

Translational Genomics Research Institute

# TGEN TODAY



**02 Dream Team Tackles Deadly Melanoma**

**04 Whole Genome Sequencing Makes Impact**

**08 Dell Donates Cloud Technology to TGen**

**11 Nationwide Bashes Benefit Diabetes Research**

**12 Volunteers Lead TGen's Fundraising Success**

## CATHERINE IVY A PATIENT-FOCUSED APPROACH

Page 6



Michael Bassoff

## A LOOK INSIDE...

Dear Friends,

Ten years ago, TGen set out to use the powerful new technologies stemming from the recently completed U.S. Human Genome Project and a patient-focused mindset to promote a new brand of medicine: one that would use a patient's own genome to treat his or her specific disease — what we like to call precision medicine.

Today, many patients facing cancer and other serious illnesses are hearing about TGen's patient-focused research and how it has begun to change the way we think about the practice of medicine. Thanks to the leadership and support of many generous friends, TGen is helping patients believe that one day there will be hope for all who face a life-threatening illness.

In this issue, you'll read about Catherine Ivy, president and founder of The Ben & Catherine Ivy Foundation and her determination to provide solutions and hope for patients with the most common and deadly form of brain tumors. Under her leadership, the Ivy Foundation chose to support TGen based on a shared belief in patient-focused research, which she hopes will enable doctors to more accurately diagnose individual patients and to match each patient with a more precise therapy.

The search for precision therapy brought San Diego-based molecular biologist Dr. Tim McDaniel to TGen when his own mother faced a rare but deadly cancer. Knowing of their patient-focused approach to research, Dr. McDaniel selected TGen scientists to help connect the best available science to his mother's treatment plan. By working together to analyze the billions of data points in her genome, they identified a unique genetic change and matched it with a therapy that improved his mother's quality of life long enough for her to celebrate her 50th wedding anniversary.

It is exactly this sort of collaborative, patient-focused approach that enabled TGen to be selected by Stand Up To Cancer and the Melanoma Research Alliance to co-lead a national dream team of scientists and physicians in an unprecedented three-year study of the most deadly form of skin cancer. They will investigate possible new individualized approaches to patients based on the specific genomic changes in their cancers.

A common thread to all of these stories is TGen's ability to sift through the billions of genetic data points generated through the sequencing of each individual genome. To accomplish this, TGen partnered with Dell, who worked with us to solve fundamental IT challenges that allow us to share patient data in real time. This enables accelerated delivery of care to patients who need it most.

Each of these stories illustrates how TGen makes a difference in the lives of patients and how genomic-based medicine is setting a new standard of care. The future looks promising as we seek to replace the diminished hope delivered by a life-threatening disease with a reversal of fortune to all who face a battle against all odds. On behalf of everyone at TGen, I'd like to thank you for helping us bring hope to many through our patient-focused approach.

A handwritten signature in dark ink, appearing to read "Michael Bassoff". The signature is fluid and cursive, written in a professional style.

Michael Bassoff  
President, TGen Foundation

Translational Genomics Research Institute

# TGEN TODAY



## 6 Cover Story

### A Patient-Focused Approach

The Ben & Catherine Ivy Foundation contributes \$10 million to TGen brain cancer research



## 2 Dream Team Tackles Deadly Melanoma

Stand Up To Cancer and Melanoma Research Alliance fund \$6 million study of skin cancer



## 4 A Scientist-Son's View

TGen's cancer genome analysis may have extended the life of one researcher's mother

## 8 Because Seconds Count

Dell cloud technology accelerates TGen's role in genomic-based clinical trials for children with cancer



## 10 TGen In-Brief

News and notables

## 11 Waylon Jennings Birthday Bashes

Nationwide series of summer concerts benefit TGen's diabetes research; more planned this summer



## 12 Cycle for the Cure

Community Leaders Recruit Riders to Raise Funds and Awareness to Battle Breast & Ovarian Cancer

