied Behavior Analysis
University of South Florida, Tampa, FL	Ph.D.	1992	(Interdisciplinary Sciences)

### A. Personal Statement

I am a Professor in the Division of Oncologic Sciences at the USF College of Medicine and Senior member in the Population Sciences Division and Director of Cancer Chemoprevention at the Moffitt Cancer Center. As a Clinical Registered Dietitian and Fellow of the American Dietetic Association by training, I established and directed the Clinical Nutrition Program, including clinical services in Thoracic Oncology Program at the Moffitt Cancer Center for over 2 decades. In addition, I have served in the Nutrition Guidelines panel at the NCCN as well as serving as an active member of Diagnosis and Management of Lung Cancer: American College of Chest Physicians: Evidence-Based Guidelines, Health and Science Policy Committee since 2005. By forging collaborations investigators in basic, population and clinical sciences, I led the foundation to develop a program to accelerate agent development and validation using botanicals and biologics at the Moffitt Cancer Center. Using a multi-disciplinary science-based approach and the rigor with which we test other therapeutic agents, she has initiated several epidemiological, laboratory, preclinical and phase I-II clinical trials evaluating safety, effectiveness and potential molecular targets of several agents including isoflavones, lycopene, green tea polyphenols, tannic acid, n-3 fatty acids, metformin, anthocyanins, curcumin and combination agents for cancer prevention, in addition to multimodal interventions to characterize and ameliorate symptoms of hypothyroidism, cancer cachexia and cognitive impairment. I am the principal author and editor of a recent textbook for health professionals titled, *Nutritional Management of Symptoms of Cancer Treatment Effects* (Springer. 2012). Currently active multi-institutional clinical trials that I leads include: (a) Phase II, Randomized, Double-blind, Multi-centered Study of Polyphenon E in Men with High-grade Prostatic Intraepithelial Neoplasia (NCI R01 CA12060-01A1); (b) Phase II Clinical Trial of Purified Isoflavones in Prostate Cancer: Comparing Safety, Effectiveness and mechanism of action between African American and Caucasian men (NIH 1 P20 MD003375-01) in addition to the; (c) Study of the Role of NOV-BYM338X2202 in Cancer Cachexia. As PI/Study Chair, she has also completed: (a) A Randomized Pilot Clinical Trial of the Action of Isoflavones and Lycopene in Localized prostate cancer (CaP): Administration Prior to Radical Prostatectomy, and; (b) The Specific Role of Isoflavones in Reducing CaP Risk. (NCI – UI0 CA81920A) and phase II trials of evaluating isoflavone in modulating hormonal and proliferative markers in breast and prostate cancers (NCI R03 CA72588-01A1). I have over two decades of experience mentoring students and junior faculty and was the Core Leader of the Center for Equal Health for the research Core as well as the Research Education and training Core. In addition to serving as an active teaching (didactic) faculty member at the Colleges of Medicine, Nursing and Public Health since 1993, I am a member of the Education Steering Committee at the Moffitt Cancer Center and serve as a mentor to several post-doctoral and junior faculty members.

### B. Positions and Honors

#### Positions and Employment

- 2006-2008 Associate Professor, Health Outcomes and Behavior, Division of Cancer Control, Director, Nutrition Research, Co-Leader, Integrative Medicine, Moffitt Cancer Center, Department of Interdisciplinary Oncology, University of South Florida, Tampa, Florida
- 2009- Senior Member, Cancer Epidemiology, Division of Population Sciences, Director, Cancer Chemoprevention, Moffitt Cancer Center, Professor, Department of Oncologic Sciences, University of South Florida College of Medicine, Tampa, Florida

#### **Other Experiences in National Advisory Committees/Panels:**

- 2009- DOD -Congressionally Directed Medical Research Program (CDMRP) Prostate Cancer (PCRP) Study Section
- 2010- Study Section: NIH-NCI-PO1 Panel.
- 2010- Cancer Center Review Panel (Cancer Center Support Grant, P30) NIH/NCI
- 2010- Author: Health & Science Policy (HSP) Integrative Oncology chapter of the Lung Cancer III Guidelines
- 2011- Indo-US cooperation in life sciences Advisory Board (Cancer Botanical Drug Development), National Cancer Institute
- 2011- China - US Cancer Symposium Panel on Traditional Chinese Medicine, National Cancer Institute.
- 2012- Study Section - French National Cancer Institute - Hospital Clinical Research Program - PHRC 2012.
- 2012- PDQ Cancer Complementary and Alternative Medicine Editorial Board, National Cancer Institute, National Institute of Health.
- 2012- Study Section. NIH-NINR: RFA-12-010 entitled "Early detection and prevention of mild cognitive impairment" (R01 mechanisms)
- 2009- Study section. NIH-NCI-CDP. Chemo/Dietary Prevention.
- 2013- Faculty, International consortium: Seminars in Cancer Biology Apoptosis Theme Review. The Halifax Project.

#### **Honors:**

- 1976 Cornelius Memorial Prize for Best Student in Nutrition, Graduating Class of 1976.
- 1997 Fellow of the American Dietetic Association
- 2013 Fellow of the Academy of Nutrition and Dietetics

#### **C. Selected peer reviewed publications**

- Kumar NB**, Dalton K, Xu P, Crocker T, Dahan K, Spiess P. The Role Purposeful Physical Activity and Steroid Hormones as Potential Modulators of Advanced Prostate Cancer Risk Reduction. Clin Med Urol. 2010.
- Kumar NB**, Kang L, Pow-Sang J, Xu P, Allen K, Riccardi D, Krischer JP. Results of a randomized phase I dose-finding trial of several doses of isoflavones in men with localized prostate cancer: administration prior to radical prostatectomy. J Soc Integr Oncol. 2010;8(1):3-13. PMID: 202-5984.
- Kumar NB**, Kazi A, Smith T, Crocker T, Yu D, Reich RR, Reddy K, Hastings S, Exterman M, Balducci L, Dalton K, Bepler, G. Cancer cachexia: traditional therapies and novel molecular mechanism-based approaches to treatment. Curr Treat Options Oncol. 2010;11(3-4):107-17. PMCID: 3016925.
- Brem S, **Kumar NB**. Management of treatment-related symptoms in patients with breast cancer. Clin J Oncol Nurs. 2011;15(1):63-71. PMID: 21278042.
- Chornokur G, Dalton K, Borysova ME, **Kumar NB**. Disparities at presentation, diagnosis, treatment, and survival in African American men, affected by prostate cancer. Prostate. 2011;71(9):985-97. PMID: 21541975. PMCID:PMC3083484.
- Connors SK, Shornokur G, **Kumar NB**. New Insights to the Mechanisms of Green Tea Catechins in the Chemoprevention of Prostate Cancer. J Nutr Cancer. 2012;64 (1):4-22. PMID: 22098273. PMCID:PMC3665011.

## BIOGRAPHICAL SKETCH

NAME Christopher I. Li, MD, PhD	POSITION TITLE Full Member,		
eRA COMMONS USER NAME (credential, e.g., agency login) CHRISLI	Fred Hutchinson Cancer Research Center		
EDUCATION/TRAINING <i>(Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable.)</i>			
INSTITUTION AND LOCATION	DEGREE <i>(if applicable)</i>	MM/YY	FIELD OF STUDY
Stanford University, Stanford, CA	BS	1995	Biological Sciences
University of California, San Francisco, CA	MD	2000	Medicine
University of Washington, Seattle, WA	MPH	2000	Epidemiology
University of Washington, Seattle, WA	PhD	2002	Epidemiology

### A. Positions and Honors

#### Positions

2002-2006	<b>Assistant Member</b> , Program in Epidemiology, Fred Hutchinson Cancer Research Center (FHCRC), Seattle, WA.
2002-2006	<b>Assistant Professor (Research)</b> , Department of Epidemiology, School of Public Health, University of Washington (UW), Seattle, WA.
2006-2010	<b>Associate Member</b> , Program in Epidemiology, FHCRC, Seattle, WA.
2002-2011	<b>Associate Professor (Research)</b> , Department of Epidemiology, UW, Seattle, WA.
2010-Present	<b>Full Member</b> , Program in Epidemiology, FHCRC, Seattle, WA.
2011-Present	<b>Full Professor (Research)</b> , Department of Epidemiology, UW, Seattle, WA.
2012-Present	<b>Program Head and Full Member</b> , Translational Research Program, FHCRC, Seattle, WA.

#### Honors and Other Professional Activities

2005	<b>Era of Hope Scholar Award</b> , Department of Defense Breast Cancer Research Program
2005	<b>Faculty Researcher Role Model Award</b> , Minority Access
2005-2010	<b>Member</b> , Scientific Advisory Committee for the Group Health Cooperative Breast Cancer Surveillance Center and the Breast Cancer Surveillance Consortium (BCSC) Statistical Coordinating Center
2009	<b>Promise of One Award</b> , Susan G. Komen for the Cure
2009-present	<b>Regular Member</b> , Subcommittee F – Manpower & Training Study Section, NCI
2010-present	<b>Member and Chair</b> , Minorities in Cancer Research (MICR) Council of the American Association for Cancer Research (AACR)
2012-present	<b>Member</b> , Department of Defense Breast Cancer Research Program Integration Panel

### B. Selected Peer-reviewed Publications (from a total of 112)

- Li CI**, Malone KE, Weiss NS, Daling JR. Tamoxifen therapy for primary breast cancer and risk of contralateral breast cancer. *J Natl Cancer Inst* 2001;93:1008-13.
- Li CI**, Daling JR, Malone KE. Incidence of invasive breast cancer by hormone receptor status from 1992 to 1998. *J Clin Oncol* 2003;21:28-34.
- Li CI**, Anderson BO, Daling JR, Moe RE. Trends in incidence rates of invasive lobular and ductal breast carcinoma. *JAMA* 2003;289:1421-4.
- Li CI**, Malone KE, Porter PL, Weiss NS, Tang MC, Cushing-Haugen KL, Daling JR. Relationship between long durations and different regimens of hormone therapy and risk of breast cancer. *JAMA* 2003;289:3254-63.
- Li CI**, Daling JR, Porter PL, Tang MC, Malone KE. Adjuvant hormonal therapy for breast cancer and risk of hormone receptor-specific subtypes of contralateral breast cancer. *Cancer Res* 2009;69:6865-70. PMID: PMC2745902



## BIOGRAPHICAL SKETCH

Provide the following information for the key personnel and other significant contributors.  
Follow this format for each person. **DO NOT EXCEED FOUR PAGES.**

NAME Maria Elena Martinez, MPH, PhD	POSITION TITLE Professor, University of California San Diego		
eRA COMMONS USER NAME mmartinez			
EDUCATION/TRAINING <i>(Begin with baccalaureate or other initial professional education, such as nursing, and include postdoctoral training.)</i>			
INSTITUTION AND LOCATION	DEGREE <i>(if applicable)</i>	YEAR(s)	FIELD OF STUDY
University of Illinois Medical Center, Chicago, IL	BS	1980	Nutrition
University of Texas School Public Health, Houston, TX	MPH	1987	Nutrition/Pop Studies
University of Texas School Public Health, Houston, TX	PhD	1993	Epidemiology
MD Anderson Cancer Center, Houston, TX	Post-Doc	1993-1994	Epidemiology
Harvard School of Public Health, Boston, MA	Post-Doc	1994-1996	Nutritional Epidemiology

### A. Personal Statement

I am a cancer epidemiologist with over fifteen years of experience in cancer epidemiology and prevention. I have worked on implementation of numerous studies of various designs and analyzing data from these, which include traditional cancer epidemiology as well as molecular epidemiology studies. I have led and participated in large multi-institutional, multi-disciplinary consortia, including PO1, SPORE, and UO1/U54 grants. During my tenure at the University of Arizona, I was Co-Leader of the Cancer Prevention and Control Program. More recently, my work has extended into cancer disparities research, primarily focusing on Hispanic/Latino populations and some of this work has involved binational and international collaborations. Nationally, I have established a strong leadership and commitment to the area of cancer health disparities; evidence of this is my recent appointment as chair of the AACR's Minorities in Cancer Research Council and my service as senior editor for the cancer disparities section of the Cancer Epidemiology, Biomarkers, and Prevention journal. I have served on the NCI's Board of Scientific Counselors and am currently a member of the Board of Scientific Advisors.

### B. Positions and Honors

2011-present Professor and Sam M. Walton Endowed Chair for Cancer Research, Family and Preventive Medicine, University of California, San Diego, La Jolla, CA

2011-present Co-leader, Reducing Cancer Disparities Program, UCSD Moores Cancer Center, La Jolla, CA

2007-2011 Co-Director, Cancer Health Disparities Institute, Arizona Cancer Center, Tucson, AZ

2005-2011 Richard H. Hollen Professor of Cancer Prevention, Arizona Cancer Center, Tucson, AZ

2005-2011 Professor of Epidemiology (Tenured), Mel and Enid Zuckerman Arizona College of Public Health and Arizona Cancer Center, University of Arizona, Tucson, AZ

2005-2011 Co-Leader, Cancer Prevention and Control Program, Arizona Cancer Center, University of Arizona, Tucson, AZ

2004-2005 Professor of Epidemiology (NT), Mel and Enid Zuckerman Arizona College of Public Health and Arizona Cancer Center, University of Arizona, Tucson, AZ

2004-2005 Associate Professor of Nutrition (adjunct faculty), Nutrition Department, College of Agriculture and Life Sciences, University of Arizona, Tucson, AZ

2001-2005 Associate Professor of Epidemiology (NT), Mel and Enid Zuckerman Arizona College of Public Health and Arizona Cancer Center, University of Arizona, Tucson, AZ

1996-2001 Research Assistant Professor of Epidemiology, College of Public Health and Arizona Cancer Center, University of Arizona, Tucson, AZ

### Honors and Awards

2013 Women Who Mean Business Award, San Diego Business Journal, San Diego, CA

2010 Distinguished Alumnus Award, MD Anderson Cancer Center, Houston, TX

2007 Researcher of the Year Award, Mel and Enid Zuckerman College of Public Health

2007 Best Poster Award, American Society of Preventive Oncology Annual Meeting

1993 University of Texas School of Public Health Alumni Association Minority Scholarship

1992 University of Texas School of Public Health Alumni Association Minority Scholarship

### C. Selected Peer-Reviewed Publications

1. **Martinez ME**, Wertheim BC, Natarajan L, Schwab R, Bondy M, Daneri-Navarro A, Meza-Montenegro MM, Gutierrez-Millan LE, Brewster A, Komenaka I, Thompson P. Reproductive factors, heterogeneity, and breast tumor subtypes in women of Mexican descent. *Cancer Epidemiol Biomarkers Prev* 2013; 22(10):1853-61. PMID 23950213. PMCID PMC3799795
2. Simpson DR, **Martínez ME**, Gupta S, Hattangadi-Gluth J, Mell LK, Heestand G, Fanta P, Ramamoorthy S, Le QT, Murphy JD. Racial disparity in consultation, treatment, and the impact on survival in metastatic colorectal cancer. *J Natl Cancer Inst* 2013;105(23):1814-20. PMID 24231453
3. Robertson DJ, Lieberman DA, Winawer SJ, Ahnen DJ, Baron JA, Schatzkin A, Cross AJ, Zauber AG, Church TR, Lance P, Greenberg ER, **Martínez ME**. Colorectal cancers soon after colonoscopy: a pooled multicohort analysis. *Gut* 2013 Jun 21. [Epub ahead of print]. PMID 23793224
4. **Martinez ME**, Thompson P, Messer K, Ashbeck EL, Lieberman DA, Baron JA, Ahnen DJ, Robertson DJ, Jacobs ET, Greenberg ER, Cross AJ, Atkin W. One-year risk for advanced colorectal neoplasia: U.S. versus U.K. risk-stratification guidelines. *Ann Intern Med* 2012;157(12):856-64. PMID 23247939. PMCID PMC3787691
5. Greenberg ER, Anderson GL, Morgan DR, Torres J, Chey WD, Bravo LE, Dominguez RL, Ferreccio C, Herrero R, Lazcano-Ponce EC, Meza-Montenegro MM, Peña R, Peña EM, Salazar-Martínez E, Correa P, **Martínez ME**, Valdivieso M, Goodman GE, Crowley JJ, Baker LH. 14-day triple, 5-day concomitant and 10-day sequential therapies for *Helicobacter pylori* infection in seven Latin American sites: a randomised trial. *Lancet* 2011;378(9790):507-14 PMID 21777974. PMCID PMC3313469
6. Nodora JN, Martz WD, Ashbeck EL, Jacobs ET, Thompson PA, **Martínez ME**. Primary care physician compliance with colorectal cancer screening guidelines. *Cancer Cause Control* 2011; 22(9):1277-87 PMID 21710193
7. Jacobs E, **Martinez ME**, Buckmeier J, Lance P, May M, Jurutka P. Circulating fibroblast growth factor-23 is associated with increased risk for metachronous colorectal adenoma. *J Carcinog* 2011;10(3). PMID 21383962. PMCID PMC3049272
8. Jacobs ET, **Martínez ME**, Campbell PT, Conti DV, Duggan D, Figueiredo JC, Haile RW, Leroy EC, Poynter JN, Thompson PA, Baron JA. Genetic variation in the retinoid X receptor and calcium-sensing receptor, and risk of colorectal cancer in the Colon Cancer Family Registry. *Carcinogenesis* 2010;31(8):1412-1416. PMID 20558521. PMCID: PMC2915636
9. Campbell PT, Jacobs ET, Ulrich CM, Figueiredo JC, Poynter JN, McLaughlin JR, Haile RW, Newcomb PA, Potter JD, Le Marchand L, Green RC, Parfrey P, Younghusband HB, Cotterchio M, Gallinger S, Jenkins MA, Hopper JL, Baron JA, Thibodeau SN, Lindor NM, Limburg PJ, **Martinez ME**, for the Colon Cancer Family Registry. Case-control study of obesity, overweight and colorectal cancer risk, overall and by tumor microsatellite instability status. *J Natl Cancer Inst* 2010;102(6):391-400. PMID 20208017. PMCID: PMC2841037
10. **Martinez ME**, Baron JA, Lieberman DA, Schatzkin A, Lanza E, Winawer SJ, Zauber AG, Jiang R, Ahnen DJ, Bond JH, Church TR, Robertson DA, Smith-Warner SA, Jacobs ET, Alberts DS, Greenberg ER. A pooled analysis of advanced colorectal neoplasia diagnoses after colonoscopic polypectomy. *Gastroenterology* 2009;136:832- 841. PMID 19171141. PMCID PMC3685417
11. Jacobs ET, Ahnen DJ, Ashbeck EL, Baron JA, Greenberg ER, Lance P, Lieberman DA, McKeown-Eyssen G, Schatzkin A, Thompson PA, **Martinez ME**. Association between body mass index and colorectal neoplasia at follow-up colonoscopy: a pooling study. *Am J Epidemiol* 2009;169(6):654-666. PMID 19147743. PMCID PMC2727215
12. **Martinez ME**, Marshall JR, Giovannucci E. Diet and cancer prevention: the roles of observation and experimentation. *Nat Rev Cancer* 2008;8(9):694-703. PMID 19143054
13. Jacobs ET, **Martinez ME**, Alberts DS, Ashbeck EL, Gapstur, SM, Lance, P, Thompson, PA. Plasma insulin-like growth factor I is inversely associated with colorectal adenoma recurrence: a novel hypothesis. *Cancer Epidemiol Biomarkers Prev* 2008;17(2):300-05. PMID 18250342

## BIOGRAPHICAL SKETCH

NAME Alexander S. Parker, Ph.D.	POSITION TITLE Associate Professor of Epidemiology Associate Professor of Urology		
Era commons name: APARKER			
EDUCATION/TRAINING			
INSTITUTION AND LOCATION	DEGREE	YEAR(s)	FIELD OF STUDY
University of North Carolina, Chapel Hill, NC	B.A.	1988-1992	Biology/Chemistry
University of Iowa, Iowa City, IA	M.S.	1996-1998	Preventive Medicine
University of Iowa, Iowa City, IA	Ph.D.	1998-2000	Epidemiology
Mayo Clinic College of Medicine, Rochester, MN	Fellow	2000-2004	Molecular Epidemiology

### A. Personal Statement

As part of this SPORE application, Dr. Yang and colleagues have described plans for a Career Development Program that will foster the growth and education of the next generation of translational lung cancer investigators. Without question, the individuals that will apply for funding through this Career Development Program will come from a broad range of disciplines and backgrounds. Related to this, I am delighted to serve as part of the committee that will select the top candidates from the diverse pool of applicants. More importantly, I am excited to also make myself available as a mentoring resource to the candidates that are ultimately selected in to the program. To this end, I will be able to draw on my training in molecular epidemiology as well my personal experiences building a successful, NIH-funded program to provide guidance and mentoring to these new investigators, particularly those proposing projects in the area of biomarkers of cancer etiology and prognosis. Moreover, I will also be able to leverage my leadership roles as Vice Chair of the Department of Health Sciences Research, Associate Director of the Center for Individualized Medicine and co-leader for Genetic Epidemiology and Risk Assessment program within the Mayo Clinic Cancer Center to provide access to additional expertise and resources that will undoubtedly have a positive impact on the development of the selected applicants. Finally, I have a history of successful collaboration with several members of the team of investigators assembled for this SPORE (including Dr. Yang) and therefore I will be able to leverage these existing relationships in my role as a member of the Career Development program proposed as part of this SPORE application.

