



ANNUAL REPORT

2012



CANCER PREVENTION & RESEARCH
INSTITUTE OF TEXAS



CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS OVERSIGHT COMMITTEE • FISCAL YEAR 2012

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LETTER FROM THE INTERIM EXECUTIVE DIRECTOR

The Honorable Rick Perry, *Governor*

The Honorable David Dewhurst, *Lieutenant Governor*

The Honorable Joe Straus, *Speaker of the House of Representatives*

Pursuant to V.T.C.A., Health and Safety Code Sec. 102.052 please accept this annual report from the Cancer Prevention & Research Institute of Texas (CPRIT). The following pages summarize how CPRIT is fulfilling its mission of reducing the burden of cancer in Texas.

Cancer is the leading cause of death among Texans younger than 85 years of age. On average, more than 100 Texans die from cancer every day. The tragic emotional and physical toll of cancer is incalculable, but in purely economic terms, Texas cancer deaths translate into a daily cost to the state of about **\$77 million** in medical expenses and lost productivity.

But Texas is rising to the challenge. Through August 2012, CPRIT has announced **423** awards for research, prevention and product development grants totaling **\$749,114,873**. Together with matching funds obligated by grant recipients, more than **\$902 million** has been invested in Texas' extraordinary commitment to the war against cancer.

Recipients of CPRIT awards include Texas academic institutions, non-profit organizations, and private companies. These awardees are advancing the health of Texans, expanding research in the state, building Texas' life-science infrastructure, and benefiting the Texas economy. In addition, CPRIT recruitment grants have helped bring research superiority to Texas through **26** outstanding researchers who, over the course of their careers, should attract significant follow-on funding to the state.

CPRIT-funded projects and programs reach Texans in all 254 counties. In addition to providing education and training to **620,000** people, CPRIT has funded clinical services for more than **230,000** Texans, including **38,000** who have never before been screened for cancer.

State law specifies several elements for annual reports. Some elements, most notably an assessment of the relationship between CPRIT's grants and the overall strategy of its research program and a statement of its strategic research and financial plans, are not provided in this report. The 83rd Legislature made numerous changes that will strengthen CPRIT's governance and operations and allow a more efficient, effective and transparent focus on combating cancer. These changes and enhancements include a requirement that the CPRIT Oversight Committee establish annual priorities for the research, prevention and product development grant programs. This enhancement, as well as others, will make it possible for future CPRIT annual reports to provide additional information to evaluate our performance and progress.

On behalf of the CPRIT Oversight Committee and the agency's staff, I thank you for this opportunity to highlight the contributions CPRIT has made in 2012. We look forward to the future and new opportunities to fulfill our mission to improve the health and lives of our fellow Texans.

Sincerely,

Wayne R. Roberts
Interim Executive Director

IMPACT OF CANCER

Age-Adjusted Invasive Cancer Incidence Rates in Texas

All Sites, 2005-2009

By County

Age-Adjusted to the 2000 U.S. Standard Population

Texas Rate: 451.0

Rate per 100,000

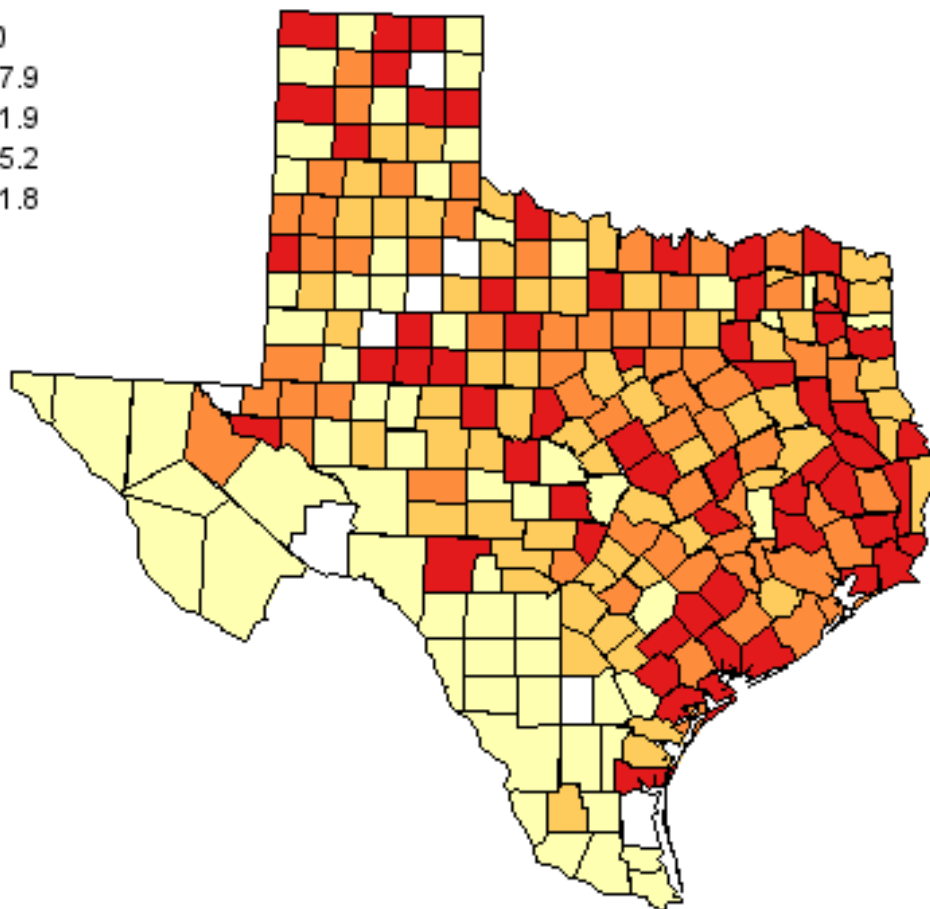
269.0 - 417.9

418.7 - 451.9

452.2 - 485.2

485.8 - 601.8

Risk Population
less than 1000



Source: Texas Cancer Registry, Cancer Incidence File, JANUARY 2012.