*Cycle for the Cure photos courtesy of Dave Siebert*

## 13 TGen's annual stepNout walk, run, dash

1,000 Participants, 31 Teams Step Out to Support TGen Pancreatic Cancer Research



## 14 Key to the Cure

Fashion Fights Back Against Women's Cancer

*Key to the Cure photos courtesy of Trends Publishing and Darrylee Cohen*

### About TGen |

The Translational Genomics Research Institute (TGen) is a non-profit organization dedicated to conducting groundbreaking research with life changing results. Research at TGen is focused on helping patients with diseases such as cancer, neurological disorders and diabetes. TGen is on the cutting edge of translational research where investigators are able to unravel the genetic components of common and complex diseases. Working with collaborators in the scientific and medical communities, TGen believes it can make a substantial contribution to the efficiency and effectiveness of the translational process. For more information, visit: [www.tgen.org](http://www.tgen.org)

# Dream Team Tackles Deadly Melanoma

Stand Up To Cancer, Melanoma Research Alliance and Gateway Foundation fund \$6.5 million study of skin cancer



Dream Team Leaders  
Dr. Patricia LoRusso and  
Dr. Jeffrey Trent

In the sun-drenched Southwest, it seems only appropriate that an Arizona-based research institute like TGen should lead a study of deadly skin cancer.

In collaboration with the Barbara Ann Karmanos Cancer Institute, TGen is leading a \$6 million, three-year international study of melanoma – the most dangerous form of skin cancer – funded by Stand Up To Cancer (SU2C) and the Melanoma Research Alliance (MRA).

This unique clinical study will pursue new therapies for a type of melanoma known as BRAF wild-type (BRAFWt), for which there are few treatment options.

The SU2C-MRA Melanoma Dream Team Translational Cancer Research Grant will accelerate the application of new therapeutic agents, quickly moving new scientific discoveries to clinics where they can immediately benefit patients. In the U.S., nearly 121,000 people are diagnosed with melanoma each year. Someone dies from melanoma every hour; about 9,000 annually.

The study, which will involve nearly 50 scientists and 200 patients at more than a dozen institutes, will be headed by SU2C-MRA Dream Team leaders Dr. Jeffrey Trent, President and Scientific Director at TGen in Phoenix, and Dr. Patricia LoRusso, Director of the Eisenberg Center for Experimental Therapeutics at the Karmanos Cancer Institute in Detroit.

“The Stand Up To Cancer-Melanoma Research Alliance grant gives us the remarkable ability to align cutting edge researchers across the globe to join forces to defeat this terrible disease,” said Dr. Trent, an internationally recognized expert in molecular-based systems biology approaches to cancer who will supervise patients’ genetic and genomic sequencing, spelling out their DNA.

“We hope to use this unique multi-stage clinical investigation to define new treatments that will produce benefits for metastatic melanoma patients, based on extensive genomic profiling. We have great scientists and clinicians from across the nation who will join forces on this,” said Dr. LoRusso, who will supervise the selection of patients, tissue samples and clinical trials research.

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THIS UNIQUE CLINICAL  
STUDY WILL PURSUE  
NEW THERAPIES FOR  
A TYPE OF MELANOMA  
KNOWN AS BRAF  
WILD-TYPE, FOR  
WHICH THERE ARE FEW  
TREATMENT OPTIONS.

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Joining TGen and Karmanos on the Dream Team are numerous clinicians and researchers from 16 Participating Institutes and Clinical Centers, a medical overseer, four patient advocates, and a four-member genomics advisory committee.

“Having a Dream Team of physicians and scientists focus on such an important and unmet need for patients who are not able to benefit from the latest breakthrough drugs is a most welcome development,” said Debra Black, co-founder and chair of the Melanoma Research Alliance.

Dr. Trent also is a Professor in TGen’s Genetic Basis of Human Disease Division and Head of the Melanoma Therapeutics Lab. His work is focused on applying genomic tools to study melanoma, and he is recognized for this as well as his work in translational medicine and will act as the principal investigator overseeing translational research of this Dream Team project.

Dr. LoRusso also is a Principal

Investigator for the Barbara Ann Karmanos Cancer Institute’s National Cancer Institute-U01-funded phase I program, and Professor of Oncology at Karmanos Cancer Institute and Wayne State University School of Medicine in Detroit. She is a leading clinical investigator in early developmental therapeutics and will act as principal investigator for the clinical trials, overseeing all clinical aspects of this Dream Team project.