### B. Positions and Honors

#### Positions and Employment

2000-2003	Research Associate, Department of Health Sciences Research, Mayo Clinic, Rochester, MN
2004-2010	Assistant Professor of Epidemiology, Mayo Clinic College of Medicine, Jacksonville, FL
2011-	Associate Professor of Epidemiology, Mayo Clinic College of Medicine, Jacksonville, FL
2011-	Associate Professor of Urology, Mayo Clinic College of Medicine, Jacksonville, FL
2011-	Vice Chair, Division of Health Sciences Research, Mayo Clinic Florida, Jacksonville, FL
2011-	Associate Director, Center for Individualized Medicine, Mayo Clinic Florida, Jacksonville, FL

#### Other Experience and Professional Memberships

2000-	Member, American Association for Cancer Research
2000-	Member, American Society of Preventive Oncology
2000-	Member, American College of Epidemiology
2004-	Finance Committee, American College of Epidemiology
2004-	Membership Committee, American College of Epidemiology
2007-2010	Chair, CHS-EPI section, Prostate Cancer Research Program, CDMRP, Department of Defense
2010, 2011	Chair, Prevention Initiative Study Section, Canadian Cancer Society Research Institute
2010-	Associate Editor, American Journal of Epidemiology
2010-2013	Associate Editor, BMC Urology

## Honors

1990	Phi Sigma Pi National Honor Fraternity, University of North Carolina at Chapel Hill
1998	Pre-doctoral Research Trainee, National Institute of Environmental Health Sciences, National Institutes of Health, Bethesda, MD
1999	Best Student Poster, American College of Epidemiology National Meeting
2000	Milford E. Barnes Outstanding Student Award, Department of Epidemiology, Univ. of Iowa
2001	American Society of Preventive Oncology/Cancer Research Foundation of America Cancer Prevention Research Fellowship
2001	Mayo Cancer Genetic Epidemiology R25 Training Program (MCGETP) Fellowship
2005	Chair, Renal Cancer Working Group, 2nd NCI Epidemiology Leadership Workshop
2007	Best of Posters, American Urological Association, Annual Meeting, Anaheim, CA

## **C. Selected peer-reviewed publications from total of 80 (in chronological order)**

1. Leibovich BC, Blute ML, Cheville JC, Lohse CM, Frank I, Kwon ED, Weaver AL, **Parker AS**, Zincke H. Prediction of progression after radical nephrectomy for patients with clear cell renal cell carcinoma - A stratification tool for prospective clinical trials. *Cancer* 2003 Apr 1; 97(7):1663-71. PMID:12655523.
2. **Parker AS**, Cheville JC, Blute ML, Igel T, Lohse CM, Cerhan JR. Pathologic T1 clear cell renal cell carcinoma: insulin-like growth factor-I receptor expression and disease-specific survival. *Cancer* 2004 Jun 15; 100(12):2577-82. PMID:15197799.
3. Kosari F, **Parker AS**, Kube DM, Lohse CM, Leibovich BC, Blute ML, Cheville JC, Vasmatazis G. Clear cell renal cell carcinoma: Gene expression analyses identify a potential signature for tumor aggressiveness. *Clin Cancer Res* 2005 Jul 15; 11(14):5128-39. PMID:16033827.
4. **Parker AS**, Kosari F, Lohse CM, Houston Thompson R, Kwon ED, Murphy L, Riehle DL, Blute ML, Leibovich BC, Vasmatazis G, Cheville JC. High expression levels of survivin protein independently predict a poor outcome for patients who undergo surgery for clear cell renal cell carcinoma. *Cancer* 2006 Jul 1; 107(1):37-45. PMID:16736510.
5. **Parker AS**, Lohse CM, Wu K, Kreinest P, Copland JA, Hilton T, Wehle M, Cheville JC, Blute M. Lower expression levels of the transforming growth factor beta receptor type II protein are associated with a less aggressive tumor phenotype and improved survival among patients with clear cell renal cell carcinoma. *Hum Pathol* 2007 Mar; 38(3):453-61. PMID:17188329.
6. Krambeck AE, Dong H, Thompson RH, Kuntz SM, Lohse CM, Leibovich BC, Blute ML, Sebo TJ, Cheville JC, **Parker AS**, Kwon ED. Survivin and b7-h1 are collaborative predictors of survival and represent potential therapeutic targets for patients with renal cell carcinoma. *Clin Cancer Res* 2007 Mar 15; 13(6):1749-56. PMID:17363528.
7. Lee JE, Hunter DJ, Spiegelman D, Adami HO, Bernstein L, van den Brandt PA, Buring JE, Cho E, English D, Folsom AR, Freudenheim JL, Gile GG, Giovannucci E, Horn-Ross PL, Leitzmann M, Marshall JR, Mannisto S, McCullough ML, Miller AB, **Parker AS**, Pietinen P, Rodriguez C, Rohan TE, Schatzkin A, Schouten LJ, Willett WC, Wolk A, Zhang SM, Smith-Warner SA. Intakes of coffee, tea, milk, soda and juice and renal cell cancer in a pooled analysis of 13 prospective studies. *Int J Cancer*. 2007 Nov 15; 121(10):2246-53. PMID:17583573.
8. **Parker AS**, Lohse C, Cheville J, Leibovich B, Igel T, Blute M. Evaluation of the association of current cigarette smoking and outcome for patients with clear cell renal cell carcinoma. *Int J Urol* 2008 Apr; 15(4):304-8. PMID:18380816.
9. **Parker AS**, Lohse CM, Leibovich BC, Cheville JC, Sheinin YM, Kwon ED. Comparison of digital image analysis versus visual assessment to assess survivin expression as an independent predictor of survival for patients with clear cell renal cell carcinoma. *Hum Pathol* 2008 Aug; 39(8):1176-84. Epub 2008 Jun 05. PMID:18538369. PMCID:2789391..
10. **Parker AS**, Leibovich BC, Lohse CM, Sheinin Y, Kuntz SM, Eckel-Passow JE, Blute ML, Kwon ED. Development and evaluation of BioScore: a biomarker panel to enhance prognostic algorithms for clear cell renal cell carcinoma. *Cancer* 2009 May 15; 115(10):2092-103. PMID:19296514. PMCID:2789398.

Clinical and Translational Cancer Research  
Margaret Tempero, M.D., Chair

Peer Review Panel Members for Approval

1. Dean Brenner, M.D.
2. Patricia LoRusso, D.O.
3. Antoni Ribas, M.D./Ph.D.

**BIOGRAPHICAL SKETCH**

Provide the following information for the Senior/key personnel and other significant contributors in the order listed on Form Page 2.  
Follow this format for each person. **DO NOT EXCEED FOUR PAGES.**

NAME Dean E. Brenner, M.D.	POSITION TITLE Professor		
eRA COMMONS USER NAME (credential, e.g., agency login) dbrenner@umich.edu			
EDUCATION/TRAINING (Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable.)			
INSTITUTION AND LOCATION	DEGREE (if applicable)	MM/YY	FIELD OF STUDY
University of Pennsylvania, Philadelphia, PA	AB	05/71	Natural Sciences
Hahnemann Medical College, Philadelphia, PA	MD	06/74	Medicine
Pennsylvania State Univ, Hershey, PA	Resident	06/77	Internal Medicine
National Cancer Institute	Fellowship	06/80	Medical Oncology

**A. Personal Statement**

I studied the clinical pharmacology of antineoplastic agents and more recently cancer preventive interventions. Biomarkers as targets and surrogate endpoints for cancer preventive interventions are derived from mechanism based carcinogenesis data such as those proposed in this application. I believe that concepts generated in one organ site should have cross organ use. For this reason, I have generalized my experience in clinical pharmacology of cancer preventive interventions and biomarkers derived from our understanding of the carcinogenesis process in the colon to other organ sites, specifically the breast, skin and the lung.

**B. Positions and Honors****Positions and Employment**

1980-1981 Scientific Expert, National Cancer Institute, Div Cancer Treatment, Baltimore MD  
 1981-1986 Assistant Professor of Medicine, Vanderbilt Univ, Nashville TN  
 1984-1986 Research Associate, Veterans Administration Medical Ctr, Nashville TN  
 1986-1989 Research Clinician, Roswell Park Memorial Inst, Buffalo NY  
 1987-1989 Associate Professor of Medicine, SUNY-Buffalo, Buffalo NY  
 1989-1996 Associate Professor of Internal Medicine, Univ of Michigan, Ann Arbor MI  
 1989-1997 Associate Professor of Pharmacology, Univ of Michigan, Ann Arbor MI  
 1996-present Professor of Internal Medicine, Univ of Michigan, Ann Arbor MI  
 1997-present Professor of Pharmacology, Univ of Michigan, Ann Arbor, MI  
 2005-present Kutsche Family Memorial Professor of Internal Medicine, Univ of Michigan, Ann Arbor, MI

**Other Experience and Professional Memberships**

1987-1991 Member, FDA Oncologic Drugs Advisory Committee  
 1992-1996 Cancer Clinical Investigations Review Committee (SubCte H), NIH, NCI  
 1999-2004 NIH, CSR, Clinical Oncology Study Section, Chair (2000-2004)  
 1995-present Director, Biomedical Prevention Program, Univ of Michigan Cancer Center.  
 2002-2005 Executive Committee and Chair, GI Collaborative, Early Detection Research Network  
 2006-2010 Peer Review Advisory Committee, NIH

**C. Selected Peer-reviewed Publications (15 out of 156)**

1. Normolle DP, Ruffin MT, Brenner DE. Design of early validation trials of biomarkers. Cancer Informatics 2005; 1:25-31.
2. Boockock DJ, Faust GES, Patel, KR, Normolle DP, Booth TD, Crowell JA, Brown VA, Ducharme MP, Schinas AM, Gescher AJ, Steward WP, Brenner DE. Phase I dose escalation pharmacokinetic study in healthy volunteers of resveratrol, a potential cancer chemoprevention agent. Cancer Epidemiol Biomark Prevent, 2007;16:1246-1252.

### BIOGRAPHICAL SKETCH

Provide the following information for the key personnel and other significant contributors in the order listed on Form Page 2.  
Follow this format for each person. **DO NOT EXCEED FOUR PAGES.**

NAME Patricia Mucci LoRusso	POSITION TITLE Professor of Medicine		
EDUCATION/TRAINING <i>(Begin with baccalaureate or other initial professional education, such as nursing, and include</i>			
INSTITUTION AND LOCATION	DEGREE <i>(if applicable)</i>	YEAR(s)	FIELD OF STUDY
Marygrove College/Univ. of Detroit, Detroit, MI	B.S.	05/77	Biology/Religious Studies
Michigan State Univ., Lansing, MI	D.O.	06/81	Osteopathic Medicine

**A. Personal Statement** For over 24 years, I have been doing early phase (phase I and II) clinical trials with a strong emphasis on clinical trial recruitment, novel trial designs, translational concepts and novel/novel drug combinations. As part of a smaller cancer center, it has been imperative that I form strong collaborations to not only execute and complete investigator initiated trials, but equally importantly to be able to do translational science. This is most recently exemplified in several collaborative grants for which I am a Co-PI, most notably our SU2C Melanoma Dream Team Award. My program has worked on over 13 agents now FDA approved. In doing so, I realize that a paradigm shift in the way cancer drugs are developed is needed to improve both therapeutic benefit and cost. The next several years hold much promise for drug development, not only due to the knowledge and science unfolding, but also the technology and tools that will lend significant impact on clinical drug development. For this UM-1 submission, I have assembled and will lead a complementary group of institutions and investigators contributing immunotherapy, genomics, molecular biology, previous pharma experience, minority recruitment and a team of investigators firmly committed to translational science and medicine. Together, we are well positioned to advance the arena of early drug development, and meet the goals and contribute significantly to the ET-CTN.

**Positions and Honors.** List in chronological order previous positions, concluding with your present position. List any honors. Include present membership on any Federal Government public advisory committee.

1989 – 1996 Assistant Professor, Division of Hematology and Oncology, Wayne State University School of Medicine, Harper Hospital, Detroit, MI.  
1996 – 2002 Associate Professor, Division of Hematology and Oncology, Wayne State University School of Medicine, Karmanos Cancer Institute, Detroit, MI.  
1997 – 1998 Acting Director, Drug Discovery Program, Karmanos Cancer Institute, Detroit, MI.  
1997 – present Director, Phase I Clinical Trials Program, Karmanos Cancer Institute, Detroit, MI.  
2002 – present Professor, Division of Hematology and Oncology, Wayne State University School of Medicine, Karmanos Cancer Institute, Detroit, MI.  
2006 – present Tenure, Wayne State University School of Medicine, Detroit, MI.  
2010 – present Director, Center for Developmental Therapeutics, Karmanos Cancer Institute, Detroit, MI  
2011 – present Division Leader, Translational Therapeutics, Department of Oncology, Wayne State University

Outstanding Resident; 1982-1983, 1984-1985

Chief Resident; 1984-1985

American Cancer Society Fellowship Award; 1985-1987

ACOI Abstract Award Recipient (3rd place); 1988

Scientific Research Award, 1998

Heroes of Breast Cancer, 1999

Bennett J. Cohen Educational Leadership Award for Medical Research, 2004

Heroes of Healthcare Award, Crain's Detroit Business, 2008

Marygrove College Distinguished Alumni Award, 2008

Michelle Christian Award, NCI, 2008

WSU Teaching Award, 2009

KCI Center Director's Quarterly Research Award, May 2010

American College of Osteopathic Internists (ACOI) Researcher of the Year Award, 2010

**B. Selected peer-reviewed publications (in chronological order).** Do not include publications submitted or in preparation. (Out of 165)