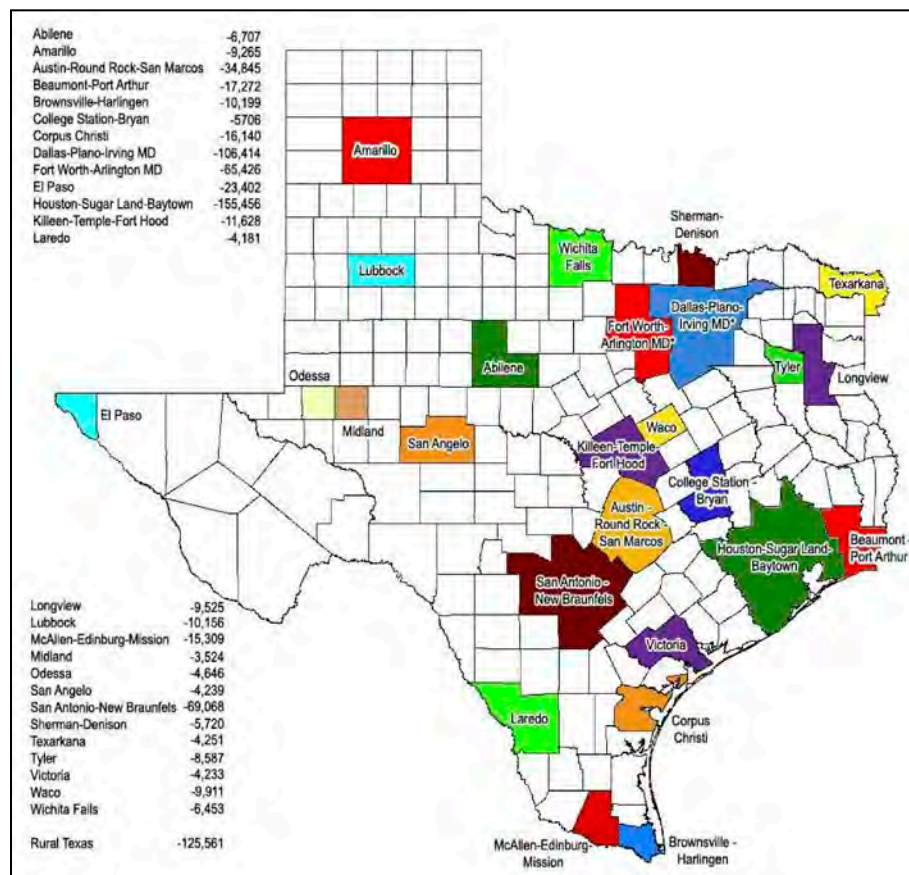
ECONOMIC IMPACT OF CANCER

On behalf of CPRIT, The Perryman Group studied the economic burden of cancer in Texas as well as the economic benefits of the agency's investments in the war against cancer. Some of the key findings:

- Cancer costs the Texas economy:
 - **\$146.5 billion** in reduced annual spending;
 - **\$72.4 billion** in output losses annually; and
 - **747,825 lost jobs** from cancer treatment, morbidity and mortality, and the associated spillover effects.
- Estimated returns on CPRIT investments in 2012 (totaling \$342.8 million in awards) include:
 - **\$2.9 billion** in economic activity (total expenditures) in 2012
 - **33,431 jobs** created through direct and indirect economic activity
 - **\$159.7 million** in state tax receipts and **\$73.5 million** in local government tax receipts

CPRIT's efforts not only decrease the costs of cancer (both human and economic) but also, by establishing Texas as a center for cancer research, enhance the economic development of the state.

Economic Cost of Cancer to Texas Metropolitan Areas: Estimated Job Losses Stemming from the Economic Cost of Treatment, Morbidity, and Mortality Associated with Cancer as of 2012



Source: The Perryman Group

TEXAS ADDRESSING CANCER: AWARDS THROUGH AUGUST 31, 2012

| Investments in Cancer Prevention | |
|---|--------------|
| Angelo State University | \$ 1,120,825 |
| Asian American Health Coalition of Greater Houston (dba Hope Clinic) | \$ 1,450,887 |
| Asian Breast Health Outreach Project at Methodist Richardson Medical Center | \$ 535,540 |
| Baylor College of Dentistry-TAMU Health Science Center | \$ 203,244 |
| Baylor College of Medicine | \$ 5,066,713 |
| Cancer and Chronic Disease Consortium | \$ 2,177,340 |
| Cancer Foundation for Life | \$ 100,000 |
| Cancer Services Network | \$ 99,581 |
| Centro San Vicente | \$ 1,937,461 |
| City of Laredo Health Department | \$ 2,497,500 |
| Daughters of Charity Health Services of Austin (dba SETON Healthcare Network) | \$ 128,640 |
| Texas Department of State Health Services | \$ 335,271 |
| Funding Solutions | \$ 157,494 |
| Healthy Tarrant County Collaboration | \$ 212,535 |
| LIVESTRONG | \$ 600,000 |
| Light and Salt Association | \$ 329,933 |
| LRGV Community Health Management Corporation, Inc. (dba El Milagro Clinic) | \$ 149,100 |
| Mercy Ministries of Laredo | \$ 608,579 |
| Methodist Dallas Medical Center | \$ 599,574 |
| MHMR of Tarrant County | \$ 2,397,784 |
| Migrant Clinicians Network | \$ 473,405 |
| National Center for Farmworker Health, Inc. | \$ 551,221 |

| Investments in Cancer Prevention | |
|---|--------------|
| SETON Family of Hospitals | \$ 562,004 |
| Shannon Business Services | \$ 255,198 |
| South Texas Rural Health Services, Inc. | \$ 149,971 |
| Texas A&M University | \$ 839,227 |
| Texas A&M University System Health Science Center | \$ 3,830,498 |
| Texas A&M University System HSC Research Foundation | \$ 339,932 |
| Texas Agrilife Extension Service | \$ 3,410,830 |
| Texas Department of State Health Services | \$ 2,936,382 |
| Texas Medical Association | \$ 967,425 |
| Texas Nurses Foundation | \$ 2,107,901 |
| Texas Tech University | \$ 592,546 |
| Texas Tech University Health Sciences Center | \$ 4,831,994 |
| The Bridge Breast Network | \$ 977,603 |
| The Cooper Institute | \$ 591,384 |
| The Rose | \$ 3,845,471 |
| The University of North Texas Health Science Center at Fort Worth | \$ 4,504,995 |
| The University of Texas at Austin | \$ 266,920 |
| The University of Texas Health Science Center at Houston | \$ 4,983,062 |
| The University of Texas Health Science Center at San Antonio | \$ 6,741,439 |
| The University of Texas MD Anderson Cancer Center | \$ 4,151,419 |
| The University of Texas Medical Branch at Galveston | \$ 1,239,025 |
| The University of Texas Southwestern Medical Center at Dallas | \$ 9,549,709 |
| University Health System | \$ 6,218,267 |
| University of Houston | \$ 272,753 |