Currently, patients who develop metastatic melanoma have a dismal prognosis, with a median survival of six to nine months and a five-year survival rate of 15 percent to 20 percent. About half of patients with metastatic melanoma have an oncogenic mutation in their tumor’s BRAF gene, but the other half of patients are BRAFwt and have no mutation in the gene. Very little progress has been made to identify new therapeutic targets to treat metastatic melanoma patients with BRAFwt disease.

This Dream Team will investigate the utility of personalized target/therapy identification in patients with BRAFwt metastatic melanoma. It will explore the efficacy of molecularly guided therapy involving numerous Food and Drug Administration-approved and investigational agents.

An ensuing clinical trial will determine whether this personalized approach significantly improves clinical outcome.

The team hopes that an individualized medicine approach to the treatment of BRAFwt metastatic melanoma will not only lead to therapeutic benefit for this patient population, but may also be beneficial to many other tumor and disease types.

On behalf of Stand Up To Cancer, the American Association for Cancer Research (AACR) will administer the grants. AACR is the oldest and largest scientific organization in the world focusing on every aspect of high-quality, innovative cancer research.



Melanoma  
Research Alliance

## SU2C Melanoma Dream Team

### Dream Team Leaders

*Dr. Jeffrey Trent, TGen*

*Dr. Patricia LoRusso, Karmanos Cancer Institute*

### Participating Institutes & Clinical Centers

#### TGen

*Dr. Michael Bittner; Dr. John Carpten;*

*Dr. David Craig; Dr. Matt Huentelman;*

*Dr. Jeff Kiefer; Dr. Christopher Kingsley;*

*Dr. Amol Tembe; & Dr. Spyro Mousses*

#### TGen/Virginia G. Piper Clinical Trials Programs at Scottsdale Healthcare

*Dr. Ramesh K. Ramanathan & Dr. Daniel D. Von Hoff*

#### Arizona State University

*Dr. Joshua LaBaer*

#### Baylor University

*Dr. C. Lance Cowey*

#### Indiana University

*Dr. Leslie Anne Fecher*

#### Karmanos Cancer Institute

*Dr. Patricia M. LoRusso & Dr. Lawrence E. Flaherty*

#### Mayo Clinic (Scottsdale, Minnesota & Florida)

*Dr. Aleksandar Sekulic; Dr. Svetomir Markovic*

*& Dr. Richard W. Joseph*

#### Memorial Sloan-Kettering Cancer Center

*Dr. Neal Rosen; Dr. David B. Solit,*

*Dr. Gary K. Schwartz; Dr. Paul B. Chapman*

*& Dr. Jedd D. Wolchok*

#### Michigan State University

*Dr. Brian J. Nickoloff*

#### National Institutes of Health

*Dr. Sally Hunsberger; Dr. Richard Simon; Dr.*

*Shivaani Kumar & Dr. Javed Khan*

#### Sanford Burnham Medical Research Institute

*Dr. Kristiina Vuori*

#### Scripps Research Institute

*Dr. Nicholas J. Schork & Dr. Ali Torkamani*

#### University of California Santa Cruz

*Dr. David Haussler*

#### University of Michigan Comprehensive Cancer Center

*Dr. Bruce Redman & Dr. Christopher Lao*

#### University of Pittsburgh Cancer Institute

*Dr. John M. Kirkwood*

#### Van Andel Institute (VAI)

*Dr. Craig P. Webb*

#### Vanderbilt University

*Dr. Jeffrey A. Sosman; Dr. William Pao & Dr. Mia Levy*

# A SCIENTIST-SON'S VIEW

## TGen joins son's fight to help mother

At his family's farm in Kentucky, Dr. Tim McDaniel arrived in December 2010 to help care for his mother, Beth, and try to unravel the cause of her mysterious illness — a rare cancer known as Sézary syndrome that caused uncontrollable itching and skin sores.

It is a poorly studied and usually fatal form of cutaneous T-cell lymphoma that is diagnosed in fewer than 100 Americans each year.