1. Blum, J.L, Jones, S.E., Buzdar, A.U., LoRusso, P., Kuter, I., Vogel, C., Burger, H.U., Brown, C.S., Griffin, T. A Multicenter Phase II Study of Capecitabine in Paclitaxel-Refractory Metastatic Breast Cancer. *J. Clin. Oncol.*, U17U(2):485-493, 1999. PMID: 10080589
2. Blum, J., Dieras, V., LoRusso, P.M., Horton, J., Burger, H.U., Osterwalder, B., Laws, S., Buzdar, A. Multicenter, phase II study of Capecitabine in taxane-pretreated metastatic breast cancer patients. *Cancer* 1;92(7):1759-68, October 2001. PMID: 11745247
3. Albanell, J., Rojo, F., Averbuch S., Feyereislova, A., Mascaro, J.M., Herbst, R., LoRusso, P., Rischin, D., Saulea, S., Gee, J., Nicholson, R.I., Baselga, J. Pharmacodynamic Studies of the Epidermal Growth Factor Receptor Inhibitor ZD1839 in Skin from Cancer Patients: Histopathologic and Molecular Consequences of Receptor Inhibition. *J. Clin. Oncol.* 20(1): 110-124, 2002. PMID: 11773160
4. Rinehart J, Adjei AA, LoRusso PM, Waterhouse D, Hecht JR, Natale RB, Hamid O, Varterasian M, Asbury P, Kaldjian EP, Gulyas S, Mitchell DY, Herrera R, Seboth-Leopld JSS, Meyer MB. A Multicenter Phase II Study of the Oral MEK Inhibitor, CI-1040 in Patients with Advanced Non-small-Cell Lung, Breast, Colon and Pancreatic Cancer. *J Clin Oncol* 22:4456-4462, 2004. PMID: 15483017
5. Alousi A.M., Boinpally R., Wiegand R, Parchment R., Gadgeel S., Heilbrun L.K., Wozniak A.J., DeLuca P., LoRusso P.M. A phase 1 trial of XK469: toxicity profile of a selective topoisomerase II $\beta$  inhibitor. *Invest New Drugs.* 2007 Apr;25(2):147-54. Epub 2006 Nov 11. PMID: 17103044
6. Johnston S, Trudeau M, Kaufman B, Boussen H, Blackwell K, LoRusso P, Lombardi DP, Ben Amed S, Citrin DL, Desilvio ML, Harris J, Westlund RE, Salazar V, Zaks TZ, Spector NL. Phase II study of predictive biomarker profiles for response targeting human epidermal growth factor receptor 2 (HER-2) in advanced inflammatory breast cancer with lapatinib monotherapy. *J Clin Oncol.* 2008 Mar 1; 26(7):1066-72. Epub 2008 Jan 22. PMID: 18212337
7. LoRusso PM, Jones SF, Koch KM, Arya N, Fleming RA, Loftiss J, Pandite L, Gadgeel S, Weber BL, Burris HA 3rd. Phase I and pharmacokinetic study of lapatinib and docetaxel in patients with advanced cancer. *J Clin Oncol.* 2008 Jun 20;26(18):3051-6. PMID: 18565892
8. Bröker LE, Valdivieso M, Pilat MJ, Deluca P, Zhou X, Parker S, Giaccone G, Lorusso PM. Effect of food on the pharmacokinetic behavior of the potent oral taxane BMS-275183. *Clin Cancer Res.* 2008 Jul 1;14(13):4186-91. PMID: 18593998
9. LoRusso PM, Eder JP. Therapeutic potential of novel selective-spectrum kinase inhibitors in oncology. *Expert Opin Investig Drugs.* 2008 Jul;17(7):1013-28. Review. PMID: 18549338
10. Eder JP, Vande Woude G, Boerner S, LoRusso PM. Novel Therapeutic Inhibitors of the c-Met Signaling Pathway in Cancer. *Clin Cancer Res.* 2009 Apr 1;15(7):2207-14. [Epub 2009 Mar 24]. PMID: 19318488
11. LoRusso PM. Phase 0 clinical trials: an answer to drug development stagnation? *J Clin Oncol.* 2009 Jun 1;27(16):2586-8. Epub 2009 Apr 13. PMID: 19364952
12. Von Hoff DD, LoRusso PM, Rudin CM, Reddy JC, Yauch RL, Tibes R, Weiss GJ, Borad MJ, Hann CL, Brahmer JR, Mackey HM, Lum BL, Darbonne WC, Marsters Jr JC, de Sauvage FJ, Low JA. Inhibition of the hedgehog pathway in advanced basal-cell carcinoma. *N Engl J Med.* 2009 Sep 17;361(12):1164-72. Epub 2009 Sep 2. PMID: 19726763
13. Demetri GD, LoRusso P, MacPherson IR, Wang D, Morgan JA, Brunton VG, Paliwal P, Agrawal S, Voi M, Evans TR. Phase I dose-escalation and pharmacokinetic study of dasatinib in patients with advanced solid tumors. *Clin Cancer Res.* 2009 Oct 1;15(19):6232-40. Epub 2009 Sep 29. PMID: 19789325
14. Heath EI, LoRusso PM, Ivy SP, Rubinstein L, Christian MC, Heilbrun LK. Theoretical and Practical Application of Traditional and Accelerated Titration Phase I Clinical Trial Designs: the Wayne State University Experience. *Journal of biopharmaceutical statistics* 2009;19(3):414-23. PMID: 19384685
15. Li J, Sausville EA, Klein PJ, Morgenstern D, Leamon CP, Messmann RA, LoRusso P. Clinical pharmacokinetics and exposure-toxicity relationship of a folate-Vinca alkaloid conjugate EC145 in cancer patients. *J Clin Pharmacol.* 2009 Dec;49(12):1467-76. Epub 2009 Oct 16. PMID: 19837906
16. Burris HA 3rd, Jones SF, Williams DD, Kathman SJ, Hodge JP, Pandite L, Ho PT, Boerner SA, Lorusso P. A phase I study of ispinesib, a kinesin spindle protein inhibitor, administered weekly for three consecutive weeks of a 28-day cycle in patients with solid tumors. *Invest New Drugs.* 2011 Jun;29(3):467-72. Epub 2010 Jan 13. PMID: 20069338
17. LoRusso PM, Boerner SA, Seymour L. An overview of the optimal planning, design and conduct of phase I studies of new therapeutics. *Clin Cancer Res.* 2010 Mar 15;16(6):1710-8. Epub 2010 Mar 9. [Review]. PMID: 20215546



**BIOGRAPHICAL SKETCH**

Provide the following information for the Senior/key personnel and other significant contributors in the order listed on Form Page 2.  
Follow this format for each person. **DO NOT EXCEED FOUR PAGES.**

NAME Ribas, Antoni	POSITION TITLE Professor of Medicine, Surgery, and Molecular and Medical Pharmacology		
eRA COMMONS USER NAME (credential, e.g., agency login) RIBAS2			
EDUCATION/TRAINING <i>(Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable.)</i>			
INSTITUTION AND LOCATION	DEGREE <i>(if applicable)</i>	MM/YY	FIELD OF STUDY
University of Barcelona, Spain	MD	1984-1990	Medicine
Autonomous University of Barcelona, Spain	PhD	1990-1993	Immunology

**A. Personal Statement**

I have a longstanding interest and track record in providing a mentored research environment for the career development of trainees interested in independent academic cancer research. I am the principal investigator of the medical oncology training grant T32 CA09297, and a training faculty of four other training grants, and I was a permanent committee member of the National Cancer Institute (NCI) subcommittee-I (NCI-I) study section reviewing K08/K22 fellowship grants for 4 years. I am now a permanent member at the Clinical Oncology (CONC) study section. Several of my trainees have gone onto productive academic careers, like Jennifer Wargo, M.D., Assistant Professor in Surgery at the Massachusetts General Hospital, Begonya Comin-Anduix, Ph.D., Associate Professor of Surgery at UCLA, Robert Prins, Ph.D., Associate Professor in Neurosurgery at UCLA, Richard C. Koya, M.D., Ph.D., Associate Professor of Medicine at Roswell Park Institute, Bartosz Chmielowski, M.D., Ph.D., Assistant Professor in Medicine at UCLA, Gregory Van Dyke, M.D., Ph.D., Assistant Professor in Pathology at UCLA, Paul Tumei, M.D., Assistant Professor in Dermatology at UCLA.

**B. Positions and Honors****Positions and Employment**

1991-94	Internship and Residency in General Medicine and Medical Oncology. University Hospital Vall d'Hebron. Autonomous University of Barcelona, Spain.
1994-1995	Clinical Instructor. University Hospital Vall d'Hebron. Barcelona, Spain.
1996-1998	Postdoctoral Fellow. Div. Surgical Oncology, UCLA. Mentor: James S. Economou, M.D., Ph.D.
1998-2001	Clinical Fellow. Div. Hematology/Oncology, UCLA. Chief: Dennis J. Slamon, M.D., Ph.D.
2001-2006	Assistant Professor in Residence of Medicine and Surgery, UCLA.
2006-2008	Associate Professor in Residence of Medicine and Surgery, UCLA.
2008-2011	Associate Professor of Medicine and Surgery, UCLA.
2011-	Professor of Medicine (with tenure), Professor of Surgery, Professor of Medical and Molecular Pharmacology, UCLA.

**Other Experience and Professional Memberships**

1998-	American Society of Clinical Oncology.
1999-	American Association for Cancer Research.
2001-	General Clinical Research Center Advisory Board Member, UCLA.
2004-	Director, UCLA Cell and Gene Therapy Core Facility.
2005-2009	NCI-Committee I Study Section Permanent Member.
2006-2010	Associate Director, Tumor Immunology Program Area, Jonsson Comprehensive Cancer Center.
2008-2011	ASCO Scientific Program Committee Member, melanoma track.
2009-2011	ASCO Cancer Education Committee, melanoma track.
2010-	NCI-Clinical Oncology Committee (CONC) Study Section Permanent Member.
2010-	Society of Tumor Immunotherapy for Cancer (SITC) Board of Directors
2010-	Director, Tumor Immunology Program Area, Jonsson Comprehensive Cancer Center.

Program Director/Principal Investigator (Last, First, Middle): Ribas, Antoni

2011- Chair, Melanoma Committee at SWOG

2013- Vice-President, Society for Melanoma Research (SMR)

### Honors

1997 Extraordinary Prize and *Suma Cum Laude*, Doctoral Thesis. Autonomous University of Barcelona.  
2000 Fellow Teaching Award, UCLA.  
2000 Amgen Oncology Fellow Award.  
2000 Clinical Research Career Development Award from the American Society of Clinical Oncology (ASCO).  
2002 K23 CA93376 Career Development Award.  
2002 Stop Cancer Career Development Award.  
2005 Melanoma Research Foundation Junior Researcher Award.  
2008 New Faculty Award from the California Institute of Regenerative Medicine (CIRM).  
2009 Elected member of the American Society of Clinical Investigation (ASCI).  
2013 Physician of the Year, Melanoma International Foundation.

### **C. Peer Reviewed Publications (out of a total of 177):**

#### Most relevant to the current application

1. M.E. Davis, J.E. Zuckerman, C.J. Choi, D. Seligson, A. Tolcher, C.A. Alabi, Y. Yen, J.D. Heidel, **A. Ribas**. Evidence of RNAi in humans from systemically administered siRNA via targeted nanoparticles. **Nature** 2010 Apr 15; 464 (7291): 1067-70. PMC2855406.
2. K.T. Flaherty, I. Puzanov, K.B. Kim, **A. Ribas**, G.A. McArthur, J.A. Sosman, P.J. O'Dwyer, R.J. Lee, J. Grippo, K. Nolop, P.B. Chapman. Selective inhibition of BRAF-V600E activating mutations induce major regressions in patients with metastatic melanoma. **New England Journal of Medicine** 2010, 363 (9): 809-19. PMCID pending.
3. G. Bollag, P. Hirth, J. Tsai, J. Zhang, P.N. Ibrahim, H. Cho, W. Spevak, C. Zhang, G. Habets, B. Burton, B.L. West, B. Powell, R. Shellooe, A. Marimuthu, H. Nguyen, K.Y.J. Zhang, D.R. Artis, J. Schlessinger, F. Su, B. Higgins, R. Iyer, A. Koehler, R.J. Lee, J. Grippo, I. Puzanov, K.B. Kim, **A. Ribas**, G.A. McArthur, J.A. Sosman, P.B. Chapman, K.T. Flaherty, K. D'Andrea, X. Xu, K.L. Nathanson, K. Nolop. Clinical efficacy of a B-RAF inhibitor requires substantial ERK pathway inhibition in *BRAF*-mutant melanoma. **Nature** Sep 30; 2010 467 (7315): 596-9. PMC2948082.
4. R. Nazarian, H. Shi, Q. Wang, X. Kong, R.C. Koya, H. Lee, Z. Chen, M.-K. Lee, N. Attar, H. Sazegar, T. Chodon, S.F. Nelson, G.A. McArthur, J.A. Sosman, **A. Ribas**, R.S. Lo. Melanomas acquire resistance to <sup>V600E</sup>B-RAF inhibition by RTK upregulation or N-RAS mutation. **Nature** 2010 Dec 16; 468 (7326): 973-7. PMCID pending.
5. R.C. Koya, S. Mok, B. Comin-Anduix, T. Chodon, M.I. Nishimura, C.G. Radu, O.N. Witte, **A. Ribas**. Kinetic Phases of Distribution and Tumor Targeting by Adoptively Transferred T Cell Receptor Engineered Lymphocytes Inducing Robust Antitumor Responses. **Proceedings of the National Academy of Sciences (PNAS)** 2010 Aug 10; 107 (32): 14286-91.
6. B. Comin-Anduix, T. Chodon, H. Sazegar, D. Matsunaga, S. Mock, J. Jalil, H. Escuin-Ordinas, B. Chmielowski, R.C. Koya, **A. Ribas**. The Raf Inhibitor PLX4032/RG7204 Preserves the Viability and Function of Human Lymphocytes Across a Wide Range of Concentrations. **Clinical Cancer Research** 2010; Dec 15; 16 (24): 6040-8. PMC3057460.
7. P.B. Chapman, A. Hauschild, C. Robert, J.B. Haanen, P. Ascierto, J. Larkin, R. Dummer, C. Garbe, A. Testori, M. Maio, D. Hogg, P. Lorigan, C. Lebbe, T. Jouary, D. Schadendorf, **A. Ribas**, S.J. O'Day, J.A. Sosman, J.M. Kirkwood, A.M.M. Eggermont, B. Dreno, K. Nolop, J. Li, B. Nelson, J. Hou, R.J. Lee, K.T. Flaherty, G.A. McArthur, for the BRIM-3 Study Group. Improved Survival with Vemurafenib in Patients with <sup>V600E</sup>BRAF Mutant Metastatic Melanoma. **New England Journal of Medicine** 2011 Jun 30; 364 (26): 2507-16. PMCID pending.
8. P.I. Poulikakos, Y. Persaud, M. Janakiraman, X. Kong, C. Ng, G. Moriceau, H. Shi, M. Atefi, B. Titz, M.T. Gabay, M. Salton, K.B. Dahlman, Tadi Madhavi, J.A. Wargo, K.T. Flaherty, M.C. Kelley, T. Misteli, J.A. Sosman, P.B. Chapman, T.G. Graeber, **A. Ribas**, R.S. Lo, N. Rosen, D.B. Solit. Acquired resistance to

Translational Cancer Research  
Richard O'Reilly, M.D., Chair

Peer Review Panel Members for Approval

1. Victor Engelhard, Ph.D.
2. Stephen Fesik, Ph.D.
3. Charles Mullighan, M.D.

**BIOGRAPHICAL SKETCH**

Provide the following information for the Senior/key personnel and other significant contributors in the order listed on Form Page 2.  
Follow this format for each person. **DO NOT EXCEED FOUR PAGES.**

NAME Engelhard, Victor H	POSITION TITLE Professor of Microbiology		
eRA COMMONS USER NAME (credential, e.g., agency login) ENGELHARD			
EDUCATION/TRAINING <i>(Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable.)</i>			
INSTITUTION AND LOCATION	DEGREE <i>(if applicable)</i>	MM/YY	FIELD OF STUDY
Rice University, Houston, TX	BA	1973	Biochemistry
University of Illinois, Urbana, IL	MS, PhD	1977	Biochemistry
Harvard University, Cambridge, MA	Postdoc	1977-80	Immunology

**A. Personal Statement**

The work of my laboratory over the last 34 years has been broadly concerned with the recognition of antigens by CD8 T lymphocytes. In the last 15 years, that work has increasingly focused on tumor-derived antigens and cancer immunology. Current activities in the laboratory are almost exclusively concerned with immune responses to antigens expressed on melanoma tumors. These activities include: 1) identification of relevant antigens on human melanomas that may serve as a basis for immunotherapy; 2) evaluation of the homing receptor programs induced on CD8 T cells by different types of antigens, and the role of different homing receptors on effective T cell infiltration; 3) investigation of the relationship between self-tolerance, autoimmunity, and immune response to tumor. Our work in these latter two areas is strongly influenced by an appreciation that immune responses initiated in different lymphoid compartments are also targeted to different body sites.

I have trained over 40 graduate students, postdoctoral fellows, and visiting scientists in my laboratory. In recognition of this mentoring role, I was awarded the Robert J Kadner Distinguished Teaching Award in 2008, and hold the title of David A. Harrison Distinguished Educator, and David A. Harrison Distinguished Professor. I am in the top 5% of recipients of aggregate NIH funding over my career. My research activities have led to over 175 peer-reviewed and invited publications. I am recognized as an ISI **Highly Cited Researcher**®, placing me among the top 250 of scientists and scholars worldwide cited in 21 broad subject categories in life sciences, medicine, physical sciences, engineering and social sciences. I am Leader of the Immunology and Immunotherapy Program of the University of Virginia Cancer Center, Co-Director of the University of Virginia Human Immune Therapy Center, and PI/PD of the NIH Training Grant in Immunology at the University of Virginia.

**B. Positions and Honors****Positions and Employment**

8/73 - 5/77	Research Assistant, University of Illinois, laboratory of Dr. D.R. Storm
8/77 - 1/80	Research Associate, Harvard University, laboratory of Dr. J.L. Strominger
1/80 - 7/85	Assistant Professor of Microbiology, University of Virginia
7/85 - 7/89	Associate Professor of Microbiology, University of Virginia
7/89 - Pres	Professor of Microbiology, University of Virginia

**Other Experience and Professional Memberships**

1/87-6/90	Member, NIH Experimental Immunology Study Section
5/90-4/94	Section Editor, <i>The Journal of Immunology</i>
1981-pres	Member, American Association of Immunologists
1995	Member, NIH Expert Panel on Transplantation Tolerance
1996-2009	Advisory Editor, <i>Journal of Experimental Medicine</i>
1999-2004	Associate Editor, <i>Immunity</i>

2001-2005 Member, *Faculty of 1000*, Immunology Section  
2004-2008 Publications Committee, American Association of Immunologists  
2005-2008 Member, NIH Tumor Transplantation and Tolerance (TTT) Study Section  
2006-2009 NIAID Epitope Discovery Working Group

### **Honors**

8/73 - 8/76 NIH Predoctoral Traineeship, University of Illinois  
8/77 - 8/79 NIH Postdoctoral Fellowship, Harvard University  
8/79 - 1/80 Medical Foundation Fellowship, Harvard University  
9/81 - 9/86 NIH Research Career Development Award, University of Virginia  
1995 Honoree, American Cancer Society, Virginia Division  
2006 David A. Harrison Distinguished Educator, University of Virginia School of Medicine  
2008 Robert J Kadner Distinguished Teaching Award, University of Virginia School of Medicine  
2013 David A. Harrison Distinguished Professorship, University of Virginia