AWARDS THROUGH AUGUST 31, 2012 CONTINUED

| Investments in Company and Academic Research | |
|---|---------------|
| Apollo Endosurgery | \$ 5,001,063 |
| Asuragen, Inc. | \$ 6,837,265 |
| Baylor College of Medicine | \$ 80,385,033 |
| Baylor Research Institute (MIRA Sub Award) | \$ 2,108,180 |
| Baylor University | \$ 200,000 |
| Baylor University Medical Center at Dallas | \$ 2,500,000 |
| Bellicum Pharmaceuticals, Inc. | \$ 5,680,310 |
| Caliber Biotherapeutics | \$ 12,808,151 |
| Cell Medica | \$ 15,571,303 |
| Clinical Trials Network (CTNeT) ¹ | \$ 25,213,675 |
| Gradalis, Inc. (MIRA Sub Award) | \$ 748,905 |
| Ingeneron, Inc. | \$ 198,111 |
| Kalon Biotherapeutics, LLC ² | \$ 7,901,420 |
| Mirna Therapeutics, Inc. | \$ 10,297,454 |
| Molecular Templates, Inc. | \$ 10,600,000 |
| Peloton Therapeutics, Inc. ³ | \$ 11,044,931 |
| Pulmotect, Inc. | \$ 7,126,398 |
| Rice University | \$ 23,472,111 |
| Rules-Based Medicine | \$ 3,024,432 |
| Scott & White Healthcare | \$ 3,584,521 |
| Texas A&M University | \$ 1,577,777 |
| Texas A&M University System Health Science Center | \$ 3,201,312 |

| Investments in Company and Academic Research | |
|---|----------------|
| Texas A&M University System Health Science Center - Institute of Biosciences and Technology | \$ 12,614,927 |
| Texas Life Science Foundation | \$ 7,745 |
| Texas Tech University | \$ 2,899,790 |
| Texas Tech University Health Sciences Center | \$ 12,153,955 |
| The Methodist Hospital Research Institute | \$ 25,283,225 |
| The University of North Texas Health Science Center at Fort Worth | \$ 179,834 |
| The University of Texas at Arlington | \$ 2,285,375 |
| The University of Texas at Austin | \$ 33,083,678 |
| The University of Texas at Dallas | \$ 5,909,898 |
| The University of Texas at El Paso (MIRA Sub Award) | \$ 999,992 |
| The University of Texas at San Antonio | \$ 898,026 |
| The University of Texas Health Science Center at Houston | \$ 7,827,104 |
| The University of Texas Health Science Center at San Antonio | \$ 17,561,348 |
| The University of Texas MD Anderson Cancer Center | \$ 124,537,092 |
| The University of Texas Medical Branch at Galveston | \$ 6,512,077 |
| The University of Texas Southwestern Medical Center at Dallas | \$ 164,030,876 |
| The University of Texas System | \$ 5,000,000 |
| University of Houston | \$ 6,597,188 |
| University of North Texas | \$ 200,000 |
| Visualase, Inc. | \$ 2,151,776 |

¹ Project terminated January, 2013; final amount expended was \$8.7 million.

² In contract negotiation

³ Project suspended; grant funds expended to date is \$3.2 million.

RESEARCH

The goal of CPRIT's research investments is to transform new and promising ideas into positive outcomes for cancer patients. The value that CPRIT adds to the fight against cancer goes well beyond the substantial dollars invested by the people of Texas. CPRIT's research programs are designed to challenge the brightest and most innovative scientists to pursue the most worthy research objectives that will help reduce the burden of cancer in Texas and throughout the world.

Research proposals submitted to CPRIT are reviewed by peer review committees comprised of some of the most outstanding cancer researchers and clinicians in the nation. These individuals bring a wealth of knowledge and experience to ensure that the funds invested are awarded to programs and projects that can make a real difference. Peer review committees are charged to follow their own independent and objective judgment, free from political or geographic influences or conflicts of interest, in providing a thorough evaluation of programs.

CPRIT's goal is to support a collection of the most creative ideas from the finest cancer researchers in Texas, and as such, there are no pre-determined quotas for the types of cancer research to be funded. CPRIT aims to support the most meritorious and promising programs and projects across the research spectrum — from basic science to translational research and clinical applications, and from short-term individual projects to complex, multi-year research programs.

CPRIT'S CANCER RESEARCH AWARDS

CPRIT Scholars in Cancer Research awards recruit superior cancer researchers at various stages of their careers to Texas academic institutions to establish laboratories or clinical research programs that add research talent to the state.

High Impact-High Risk Research Awards provide seed money for investigators to try out new ideas at the cutting edge of cancer research.

Individual Investigator Research Awards support innovative research projects directed by one scientist that can significantly advance knowledge of the causes, prevention, diagnosis, and/or treatment of cancer.

Multi-Investigator Research Awards fund large-scale cross-disciplinary research projects that promise to deliver significant advances through innovation and collaboration.

Research Training Awards support programs designed to educate the next generation of cancer researchers. Individuals from underrepresented racial and ethnic groups or disadvantaged backgrounds, as well as persons with disabilities, are encouraged to participate in these training programs.

Shared Instrumentation Awards underwrite the cost of major research equipment at Texas institutions to support the work of multiple investigators and the goals of scientifically significant projects.

Core Facilities Support Awards support centralized laboratories performing widely used technologies that serve the needs of multiple researchers.

Early Translational Research Awards support projects that "bridge the gap" between the research laboratory and potential clinical applications, such as proof-of-principle research to guide the development of therapeutics, devices, or diagnostic assays.

Through August 2012, the CPRIT research program has announced awards totaling \$565 million for 305 programs and projects across 26 academic institutions. Funding has allowed for not only academic advancement in research, but has had an economic impact by creating hundreds of new jobs in the state.

RESEARCH HIGHLIGHTS — GROUNDBREAKING DISCOVERIES TODAY

1. Developing new ways to predict and block metastasis



Sean Morrison, Ph.D.

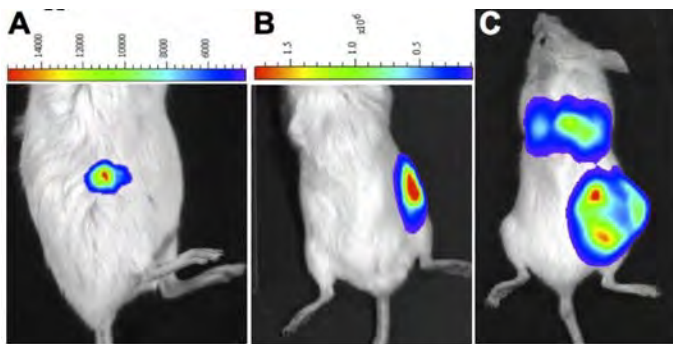
Children's Medical Center Research Institute at UT Southwestern

Human melanoma — the most severe form of skin cancer — is curable through surgery to remove the tumor unless the cancer has spread elsewhere in the body (what's known as metastasis). Knowing how to predict and prevent metastasis makes a substantial difference in the effectiveness of melanoma treatment and patient outcomes. However, it has proven difficult to reproduce in the laboratory the ways in which melanoma cells actually form and spread in human patients. CPRIT Scholar Dr. Sean Morrison's laboratory at UT Southwestern has developed a specialized mouse model that promises to overcome this challenge.

The Morrison laboratory's "xenograft" model allows small numbers of melanoma cells (even single cells) obtained directly from patients to be transplanted into specially bred laboratory mice (known as NSG mice), forming human melanomas. The mice are then used to study the biology of human melanomas, to identify new biomarkers, and to test new therapies.