At first, Dr. McDaniel did not have a complete plan. He was on leave from his job as a Ph.D. senior scientist and product-development team-leader at the San Diego-based molecular sequencing giant, Illumina Inc.

"I had amazing support from scientists at my work, and my mom had great doctors, but I lacked a team that knew how to connect the science to patient care," he said.

Over several months — and a series of fortunate relationships — Dr. McDaniel eventually connected with TGen and a path of discovery that led to "nothing but hope, inspiration and wonder."

Dr. John Carpten, TGen's Deputy Director of Basic Science, Dr. David Craig, TGen's Deputy Director of Bioinformatics, and Mayo Clinic physician/researcher, Dr. Aleksandar Sekulic anchored the TGen/Mayo team. They used whole-genome DNA sequencing that Dr. McDaniel's family obtained from Illumina, as well as conducted their own RNA sequencing, to spell out the billions of chemical letters in Mrs. McDaniel's genome.

Crucially, working with Dr. McDaniel, they discovered that her tumor contained a fusion of the CD28 and CTLA4 genes, both of which are important in regulating the body's immune system T-cells — white blood cells that seek out invading pathogens. At its simplest level, CD28 activates T-cells, while CTLA4 turns them off.

Under normal conditions, CD28 initiates immune responses, starting a cascade of events that initiates the creation of T-cells. CTLA4 also is activated to tell the T-cells when to stop multiplying. "The balance of these two contrasting signals controls T-cell proliferation in an appropriate immune response," Dr. McDaniel explained.

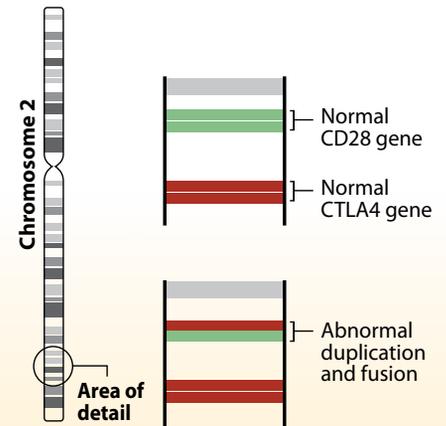
With the fusion of the two genes, he said, "You had the brake pedal of the T-cells wired to the accelerator. Instead of sending growth suppression signals, it would seem to be telling the cell to 'Go, go, go.'"

Out-of-control cellular growth is one of the defining characteristics of cancer.

In March 2011, about the time TGen completed its genomic analysis, the U.S. Food and Drug Administration approved a monoclonal antibody drug for late-stage melanoma called ipilimumab, which targets CTLA4.

TGen's whole-genome sequencing indicated that ipilimumab might also work for Sézary syndrome.

"The fusion (TGen discovered) suggested that this recently approved drug, which otherwise would not have been considered for the disease, might have growth-inhibitory effects on the



TGen researchers found that two tumor cell genes, CD28 and CTLA4, had fused, causing the patient's immune system T-cells to grow uncontrollably, like "the brake pedal...wired to the accelerator."

cancerous cells." Dr. McDaniel said.

After his mother's condition worsened, and after consultations by a panel of experts, Dr. McDaniel's mother began receiving ipilimumab in late July 2011.

A week later, his mother's deeply red skin had turned pink. Her lymph nodes in her neck, swollen for 6 years since her initial diagnosis, couldn't be felt.

After another week, four of the five ulcerated tumors on her legs had healed, and the remaining one was smaller.

Most importantly, Dr. McDaniel said, "She started living again. She had been housebound for months." Now, she was going to restaurants, movies and church. "She was going out with friends every day."

But within a few weeks, the disease began coming back. By November 2011,



Left to right: Dr. Tim McDaniel of Illumina, and TGen Deputy Directors Dr. John Carpten and Dr. David Craig.

it had accelerated, and “by the end of the month, my mother had passed away,” Dr. McDaniel said.

There is no conclusive proof that the drug made his mother better, he told a gathering of TGen employees in March. More study is needed. Even if it were the cause of his mother’s improvement, some still might question the value of extending a life maybe 6 or 8 weeks.

For Dr. McDaniel, his voice choking a bit, those few weeks were eminently significant. It meant his mother, Beth, and father, Roger, were able to celebrate their 50th wedding

anniversary, and that she got to spend one more Thanksgiving with her family.