### **C. Selected Peer-reviewed Publications**

#### **Most relevant to the current application**

1. Colella, T. A., Bullock, T.N.J., Russell, L.B., Mullins, D. W., Overwijk, W., Luckey, C.J., Pierce, R.A., Restifo, N.P., and Engelhard, V.H. 2000. Self-tolerance to the murine homologue of a tyrosinase-derived melanoma antigen: implications for tumor immunotherapy. *J Exp Med* 191: 1221-1231. PMID: 10748239. PMC2193167
2. Zarling, A.L., Ficarro, S.B., White, F.M., Shabanowitz, J., Hunt, D.F., and Engelhard, V.H. 2000. Phosphorylated peptides are naturally processed and presented by MHC class I molecules *in vivo*. *Journal of Experimental Medicine* 192: 1755-1762.
3. Mullins, D.W., Bullock, T.N.J., Colella, T.A., Robila, V.V., and Engelhard, V.H. 2001. Immune responses to the HLA-A\*0201-restricted epitopes of tyrosinase and gp100 enable control of melanoma outgrowth in HLA-A\*0201-transgenic mice. *J Immunol* 167:4853-4860. PMID: 11673489
4. Mullins, D.W., Sheasley, S.S., Ream, R.M., Bullock, T.N.J., Fu, Y.-X., and Engelhard, V.H. 2003. Route of immunization with peptide-pulsed dendritic cells controls the distribution of memory and effector T cells in lymphoid tissues and determines the pattern of regional tumor control. *J. Exp. Med.* 198: 1023. PMID: 14530375. PMC2194213
5. Bullock TN, Mullins DW, Engelhard VH. 2003. Ag density presented by dendritic cells *in vivo* differentially affects the number and avidity of primary, memory and recall CD8 T cells. *J. Immunol.* 170: 1822. PMID: 12574347
6. Slingluff, C.L., Petroni, G. R., Yamshchikov, G. V., Barnd, D. L., Eastham, S., Galavotti, H., Patterson, J. W., Deacon, D. H., Hibbitts, S., Teates, D., Neese, P. Y., Grosh, W. W., Chianese-Bullock, K. A., Woodson, E. M., Wiernasz, C. J., Merrill, P., Gibson, J., Ross, M., Engelhard, V. H. 2003. Clinical and immunologic results of a randomized phase II trial of vaccination utilizing four melanoma peptides either administered in GMCSF-in-adjuvant or pulsed on dendritic cells. *J. Clin. Oncol.* 21: 4016.
7. Hargadon KM, Brinkman CC, Sheasley-O'Neill SL, Nichols LA, Bullock TNJ and Engelhard, VH. 2006. Incomplete differentiation of antigen-specific CD8<sup>+</sup> T cells in tumor-draining lymph nodes. *J. Immunol.* 177:6081. PMID: 17056534
8. Zarling, A.L., Polefrone, J.M., Evans, A.M., Hopkins, L.M., Shabanowitz, J., Lewis, S.T., Hunt, D.F., and Engelhard, V.H. 2006. Identification of novel MHC-associated phosphopeptides as potential immunotherapeutic targets for cancer. *Proceedings of the National Academy of Sciences USA* 103:14889-14894.
9. Slingluff, Jr., C.L., Bullock, K., Bullock, T.N.J., Grosh, W.W., Mullins, D., Nichols, L., Olson, W., Petroni, G., Smolkin, M., and Engelhard, V.H. 2006. Immunity to melanoma antigens: from self-tolerance to immunotherapy. *Adv. Immunol.* 90:243. PMID: 16730266

**BIOGRAPHICAL SKETCH**

NAME Stephen W. Fesik	POSITION TITLE Professor of Biochemistry, Pharmacology, and Chemistry Vanderbilt University School of Medicine		
eRA COMMONS USER NAME (credential, e.g., agency login) FESIKSW			
EDUCATION/TRAINING <i>(Begin with baccalaureate or other initial professional education, such as nursing, and include postdoctoral training.)</i>			
INSTITUTION AND LOCATION	DEGREE <i>(if applicable)</i>	YEAR(s)	FIELD OF STUDY
College of the Holy Cross; Worcester, MA	BA	1975	Chemistry
University of CT School of Pharmacy; Storrs, CT	PhD	1981	Medicinal Chemistry
Yale University, New Haven, CT	Postdoc	1981-83	Biophysical Chemistry

**A. Personal Statement**

I have 26 years of drug discovery experience while working at Abbott Laboratories where I achieved the highest level of the scientific ladder (Volwiler Society) and for the last nine years served as the Divisional Vice President of Cancer Research. At Abbott, I developed new NMR methods, solved the three-dimensional structures of many proteins and protein/ligand complexes, pioneered the use of fragment-based methods for drug discovery, and as a VP was responsible for bringing several compounds into cancer clinical trials. I have extensive leadership experience, knowledge of drug discovery and development, and the development and application of fragment-based methods and structure-based approaches for drug design. Currently, I lead a team of about 25 scientists at Vanderbilt University with the goal of cancer drug discovery using fragment-based methods and structure-based design.

**B. Positions and Honors****Positions and Employment**

1983-1985: NMR Spectroscopist, Abbott Laboratories  
 1985-1988: Group Leader of NMR Research, Abbott Laboratories  
 1988-1992: Assoc. Res. Fellow, Volwiler Society, Abbott Laboratories  
 1992-1996: Res. Fellow, Volwiler Society, Abbott Laboratories  
 1996-2003: Senior Res. Fellow, Volwiler Society, Abbott Laboratories  
 2000-2009: Divisional Vice President of Cancer Research, Abbott Laboratories  
 2003-2009: Distinguished Res. Fellow, Volwiler Society, Abbott Laboratories  
 2009-present: Professor of Biochemistry, Pharmacology, and Chemistry, Vanderbilt University  
 2010-present: Orrin H. Ingram II Chair in Cancer Research, Vanderbilt University School of Medicine

**Editorial Boards**

1991-1995: Journal of Medicinal Chemistry  
 1991-2006: Journal of Biomolecular NMR  
 1997-2000: Biophysical Journal  
 2000-2003: Nature Reviews Cancer (Highlights Advisory Panel)  
 2001-present: Molecular Cell  
 2005-2012: Chemical Biology & Drug Design (Senior Editor)  
 2005-present: ChemMedChem  
 2007-present: Current Molecular Pharmacology (Assoc. Editor)  
 2008-present: Molecular Cancer Therapeutics  
 2009-present: Oncogene  
 2009-present: Combinatorial Chemistry and High Throughput Screening  
 2012-present: Journal of Biomolecular NMR  
 2013-present: Cancer Research

**Scientific Advisory Boards and Boards of Directors**

1998-2010: Scientific Advisory Board, Keystone Symposia  
 2003-2006: Scientific Advisory Board, Bruker BioSpin  
 2003-2010: Board of Directors, Keystone Symposia  
 2004-2011: Scientific Advisory Board, Abramson Cancer Center, UPenn

2007-present: Board of Directors, Bruker Corporation

2010-2012: Board of Directors, Inhibikase Therapeutics, Inc.

2011-present: Scientific Advisory Board, Aileron Therapeutics, Inc.

### **Selected Awards**

1997: Outstanding Researcher of the Year Award, Abbott Laboratories

1998: Servier Lecturer Award, University of Montreal

1999: ASBMB-Fritz Lipmann Award

2003: Eastern Analytical Society, Life time Achievement Award in Nuclear Magnetic Resonance

2008: Outstanding Research Team of the Year Award, Abbott Laboratories

2010: SBS 2010 Technology Innovation Award, Society for Biomolecular Sciences

2010: The 14<sup>th</sup> Annual Andrew H. Weinberg Memorial Lecture, Dana-Farber Cancer Institute, Harvard Medical School

2010: NIH Director's Pioneer Award

2010: 2010 AAAS Fellow

2012: 2012 AACR Award for Outstanding Achievement in Chemistry in Cancer Research

2012: The 13th Annual Gordon L. Hodgson Jr. Memorial Lecture, GlaxoSmithKline, RTP, NC

### **C. Selected Peer-Reviewed Publications (from over 240 total)**

1. Theriault, Y., T.M. Logan, R. Meadows, L. Yu, E. T. Olejniczak, T.F. Holzman, R.L. Simmer, and S.W. Fesik. Solution structure of the cyclosporin A/cyclophilin complex by NMR. *Nature*, 361, 88-91 (1993).
2. Yoon, H.S., P.J. Hajduk, A.M. Petros, E.T. Olejniczak, R.P. Meadows, and S.W. Fesik. Solution structure of a pleckstrin homology domain. *Nature*, 369, 672-675 (1994).
3. Harlan, J.E., P.J. Hajduk, H.S. Yoon, and S.W. Fesik. Pleckstrin homology domains bind to 5. Zhou, M.-M., K.S. Ravichandran, E.T. Olejniczak, A.M. Petros, R.P. Meadows, M. Sattler, J.E. Harlan, W.S. Wade, S.J. Burakoff, and S.W. Fesik. Structure and ligand recognition of the phosphotyrosine binding domain of Shc. *Nature*, 378, 584-592 (1995).
4. Muchmore, S.W., M. Sattler, H. Liang, R.P. Meadows, J.E. Harlan, H.S. Yoon, D. Nettlesheim, B.S. Chang, C.B. Thompson, S.-L. Wang, S.-C. Ng, and S.W. Fesik. X-ray and NMR structure of human Bcl-XL, an inhibitor of programmed cell death. *Nature*, 381, 335-341 (1996).
5. Shuker, S.B., P.J. Hajduk, R.P. Meadows, and S.W. Fesik. Discovering high-affinity ligands for proteins: SAR by NMR. *Science*, 274, 1531-1534 (1996).
6. Sattler, M., H. Liang, D. Nettlesheim, R.P. Meadows, J.E. Harlan, M. Eberstadt, H. Yoon, S.B. Shuker, B. Chang, A.J. Minn, C.B. Thompson, and S.W. Fesik. Structure of Bcl-xL/Bak peptide complex: Recognition between regulators of apoptosis. *Science*, 275, 983-986 (1997).
7. Eberstadt, M., B. Huang, Z. Chen, R. P. Meadows, S. Ng, L. Zheng, and S. W. Fesik. NMR structure and mutagenesis of the FADD (Mort1) death-effector domain. *Nature*, 392, 941-945 (1998).
8. Sun, C., M. Cai, A. H. Gunasekera, R. P. Meadows, H. Wang, J. Chen, H. Zhang, W. Wu, N. Xu, S.-C. Ng, and S. W. Fesik. NMR structure and mutagenesis of the inhibitor of apoptosis protein XIAP. *Nature*, 401, 818-822 (1999).
9. Oltschdorf, T., S.W. Elmore, A.R. Shoemaker, R.C. Armstrong, D.J. Augeri, B.A. Belli, M. Bruncko, T.L. Deckwerth, J. Dinges, P.J. Hajduk, M.K. Joseph, S. Kitada, S.J. Korsmeyer, A.R. Kunzer, A. Letai, C. Li, M.J. Mitten, D.G. Nettlesheim, S. Ng, P.M. Nimmer, J.M. O'Connor, A. Oleksijew, A.M. Petros, J.C. Reed, W. Shen, S.K. Tahir, C.B. Thompson, K.J. Tomaselli, B. Wang, M.D. Wendt, H. Zhang, S.W. Fesik and S.H. Rosenberg. An inhibitor of Bcl-2 family proteins induces regression of solid tumors. *Nature* 435, 677-681 (2005).
10. Sun Q., Burke J.P., Phan J., Burns M.C., Olejniczak E.T., Waterson A.G., Lee T., Rossanese O.W., Fesik S.W. Discovery of Small Molecules that Bind to K-Ras and Inhibit Sos-Mediated Activation. *Angew. Chem. Int. Ed. Engl.* 51(25): 6140-3 (2012). PMID: PMC3620661
11. Friberg A, Vigil D, Zhao B, Daniels RN, Burke JP, Garcia-Barrantes P, Camper D, Chauder B, Lee T, Olejniczak ET, Fesik SW. Discovery of potent myeloid cell leukemia-1 (Mcl-1) inhibitors using fragment-based methods and structure-based design. *J. Med. Chem.* 56(1): 15-30 (2013). PMID: PMC3646517
12. Patrone J.D., Kennedy J.P., Frank A.O., Feldkamp M.D., Vangamudi B., Pelz N.F., Rossanese O.W., Waterson A.G., Chazin W.J., Fesik S.W. Discovery of Protein-Protein Interaction Inhibitors of Replication Protein A. *ACS Med. Chem. Lett.* 4(7): 601-605. (2013) PMID: PMC3728914

**BIOGRAPHICAL SKETCH**

Provide the following information for the key personnel and other significant contributors.  
Follow this format for each person. **DO NOT EXCEED FOUR PAGES.**

NAME MULLIGHAN, Charles G.		POSITION TITLE Member, Department of Pathology Co-Leader, Hematological Malignancies Program Medical Director, Tissue Resources Core Facility	
eRA COMMONS USER NAME cgmullighan			
EDUCATION/TRAINING (Begin with baccalaureate or other initial professional education, such as nursing, and include postdoctoral training.)			
INSTITUTION AND LOCATION	DEGREE (if applicable)	YEAR(s)	FIELD OF STUDY
University of Adelaide, South Australia	B.Med. & B.Surg. (Hons)	1993	Medicine
University of London, United Kingdom	M.Sc.	1997	Immunology
University of Adelaide, South Australia	M.D.	1998	Immunogenetics
Haematology Joint Physician/Pathology Trainee, Institute of Medical and Veterinary Science		2001-2003	Haematology, Haematopathology
Fellowship of the Royal Australasian College of Physicians		2004	Haematology
Fellowship of the Royal College of Pathologists of Australasia		2004	Haematopathology

**A. Personal Statement**

I am an academic hematologist and hematopathologist dedicated to using genomic approaches and experimental modeling to understand the genetic factors driving the pathogenesis and outcome of acute leukemia. My work has defined the landscape of genetic alterations in acute lymphoblastic leukemia and related disorders including chronic myeloid leukemia. My current work is defining the genomic basis of high risk ALL. Recent highlights include the identification of *IKZF1* (IKAROS) mutations in multiple subtypes of high-risk ALL and the identification of novel mutations resulting in aberrant cytokine receptor signaling in high risk ALL, including rearrangement of *CRLF2* and activating JAK mutations, and novel rearrangements of *PDGFRB*, *ABL1* and *JAK2*; and the first whole genome sequencing studies of a childhood leukemia including, early T-cell precursor, BCR-ABL1-like and hypodiploid acute lymphoblastic leukemia. Each of these studies have identified important new targets for therapeutic intervention that are being vigorously pursued in experimental studies and clinical trials.

**B. Positions and Honors****Positions and Employment**

1993	Intern, Royal Adelaide Hospital, South Australia
1994-1997	University of Adelaide George Murray Scholar and Immunology Registrar, Departments of Immunology and Transplantation Immunology, Oxford Radcliffe Hospitals and University of Oxford, United Kingdom
1998-2000	Physician Trainee (Internal Medicine Resident), Royal Adelaide Hospital, South Australia
2001	Chief Medical Resident, Royal Adelaide Hospital
2001-2003	Haematology and Haematopathology Advanced Trainee, Department of Haematology, Institute of Medical and Veterinary Science and Royal Adelaide Hospital
2004-2008	NH&MRC (Australia) CJ Martin Travelling Postdoctoral Fellowship (top ranked application); Physician Scientist Postdoctoral Fellow, Department of Pathology, St. Jude Children's Research Hospital
2008-	Assistant Member, Department of Pathology, St. Jude Children's Research Hospital
2011-	Associate Member, Department of Pathology, St Jude Children's Research Hospital
2011-	Co-leader, Hematologic Malignancies Program, St Jude Children's Research Hospital
2013-	Affiliate Professor, Department of Medicine, University of Adelaide
2014-	Member, Department of Pathology, St Jude Children's Research Hospital

**Other Experience and Professional Memberships**

2001-	Haematology Society of Australia and New Zealand
2001-	American Society for Bone Marrow Transplantation



2001- American Society of Hematology  
 2001- Australian and New Zealand Society for Blood Transfusion  
 2001- Australasian Society for Thrombosis and Haemostasis  
 2004- Royal Australasian College of Physicians  
 2004- Royal College of Pathologists of Australasia  
 2005- International Society for Stem Cell Research  
 2005- American Association for Cancer Research  
 2006- American Society of Clinical Oncology  
 2007- Children's Oncology Group  
 2009- American Society of Human Genetics  
 2009- American Society of Pediatric Hematology and Oncology  
 2009- Section Editor, *Leukemia*  
 2010- Editor, *Blood*  
 2010- Founding Fellow, Faculty of Science, Royal College of Pathologists of Australasia  
 2010- Founding Member of ASH Working Committee on Scientific Affairs.  
 2011- NIH/NCI ALL Working Group  
 2011- Editor, Journal of Adolescent and Young Adult Oncology  
 2011- Editor, Frontiers in Pediatric Oncology  
 2011- Alliance (Cancer and Leukemia Group B) Correlative Sciences Committee Member  
 2011- Guest Editor, PLoS Genetics  
 2012- Editorial Board, Clinical and Translational Immunology  
 2012- Faculty of 1000

### Honors

1992 Deans List, Honors, University of Adelaide Medical School  
 2001 Chief Medical Resident, Royal Adelaide Hospital, South Australia  
 2001 Royal Australasian College of Physicians (SA Branch) Professor John Chalmers Prize  
 2001 Haematology Society of Australia and New Zealand Albert Baikie Memorial Award  
 2001-2002 Advanced Training Representative, Royal Australian College of Physicians, South Australia  
 2002 Royal Australasian College of Physicians Pfizer Advanced Trainee Prize  
 2002 Royal College of Pathologists of Australasia D.S. Nelson Prize  
 2003 Royal College of Pathologists of Australasia Kanematsu Award  
 2007 American Society of Hematology Merit Award  
 2007 American Society of Hematology Scholar Award  
 2007 American Association of Cancer Research / Aflac Career Development Award  
 2008 American Society of Hematology Joanne Levy, MD, Memorial Award for Outstanding Achievement  
 2009 Society for Pediatric Pathology Lotte Strauss Prize  
 2009 American Association for Cancer Research Team Science Award  
 2009 Pew Scholar in the Biomedical Sciences  
 2010 Founding Fellow, Faculty of Science, Royal College of Pathologists of Australasia  
 2012 American Society of Clinical Investigation  
 2012 Meyenburg Prize for Cancer Research

### C. 15 most relevant peer-reviewed publications (Selected from 139 indexed peer-reviewed publications; 8560 citations, *h* factor 46).