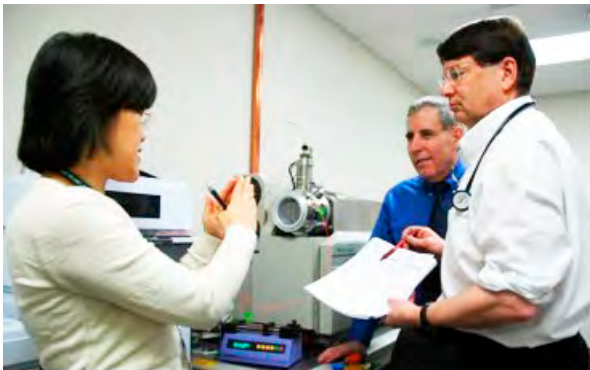
The Morrison laboratory recently showed that Stage III melanoma cells did, in fact, metastasize in these mice in the same ways they did in the patients from which the cells came — with some spreading rapidly and widely ("efficient") and others more slowly ("inefficient"). These differences correlated with the frequency of circulating melanoma cells in the blood of the mice, suggesting that the rate of entry into the blood is one factor that governs how quickly and widely melanomas spread.

The Morrison laboratory is currently testing new potential therapies to block the growth and spread of melanoma, as well as exploring genetic approaches to compare “efficient” and “inefficient” melanomas and identify the molecular mechanisms that regulate metastasis. New potential small molecule therapies are being tested now for their ability to cure or to prevent the spread of human melanoma in mice. The therapies that prove most promising in mice will ultimately be tested in clinical trials in patients.



The Morrison lab has used these mice to study the mechanisms that regulate metastasis.

2. A novel target for cancer chemotherapy: the building blocks of cell membranes



C. Patrick Reynolds, M.D., Ph.D., Barry Maurer, M.D., Ph.D., and Min Kang, PharmD, Texas Tech University Health Sciences Center (TTUHSC) School of Medicine Cancer Center

Many new cancer chemotherapy drugs focus on precise targets, often related to changes (mutations) in cancer cell DNA that produce molecules that can be attacked by new drugs. This new generation of targeted drugs is less toxic to a patient’s healthy cells than traditional chemotherapy, but cancer cells can rapidly overcome these narrowly targeted drugs by additional mutations. A team of CPRIT-funded investigators at the Texas Tech University Health Sciences Center (TTUHSC) School of Medicine Cancer Center developed a novel way of targeting cancer cells that could overcome some of the limitations of both traditional chemotherapy and the new targeted drugs.

The starting point for the work of Drs. Reynolds, Maurer and Kang is a well-tolerated drug, derived from vitamin A, called fenretinide. This drug tricks cancer cells — but not normal cells — into overproducing ceramides, one of the building blocks of cell membranes. This is highly toxic to many cancer cells. This approach was initially developed to treat neuroblastoma, the most common type of cancer in infants, but was soon found to be effective against certain adult lymphomas (cancer of lymph nodes). The TTUHSC investigators were able to move their research forward much faster by carrying out simultaneous clinical trials in both pediatric and adult patients.

Fenretinide has achieved complete remissions in children with neuroblastoma and adults with T-cell lymphoma, but many cancers do not respond to fenretinide alone. Therefore, Drs. Reynolds, Maurer, and Kang are developing next-generation drugs to combine with fenretinide and amplify its effects. Laboratory work with one such drug, safingol, has been promising, with a phase I clinical trial at UT Southwestern being ramped up for use on patients. A CPRIT grant to Dr. Reynolds provides partial support for this clinical trial as well as for other ongoing fenretinide trials in both adults and children.

Drs. Maurer and Kang have identified another drug (called PPMP) that also enhances the effect of fenretinide and safingol. With support from a CPRIT Early Translational Grant, critical manufacturing, formulation, and toxicity studies are under way to bring PPMP to early-phase clinical trials.

3. Exploring the Origins of Liver Cancer in Children

Gail Tomlinson, MD, PhD, lead investigator

Greehey Children's Cancer Research Institute, UT Health Science Center at San Antonio

In 2012, about 1,300 children were diagnosed with cancer in Texas, and about 200 children died of cancer. Pediatric cancer is different from adult cancer and requires different treatment strategies. Whereas the genetic damage that leads to adult cancers can accumulate over decades of aging and exposure to carcinogens, cancer in young children is thought to stem from a handful of acquired genetic alterations at specific stages of development.

As a result, pediatric cancer is rare, affecting only a few thousand patients in the U.S. each year. To ensure that pediatric cancers are not left behind in the ongoing revolution in targeted therapy, it is critical that researchers from different institutions work together to share their knowledge and focus their energies and expertise. CPRIT's multi-investigator research awards (MIRAs) are designed precisely to support this crucial collaboration; since 2009, CPRIT has awarded three MIRAs that focus on specific types of pediatric cancer.

One of these is hepatoblastoma, a rare type of liver cancer that almost always occurs in children under 5; the average age of onset is 20 months. This type of cancer is occasionally associated with both very low birth weight (as in premature birth) and unusually high birth weight. It is thought that genes involved in early growth and development of the prenatal liver contribute to the disease.

Hepatoblastoma exemplifies all of the challenges of treating cancer in young children. Current therapies involve standard chemotherapy, which is toxic to the entire body and can result in life-long damage to vital organs. There is a clear need for targeted therapies, as well as for markers that predict whether children with hepatoblastoma need the most intensive and potentially damaging therapies.

Dr. Gail Tomlinson, a physician-scientist at the Greehey Children's Cancer Research Institute, is an expert in hepatoblastoma biology and genetics and leads this MIRA project. Her collaborators include:

- Dr. D. Will Parsons at the Baylor College of Medicine Human Genome Center, who has directed genetic analysis of 35 hepatoblastoma tumors, the first such study of pediatric liver cancer;
- Dr. Dolores Lopez-Terrada, director of the Molecular Oncology Laboratory at Texas Children's Hospital, whose work allows distinct groups of tumors to be categorized based on gene expression;
- Dr. Milton Finegold, a world leader in liver pathology at Texas Children's Hospital, who analyzes tumor pathology;
- Dr. Dinesh Rakheja, a pediatric anatomic pathologist at UT Southwestern, who is pursuing additional

studies of genes on tumor tissues;

- Drs. Sarah Comerford and Robert Hammer at UT Southwestern, who are constructing the first mouse model of hepatoblastoma to use to study the role of developmental genes in the liver;
- Dr. Yidong Chen and his team at the Greehey Children's Cancer Research Institute, who are compiling a complete integrated genomic profile out of the data collected throughout the MIRA.

The end result of this project will be the most comprehensive knowledge to date of the genetic factors contributing to hepatoblastoma, which will allow for the development of targeted therapies that improve patient outcomes.

CPRIT SCHOLARS

The CPRIT Scholars in Cancer Research program recruits exceptional researchers to Texas academic and research institutions. These awards support the work and the laboratories of promising outstanding established investigators, first-time tenure-track faculty, missing links, and rising stars, and are a key component of CPRIT's strategic investment in building Texas' research infrastructure. For full biographies go to <http://www.cprit.state.tx.us/funded-grants/cprit-scholars/>.