Dr. Jeffrey Trent, TGen’s President and Scientific Director, said Dr. McDaniel’s story is “one of the most important and timely messages for TGen to hear. It’s a message of home, loss, and recognition of the importance of the work that all of us do here at TGen, and at other centers focused on genomic medicine.”

Dr. McDaniel wonders what might have happened if the drug were available six years earlier, when his mother was first diagnosed. “I dream

of the day when this will become part of mainstream medicine,” he said of the molecular sequencing and analysis performed by TGen.

“Thank you so much for turning this research into something that is actually applied to help people,” Dr. McDaniel told TGen employees. “My friends, what you do, it matters.”

Other TGen researchers who worked on this project include: Dr. Winnie Liang, Dr. Waibhav (Amol) Tembe, Rebecca Reiman, Lori Phillips, Tyler Izatt, Ahmet Kurdoglu, and Alexis Christoforides.



Catherine Ivy, Founder and President of The Ben & Catherine Ivy Foundation, with TGen Deputy Directors Drs. David Craig (left) and John Carpten (right).

# A PATIENT-FOCUSED APPROACH

The Ben & Catherine Ivy Foundation  
contributes \$10 million to TGen brain cancer research

**C**atherine (Bracken) Ivy, Founder and President of The Ben & Catherine Ivy Foundation, has traveled the world learning all she can about brain cancer — specifically, how to cure it.

She is determined, through the efforts of her foundation, to find better treatment options and improve the quality of life for patients with brain tumors, especially those with glioblastoma multiforme (GBM),

the most common and aggressive form of malignant primary brain tumor. Ninety-eight percent of people diagnosed with GBM live less than 18 months.

Because brain cancer is rare compared to many other cancers, research into the disease receives little federal funding, pharmaceutical industry support or media attention. Today, standard of care currently involves removal of the tumor (though

surgery most often fails to remove all the cancer) followed by radiation treatments and chemotherapy involving a drug with limited effect for the majority of patients. Sadly, little else remains to extend life expectancy or remission.

This status quo is not acceptable to Ms. Ivy. More than anything, she wants the Ivy Foundation to provide solutions —and hope— for people diagnosed with brain cancer.

“I want people with brain cancer and brain tumors to know that there is a community of people working very hard to try and help them,” Ivy said. “We’re not saying we’re going to cure it tomorrow, but at least we’re moving the needle. Our immediate goal is to double life expectancy in the next seven years for people diagnosed with brain cancer. We will never give up until we find a cure.”

### Leading the Charge

The Ivy Foundation’s overarching goal over the next seven years is to double the life expectancy of brain cancer patients from 18 to 36 months. And in working with TGen, Ivy said she has found three key values that align both organizations:

- Patient-focused research.
  - Conducting the best research possible in a cost-effective manner.
  - Making progress immediately.
- “Those three things are not simple,” Ivy said.

But, she says, the innovative ideas of TGen President and Scientific Director Dr. Jeffrey Trent make her believe her efforts are worthwhile. The two first met at a brain cancer conference in Tucson, and at subsequent meetings Dr. Trent outlined research that would help the Ivy Foundation achieve its goal of advancing patient care.

As a result, the Ivy Foundation recently granted \$10 million to TGen: \$5 million each for two new groundbreaking brain cancer projects.

### Discovering why some patients live longer

One \$5-million-project is titled “Outliers in Glioblastoma Outcome: Moving the curve forward.” This five-year investigation seeks to discover why approximately two percent of glioblastoma multiforme (GBM) patients — the outliers — live far beyond the average survival time of 18 months.

“A major challenge with brain cancer is that people survive such a short time,” Ivy said. “If this research enables patients to live longer, clinicians and researchers will gain a better understanding of how this disease works, which will bring us time to study the disease providing the opportunity to move closer to a cure.”

By precisely identifying the billions of molecular building blocks in each

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WITH BRAIN CANCER.

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patient’s DNA through whole genome sequencing, TGen researchers hope to discover the genetic differences between those patients who survive only a few months, and those who survive longer because their brain cancer develops more slowly.