1. **Mullighan CG**, Goorha S, Radtke I, Miller CB, Coustan-Smith E, Dalton JD, Girtman K, Mathew S, Ma J, Pounds SB, Su X, Pui C-H, Relling MV, Evans WE, Shurtleff SA, Downing JR. Genome-wide analysis of genetic alterations in acute lymphoblastic leukaemia. *Nature* 446:758-764, 2007.
2. **Mullighan CG**, Miller CB, Radtke I, Phillips LA, Dalton J, Ma J, White D, Hughes TP, Le Beau MM, Pui C.-H., Relling MV, Shurtleff, SA, Downing JR. BCR-ABL1 lymphoblastic leukaemia is characterized by the deletion of Ikaros. *Nature* 453:110-114, 2008.
3. **Mullighan CG**, Phillips LA, Su X, Ma J, Miller CB, Shurtleff SA, Downing JR. Genomic analysis of the clonal origins of relapsed acute lymphoblastic leukemia. *Science* 2008;322:1377-80. PMID: PMC2746051
4. **Mullighan CG**, Su X, Zhang J, Radtke I, Phillips LA, Miller CB, Ma J, Liu W, Cheng C, Schulman BA, Harvey RC, Chen I-M, Clifford RJ, Carroll WL, Reaman G, Bowman WP, Devidas M, Gerhard DS, Yang W, Relling MV, Shurtleff SA, Campana D, Borowitz M, Pui C-H, Smith M, Hunger SP, Willman C, Downing JR, and the Children's Oncology

## **Product Development**

### **Review Panel Members for Approval**

1. James Foley, PhD; Managing Director of Aqua Partners, LLC
2. Ray DuBois, MD, PhD; Executive Director of The Biodesign Institute, Arizona State University
3. Yueming Li, PhD; Professor, Molecular Pharmacology and Chemistry Program, Memorial Sloan –Kettering Cancer Center
4. Subramanian Vaitheeswaran (Vaithee) – Advocate Reviewer
5. Gwen Harding-Peets – Advocate Reviewer
6. Mara B. Ginsberg, Esq. – Advocate Reviewer
7. Michael S. Katz. – Advocate Reviewer



# Curriculum Vitae

**James E. Foley**  
114 Glenn Road  
Ardmore, Pennsylvania 19003

Home tel: (610)-896-9936

Home fax: (610)-896-9926

Mobile tel: (610)-618-7660

E-mail (personal): [james.foley@bioglenn.com](mailto:james.foley@bioglenn.com)

EDUCATION	<b>Rutgers University</b>	1962-1966 (B.S.)
	<b>New York University</b>	1967-1971
	Graduate Studies Department of Biology	
	<b>Thomas Jefferson University</b>	1971-1975 (Ph.D.)
	College of Graduate Studies Department of Physiology Cardeza Foundation for Hematological Research	
PROFESSIONAL CERTIFICATION	<b>Certified Licensing Professional</b> Licensing Executives Society Certification number 1674 <sup>a</sup>	2008
EXPERIENCE	<b>Tulane University School of Medicine</b>	1975-1977
	Department of Pharmacology (James W. Fisher, Ph.D.) <i>NIH Post-Doctoral Fellowship</i>	
	<b>E.R. Squibb and Sons, Inc.</b> The Squibb Institute for Medical Research Human Pharmacology	
	<i>Assistant Director</i>	1977-1981
	<i>Associate Director</i>	1981-1987
	<ul style="list-style-type: none"><li>• Responsible for worldwide Phase I and early Phase II development of new chemical entities, primarily cardiopulmonary.</li><li>• Represented Squibb science as a participant in the Institutional Review Committee of the Medical Center at Princeton, which operated a Clinical Pharmacology Unit devoted exclusively to the early evaluation of Squibb compounds in man.</li><li>• Represented Squibb and their cosmetic/personal-care subsidiary, Charles of the Ritz Group, on the Pharmacology and Toxicology Committee of the Cosmetic, Toiletry and Fragrance Association, making significant contributions to the safety defense of various dyes including Reds 9 and 19 under threat of de-listing as cosmetic, pharmaceutical and food ingredients.</li><li>• Provided and managed in house human safety assessment services to Charles of the Ritz for their cosmetic, toiletry and sunscreen products.</li><li>• Served as Secretary, Vice President and President of the Squibb Management Association.</li></ul>	
	<b>Director- Biomedical Evaluation</b>	1987-1989
	<ul style="list-style-type: none"><li>• Established a development group dedicated to support of various Squibb business units to answer go/no-go development decisions.</li><li>• Provided support in generating preliminary clinical data supporting or confirming claims for in-licensing candidates.</li><li>• Responsible for a project team focused on completing a clinical summary reporting 3500 patients for an NDA supporting an ACE inhibitor, which included clinical pharmacology (27 studies), two pivotal double-blind, placebo-controlled Phase III studies (500 patients each) and six comparative Phase III studies. Data analysis and final Clinical Summary completed over a 5-month period 1 month ahead of an already aggressive schedule.</li></ul>	

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<sup>a</sup> The Certified Licensing Professional (CLP™) designation is intended to distinguish those who have demonstrated experience, proficiency, knowledge and

Worldwide Licensing and Business Analysis  
**Director- Scientific Liaison, Japan**

1989-1990

- Established a licensing/business development office in Tokyo for worldwide pharmaceuticals to search out product opportunities and potential alliances with Japanese companies of worldwide interest to Squibb and later B-MS.
- Established an in depth professional network within the Japanese pharmaceutical industry.

**Bristol-Myers Squibb Company**  
 Worldwide Licensing  
**Director- Licensing, Japan**

1990-1991

- Extended B-MS business development reach to include mainland China, Korea and Australia.
- Cultivated the beginnings of a business/research relationship with a major Japanese company.
- Provided business-development services to B-MS KK.

**SmithKline Beecham, p.l.c.**  
 Worldwide Business Development  
**Vice President and Director-  
 Business Development**

1991- 2001

**GlaxoSmithKline, p.l.c.**  
 Worldwide Business Development  
**Vice President  
 Business Development- Japan/Pacific**

2001- 2002

- Based in Tokyo (September, 1991-August, 1995) responsible for identification and progression of scientific and business opportunities of worldwide pharmaceutical interest from Japan and other Pacific countries (Australia, Korea, PRC, Taiwan, Hong Kong and Singapore).
- Developed sound scientific and business relationships with industrial and academic groups in the region and initiated discussions towards strategic business and/or scientific alliances with both local and worldwide reach.
- Identified and facilitated a total equity investment (over two rounds) of \$1.3 million from SR One in Sosei and Co., a 6-year old Japanese biotech/technology transfer company. The initial investment provided sufficient credibility for second-round investments by Stirred PTV, Rothschild and Oxford Bioscience Partners. In return, SB obtained a "window" on world-class academic biomedical research activities with commercial potential.
- Member of SB top-management Japan Strategy Committee to develop the 10-year vision for SmithKline Beecham Seiyaku, SB's 100% owned local business.
- Ad hoc member- International Management Committee.
- Participated on Japan Development Planning Committee and Japan Development Strategy Committee.
- Successfully represented SB pharmaceutical assets for out licensing in Japan (levromakalim, famciclovir, renzapride).
- Completed a 'sabbatical' in SB's corporate venture capital subsidiary SR One focused on investments in start up and emerging healthcare companies (biotechnology, e-health, etc.). Participated in new investment selection and initial fund raising activities for EuclidSR. (February 2000)
- Completed agreements include:
  - In license worldwide rights for tranilast (Rizaben from Kissei Pharmaceutical Co., Ltd.) to develop outside Japan for the prevention of restenosis. (May 1997).
  - In license worldwide rights to LB 20304a, a potent broad-spectrum quinolone from LG Chemical (Korea) for worldwide development outside Korea. (May 1997). SB filed NDA December 1999 with approval expected December 2000.
  - In-license agreement with Hayashibara for worldwide rights, including Japan, to the therapeutic applications for the human genes for IL-18 and its receptor in the fields of cancer, infection and autoimmune disease (excluding MAbs). (May 1999).
  - Termination of worldwide pranlukast license agreement with Ono with agreement on IND transfer to Ono and support to be provided by SB in recruiting and supporting a new worldwide development partner outside Japan. (June 1999).
  - With the advice and consent of SB Seiyaku (SBS) negotiated termination without penalties to SB of license agreement between SB, Yamanouchi and Avant Immunotherapeutics for co-development of TP 10 in Japan. (November 1999).
  - In-licensing agreement with Asahi Chemical Industries, Ltd. for exclusive worldwide rights to develop and commercialize AZ 40140, a selective beta-3 agonist for the treatment of type 2 diabetes and obesity. In Japan, SB and Asahi will co-distribute Avandia and AZ 40140 (February 2000).
  - In-licensing agreement with Taiho Pharmaceutical Company for exclusive worldwide rights outside Japan for TAS 106, a novel anti-cancer compound (May 2000).

- In-licensing agreement with Tanabe Seiyaku for exclusive worldwide rights outside Japan and certain Asian countries for TR 14035 (and related compounds), a novel  $\alpha_4\beta_1/\alpha_4\beta_7$  integrin antagonist targeted for asthma, rheumatoid arthritis, IBD and psoriasis (December 2000)

**Bristol-Myers Squibb Company** 2002- 2006  
 Corporate and Business Development  
*Vice President*  
*Business Development- Japan/Pacific*

- Responsible for sourcing and negotiating novel in-licensing opportunities of worldwide interest to the Company from Japan, Asia, and Australia/New Zealand.
- Management of post-agreement relationships with Japanese and Asian companies.
- Termination of UFT worldwide licensing agreement with Taiho and transfer of the worldwide business to E. Merck, Taiho's new partner.

**SMART Biosciences, Inc.** 2006- 2008  
 Philadelphia, Pennsylvania  
*President and Chief Executive Officer*

**Aqua Partners, LLC** 2008- present  
 Philadelphia, Pennsylvania  
*Managing Director*

## DEALS<sup>b</sup>

**Ono/SmithKline Beecham** (pranlukast, LTD4 antagonist, licensing agreement, 1992)  
**Takeda/SmithKline Beecham** (HGS Far-East R&D collaboration agreement, 1993)  
**KRICT/SmithKline Beecham** (SB quinolone discovery collaboration agreement, 1995)  
**LG Chem/SmithKline Beecham** (Factive, quinolone antibiotic, licensing agreement, 1997)  
**Kissei/SmithKline Beecham** (tranilast, prevention of post-PTCA restenosis, licensing agreement, 1997)  
**Hayashibara/SmithKline Beecham** (IL-18, therapeutic indications, licensing agreement, 1999)  
**Asahi/SmithKline Beecham** (AZ 40140, beta-3 agonist, licensing agreement, 2000)  
**Taiho/SmithKline Beecham** (TAS 106, RNA polymerase inhibitor, licensing agreement, 2000)  
**Tanabe/SmithKline Beecham** (dual  $\alpha_4$ -integrin antagonist, licensing agreement, 2000)  
**Tanabe/SmithKline Beecham** (multiple products, licensing agreement, 2001)  
**Taiho/Bristol-Myers Squibb** (UFT, termination agreement, 2005)  
**E. Merck/Bristol-Myers Squibb/Taiho** [transfer of UFT worldwide business (registered in 60 countries for first-line treatment of metastatic colorectal cancer) from BMS to E. Merck, 2005-2006]  
**KRICT/SMART Biosciences** (oncology discovery collaboration agreement, 2006)  
**Takeda/Pieris Ag** (anticalin discovery collaboration, 2011)  
**TetraLogic/Daiichi Sankyo** (clinical development collaboration/feasibility program, 2013)  
**Melior Discovery/Bukwang Pharmaceuticals** (MLR 1023 development collaboration and license agreement- Korea/Asia ex Japan, 2013)

## OUTSIDE PROFESSIONAL ACTIVITIES

**Sosei and Company, Inc.** 1993- 2002  
 Tokyo, Japan  
*Member, Board of Directors*

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<sup>b</sup> Deals identified through JEF and progressed in teams with transactional, scientific, regulatory, legal, manufacturing, finance, commercial and alliance management membership.

<b>Japan Pharmaceutical Licensing Association</b>	1990- 1995
Tokyo, Japan	
<i>Member, Organizing Committee</i>	1991-1995
<b>Japan America Society of Greater Philadelphia</b>	1996- present
Philadelphia, Pennsylvania	
<i>Member, Board of Directors</i>	
<i>Co-Chairman, Organizing Committee</i>	1999- present
<i>US- Japan Health Sciences Dialogue</i>	
<b>New York Pharma Forum</b>	1996-2006
New York, New York	
<i>Member, Board of Directors</i>	1997- 2006
<b>Thomas Jefferson University</b>	1998- 2002
College of Graduate Studies	
<i>Member, Advisory Committee for the Graduate Center</i>	
<b>St. Joseph's University</b>	2000- 2004
Haub School of Business	
<i>Member, Advisory Committee</i>	
<b>Biotechnology Industry Organization</b>	2003- 2007
<i>Member- Business Development Committee</i>	
<i>Member- Business Forum Committee (BIO 2005 Annual Meeting)</i>	
<i>Member- International Committee (BIO 2005 Annual Meeting)</i>	
<i>Member- Program Committee (BioAsia 2007, BioAsia 2008)</i>	
<b>University of Southern California</b>	2005- 2006
Marshall School of Business	
Global BioBusiness Initiative	
<i>Member, Advisory Board</i>	
<b>Traxion Therapeutics</b>	2006- 2009
Baltimore, Maryland	
<i>Member, Advisory Board</i>	
<b>Science Center Capital I</b>	2006- 2007
The University City Science Center	
Philadelphia, Pennsylvania	
<i>Member, Advisory Committee</i>	
<b>SFJ Pharmaceuticals</b>	2009- present
San Francisco, California	
<i>Member, Board of Directors</i>	
<b>Rutgers, The State University of New Jersey</b>	2010- present
School of Management and Labor Relations	
Master of Business and Science Program	
<i>Member, Industrial Advisory Board- Drug Discovery and Development</i>	

**Thomas Jefferson University**

2013- present

Jefferson Graduate School of Biomedical Sciences Alumni Association  
Member, Executive Board

**MEMBERSHIPS**

American Association for the Advancement of Science  
American Society of Clinical Pharmacology and Therapeutics  
American Chamber of Commerce in Japan  
American College of Clinical Pharmacology  
American Heart Association  
Australian Biotechnology Association  
Japan America Society of Greater Philadelphia (Member, Board of Directors)  
Japan Pharmaceutical Licensing Association  
Licensing Executives Society  
New York Academy of Sciences

**PROFESSIONAL  
AWARDS**

**Frank Barnes Mentorship Award**  
Licensing Executive Society  
October 18, 2004





## BIOGRAPHICAL SKETCH

Provide the following information for the key personnel and other significant contributors in the order listed on Form Page 2.  
Follow this format for each person. **DO NOT EXCEED FOUR PAGES.**

NAME	POSITION TITLE		
DuBois, Raymond N., Jr.	Biodesign Institute Executive Director		
eRA COMMONS USER NAME (credential, e.g., agency login)			
duboisrn			
EDUCATION/TRAINING (Begin with baccalaureate or other initial professional education, such as nursing, and include postdoctoral training.)			
INSTITUTION AND LOCATION	DEGREE (if applicable)	YEAR(s)	FIELD OF STUDY
Texas A&M University, College Station, TX	BS	1977	Biochemistry
UT Health Science Center at Dallas, Dallas, TX	PHD	1981	Biochemistry
UT Health Science Center at San Antonio, TX	MD	1985	Medicine
The Johns Hopkins Hospital, Baltimore, MD	Osler Med Intern & Residency	1988	
The Johns Hopkins Hospital, Baltimore, MD	Gastroenterology Fellowship	1991	

### A. Personal Statement

My research is focused on understanding the molecular, genetic, and cellular bases for normal and neoplastic intestinal biology. As part of this focus, my laboratory is extremely interested in understanding how inflammation and inflammatory mediators contribute to colorectal carcinogenesis. Discoveries we have made over the life of this R01 have improved our understanding of the central role of PGE<sub>2</sub> signaling in CRC in colorectal carcinogenesis. More specifically, our recent work uncovers a previously unrecognized role of prostaglandin E<sub>2</sub> (PGE<sub>2</sub>) in promoting intestinal tumor growth by silencing certain tumor suppressor and DNA repair genes via DNA methylation. Moreover, our studies reveal that PGE<sub>2</sub> contributes to the adaptive responses of colorectal carcinoma cells encountering hypoxia during cancer progression via ANGPTL4 and/or HEF1. Although my group and others have extensively investigated the mechanisms by which PGE<sub>2</sub> promotes colorectal cancer progression, it is still not fully understood how PGE<sub>2</sub> accelerates CRC formation and progression. This renewal application is a logical extension of my previous work. I plan to continue to address many exciting and challenging questions outlined in the proposed research plan. Answers to these questions will not only reveal comprehensive insights of how PGE<sub>2</sub>, CXCR2, MDSCs, and NK cells coordinately contribute to CRC initiation, progression, and metastasis, but also provide a rational for applying adoptive transfer of allogeneic NK cells with subverting tumor-induced immunosuppression as novel therapeutic approaches in metastatic and adjuvant therapies. My scientific background and administrative skill set make me fully qualified to serve as the PI on this R01.

### B. Positions and Honors.

#### Positions and Employment

1988-1991	Howard Hughes Research Associate, Molecular Biology, Johns Hopkins School of Medicine, Baltimore, MD
1991-1994	Assistant Professor, Vanderbilt University Medical Center, Nashville, TN
1994-2004	Director, Vanderbilt Digestive Disease Research Center, Nashville, TN
1994-1997	Associate Professor, Vanderbilt University Medical Center, Nashville, TN
1996-2004	Director, Cancer Prevention Program, Vanderbilt-Ingram Cancer Center, Nashville, TN
1997-2003	Mina C. Wallace Professor, Medicine & Cell Biology, VUSM, Nashville, TN
1998-2005	Associate Director, Department of Cancer Prevention, VICC, Nashville, TN
1998-2003	Director, Gastroenterology, Vanderbilt University Medical Center, Nashville, TN
2003-2004	Hortense B. Ingram Professor, Molecular Oncology, VUSM, Nashville, TN
2005-2007	Director, Vanderbilt-Ingram Cancer Center, Nashville, TN
2007-2012	Professor of Cancer Biology & Cancer Medicine, UTMDACC, Houston, TX
2007-2012	Provost & Executive Vice President, UTMDACC, Houston, TX
2012-present	Executive Director, Biodesign Institute, Arizona State University, Tempe, AZ

#### Other Experience and Professional Memberships

1989	Member, American Gastroenterological Association (AGA)
1991	Member, American Society for Biochemistry and Molecular Biology (ASBMB)
1994	Member, American Association for Cancer Research (AACR)

1997 Member, American Society for Clinical Investigation (ASCI)  
 1999-2003 AGA Council, Vice Chair and Chair, GI Oncology Section, AGA  
 2003-present Board of Directors, AACR  
 2004-2005 AACR Task Force on Cancer Prevention, AACR  
 2005-2006 Cancer Research Editor Search Committee, AACR  
 2005-present AACR Steering Committee Clinical/Translational Research, Science Policy and Legislative Affairs Committee, AACR  
 2006 AGA William Beaumont Prize Selection Committee, AGA  
 2006-present Special Conferences Committee, Publications Committee, AACR  
 2007-2009 President Elect, American AACR  
 2007-2012 Chair, Research Council, The University of Texas M. D. Anderson Cancer Center, Houston, TX  
 2007-2012 Member, Executive Committee, UTMDACC, Houston, TX  
 2007-2012 Member, M. D. Anderson Lung P01 Advisory Board, UTMDACC, Houston, TX  
 2008-2012 Ex Officio Member, Graduate Education Committee, UTMDACC, Houston, TX

### **Honors**

2000 E.V. Newman Research Prize, Vanderbilt University Department of Medicine  
 2000 Outstanding Investigator Award, American Federation for Medical Research  
 2000 President, Southern Society for Clinical Investigation  
 2000 President, Gastroenterology Research Group of the AGA  
 2000 Royal College of Physicians (by distinction), London UK  
 2002 Board of Scientific Advisors for the Director of the National Cancer Institute, NIH  
 2002 Richard and Hinda Rosenthal Foundation Cancer Research Award, AACR  
 2003 Board of Directors, American Association for Cancer Research  
 2003 National Diabetes, Digestive and Kidney Diseases Advisory Council, NIH  
 2004 Distinguished Achievement Award, American Gastroenterological Association (AGA)  
 2004 Dorothy P. Landon Cancer Research Prize, AACR  
 2004 Fellow, American Association for the Advancement of Science  
 2006 Anthony Dipple Carcinogenesis Award, Oxford University Press  
 2007 Johns Hopkins Society of Scholars, Johns Hopkins  
 2008-2009 President, AACR  
 2009-2012 Ellen F. Knisely Distinguished Chair in Colon Cancer Research, MD Anderson Cancer Center

### **C. Selected peer-reviewed publications (in chronological order).**

(Publications selected from 219 peer-reviewed publications)

#### **Most relevant to the current application**

1. Wang D, Wang H, Shi Q, Katkuri S, Walhi W, Desvergne B, Das SK, Dey SK, DuBois RN. Prostaglandin E(2) promotes colorectal adenoma growth via transactivation of the nuclear hormone receptor PPRA $\delta$ . *Cancer Cell* 6:285-295, 9/2004. PMID: 15380519.
2. Wang, D., Wang, H., Brown, J., Daikoku, T., Ning, W., Shi, Q., Richmond, A., Strieter, R., Dey, S.K., and DuBois, R.N. CXCL1 induced by prostaglandin E2 promotes angiogenesis in colorectal cancer. *J Exp Med* 203:941-951, 4/2006. PMID: 16567391.
3. Kim SH, Xia D, Kim SW, Holla V, Menter DG, Dubois RN. Human enhancer of filamentation 1 Is a mediator of hypoxia-inducible factor-1 $\alpha$ -mediated migration in colorectal carcinoma cells. *Cancer research*.70(10):4054-63, 2010. PMCID: 2871069.
4. Kim SH, Park YY, Kim SW, Lee JS, Wang D, Dubois RN. ANGPTL4 induction by prostaglandin E2 under hypoxic conditions promotes colorectal cancer progression. *Cancer Res.* 71(22):7010-20, 11/2011. e-Pub 9/2011. PMID: 21937683.
5. Xia D, Wang D, Kim SH, Katoh H, Dubois RN. Prostaglandin E(2) promotes intestinal tumor growth via DNA methylation. *Nat Med.* 18(2):224-6, 2012. e-Pub 1/2012. PMID: 22270723.