ESTABLISHED INVESTIGATOR AWARDS

Lynda Chin

Institution: The University of Texas MD Anderson Cancer Center

Recruited from: Harvard Medical School

Degrees and positions held:

- M.D., Albert Einstein College of Medicine
- Chief Resident of Dermatology, Columbia Presbyterian Medical Center
- Professor of Dermatology, Harvard Medical School
- Senior Associate Member of the Broad Institute of MIT and Harvard
- Scientific Director, Belfer Institute for Applied Cancer Science at the Dana-Farber Cancer Institute
- Co-Leader, Dana-Farber Cancer Institute/Harvard Cancer Center's Melanoma Program
- Chair, Department of Genomic Medicine, MD Anderson Cancer Center
- Scientific Director, Institute for Applied Cancer Science, MD Anderson Cancer Center
- Founder, AVEO Pharmaceuticals and Metamark Genetic

Honors: The Cancer Genome Atlas (TCGA) Executive Subcommittee, GBM and Melanoma Disease Working Groups, Scientific Steering Committee of the International Cancer Genome Consortium, Member, Institute of Medicine

Research interests: Transcription, telomere biology, mouse models of human cancer, oncogenomics, and personalized cancer medicine

Neal Copeland

Institution: The Methodist Hospital Research Institute

Recruited from: Institute of Molecular and Cell Biology, Singapore

Degrees and positions held:

- Ph.D., Biochemistry, University of Utah, Harvard Medical School
- Director, Mouse Cancer Genetics, National Cancer Institute
- Director, Institute of Molecular and Cell Biology, Singapore
- Co-director, Methodist Hospital Research Institute

Honors: National Academy of Sciences

Research interests: Modeling human cancer in mice; molecular biology of retroviruses; identifying candidate cancer genes in hematopoietic tumors; transposon-based insertional mutagenesis to identify drug resistant genes.

Nancy Jenkins

Institution: The Methodist Hospital Research Institute

Recruited from: Institute of Molecular and Cell Biology, Singapore

Degrees and positions held:

- Ph.D., Molecular and Cellular Biology, Indiana University
- Postdoctoral Fellow, Dana-Farber Cancer Institute, Harvard Medical School
- Head, Molecular Genetics of Development, National Cancer Institute
- Deputy Director, Institute of Molecular and Cell Biology, Singapore
- Co-director, Methodist Hospital Research Institute

Honors: National Academy of Sciences
Research interests: Molecular biology of retroviruses; retroviruses as insertional mutagens in mice; transposon screening; lung and ovarian cancer

David Johnson

Institution: The University of Texas Southwestern Medical Center

Recruited from: Vanderbilt University

Degrees and positions held:

- M.D., Medical College of Georgia, Vanderbilt University
- Director, Division of Hematology and Medical Oncology, Vanderbilt University
- Deputy Director, Vanderbilt-Ingram Cancer Center
- Distinguished Chair, Department of Internal Medicine, UT Southwestern

Honors: Chair-Elect, American Board of Internal Medicine

Research interests: Biology of lung cancer

Herbert Levine

Institution: Rice University

Recruited from: University of California, San Diego

Degrees and positions held:

- Ph.D., Physics, Princeton University, Harvard University
- Distinguished Professor, University of California, San Diego
- Professor, Bioengineering and Physics, Rice University
- Co-director, Center for Theoretical Biological Physics

Honors: Fellow of the American Physical Society, National Academy of Sciences

Research interests: Application of non-equilibrium physics to cancer

Sean Morrison

Institution: The University of Texas Southwestern Medical Center

Recruited from: University of Michigan

Degrees and positions held:

- Ph.D., Immunology, Stanford University, California Institute of Technology
- Director, Center for Stem Cell Biology, University of Michigan
- Director, Children's Research Institute, UT Southwestern

Honors: Searle Scholar; Presidential Early Career Award for Scientists and Engineers; McCulloch and Till Award; Harland Mossman Award; National Institute on Aging

Research interests: Stem cell aging and self-renewal

Jose Onuchic

Institution: Rice University

Recruited from: University of California, San Diego

Degrees and positions held:

- Ph.D., Physics, California Institute of Technology
- Professor, University of California, San Diego
- Professor, Physics and Astronomy, Chemistry and Biochemistry, Rice University
- Co-director, Center for Theoretical Biological Physics

Honors: International Center for Theoretical Physics Prize; Fellow of the American Physical Society, National Academy of Sciences; Fellow of the American Academy of Arts and Sciences; Brazilian Academy of Sciences; Fellow of the Biophysical Society

Research interests: Theoretical and computational methods for molecular biophysics

Jeffrey Chang

Institution: The University of Texas Medical School at Houston
Recruited from: Duke University
Degrees: Ph.D., Biomedical Informatics, Stanford University
Research interests: Genomics-based investigation of cancer

Guangbin Dong

Institution: The University of Texas at Austin
Recruited from: California Institute of Technology
Degrees: Ph.D., Chemistry, Stanford University
Research interests: Synthetic technology to construct small molecule agents for cancer research

Lauren Ehrlich

Institution: The University of Texas at Austin
Recruited from: University of California, San Francisco
Degrees: Ph.D., Immunology, Stanford University
Research interests: T-cell lymphomas and leukemias

Dmitri Ivanov

Institution: The University of Texas Health Science Center at San Antonio
Recruited from: Harvard Medical School
Degrees: Ph.D., Biophysics and Structural Biology, Brandeis University
Research interests: HIV-related cancers

Ning Jiang

Institution: The University of Texas at Austin
Recruited from: Stanford University
Degrees: Ph.D., Biology, Georgia Institute of Technology
Research interests: Metrics for cancer progression prediction and monitoring

Ralf Kittler

Institution: The University of Texas Southwestern Medical Center
Recruited from: University of Chicago
Degrees: Ph.D., Dresden University of Technology and Max Planck Institute
Research interests: New targets for detection and treatment of prostate and lung cancer

Li Ma

Institution: The University of Texas MD Anderson Cancer Center
Recruited from: Massachusetts Institute of Technology
Degrees: Ph.D., Cornell University
Research interests: Roles and mechanisms of microRNAs

Kyle Miller

Institution: The University of Texas at Austin
Recruited from: Cambridge University
Degrees: Ph.D., University College London
Research interests: Chromatin and DNA repair in human cells

Daisuke Nakada

Institution: Baylor College of Medicine
Recruited from: University of Michigan
Degrees: Ph.D., Nagoya University
Research interests: Stem cell maintenance and mutation

Kathryn O'Donnell

Institution: The University of Texas Southwestern Medical Center
Recruited from: Johns Hopkins University
Degrees: Ph.D., Human Genetics and Molecular Biology, Johns Hopkins University
Research interests: Genetic mechanisms in cancer

Patrick Potts

Institution: The University of Texas Southwestern Medical Center
Recruited from: The University of Texas Southwestern Medical Center
Degrees: Ph.D., Biochemistry, UT Southwestern
Research interests: Biochemical and molecular mechanisms behind cellular processes

Lidong Qin

Institution: The Methodist Hospital Research Institute
Recruited from: California Institute of Technology
Degrees: Ph.D., Chemistry, Northwestern University
Research interests: Nanomedicine, prostate cancer

Jin Wang

Institution: Baylor College of Medicine
Recruited from: University of North Carolina at Chapel Hill
Degrees: Ph.D., Chemistry, The Ohio State University
Research interests: Nanotechnology in cancer research

Yonghao Yu

Institution: The University of Texas Southwestern Medical Center
Recruited from: Harvard Medical School
Degrees: Ph.D., Chemistry, University of California, Berkeley
Research interests: Mass spectrometric technologies in signal transduction networks

MISSING LINKS AWARDS**Robert Lenkinski**

Institution: The University of Texas Southwestern Medical Center
Recruited from: Harvard Medical School
Degrees and positions held:

- Ph.D., Chemistry, University of Houston, Weizmann Institute of Science
- Professor, Radiology, University of Pennsylvania
- Professor, Radiology, Harvard Medical School

Research interests: Molecular imaging

Hamid Mirzaei

Institution: The University of Texas Southwestern Medical Center
Recruited from: Institute for Systems Biology, Seattle
Degrees: Ph.D., Analytical Chemistry, Purdue University
Research interests: Development of new technologies for purification of proteins involved in gene regulatory complexes.