Using these genetic targets, TGen researchers will identify those patients most likely to benefit from the current standard of care, and those who might best benefit from alternative or new experimental treatments.

### First-in-patient clinical trial studies

In the second \$5-million-project, “Genomics Enabled Medicine in Glioblastoma Trial,” TGen and its clinical partners will lead first-in-patient clinical trial studies that will test promising new drugs that might

extend the survival of GBM patients.

This multi-part study will take place in clinics across the country and TGen laboratories.

This project begins with a pilot study of 15 patients, using whole genome sequencing to study their tumor samples to help physicians determine what drugs might be most beneficial.

To support molecularly informed clinical decisions, TGen labs also will examine genomic data from at least 536 past cases of glioblastoma, as well as tumor samples from new cases, developing tools that will produce more insight into how glioblastoma tumors grow and survive. TGen also will conduct a series of pioneering lab tests to measure cell-by-cell responses to various drugs.

To get new treatments to patients as quickly as possible, this five-year study will include a feasibility study involving up to 30 patients, followed by Phase II clinical trials with as many as 70 patients. TGen intends to team with the Ivy Early Phase Clinical Trials Consortium that includes: University of California, San Francisco; University of California, Los Angeles; the MD Anderson Cancer Center; Memorial Sloan Kettering Cancer Center; University of Utah; and the Dana-Farber/Harvard Cancer Center.

The results of these clinical trials should not only help the patients who join them, but also provide the data needed for FDA approval and availability of new drugs that could benefit tens of thousands of brain cancer patients in the future.

“It’s a tremendous opportunity to find more solutions for the patient diagnosed with brain cancer,” said Ivy, who also is working to establish additional clinical trials in the Phoenix area, giving local patients more treatment options. “The clinical trials are very exciting because they can impact the patient today.”



**W**hen children develop cancer, they don't have time to wait. Their cancers move quickly, and death comes all too soon.

But what if there was a system that could quickly analyze a child's tumor, and inform the patient's oncologist of the best available options, based on the child's complete genomic profile?

That system is on its way.

The Neuroblastoma and Medulloblastoma Translational Research Consortium (NMTRC), joining with the Translational Genomics Research Institute (TGen), has launched personalized medicine clinical trials focused first on neuroblastoma (cancer of the nervous tissue), and eventually expanding to other children's cancers, including medulloblastoma (a type of brain tumor).

In partnership with Dell, who committed funding, employee engagement and cloud computing technology to support the initiative at TGen, the NMTRC is coordinating clinical tests that include whole-genome sequencing, the spelling out, of each patient's entire 3-billion-letter DNA.

Whole-genome sequencing aligns a patient's normal DNA with the cancerous DNA to locate changes as well as potential targets that could be matched to existing FDA-approved drugs, or point researchers toward the development of effective new drugs.

While the cost and the time to conduct whole-genome sequencing continues to drop, another hurdle remains for researchers and physicians: How to quickly access the ultra-high volumes of data involved in these comprehensive genomic tests, while at the same time ensuring that the information is secure and the patient's privacy is protected.

Children with cancer don't have time for hard-drives to be shipped across the nation, or around the globe. Their doctors need the information now.

Because of Dell's cloud solution, the data can reside at TGen and yet be quickly accessed globally by physicians and scientists in the NMTRC system. Dell's cloud has the capacity to handle the high volumes of data, and at the same time support timely processing of patient tumors, helping predict the best drug therapy for each patient.

## Because Seconds Count

Dell cloud technology accelerates genomic-based trials for children with cancer

"Even at this earliest moment in genomics-guided therapy, there is universal recognition that the amount and complexity of data is overwhelming," said Dr. Jeffrey Trent, TGen's President and Scientific Director. "Dell's commitment to helping children with cancer, coupled with

its expertise in developing cloud-based solutions for health information, will provide great benefit in terms of helping us manage and share information rapidly, and help us arrive at the optimal treatment decision for each child battling cancer.”

Dell is expanding its Powering the Possible program to focus on pediatric cancers because of the devastating nature of these diseases, and to address the void of new and innovative treatments available for children.