#### **Additional recent publications of importance to the field (in chronological order)**

6. Buchanan, F.G., Gorden, D.L., Matta, P., Shi, Q., Matrisian, L.M., and DuBois, R.N. Role of beta- arrestin 1 in the metastatic progression of colorectal cancer. *Proc Natl Acad Sci U S A* 103:1492-1497, 2006. PMCID:1360588.
7. Cha, Y.I., Kim, S.H., Sepich, D., Buchanan, F.G., Solnica-Krezel, L., and DuBois, R.N. Cyclooxygenase-1-derived PGE2 promotes cell motility via the G-protein-coupled EP4 receptor during vertebrate gastrulation.

Genes & development 20:77-86, 2006. PMCID:1356102.

8. Buchanan, F.G., Holla, V., Katkuri, S., Matta, P., and DuBois, R.N. Targeting cyclooxygenase-2 and the epidermal growth factor receptor for the prevention and treatment of intestinal cancer. *Cancer research* 67:9380-9388, 10/2007. PMID: 17909047.
9. Backlund, M.G., Mann, J.R., Holla, V.R., Shi, Q., Daikoku, T., Dey, S.K., and DuBois, R.N. Repression of 15-hydroxyprostaglandin dehydrogenase involves histone deacetylase 2 and snail in colorectal cancer. *Cancer research* 68:9331-9337, 2008. PMID: 19010907.
10. Holla, V.R., Backlund, M.G., Yang, P., Newman, R.A., and DuBois, R.N. Regulation of prostaglandin transporters in colorectal neoplasia. *Cancer prevention research* 1:93-99, 2008. PMID: 19138942.
11. Wang D, Dubois RN, Richmond A. The role of chemokines in intestinal inflammation and cancer. *Curr Opin Pharmacol.* 9(6):688-96. 9/2009. e-Pub 9/2009. PMCID: 2887713.
12. Xia, D., Holla, V.R., Wang, D., Menter, D.G., and DuBois, R.N. HIF1 is a crucial mediator of the proliferative effects of prostaglandin E(2) on colon cancer cells. *Cancer research* 70:824-831, /12/2010. PMID: 20068165.
13. Wang D, Dubois RN. Eicosanoids and cancer. *Nat Rev Cancer* 10(3):181-93, 3/2010. e-Pub 2/2010. PMID: 20168319.
14. Wang D, DuBois RN. The Role of the PGE<sub>2</sub>-Aromatase Pathway in Obesity-Associated Breast Inflammation. *Cancer Discov* 2(4):308-10, 4/2012. PMID: 22576207.
15. Wang D, Dubois RN. The role of anti-inflammatory drugs in colorectal cancer. *Annu. Rev. Med.* e-Pub 09/2012. PMID: 23020877.

#### **D. Research Support.**

##### **ACTIVE**

<b>P01 CA77839-11A1 (PI)</b>	<b>2/01/2000-5/31/2017</b>	<b>1.92 calendar</b>
<b>NIH/NCI</b>	<b>\$1,204,017</b>	

##### **The Role of Bioactive Lipids in Inflammation and Cancer**

Major goals: The goal of this P01 is develop mechanism based chemoprevention strategies that utilize alternative approaches leading to COX-2 independent suppression of PGE<sub>2</sub> production or to block the downstream pro-carcinogenic effects of this bioactive lipid. The significance of the proposed study is very high and there is a potential of gaining new knowledge about bioactive lipid signaling networks. Four projects at three different performance sites are proposed that share a common goal of developing combination cancer chemoprevention strategies.

<b>RP100960 (PI)</b>	<b>4/1/2010-4/1/2013</b>	<b>1.2 calendar</b>
<b>Cancer Prevention &amp; Research Institute of Texas (CPRIT)</b>	<b>\$399,431</b>	

##### **Prostaglandins and Inflammation in Colorectal Cancer**

Major goals: Considering the importance of PGE<sub>2</sub> signaling in inflammation and colon carcinogenesis, the approach in this proposal is to determine the molecular mechanisms by which PGE<sub>2</sub> promotes tumor formation and growth by regulating DNA methylation machinery and adapting the microenvironment in order to support tumor growth.

<b>5R37-DK047297-16 (PI)</b>	<b>8/1/1994-7/31/2013</b>	<b>1.2 calendar</b>
<b>NIH/NCI</b>	<b>\$250,000</b>	

##### **Role of Eicosanoids in Intestinal Epithelial Growth and Function**

Major goals: The specific aims of this project are to determine the role of eicosanoids in colorectal cancer carcinogenesis. These studies will examine the mechanism(s) by which COX-2 derived PGE<sub>2</sub> regulates the motility and invasiveness of colorectal carcinoma cells. They will also delineate the role of the nuclear PGI<sub>2</sub> receptor, PPARdelta, in the development of colorectal cancer. Furthermore they will determine the relative contribution of COX-2 expressed in malignant epithelial cells versus the surrounding stroma (host) in promoting colorectal cancer.



**BIOGRAPHICAL SKETCH**

Provide the following information for the Senior/key personnel and other significant contributors in the order listed on Form Page 2.  
Follow this format for each person. **DO NOT EXCEED FOUR PAGES.**

NAME Yueming Li	POSITION TITLE Member and Professor		
eRA COMMONS USER NAME (credential, e.g., agency login) LIYUEMING			
EDUCATION/TRAINING (Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable.)			
INSTITUTION AND LOCATION	DEGREE (if applicable)	MM/YY	FIELD OF STUDY
Shanxi Agriculture University, China	B.S.	01/82	Plant Biology
Chinese Academy of Sciences	M.S.	10/84	Toxicology
University of California, Berkeley	Ph.D.	05/92	Comparative Biochem
Harvard Medical School	Postdoc	09/97	Biochemistry & Pharma.

**A. Personal Statement**

The main interests of my laboratory are to elucidate the mechanism of  $\gamma$ -secretase-dependent Notch signaling and amyloid precursor protein (APP) processing under physiological and pathological conditions and to develop target-based therapies.  $\gamma$ -Secretase represents a novel class of proteases that catalyze proteolysis of substrates within the transmembrane domain, an apparently hydrophobic environment, wherein water is required for peptide hydrolysis. Intramembranous proteases, including  $\gamma$ -secretase, site 2 proteases, rhomboids and signal peptide peptidase, play an essential role in many biological processes ranging from sterol regulation to development. Unraveling the intricacies of these enigmatic proteases is a formidable challenge that requires not only multi-disciplinary approaches, but also development of novel technologies that are suitable to study the structure and catalysis of membrane proteins. I have brought chemical biology into intramembranal proteolysis that significantly advances our understanding of  $\gamma$ -secretase and other proteases. We developed the first *in vitro*  $\gamma$ -secretase activity assay and made a groundbreaking discovery that  $\gamma$ -secretase activity is catalyzed by the presenilin containing macromolecular complex, providing a molecular basis to search for additional subunits. More significantly, my group has demonstrated that presenilin contains the active site of  $\gamma$ -secretase, revealing the first biochemical identity of  $\gamma$ -secretase using photoaffinity labeling approaches. Publication of this work in *Nature* was recognized as a "hot paper" by The Scientist, due to the large volume of citations. Furthermore, my lab has reconstituted  $\gamma$ -secretase and presenilinase using recombinant proteins and provided the final proof that presenilin is  $\gamma$ -secretase, which has offered a novel platform for studying the structure and function of  $\gamma$ -secretase at both molecular and atomic levels. This work on the reconstitution of  $\gamma$ -secretase published in *PNAS* was selected as one of the Alzheimer Research Forum's 13 Top AD Trends in 2010. Recent clinical disappointment of  $\gamma$ -secretase inhibitor studies indicates that development of  $\gamma$ -secretase based therapy is an enormous challenge that requires a comprehensive understanding of the target. We have focused on development of  $\gamma$ -secretase modulators (GSMs) and elucidation of their mechanism of action using an integrated approach of chemical biology and proteomics. We have demonstrated that GSMs target  $\gamma$ -secretase and structurally distinct GSMs occupy different sites of  $\gamma$ -secretase, offering a molecular basis for drug combination studies.

**B. Positions and Honors**

1984-1986	Research Associate, Institute of Zoology, Chinese Academy of Science
1997-2000	Research Fellow, Department of Biological Chemistry, Merck Research Laboratories
2000-2002	Senior Research Fellow, Department of Biological Chemistry, Merck Research Laboratories
2002-2005	Assistant Member, Molecular Pharmacology & Chemistry Program, Sloan-Kettering Institute
2002-2005	Assistant Member, Memorial Sloan-Kettering Cancer Center (MSKCC)
2005-2011	Associate Member, Molecular Pharmacology & Chemistry Program, Sloan-Kettering Institute
2005-2011	Associate Member, Memorial Sloan-Kettering Cancer Center

2011-present Member and Professor, Molecular Pharmacology & Chemistry Program, Sloan-Kettering Institute  
2011-present Member, Memorial Sloan-Kettering Cancer Center  
2011-present Director, Pharmacology Graduate Program, Weill Medical College of Cornell University

#### **Other Experience and Professional Memberships**

1997-2002 Member, Protease Task Force Committee, Merck Research Laboratories  
2002-Present Member, Experimental Therapeutics Center, MSKCC  
2003-2007 Member, Institutional Animal Care and Use Committees, MSKCC  
2002-Present Member, Tri-Institutional Chemical Biology Training Program  
2002-Present Curriculum Committee, Pharmacology Depart, Weill Graduate School of Medical Sciences  
2008-present Member, Technology Development Executive Committee, MSKCC  
2007-present Member, Clinical& Translational Science Center, the Weill Medical College  
2002-2005 Assistant Professor, the Weill Medical College of Cornell University  
2005-2012 Associate Professor, the Weill Medical College of Cornell University  
2005-2011 Associate Professor, Gerstner Sloan-Kettering Graduate School  
2011-present Professor, Gerstner Sloan-Kettering Graduate School  
2012-present Professor, the Weill Medical College of Cornell University  
2004-2005 AD Hoc Member, NIH Study Section, CDIN (Death and Injury in Neurodegeneration Study Section)  
2008 AD Hoc Member, NIH SBCB Study Section, "Synthetic and Biological Chemistry B"  
2010-present AD Hoc Member, Drug Discovery for the Nervous System Study Section (ZRG1 MDCN-C)  
2011 AD Hoc Member, NCI P01 "Mechanisms of Cell Signaling in Cancer" Special Emphasis Panel

#### **Honors**

2002 "Hot Paper" by The Scientist 16:34 (for a paper published in **Nature** 405: 689, 2000)  
2004-2006 The Zenith Fellows Award, Alzheimer's Association  
2013 The MetLife Foundation Award for Medical Research

#### **C. Selected Peer-reviewed Publications (Selected from 85 peer-reviewed publications)**

1. Li YM\*, Lai MT, Xu M, Huang Q, DiMuzio-Mower J, Sardana MK, Shi XP, Yin KC, Shafer JA, Gardell SJ. Presenilin 1 is linked with gamma-secretase activity in the detergent solubilized state. *Proc Natl Acad Sci U S A* 2000;97(11):6138-43 PMID: 10801983 (\*Corresponding author)
2. Li YM\*, Xu M, Lai MT, Huang Q, Castro JL, DiMuzio-Mower J, Harrison T, Lellis C, Nadin A, Neduvellil JG, Register RB, Sardana MK, Shearman MS, Smith AL, Shi XP, Yin KC, Shafer JA, Gardell SJ. Photoactivated gamma-secretase inhibitors directed to the active site covalently label presenilin 1. *Nature* 2000;405(6787):689-94 PMID: 10864326 (\*co-Corresponding author)
3. Chun J, Yin YI, Yang G, Tarassishin L, Li YM. Stereoselective synthesis of photoreactive peptidomimetic gamma-secretase inhibitors. *J Org Chem*. 2004;69(21):7344-7. PMID: 15471490
4. Tarassishin L, Yin YI, Bassit B, Li YM. Processing of Notch and amyloid precursor protein by gamma-secretase is spatially distinct. *Proc Natl Acad Sci U S A*. 2004;101(49):17050-5. PMID: 15563588
5. Placanica L, Tarassishin L, Yang G, Peethumnongsin E, Kim SH, Zheng H, Sisodia SS, Li YM. Pen2 and presenilin-1 modulate the dynamic equilibrium of presenilin-1 and presenilin-2 gamma-secretase complexes. *J Biol Chem*. 2009;284(5):2967-77. PMID: 19036728
6. Placanica L, Zhu L, Li YM. Gender- and age-dependent gamma-secretase activity in mouse brain and its implication in sporadic Alzheimer disease. *PLoS One*. 2009;4(4):e5088. PMID: 19352431
7. Shelton CC, Zhu L, Chau D, Yang L, Wang R, Djaballah H, Zheng H, Li YM. Modulation of gamma-secretase specificity using small molecule allosteric inhibitors. *Proc Natl Acad Sci U S A*. 2009;106(48):20228-33. PMID: 19906985
8. Yang G, Yin YI, Chun J, Shelton CC, Ouerfelli O, Li YM. Stereo-controlled synthesis of novel photoreactive gamma-secretase inhibitors. *Bioorg Med Chem Lett*. 2009;19(3):922-5. PMID: 19097779
9. Ahn K, Shelton CC, Tian Y, Zhang X, Gilchrist ML, Sisodia SS, Li YM. Activation and intrinsic gamma-secretase activity of presenilin 1. *Proc Natl Acad Sci U S A*. 2010;107(50):21435-40. PMID: 21115843

10. Tian Y, Bassit B, Chau D, Li YM. An APP inhibitory domain containing the Flemish mutation residue modulates gamma-secretase activity for Abeta production. *Nat Struct Mol Biol.* 2010;17(2):151-8 PMID: 20062056
11. Crump CJ, Fish BA, Castro SV, Chau DM, Gertsik N, Ahn K, Stiff C, Pozdnyakov N, Bales KR, Johnson DS, Li YM. Piperidine acetic acid based gamma-secretase modulators directly bind to Presenilin-1. *ACS Chem Neurosci.* 2011;2(12):705-10. PMID: 22229075
12. Crump CJ, Castro SV, Wang F, Pozdnyakov N, Ballard TE, Sisodia SS, Bales KR, Johnson DS, Li YM. BMS-708,163 Targets Presenilin and Lacks Notch-Sparing Activity. *Biochemistry.* 2012;51(37):7209-11. PMID: 22931393
13. Chau DM, Crump CJ, Villa JC, Scheinberg DA, Li YM. Familial Alzheimer Disease Presenilin-1 Mutations Alter the Active Site Conformation of gamma-secretase. *J Biol Chem.* 2012;287(21):17288-96. PMID: 22461631
14. Pozdnyakov N, Murrey HE, Crump CJ, Pettersson M, Ballard TE, Am Ende CW, Ahn K, Li YM, Bales KR, Johnson DS. gamma-Secretase modulator (GSM) photoaffinity probes reveal distinct allosteric binding sites on presenilin. *J Biol Chem.* 2013;288(14):9710-20. PMID: 23396974
15. Gertsik N, Ballard TE, am Ende CW, Johnson DS, Li Y-M. Development of CBAP-BPyne, a probe for [gamma]-secretase and presenilinase. *MedChemComm.* 2014;DOE 10.1039/C3MD00281K.

#### D. Ongoing Research Support

5R01 NS076117 Yueming Li (PI, 20%) 6/01/2011-05/31/2016  
NIH/NINDS (Co-PI: Dr. Hui Zheng at Baylor Medical College)

Project Title: Mechanism and therapy of A $\beta$ -42-specific gamma-secretase

This proposal directly addresses the mechanisms and functional role of A $\beta$ 42-specific inhibition in synaptic plasticity and learning and memory. It will greatly facilitate the understanding and development of  $\gamma$ -secretase-based AD therapy.

5R01 AG026660 Yueming Li (PI, 20%) 8/1/2011 - 8/31/2016  
NIH/NIA

Project Title: Regulation and Function of Gamma-Secretase

The overall objective of the proposed study is to characterize endogenous  $\gamma$ -secretase from various cell lines and tissues and determine the individuality and commonality of the complexes for their function and regulation.