Carol Nilsson

Institution: The University of Texas Medical Branch at Galveston
Recruited from: Pfizer Global Research and Development, San Diego
Degrees and positions held:

- M.D., Ph.D., Clinical Neurochemistry, Goteborg University
- Associate Professor, Goteborg University
- Director, Ion Cyclotron Resonance User Program, National High Magnetic Field Laboratory
- Senior Principal Scientist, Pfizer Global Research and Development

Research interests: Use of quantitative phosphoproteomics and related systems biological techniques in neuro-oncology

RISING STAR AWARDS

Taiping Chen

Institution: The University of Texas MD Anderson Cancer Center
Recruited from: Harvard Medical School
Degrees: Ph.D., Molecular and Cell Biology, McGill University
Research interests: Role of epigenetic modifications in cancer

Joshua Mendell

Institution: The University of Texas Southwestern Medical Center
Recruited from: Johns Hopkins University
Degrees: M.D., Ph.D., Johns Hopkins University
B.A., Biology, Cornell University
Research interests: Activity of microRNAs

Jessica Tyler

Institution: The University of Texas MD Anderson Cancer Center
Recruited from: University of Colorado School of Medicine
Degrees: Ph.D., Virology, University of Glasgow
B.A., Biochemistry, University of Sheffield
Research interests: Chromatin and DNA repair

PRODUCT DEVELOPMENT

Groundbreaking science matters most when it is translated into products that help patients. CPRIT fulfills its mission by accelerating the progression of new cancer drugs, diagnostics, and therapies from the laboratory into clinical practice. In addition to improving patient care, CPRIT's Product Development Initiative fosters economic development in Texas' emerging life sciences industry and, through intellectual property and revenue sharing, provides a direct return on the investments made by the people of Texas.

CPRIT funds product development projects based upon both scientific merit and significant commercial potential. In addition to the scientific peer review process used by all CPRIT initiatives, product development proposals are subjected to a thorough due-diligence analysis to evaluate commercial prospects for new oncology products and services.

In addition to projects funded through grant awards, CPRIT's Product Development Initiative includes support programs such as the CPRIT Accelerator Program and the Entrepreneur in Residence Program. The CPRIT Accelerator Program attracts industry partners to work with CPRIT grantees on promising oncology products and services whose development has been funded by CPRIT. The Entrepreneur in Residence Program is designed to attract venture partners and strong management teams to Texas.

CPRIT'S PRODUCT DEVELOPMENT AWARDS

Company Awards support Texas-based companies with at least one round of professional institutional investment in developing marketable oncology products or services.

Company Formation Awards help underwrite new start-up companies, with no previous rounds of professional institutional investment, seeking to develop marketable oncology products or services. Companies must either be currently based in Texas or be willing to relocate to Texas.

Company Relocation Awards supports non-Texas-based companies with at least one round of professional institutional investment that are willing to relocate to Texas to develop commercially oriented oncology products or services.

CPRIT'S PRODUCT DEVELOPMENT PORTFOLIO

- CPRIT has announced awards totaling \$98 million in product development grants.
- More than 150 companies applied for CPRIT funding; 13 Texas-based companies were selected for funding (two companies were selected by the research program prior to launch of CPRIT's commercialization program).
- To date these 13 companies have leveraged CPRIT's investment to attract \$200 million in additional capital to Texas, both in matching funds and in subsequent financing.
- Commitments as of 2011 are projected to create or maintain approximately 140 life science-specific jobs in Texas over the next 3 years.
- Combined CPRIT and private capital of \$260 million could result in more than 3,380 new jobs in Texas over the next three years.
- CPRIT-funded company projects include promising drugs, diagnostics, and devices targeting a variety of

cancers, including cancers of the blood (leukemia, lymphoma, and myeloma), colon and rectum, esophagus, stomach, lung, and prostate. In addition, some companies are developing approaches applicable to multiple cancer types.

PRODUCT DEVELOPMENT HIGHLIGHTS

| Company | CPRIT Investment | Potential ROI (up to 5 years post marketing) | Follow-on Capital Attracted | Jobs Created and Maintained |
|--|------------------|--|--|-----------------------------|
| Apollo Endosurgery, Inc. <i>Austin</i> | \$5,001,063 | \$15 million | \$52 million | 43 |
| Asuragen, Inc. <i>Austin</i> | \$6,837,265 | (to be determined) | \$6.8 million | none reported |
| Bellicum Pharmaceuticals, Inc. <i>Houston</i> | \$5,680,310 | \$18 million | \$22 million | 14 |
| Caliber Biotherapeutics, Inc <i>College Station</i> | \$12,808,151 | (to be determined) | \$6.4 million | none reported |
| Cell Medica, Inc. <i>Houston</i> | \$15,571,303 | \$80 million | \$11 million | none reported |
| InGeneron, Inc. <i>Houston</i> | \$198,111 | \$221,000 | \$0.2 million | 4 |
| *Kalon Biotherapeutics, LLC <i>College Station</i> | \$7,901,420 | \$10 million | \$3.95 million | none reported |
| Mirna Therapeutics, Inc. <i>Austin</i> | \$10,297,454 | \$15 million | \$39.6 million | 5 |
| Molecular Templates, Inc. <i>Georgetown</i> | \$10,600,000 | \$42 million | \$5.3 million | none reported |
| **Peloton Therapeutics, Inc. <i>Dallas</i> | \$11,044,931 | \$113 million | \$18 million | 21 |
| Pulmotect, Inc. <i>Houston</i> | \$7,126,398 | (to be determined) | \$3.56 million | none reported |
| Rules-Based Medicine, Inc. <i>Austin</i> | \$3,024,432 | \$25 million | \$1 million + \$80 million (acquisition) | 4 |
| Visualase, Inc. <i>Houston</i> | \$2,151,776 | \$2.4 million | \$1.8 million | 5 |

* Pending contract negotiation.

** Project suspended; grant funds expended to date is \$3.2 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. In addition, using standard multipliers for the life science industry, the \$260 million in combined CPRIT and private capital associated with these projects should generate approximately 3,380 indirect jobs in Texas over the same period.