“Time is critical when treating these types of cancer. Dell’s technology, coupled with the groundbreaking research is needed to help reduce the amount of time it takes to create these specialized treatment plans,” said Dr. James Coffin, Vice President and General Manager of Dell Healthcare & Life Sciences.

Since the 1980s, the Food and Drug Administration (FDA) has approved only one new treatment for any type of childhood cancer, compared with 50 approved treatments for adult cancers in the same time period.

Neuroblastoma strikes one in 100,000 children annually, usually before the age of 5, and despite it being so rare, it is so deadly that it is responsible for one in seven pediatric cancer deaths. It attacks the sympathetic nervous system, and because of the unique and aggressive nature of neuroblastoma tumors, conventional approaches to developing a one-size-fits-all treatment are ineffective.

With little commercially or federally funded research underway, parents and pediatric oncologists have relied largely on “trial and error” in their search for a treatment that will work from among the hundreds of available adult cancer trials.

“This trial offers hope to those children facing what is among the worst of all pediatric cancers,” said Dr. Giselle Sholler, NMTRC Chair and Co-Director of the Pediatric Cancer Translational Research Program at the Van Andel Institute (VAI). “We are confident the

genomic-based personalized medicine approach is the right one, and Dell’s contribution will help remove barriers that currently exist in how rapidly and easily we can analyze and share information to benefit our patients.”

Because there are about 3 billion chemical bases of DNA in each cell, sequencing of each patient’s normal DNA, and tumor DNA — a process repeated numerous times to ensure accuracy — generates more than 200 billion measurements per patient that must be analyzed, shared and stored. The computation and analysis of this information can take weeks, and even months, to process.

Dell’s cloud solution provides the computing power needed to help increase TGen’s gene sequencing and analysis capacity by 1,200 percent. The cloud is expected to accelerate genetic analysis and identification of targeted treatments for each patient, reducing the information processing time from months, to just a few days.

These advancements will improve collaboration among the team of physicians, genetic researchers, pharmacists and computer scientists working on the trial. The additional computing power will also improve the availability of critical information and allow researchers to develop a real-time knowledge repository of the latest findings on the most effective treatments for oncologists to use globally.

The researchers also intend to use the cloud to expand the program’s participation from a handful of children today to hundreds of children over the next three years, with the goal of establishing an information framework that, subject to regulatory approval, could one day help thousands of pediatric cancer patients.

The new TGen cloud will also facilitate rapid transfer of information to international partners and lay the groundwork for expansion of the trial to additional types of childhood cancers in the future.



The Neuroblastoma and Medulloblastoma Translational Research Consortium (NMTRC) is a group of universities and children’s hospitals across the U.S. that offer a nationwide network of childhood cancer clinical trials. These trials are based on the research from a group of closely collaborating investigators who are linked with laboratory programs developing novel therapies for high-risk patients battling neuroblastoma and medulloblastoma.

**Phoenix Children’s Hospital**  
Phoenix, Arizona

**The Helen DeVos Children’s Hospital**  
Grand Rapids, Michigan

**Levine Children’s Hospital**  
Charlotte, North Carolina

**M.D. Anderson Cancer Center**  
Orlando, Florida

**National Cancer Institute**  
Bethesda, Maryland

**SSM Cardinal Glennon Children’s Medical Center**, St. Louis, Missouri

**Children’s Mercy Hospital and Clinics**  
Kansas City, Missouri

**Connecticut Children’s Medical Center**  
Hartford, Connecticut

**Rady Children’s Hospital**  
San Diego, California

**The Medical University of South Carolina**,  
Charleston, South Carolina

**Monroe Carrell Jr. Children’s Hospital at Vanderbilt**, Nashville, Tennessee

**Dell Children’s Blood and Cancer Center**,  
Austin, Texas

**Primary Children’s Hospital**,  
Salt Lake City, Utah



Dr. Paul Keim

### Dr. Keim named Arizona's 2012 Bioscience Researcher of the Year

Dr. Paul Keim, Director of TGen's Pathogen Genomics Division in Flagstaff and the Cowden Endowed Chair of Microbiology at Northern Arizona University (NAU), received the 2012 Bioscience Researcher of the Year award from the Arizona BioIndustry Association (AZBio).