# Subramanian Vaitheeswaran (Vaithee)

Department of Chemistry and  
Department of Chemical Engineering  
University of Massachusetts Amherst  
Amherst, MA 01003

Res: 48 Long Plain Road  
Leverett, MA 01054  
Tel: 301-787-8100  
vaithee05@gmail.com

## Honors

- Pre-doctoral Visiting Fellowship 2002  
National Institute of Diabetes and Digestive and Kidney Diseases (NIDDK),  
National Institutes of Health; Bethesda, MD
- Frank H. Todd Scholarship 1999 - 2002  
Dept. of Physics and Astronomy, University of Maine; Orono, ME
- Sigma Pi Sigma, Physics Honor Society
- Junior Research Fellowship; University of Mumbai; Mumbai, India 1993 - 1994

## Work History

- **Lecturer;** Department of Chemical Engineering, University of Massachusetts Amherst; Amherst, MA Fall 2013
  - Instructor for Chem-Eng 475, Physical Chemistry: Quantum Mechanics, Spectroscopy and Statistical Thermodynamics.
  - Responsible for the entire course, including syllabus, lectures, homeworks and exams for a class of 94 undergraduates.
- **Senior Research Fellow;** Department of Chemistry and Department of Chemical Engineering, University of Massachusetts Amherst; Amherst, MA 2011 - 2013
  - QM/MM models of the conversion of biomass to liquid fuels.
  - Experimental and theoretical study of the zeolite-catalyzed conversion of furan to biofuels.
  - DFT study of the mixed aldol condensation reaction between acetone and formaldehyde, catalyzed by HZSM-5 and HY zeolites.
- **Post-doctoral Research Associate;** Center for Biotechnology and Interdisciplinary Studies, Rensselaer Polytechnic Institute; Troy, NY 2008 - 2011
  - Theoretical study of multivalent ligand-receptor interactions in biological systems.
  - Coarse-grained simulations of protein-carbon nanotube complexes.
  - Theoretical modeling of the terahertz spectra of proteins.
- **Post-doctoral Research Associate;** Institute for Physical Science and Technology, University of Maryland; College Park, MD 2004 - 2008
  - Molecular dynamics and Monte Carlo simulations of water and small molecules in nanopores.
  - Molecular dynamics studies of hydration effects on proteins in confinement.

## Peer-reviewed Publications

1. S. Vaitheeswaran, S. K. Green, P. Dauenhauer and S. M. Auerbach. On the Way to Biofuels from Furan: Discriminating Diels-Alder and Ring-Opening Mechanisms. *ACS Catal.* 2013, **3**, 2012-2019.
2. S. Vaitheeswaran, J. Chen and D. Thirumalai. Hydrophobic and Ionic Interactions in Bulk and Confined Water with Implications for Collapse and Folding of Proteins. *J. Stat. Phys.* 2011, **145**, 276-292.
3. S. Vaitheeswaran and A. E. Garcia. Protein Stability at a Carbon Nanotube Interface. *J. Chem. Phys.* 2011, **134**, 125101.
4. S. Vaitheeswaran, G. Reddy and D. Thirumalai. Water-mediated Interactions between Hydrophobic and Ionic Species in Cylindrical Nanopores. *J. Chem. Phys.* 2009, **130**, 094502.
5. S. Vaitheeswaran and D. Thirumalai. Interactions between Amino Acid Side Chains in Cylindrical Hydrophobic Nanopores with Applications to Peptide Stability. *Proc. Natl. Acad. Sci. U.S.A.* 2008, **105**, 17636-17641.
6. S. Vaitheeswaran and D. Thirumalai. Hydrophobic and Ionic Interactions in Nanosized Water Droplets. *J. Am. Chem. Soc.* 2006, **128**, 13490-13496.
7. S. Vaitheeswaran, H. Yin and J. C. Rasaiah. Water between Plates in the Presence of an Electric Field in an Open System. *J. Phys. Chem. B.* 2005, **109**, 6629-6635.
8. S. Vaitheeswaran, H. Yin, J. C. Rasaiah and G. Hummer. Water Clusters in Nonpolar Cavities. *Proc. Natl. Acad. Sci. U.S.A.* 2004, **101**, 17002-17005.
9. S. Vaitheeswaran, J. C. Rasaiah and G. Hummer. Electric Field and Temperature Effects on Water in the Narrow Nonpolar Pores of Carbon Nanotubes. *J. Chem. Phys.* 2004, **121**, 7955-7965.

## Education

- Ph. D., Physics; University of Maine; Orono, ME 2004  
Thesis title: Computer Simulations of Partially Confined Water.
- M. Sc., Physics; University of Mumbai; Mumbai, India 1993  
Thesis title: Empirical Rules for Phase Formation in Ion Implanted Alloys.
- B. Sc., Physics; R. Jhunjhunwala College; Mumbai, India 1990

## Activities

- Reviewer for the Congressionally Directed Medical Research Program (CDMRP) of the U. S. Department of Defense, 2009-2012.
- Volunteer for *The Leukemia & Lymphoma Society's* First Connection program, a Peer-to-Peer program for newly diagnosed patients with blood cancers.
- An avid runner, I ran the Marine Corps Marathon in 2006 to raise funds for *Asha for Education*, a non-profit that supports children in India who have limited access to education.

# Gwen Harding-Peets, PhD

## Ovarian Cancer Survivor

### Curriculum Vitae

41 West Pine Road  
Staatsburg, NY 12580

(845) 889-4860 (home)  
(914) 475-3983 (cell)

#### Diagnosis and Treatments

2/05	1 <sup>st</sup> Debulking, surgery, dx stage IIIC ovarian papillary serous carcinoma, high grade
2/05-6/05	First line treatment: 6 cycles IV Carboplatin/Taxol
8/05-7/06	Maintenance therapy: 12 cycles Taxol
5/08	2 <sup>nd</sup> Debulking surgery for recurrence
6/08 -11/08	2 <sup>nd</sup> line chemotherapy: 6 cycles Carboplatin/Gemzar
6/09-8/09	Vaccine clinical trial: NCT00616941 - Phase I Study of NY-ESO-1 OLP4 + Montanide
Current status	NED

#### *Research Advocacy*

2008 - 2013

##### Department of Defense (DoD) Ovarian Cancer Research Program (OCRP)

- Served on six panels as a Consumer Reviewer representing the ovarian cancer community's interest during the peer review of proposals submitted to the DOD-OCRP.

##### Society of Gynecologic Oncologists (SGO)

2010, 2011

- Attended 2010 and 2011 conferences with support from SHARE.
- Reported back lessons learned via written reports and a teleconference that was made available to the ovarian cancer community in general and SHARE Ovarian Cancer Hotline Volunteers in particular, the latter having access to an archived recording.

##### American Society of Clinical Oncology (ASCO)

2011, 2012

- Attended 2011 and 2012 conferences with support from SHARE and Research Advocacy Network respectively. In 2012 attended as a Focus on Research Scholar, a program of the Research Advocacy Network.
- Attended Society for Immunotherapy of Cancer (SITC) pre-ASCO conference "Primer on Tumor Immunology and Cancer Immunotherapy"
- Reported back lessons learned via written reports and participation in a teleconference.

##### Ovarian Cancer National Alliance (OCNA)

2008, 2011, 2013

- Attended the annual Ovarian Cancer National Alliance (OCNA) conferences and reported back lessons learned on a Support Connection sponsored toll-free teleconference that was open to the ovarian community at large and in written reports summarized in "Celebrate Life", the newsletter of the Oncology Support Program – HealthAlliance of the Hudson Valley.

##### Genentech

2011-2012

- Served as the Patient Advocate on Genentech's PRO/PRECISION Study Steering Committee

## ***Outreach and Awareness Education***

**SHARE – Self-help for Women with Breast and Ovarian Cancer** 2010 – present

- Appointed Hudson Valley Regional Coordinator expanding SHARE’s reach into this region and increasing awareness in the general public about ovarian cancer through media exposure, health fairs, and presentation to various groups

**Survivors Teaching Students, Saving Women’s Lives® (STS®) – a program of the Ovarian Cancer National Alliance (OCNA)** 2006 – present

- Present in both the New York City area and Albany to medical students, physician assistant students, nursing students and nursing practitioner students
- In 2012 appointed the New York/New Jersey Regional Coordinator overseeing the STS® program in this region – ensuring the volunteer pool is maintained, current programs comply with policies of the Alliance, and foster relations with additional schools to expand the program.

**C.A.S.T. (Cancer Awareness Survivor Team) – reaching the west side of the Mid-Hudson River Valley** 2009 – present

- Speak to local nursing students and nurse practitioner students (similar to OCNA’s Survivors Teaching Students Program® )
- Participate in area health fairs for college students and the general public in Ulster and Orange Counties in NY
- Participate in “Paint the Town Teal” Ovarian Cancer Awareness Campaign in Montgomery, NY

## ***Cancer Patient Support***

**Linda Young Ovarian Cancer Support Program – Kingston, NY** 2006 – present

- Active member of the support group
- Active member of the Program’s Advisory Committee since 2007
- Contribute to the Oncology Support Program’s newsletters

**Support Connection – Yorktown, NY** 2006 – present

- Facilitate the monthly Ovarian Cancer National Toll-Free Telephone Support Group
- Serve as a peer contact for women wanting to speak one on one with a survivor

**SHARE – Self-help for Women with Breast and Ovarian Cancer - NYC** 2006 – present

- Volunteer on the ovarian cancer hotline, receiving calls nationwide

## ***Political Advocacy***

- Participated in OCNA’s Advocacy Day in Washington, DC, meeting with state senators and congressmen/congresswomen. 2011, 2013
- Contact and lobby state and federally elected officials by email and phone as well as in person per OCNA’s action alerts and opportunities identified by SHARE On-going

**MARA B. GINSBERG, Esq.**

49 Darnley Greene  
Delmar, NY 12054  
Home: 518.439.8643  
Cell: 518.598.3317  
Work: 518.436.0751  
mginsberg@verizon.net  
mginsberg@hinmanstraub.com

**TO LIFE! (www.tolife.org)**

1998 – Present      *Founder & President*

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*To Life!* is the only nonprofit organization in the Capital Region of New York providing comprehensive education programs and support services relating to breast cancer and women's health free of charge and in non-clinical settings. The Founder & President provides vision and direction for the organization; represents *To Life!* in legislative matters and supervises the organization's fundraising efforts. The Founder & President has been recognized locally and nationally as both a community leader and as an expert resource for consumer driven health care improvement. The Founder & President is in regular contact with legislators, NYS agency executives at the Department of Health and the NYS Attorney General, statewide associations, health plans, pharmaceutical company executives, physicians and hospitals, media as well as consumer organizations working collectively and collaboratively to both improve healthcare and the delivery of healthcare to consumers. The Founder & President speaks frequently on issues relating to healthcare for local and statewide organizations. Some of the Founder & President's health policy affiliations:

- 2001 – 2014 **NYS Advisory Council for Breast Cancer Education and Research**
- 2003 – 2014 **United States Department of Defense** (*Consumer Grant Reviewer*)
- 2003 – 2011 **Advisory Review Panel for Pesticide Information Release**

**Hinman Straub P.C.**

Present      *Principal*

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As a partner in the Albany law firm of Hinman Straub, P.C., Ms. Ginsberg has a legal and legislative practice including areas relating to health care and Medicaid compliance (OMIG), procurement, technology and education.

**AmeriChoice of New York & UnitedHealthcare of New York**

*Vice President Government Affairs*

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Lead legislative and regulatory health policy for this Medicaid managed Care Company in New York State. The company, as part of United Health Group serves over 200,000 members in New York with Medicaid, Family Health Plus and Child Health Plus. The VP handled legal work for New York, New Jersey and Rhode Island for matters associated with managed care. These responsibilities included the drafting and filing of management service agreements and provider contracts, revising provider contracts across corporate segments to ensure contract language accurately represents the numerous lines of business. Responsibilities also included regulatory work particularly with the Department of Health on matters pertaining to UnitedHealth Group's commercial matters.

## **NYS Office of Cyber Security & Critical Infrastructure Coordination**

### *Counsel*

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This office was created from the NYS Office for Technology (OFT) in 2002. Counsel was the number three person in charge of this NYS Office and had multiple responsibilities including directing the Legislative program and negotiating all contracts. In the 2005 legislative session Counsel negotiated the New York Information Security Breach and Notification Act, which was signed into law on August 9, 2005. Additionally, Counsel directed all procurements and routinely negotiated contracts in excess of one million dollars. To forge collaboration and information sharing among and between states, Counsel created a national legal work group involving public and private attorneys for cyber security related issues and succeeded in having 50 states sign an identical agreement related to confidential information sharing. Counsel was a frequent public speaker, most recently on the Notification Act.

## **NYS Office for Technology**

### *Deputy Director and Counsel*

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Directed the Office for Technology legislative program, including drafting and negotiating bills; negotiated technology contracts; provided research, advice and counsel on policy and legal issues to OFT executive staff; drafted and revised agency and statewide policies; negotiated and resolved significant litigation; addressed agency ethics and Freedom of Information Law (FOIL) matters; addressed personnel matters before the U.S. Equal Employment Opportunities Commission, State Division of Human Rights and NYS Department of Civil Service; supervised staff of ten attorneys and additional support staff.

## **NYS Department of Law**

### *Assistant Attorney General, Bureau of Appeals & Opinions*

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Appellate attorney representing many State agencies, including the Education Department and the Department of Health. Many of these cases involved Article 78 appeals relating to physician licensing and teacher discipline and certification. Representation involved all aspects of appellate practice, including research, preparation of briefs, oral arguments and settlement negotiations.

## **Consulting**

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*Mediator* for employment discrimination claims for the **United States EEOC**; *Consultant* for the **New York State Education Department (NYSED)** and **New York State Review Officer** involving students with disabilities; *Hearing Officer* for moral character hearings for the **Bureau of Teacher Certification of NYSED**.

## **NYS Education Department**

### *Assistant Counsel*

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Reviewed, drafted and negotiated legislation relating to Education Law and negotiated state aid to school district formulas; Researched and wrote Commissioner's decisions for the Commissioner of Education. Decisions required knowledge of the New York State Education Law, regulations of the Commissioner, rules of the Board of Regents and Federal Laws relating to education as well as special education. Duties also included representing the Department in litigation, including Article 78 proceedings and administrative hearings; providing counsel to department personnel on application of the Education Law

and department regulations

### **College of St. Rose**

*Adjunct Professor, Department of Education Administration*

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Taught education and constitutional law to teachers and school administrators seeking administrative certification. Course dealt with practical application of legal principles, including the development of school district policies and procedures.

### **NYS School Boards Association**

*Attorney*

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Duties included acting as a mediator in a dispute resolution program conducted by NYSSBA for school boards throughout New York State, writing amicus briefs, providing legislative analysis and responding to daily legal inquiries from association members.

### **NYS Commission of Correction**

*Deputy Counsel*

*Assistant Counsel*

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Drafted, reviewed and negotiated Commission legislation; Represented commission employees at depositions and grand jury proceedings; drafted directives to county officials mandating compliance with Commission regulations and applicable laws; and prepared affidavits and pleadings for the judicial enforcement of Commission directives. Supervised professional employees having responsibility for the development of Commission rules and regulations.

### **Education**

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**Albany Law School of Union University** J.D. 1983

Admitted to the New York State Bar 1984

Admitted to the Federal District Court 1987

**Union College** B.S. cum laude 1980

### **Other Community Affiliations**

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#### **Ferre Institute**

2011- present *Board Member*

**United Jewish Federation-** board member

2003 – 2007; 2013- present *Board Member*

#### **Empire State Youth Orchestra**

2006 – 2009 *Board Member*

#### **New York State Bar Association**

2003 – 2007 *Appointed Member, Cyber Space Law Committee; Attorneys in Public Service*

#### **Capital District Women's Bar Association**

2001 – 2003 *Board Member*

### **Awards**

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- Circle of Humanity Award- Temple Israel



- Business and Professional *Woman of the Year*
- Hadassah Capital District- “*Women Inspiring Hope and Possibility*”
- Yoplait, Self Magazine and Susan Komen National Award- *25 Champions*
- American Institute for Public Service- finalist *Thomas Jefferson award*
- New York Governor Pataki *Women’s History Month honoree*
- Bethlehem Chamber of Commerce, *Citizen of the Year*
- Albany-Colonie Regional Chamber of Commerce, *100 Women of Excellence*
- Greater Capital Region Business and Professional Women's Organization, *Woman of the Year*
- New York State *Innovation in Breast Cancer Research and Education Award* for To Life!
- Susan G. Komen Foundation, *Local Hero Award*
- Albany Race for the Cure, Susan G. Komen Affiliate, *You Can Make a Difference Award*

## BIOGRAPHICAL SKETCH

Provide the following information for the Senior/key personnel and other significant contributors in the order listed on Form Page 2.  
Follow this format for each person. **DO NOT EXCEED FOUR PAGES.**

NAME <b>Michael S. Katz</b>	POSITION TITLE <b>Co-Chair, Cancer Research Advocate Committee, ECOG-ACRIN</b> <b>Vice President, International Myeloma Foundation</b>		
eRA COMMONS USER NAME (credential, e.g., agency login)			
EDUCATION/TRAINING <i>(Begin with baccalaureate or other initial professional education, such as nursing, include postdoctoral training and residency training if applicable.)</i>			
INSTITUTION AND LOCATION	DEGREE <i>(if applicable)</i>	MM/YY	FIELD OF STUDY
Columbia University School Of Engineering and Applied Science	BS	06/75	Electrical Engineering/ Computer Science
Columbia University Graduate School Of Business	MBA	01/77	Management Science & Finance

### A. Personal Statement

I am a twenty-two-year survivor of Multiple Myeloma and a five-year survivor of rectal cancer. In the past twenty years, I have worked as a Patient Advocate across a broad spectrum of Cancers, in Research, Education and Support.

Highlights include chairing the Patient Representatives Committee at ECOG, the NCI's Director's Consumer Liaison Group and the Association of Cancer Online Resources, and serving as Vice President of the International Myeloma Foundation. In these roles, I have been privileged to be able to actively contribute to improved outcomes for cancer patients, through in-person, on the phone and online educational programs and support, as well as adding the patient voice to the dialog on cancer research. I am thrilled to have been a part of the registration trials for two of the iMids currently being used for multiple myeloma and to have been the catalyst for the trial that replaced high dose dose dexamethasone with a safer, equally-effective lower dose alternative.

### B. Positions and Honors. List in chronological order previous positions, concluding with your present position. List any honors. Include present membership on any Federal Government public advisory committee.