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' investments without impeding the ability to attract future commercial ventures. Like any interested investor, CPRIT is an engaged partner who can help bridge the gap between early stage discoveries and product development and hold award recipients accountable for their efforts to bring products to market.

PREVENTION

Ten percent of CPRIT's annual funding supports cancer prevention programs and services in Texas. These grants make it possible for proven services and interventions to reach many more Texans and decrease the burden of cancer statewide.

There are diverse and complex cancer prevention and control needs across the state, and CPRIT only funds projects that are results-oriented, evidence-based, non-duplicative, and innovative in delivery. These projects include:

- Primary prevention efforts, from vaccination to healthy lifestyle and obesity prevention initiatives, tobacco control, and sun protection;
- Early detection, screening, and diagnostic services primarily for breast, cervical, and colorectal cancers; and
- Survivor services, including physical rehabilitation and therapy, behavioral health, and support services.

CPRIT's Prevention Initiative focuses on the delivery of proven programs and services for people of Texas in the greatest need – those who are uninsured or underinsured, those in medically underserved areas of the state, or those at highest risk of cancer.

CPRIT'S PREVENTION AWARDS

Cancer Prevention Microgrant Awards focus on policy or systems change for tobacco cessation and increasing access to prevention services

Evidence-Based Cancer Prevention Services Awards provide for the delivery of evidence-based prevention services (e.g., prevention vaccine, screening, diagnostic, survivorship services)

Health Behavior Change Through Public and Professional Education and Training Awards focus on public health promotion, education, and outreach programs and/or professional education and training programs

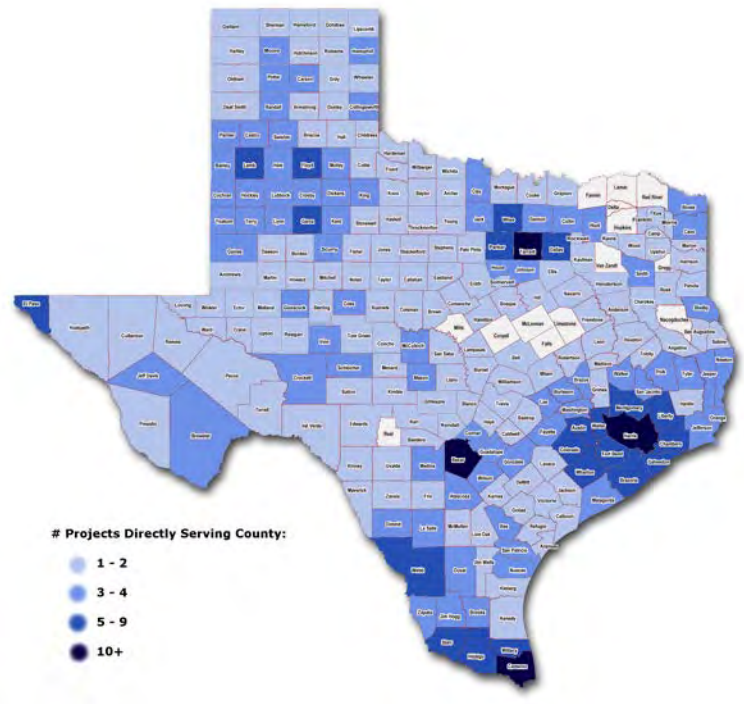
CPRIT'S PREVENTION PORTFOLIO

CPRIT's prevention work spans across Texas; the vast majority of counties have targeted projects serving their area of the state. Through August 2012, the prevention program has announced 105 awards totaling over \$85 million. To date, these projects have served over 850,000 Texans:

- o More than 620,000 people have received education, outreach, support services, and training
- o More than 230,000 people have received direct clinical services, including vaccination, screening and diagnosis, and survivor services. These include:
 - More than 31,000 tobacco-cessation clients
 - Almost 7,000 preventive vaccinations

- More than 184,000 people screened for colorectal, cervical, or breast cancer. Of these, more than 38,000 — 21 percent — had never before been screened. These screenings have detected at least 1,145 cancer precursors and 534 actual cancers.

Counties Served: Active Prevention Projects (75)
13 Projects Serve ALL Counties



PREVENTION HIGHLIGHTS

1. Moncrief Cancer Center



Faith Walker lives on a small ranch in Johnson County, south of Fort Worth. Through a CPRIT-funded project at UT Southwestern Medical Center's Moncrief Cancer Institute, Faith and other uninsured or underinsured women from Denton, Wise, Parker, Hood and Johnson Counties are now able to access state-of-the art breast cancer screening services close to home. These services are brought to patients' communities through mobile mammography and navigation services. Moncrief was able to diagnose Faith's cancer quickly, and because of Moncrief's excellent patient navigation services, Faith found out that she qualified for treatment under Medicaid. Better yet, she was able to get her surgery, chemotherapy, and radiation in her home county.

"It was a miracle for me to get treatment, a godsend to me, really. I can't thank them enough for all their help, and I'll never forget it." – Faith Walker

2. A Healthier San Antonio: Salud San Antonio!

Salud San Antonio!, a program led by the Institute for Health Promotion Research at the UT Health Science Center, is creating a healthier San Antonio. Often facing low income and little education on how to fight cancer, Latinas have few options for support, which means that breast or cervical cancer is often not detected until it has reached an advanced stage. One such woman attended a Salud event and shared that she had been diagnosed with precancerous cells in her cervix but could not afford to pursue treatment. With the assistance of Salud, the woman was shown how to receive the help she needed.

3. HOPE Where Hope is Scarce: The Asian American Health Coalition

A 44-year old Vietnamese man with a low income and no healthcare felt lost in Houston's 200,000-strong Asian American population. Like many in this demographic, financial and cultural limitations left him with limited access to cancer education and prevention programs. He had never been to a doctor. Through a friend, he learned about and attended a HOPE Clinic and Asian American Health Coalition program that incorporates a variety of activities to promote cancer awareness in the community. Through this program, he was able to register for a multitude of cancer and chronic disease screenings and to gain access to the care he needed.