1977 – 2011	Senior Vice President, Booz & Co., New York, New York Practice focused in media/entertainment, financial services and e-commerce
1993 – present	Vice President, International Myeloma Foundation, Los Angeles, California
1996 – present	List Owner, Association of Cancer Online Resources Myeloma and Amyloid Listservs, total membership over 2,000 subscribers, New York, New York
1997 – 2003	Chair (3 yrs.), Member (4 yrs.), NCI Directors Consumer Liaison Group, Bethesda Maryland
1998	Patient Representative, Biological Response Modifiers Advisory Committee Meeting, Food and Drug Administration, Rockville, Maryland
1998 – present	Co-Chair, Patient Representative Committee, Executive Committee Member, Eastern Cooperative Oncology Group(ECOG), Philadelphia, Pennsylvania (now ECOG-ACRIN)
1999 – 2008	Patient Advisory Board, National Coalition of Cancer Cooperative Groups, Philadelphia, Pennsylvania
1999 – 2001	Member, Communications Opportunity Leadership Team, National Cancer Institute, Bethesda, Maryland
2000 – 2002	Chairman, Association of Cancer Online Resources
2000 – 2001	Consumer Representative, Leukemia, Lymphoma, Myeloma Progress Review Group, National Cancer Institute Bethesda Maryland
2000 – present	Leader, Manhattan multiple myeloma support group, New York, NY
2000 – present	Leader, White Plains multiple myeloma support group, New York, NY
2001 – present	Patient Consultant, Food and Drug Administration, Rockville, Maryland

2003	Patient Representative, Oncologic Drugs Advisory Committee Meeting on Cancer Clinical Trial Endpoints, Food and Drug Administration, Rockville, Maryland
2005 – 2010	Operations Manager, Bank On A Cure, International Myeloma Foundation DNA data bank project
2007 – 2009	Steering Committee Member, New York State Cancer Control Consortium
2009 – present	Scientific Advisory Board, Observation Medical Outcomes Partnership, Foundation for the National Institutes of Health
2008 – 2010	American Society of Clinical Oncology, Cancer Research Committee
2009 – present	Chair, Patient Advisory Board, Coalition of Cancer Cooperative Groups
2010 – present	National Cancer Institute, Myeloma Steering Committee, Patient Advocates Committee
2010 – present	Hoosier Oncology, Patient Advocate
2010 – 2011	Co-Chair, Steering Committee, Executive Committee Member, Clinical Trials Transformation Initiative
2010 – present	National Cancer Institute, Myeloma Steering Committee, Patient Advocates Committee
2010 – present	Department of Defense, Patient Advocate, Congressionally-Directed Medical Research Program
2013 – present	Mayo Clinic, Myeloma SPORE, Patient Advocate

**C. Selected peer-reviewed publications (in chronological order).** Do not include publications submitted or in preparation. For publicly available citations, URLs or PMC submission identification numbers may accompany the full reference; copies of publicly available publications are not accepted as appendix material.

1. Durie, B, Van Ness, B., Ramos, C. Stephens, O. Haznadar, M., Hoering, A., Haessler, A. , Katz, M., Mundy, G., Kyle, R., Morgan, G., Crowley, J., Barlogie, B., Shaughnessy, J., Genetic Polymorphisms Identify the Likelihood of Bone Disease in Myeloma: Correlations with Myeloma Cell DKK1 Expression and High Risk Gene Signatures. Session Type: Oral Session [800], American Society Of Hematology, 2007 Annual Meeting
2. Durie B.G., Katz M., Crowley J. Osteonecrosis Of The Jaw And Bisphosphonates. *N Engl J Med*, 353(1):99-102.
3. Katz M., Bergsagel K.I., Bergsagel D. (2003). Patient and Caregiver. In J.S. Malpass, D.E. Bersagel, R.A. Kyle, & K.C. Anderson, (Eds.), Myeloma. (3<sup>rd</sup> Edition). Oxford: Elsevier.
4. Stephenson J, Katz M, Tcherednichenko T, Wu Q, Lynn H, Ward D, Ellis P. Cancer Care: What are the Priorities? *Lancet Oncol*, 2(10):636-641.
5. Katz, M, Smith, ML, Sparano, JA, Giantonio, BJ, Comis, RL, “Are We Asking Too Much? The Challenges of Mandatory Research Biopsies within Clinical Trials”, ASCO 2010 Educational Session
6. Katz MS, Smith ML., Central institutional review board-facilitated review metrics omit critical components, *J Clin Oncol*. 2010 Feb 20;28(6):e105
7. Vesole DH, Oken MM, Heckler C, Greipp PR, Katz MS, Jacobus S, Morrow GR; University of Rochester Cancer Center and the Eastern Cooperative Oncology Group, Oral antibiotic prophylaxis of early infection in multiple myeloma: a URCC/ECOG randomized phase III study, *Leukemia*. 2012 Dec;26(12):2517-20
8. Durie BG, Van Ness B, Ramos C, Stephens O, Haznadar M, Hoering A, Haessler J, Katz MS, Mundy GR, Kyle RA, Morgan GJ, Crowley J, Barlogie B, Shaughnessy J Jr., Genetic polymorphisms of EPHX1, Gsk3beta, TNFSF8 and myeloma cell DKK-1 expression linked to bone disease in myeloma, *Leukemia*. 2009 Oct;23(10):1913-9.

# CPRIT Peer Review and Grants Management

## Services Provided by SRA International, Inc.

Steven Goldberg, Ph.D., M.B.A., PMP  
Director, Peer Review and Science Management

Rajan Munshi, Ph.D., M.S.I.S.  
Project Director, CPRIT Grants Management

19 February 2014



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- Meeting facilitation
- Travel and logistics support
- Technical writing/editing

## Representative Clients

- Cancer Prevention and Research Institute of Texas (CPRIT)
- DOD Congressionally Directed Medical Research Programs (CDMRP)
- ED Institute of Education Sciences (IES)
- HHS National Institutes of Health (NIH)



# Supporting CPRIT's Mission

- **Rigorous process**
  - Fair
  - Thorough
  - Transparent
- **Review integrity**
  - Free of bias or conflict of interest
  - Independent expert evaluations
- **Sound decision-making**
  - Based on best available expertise
  - Clear documentation

***Outcomes aligned with CPRIT's purpose***



# CPRIT's Grants Lifecycle

## Application Phase

- Eligible
- Responsive
- High quality

## Evaluation Phase

- Fair
- Thorough
- Transparent

## Post Award Phase

- Monitoring
- Oversight
- Measurement





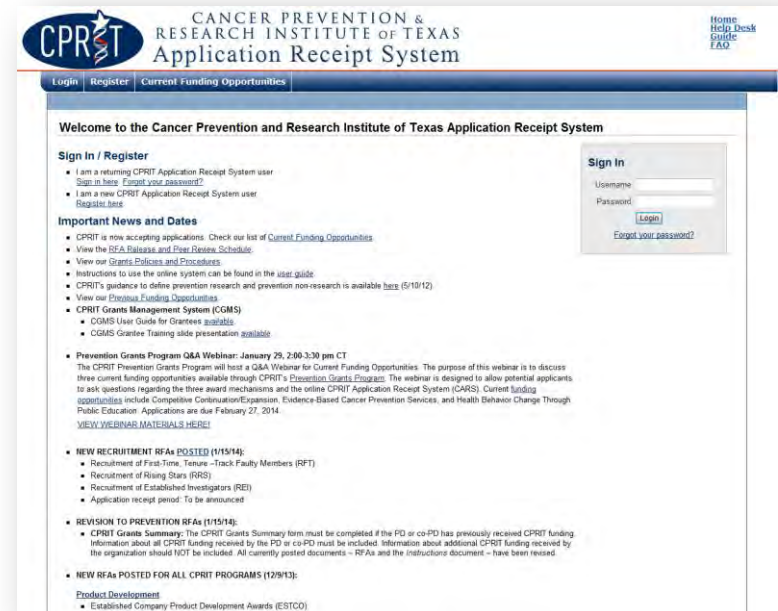
# Grants Lifecycle: Application

- Request for Application (RFA)
  - Informs research community of intent
  - Articulates (measurable) program goals
  - Defines eligibility
  - Defines required documents
  - Establishes review criteria
  - Defines grant (amount, duration)
- Help Desk
- Application Receipt
  - Applications are responsive and qualified
  - Diversity/breadth of subject matter and approaches



# CPRIT Application Receipt System (CARS)

- Official portal for submission of applications to CPRIT
- Customized for every CPRIT program/award mechanism
- Central source of information to applicant community
- Enforces consistent application process
- >3,000 applications submitted



# Grants Lifecycle: Evaluation

- **Expert Recruitment**
  - Subject matter experts (SMEs)
  - Free of bias/conflict of interest (COI)
- **Peer Review**
  - Criteria based
  - Assesses technical merit
  - Informs funding decisions
- **Program Review**
  - Ensures alignment with objectives
  - Balances portfolio/addresses gaps
  - Allocates funds



# Grants Lifecycle: Post Award

- **Award Negotiation**
- **Progress Monitoring**
- **Grant Close-Out**



# CPRIT Grants Management System (CGMS)

- Official repository of grant data
- Structured management of grants
- Workflows support CPRIT process
- Assists grantees with reporting:
  - Expenditures
  - Technical progress
- Compliance verification
- Management reporting

The screenshot displays the CPRIT Grants Management System (CGMS) web application. At the top, the header includes the CPRIT logo and the text "CANCER PREVENTION & RESEARCH INSTITUTE OF TEXAS Grants Management System". Below the header is a navigation bar with tabs: "My Profile", "My Applications", "My Grants", "Current Funding Opportunities", and "Feedback". A secondary navigation bar contains links: "Home", "Grants", "Grants Admin", "Correspondence & Notifications", "Reports", "Users", and "Admin". The main content area is titled "Search for Grants" and features a "Filter Options" section. This section includes fields for "Program", "Fiscal Year", "Award Mechanism", and "Cycle". Below these are fields for "PI/PO/CR Last Name" and "Grant ID". A "Grant" section contains fields for "Project Title", "Organization", and "Grant Status". The "Grant Status" dropdown menu is open, showing options: "Initiating", "In Negotiation", "Active", "In Early Termination", "Early Termination", "In Close Out", and "Closed". At the bottom of the filter section are date pickers for "Contract Start Date" and "Contract End Date", each with "From" and "To" fields. A "Reset" button and a "Search Grants" button are located at the bottom of the filter section.



# SRA Is Proud To Partner With CPRIT

- Supporting CPRIT since 2009
- Mission-focused support of
  - Scientific management
  - Administration and logistics
  - Information technology
- Leveraging deep knowledge of best practices in peer review
- Scalable and flexible solutions
- Innovations to drive efficiency and quality
- Rigorous, robust, and transparent processes



CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS



**No material available at this time.**



CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS

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MEMORANDUM

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**TO:** AUDIT SUBCOMMITTEE MEMBERS  
**FROM:** HEIDI MCCONNELL, CHIEF OPERATING OFFICER  
**SUBJECT:** AMENDMENT TO GRANT THORNTON FY 2014 INTERNAL AUDIT SERVICES CONTRACT  
**DATE:** FEBRUARY 12, 2014

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**Additional Audit on SRA-Managed Information Technology Systems**

The 2013 State Auditor's management report and the 2013 *Information Technology Internal Audit Report* made recommendations for audits of the security, availability, processing integrity, confidentiality and privacy controls used on the SRA-managed, proprietary systems. The systems include the CPRIT Application Receipt System (CARS), which incorporates the CPRIT Grants Management System (CGMS), and the Peer Review Management Information System (P<sup>2</sup>RMIS). There are regular examinations of the controls at the data center where the SRA systems are housed, but an evaluation of the controls that directly affect the CPRIT-specific systems have not been performed.

The following are the recommendations from the audit reports:

- State Auditor's Management Report No. 13-018, Chapter 5: Audit of the controls used in SRA's proprietary Peer Review Management Information System (P<sup>2</sup>RMIS) that records peer review conflict of interest and evaluation comments and scores on grant proposals to CPRIT to ensure the reliability of data entered into and processed by P<sup>2</sup>RMIS.
- State Auditor's Management Report No. 13-018, Chapter 5: Audit of the controls used in SRA's proprietary CPRIT Application Receipt System (CARS) that allows grant applicants to submit applications electronically and acts as a reporting system to facilitate the receipt and distribution of grant performance and expenditure reports as well as other reports to comply with grant contract terms to ensure the reliability of data entered into and processed by CARS [including the CGMS component].
- Internal Audit Report #2013-01, Additional Recommendations: Obtain Service Organization Controls (SOC) 2 Report on controls relevant to security, availability, processing integrity, confidentiality and privacy of the CPRIT Grants Management System (CGMS) managed by SRA International.



After further discussion with our Grant Thornton auditors about the scope of the audit, their guidance based on experience with other organizations is to focus on two principles the first year and add principles in future years. Their recommendation is that CPRIT focus on security, which is usually a baseline evaluation, and processing integrity this year to address the issues raised in the State Auditor's management report.

The cost of an audit focused on these two principles is approximately \$45,000. With the addition of this audit to the scope of work, the total value of the contract with Grant Thornton would be \$245,000.



## CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS

## MEMORANDUM

**TO:** OVERSIGHT COMMITTEE MEMBERS  
**FROM:** ANGELOS ANGELOU, CHAIR, AUDIT SUBCOMMITTEE  
**SUBJECT:** AUDIT SUBCOMMITTEE REPORT  
**DATE:** FEBRUARY 17, 2014

**Meeting Discussion and Actions**

At its meeting on February 13, 2014, the subcommittee received updates from CPRIT staff on the Internal Auditor job posting, status of legislative oversight approvals of the internal audit services contract with Grant Thornton LLP, and the management dashboard project.

- The agency has made some changes to the Internal Auditor job description, including revising the title to Manager of Internal Audit. The position is currently open for applications through February 28, 2014.
- The State Auditor's Office has provided audit delegation for CPRIT's internal audit services contract with Grant Thornton, but the agency is still awaiting approval of the contract from the Legislative Budget Board to proceed with its execution and implementation of the 2014 internal audit plan.
- The staff drafted management dashboard is organized into three areas that focus on accountability, mission, and transparency and incorporates several metrics in each area to provide some information about agency workload and grant performance.

Staff discussed the need to procure services to develop a plan for CPRIT's Compliance Program. The plan would provide strategic guidance and direction to define the optimal structure and level of staffing as well as procurement of services to fulfill the necessary capabilities in: ongoing compliance risk assessment, compliance monitoring, anonymous compliance reporting (hotline service), investigation and follow-up, enforcement, internal auditing, and education and training of CPRIT's governing body, staff, grant recipients, and contractors. Staff anticipates the cost of developing the plan will be less than \$100,000 per year, so it does not require approval of the subcommittee or the Oversight Committee. However, the subcommittee generally supported the idea and concept of the approach presented by staff. With the guidance from the plan, staff will have a basis to develop future procurements for necessary third-party vendor services and bring those forward to the subcommittee for discussion and approval as needed.

There was also discussion about the need for an audit of the SRA-managed, proprietary information technology systems used by CPRIT to process and review grant applications and manage grant

awards to address the recommendations outlined in the State Auditor's management report for audits on the controls of these systems and a similar recommendation in the FY 2013 *Information Technology Internal Audit Report* (Report #2013-01). There are five principles that can be evaluated in information technology systems according to the American Institute of Certified Public Accountants guidelines: security, availability, processing integrity, confidentiality, and privacy controls. CPRIT staff, in consultation with the internal auditor Grant Thornton, recommends that the audit this year focus on two principles, security and processing integrity, following the practice that other organizations use and address other principles in future years. The audit of these two principles covers significant scope and will cost approximately \$45,000 to complete. An audit of all five principles would cost a minimum of \$80,000, but would more likely be on the order of \$100,000 to complete.

### **Subcommittee Recommendation**

The subcommittee recommends that the Oversight Committee approve an amendment to the Grant Thornton contract for internal audit services to add an audit of SRA-managed, proprietary information technology systems used by CPRIT to process and review grant applications and manage grant awards. The additional audit is estimated to cost up to \$45,000, bringing the total value of the contract with Grant Thornton to \$245,000. Should the Oversight Committee approve the contract amendment, the agency must forward it to the Legislative Budget Board for approval as required by the General Appropriations Act because the contract exceeds \$100,000. CPRIT will also notify the State Auditor's Office of the contract amendment to comply with the audit delegation authorization received from that office.



**CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS**

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**MEMORANDUM**

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**TO:** OVERSIGHT COMMITTEE MEMBERS  
**FROM:** DAVID A. REISMAN, CHIEF COMPLIANCE OFFICER  
**SUBJECT:** COMPLIANCE OFFICER REPORT  
**DATE:** FEBRUARY 1, 2014

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The Chief Compliance Officer is responsible for creating, supporting, and promoting an effective Ethics and Compliance Program and assuring the CPRIT Oversight Committee that controls are in place to prevent, detect and mitigate compliance risk. One of CPRIT's proposed administrative rules, Rule 701.7, provides in part that, "The Chief Compliance Officer is responsible and will be held accountable for apprising the Oversight Committee and the Chief Executive Officer of the institutional compliance functions and activities." The required reporting includes quarterly updates to the Oversight Committee on CPRIT's compliance with applicable laws, rules and agency policies (701.7(c)(2)(A)). In addition, the compliance officer must inquire into and monitor the timely submission status of required grant recipient reports and notify the Oversight Committee and General Counsel of a grant recipient's failure to meaningfully comply with reporting deadlines.

**Monitoring Submission Status of Required Grant Recipient Reports:**

As of February 11, 2014, the date the report was run, information regarding delinquent grant recipient reports is as follows:

- 20 active grant projects, at 10 separate entities, have not filed required quarterly financial status (FSR) reports by the deadline. At the last Oversight Committee meeting, on January 24, 2014, I reported that 52 grant projects had not filed required FSRs by the deadline. An FSR is due to CPRIT within 90 days following the close of the fiscal quarter. Of the 20 delinquent reports, 0 are less than 30 days overdue. 17 are more than 30 days but less than 90 days overdue. 3 FSRs are currently 90+ days overdue. For purposes of this report, I have excluded grant projects where contract execution was affected by the moratorium on new CPRIT awards.
- 7 active grant projects have not filed required progress reports by the deadline. All grant projects must file annual progress reports; prevention projects are also required to file quarterly progress reports. Annual progress reports must be filed with CPRIT within 60 days following the anniversary of the contract effective date. Of the 6 delinquent progress reports, 5 are less than 30 days overdue and 1 is currently 90+ days overdue. For purposes of this report, I have excluded grant projects where contract execution was affected by the moratorium on new CPRIT awards.

CPRIT staff will follow up with the grant projects that have delinquent reports. Also, it should be noted that CPRIT's recently adopted administrative rules provide new options to address delinquent reports. For example, rule 703.21(b)(2) provides, "...The Grant Recipient waives the right to reimbursement of project costs incurred during the reporting period if the financial status report for that quarter is not submitted to the Institute within 30 days of the due date." The Chief Executive Officer may approve an extension of the submission deadline if, prior to the FSR due date, the grant recipient submits a written explanation for the grant recipient's inability to complete a timely submission of the FSR." (emphasis added). However, pursuant to the Oversight Committee's direction at the January 24, 2014 Oversight Committee meeting, these options will not be implemented until after the staff has an opportunity to train grant recipients on the recently adopted rules.

CPRIT is still in the process of hiring additional staff authorized by the legislature, such as grant monitors. The addition of new grant monitoring staff, together with the automatic notification features in CGMS, as well as the addition of necessary risk management, audit and reporting capabilities and tools, should work together so that CPRIT can ensure that grant recipients are achieving full compliance with applicable rules, requirements and policies.