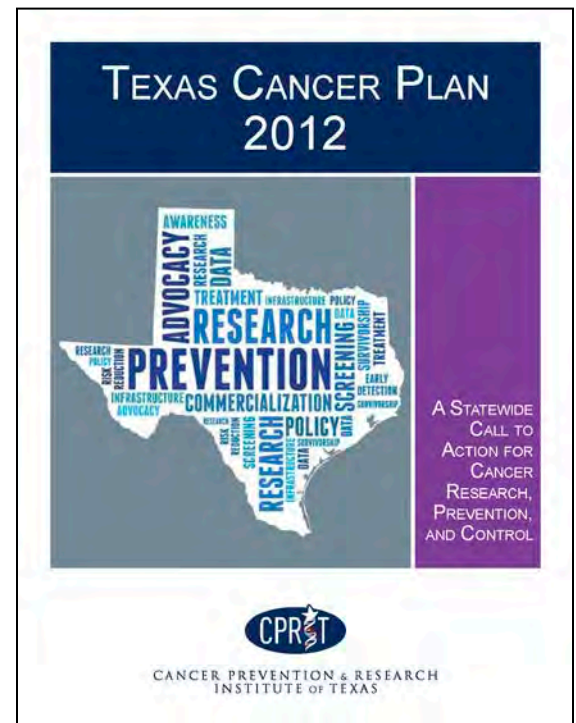
SAVING MONEY WHILE SAVING LIVES

Early detection of breast, cervical and colorectal cancer reduces the cost of patient care by as much as 50 percent, according to Texas Cancer Registry data. This allows scarce health care dollars to go farther to meet the needs of Texans. In addition, CPRIT's Prevention Initiative has helped providers to leverage federal, state and local resources to build the most effective programs. For example, Dr. Samir Gupta of The University of Texas Southwestern Medical Center and his colleagues used preliminary data from a CPRIT-funded prevention program to obtain more than \$6 million in funding from the National Cancer Institute. "It is notable that our site is one of only three colorectal cancer sites nationwide, the only site in Texas, and the only site exclusively focused on improving colorectal cancer screening for the uninsured," Dr. Gupta writes. "Receiving this prestigious NCI grant will allow Texas to be at the forefront of efforts to optimize colorectal cancer screening for the underserved, and could not have been possible without CPRIT support."

TEXAS CANCER PLAN

By state statute, CPRIT is charged with facilitating the development and implementation of the *Texas Cancer Plan*. As the statewide action plan for cancer prevention and control, the *Plan* identifies the challenges and issues that affect our state and presents a set of goals, objectives, and strategies to help inform and guide communities in the fight against cancer. The 2012 revision of the plan includes:

- Sixteen specific goals
- Measureable objectives, baselines, and targets for change
- Strategic actions for implementation
- Research and Commercialization section
- *Call to Action* section – What Can YOU Do?



ANNUAL CONFERENCE



Researchers, health professionals, and advocates from throughout Texas and the nation flocked to Austin in October 2012 for CPRIT's third annual *Innovations in Cancer Prevention and Research Conference*. The most up-to-date trends, debates, topics, and issues related to cancer research, prevention, and product development were presented during the 3-day conference. Speakers, CPRIT grantees, sponsors, and guests discussed experiences, current challenges, and goals for the future in the ongoing fight against cancer.

Dr. Brian J. Druker, the renowned leukemia specialist whose research led to the development of the drug Gleevec, keynoted the conference and gave attendees insight into the process of turning scientific breakthroughs into patient-ready treatments. Governor Rick Perry and Lieutenant Governor David Dewhurst both made unannounced appearances during the conference, thanking the attendees for their work as well as sharing their support and encouragement for CPRIT's future impact on Texas.

ADVISORY COMMITTEES

In carrying out CPRIT's mission, the Oversight Committee benefits from advice and input from four standing committees that are external to the governing body. These committees meet at least semi-annually and report to the CPRIT executive director and Oversight Committee executive leadership. Committee updates and reports are presented to the Oversight Committee at its quarterly meetings.

Advisory Committee on Childhood Cancers

The Advisory Committee on Childhood Cancers (ACCC) was created by statute to provide input and advice to CPRIT regarding the prevention, control and cure of childhood cancers. ACCC membership includes childhood cancer advocates and scientists whose research focus targets issues in pediatric oncology.

Product Development Strategy Committee

The Product Development Strategy Committee was created by the Oversight Committee to provide tactical advice regarding CPRIT's product development efforts and enhancing Texas' ability to move innovative products from the laboratory into clinical practice.

Scientific and Prevention Advisory Committee

The Scientific and Prevention Advisory Committee (SPAC) was created by the Oversight Committee to provide advice and support services to the Oversight Committee. The 22 SPAC members represent cancer-related fields including research, clinical trials, health care delivery, prevention programs, advocacy, and cancer survivorship.

University Advisory Committee

The University Advisory Committee was created by statute to advise the Oversight Committee regarding the role of institutions of higher education in cancer research. Membership is comprised of representation from the following university systems or institutions:

- University of Texas
- Texas A&M University
- Texas Tech University Health Sciences Center
- University of Houston
- Texas State University
- University of North Texas
- Baylor College of Medicine
- Rice University

FINANCIALS

| CANCER PREVENTION AND RESEARCH INSTITUTE OF TEXAS FINANCIAL SUMMARY (UNAUDITED) For the Year Ended August 31, 2012 | | |
|--|-----------|--------------------|
| REVENUES | | |
| Legislative Appropriations | \$ | 297,072,446 |
| License, fees and permits | | 76,896 |
| Interest income | | 1,345 |
| Other | | 64,728 |
| Total Revenues | \$ | 297,251,415 |
| EXPENSES | | |
| Salaries and Wages | \$ | 2,400,734 |
| Other Personnel Cost | | 26,200 |
| Professional Fees and Services | | 9,583,665 |
| Consumable Supplies | | 19,070 |
| Utilities | | 50,663 |
| Travel | | 59,224 |
| Rent – Building | | 462,813 |
| Rent – Machine and Other | | 20,923 |
| Other Operating Expenses | | 341,642 |
| Grant | | 281,565,685 |
| Capital Expenditures | | – |
| Total Expenses | \$ | 294,530,619 |
| EXCESS OF REVENUES OVER EXPENSES | \$ | 2,684,796 |

Financial Position of the Cancer Prevention and Research Institute of Texas

Management of the Cancer Prevention and Research Institute of Texas (CPRIT) is responsible for establishing and maintaining adequate internal control over financial reporting and compliance with certain provisions of laws, regulations, contracts, and grant agreements and other matters.

Clifton Larsen Allen LLP, an independent public accounting firm, has audited CPRIT's internal control over

financial reporting and compliance for the year ended August 31, 2012. As a result of the audit, Clifton Larsen Allen LLP has ascertained that the financial statements of CPRIT “present fairly, in all material respects, the respective financial position of the governmental activities and governmental funds of CPRIT as of August 31, 2012, and the respective changes in financial position and the discretely presented component unit for the year then ended in conformity with accounting principles generally accepted in the United States of America.”



2013 Executive Team

Wayne R. Roberts

Interim Executive Director

Margaret Kripke, Ph.D.

Chief Scientific Officer

Rebecca Garcia, Ph.D.

Chief Prevention Officer

Heidi McConnell

Chief Operating Officer

Kristen Pauling Doyle

General Counsel

Patricia Vojack

Chief Compliance Officer

Sandra Balderrama

Senior Advisor to the Executive Director

Laurie Baker

Receptionist

Ellen Read

Information Specialist

Michael Brown

Research Program Director

Sandra Reyes

Executive Assistant

Michelle Frerich

Prevention Program Manager

Alfonso Royal

Finance Manager

Michelle Huddleston

Accountant

Therry Simien

Information Technology Officer

Yvette Jimenez

Administrative Assistant

Ramona Magid

Prevention Program Director

Lisa Nelson

Operations Manager