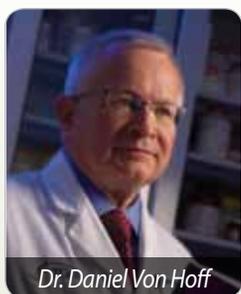
Dr. Keim is a world-renowned expert in anthrax and other infectious diseases. At TGen and NAU he directs investigations into how to bolster the nation's biodefense, and to prevent outbreaks — even pandemics — of such contagions as flu, cholera, E. coli, salmonella, and even the plague.

"Our science has been completely transformed by the rapid advancements of technology. Now, TGen's job is to rapidly advance our science to make great impacts on human health. We have that ability, therefore, we feel that we have that responsibility," said Dr. Keim, a Professor at TGen and Regents Professor of Microbiology at NAU. Dr. Keim also is Director of NAU's Microbial Genetics & Genomics Center, a program that works with numerous government agencies to help thwart bioterrorism and the spread of pathogen-caused diseases.

## IN BRIEF

### TGen-UA awarded \$600,000 for pancreatic cancer research

TGen and the University of Arizona share a \$600,000 grant from the National Foundation for Cancer Research to continue pancreatic cancer research. The grant enables TGen and the UA to continue its NCFR Center for Targeted Cancer Therapies (NCTCT), created in 2002, which is dedicated to discovering new therapies to treat pancreatic cancer, the nation's fourth leading cause of cancer death.



Dr. Daniel Von Hoff

### Dr. Von Hoff called 'leading authority' in U.S. for pancreatic cancer

In a book published earlier this year, *The Creative Destruction of Medicine*, Dr. Eric Topol describes TGen's Physician-In-Chief Dr. Daniel Von Hoff as the nation's "leading authority on pancreatic cancer." Dr. Topol is Director of the Scripps Translational Science Institute in La Jolla, California.

### TGen called one of three drivers of Arizona economy

In *Business Magazine* this year listed TGen, Arizona Public Service and Intel as three businesses that drive Arizona's economy.

"As we look forward, these organizations are sure to influence our state's economic engine for years to come," the magazine said.



Dr. Jeffrey Trent

### Dr. Trent helps guide research aboard the International Space Station

Dr. Jeffrey Trent is one of six world-class scientists named this year by CASIS to its Biological Sciences Review Panel, responsible for reviewing experiments conducted on the International Space Station.

CASIS is the Center for the Advancement of Science in Space, the non-profit organization managing research on the Space Station's U.S. National Laboratory.



Dr. Heather Cunliffe

### TGen receives \$75,000 grant for ovarian cancer research

The Marsha Rivkin Center for Ovarian Cancer Research has awarded \$75,000 to TGen for a pilot study of Small Cell Carcinoma of the Ovary (SCCO), led by Dr. Heather Cunliffe, Head of TGen's Breast and Ovarian Cancer Research Unit.

TGen's study will attempt to identify the full spectrum of molecular changes associated with SCCO. The average age of diagnosis of only 23 years old.

"We are optimistic our study will reveal a potential therapeutic vulnerability for these patients," Dr. Cunliffe said.

In addition to identifying potential drug targets, Dr. Cunliffe proposes to develop preclinical laboratory models of SCCO to enable future studies designed to confirm mechanisms of targeted drug sensitivity, and accelerate the translation of laboratory discoveries into to clinical practice.

Pilar Ramos and Megan Russell, Research Associates in the Cunliffe Lab are conducting the molecular studies with collaborative support from Michael Barrett's Lab, TGen's Collaborative Sequencing Center and Collaborative Bioinformatics Center.



Dr. John Carpten

### TGen helps identify at-risk gene in prostate cancer

TGen and its collaborators found the first major gene mutation associated with a significantly higher risk of hereditary prostate cancer risk, according to a study published in the *New England Journal of Medicine*.

Led by TGen Deputy Director Dr. Carpten, TGen sequenced the DNA of more than 200 genes on a human chromosome region known as 17q21-22. This followed a 20-year quest to find a genetic driver for an aggressive type of prostate cancer that strikes men at younger ages.

"The results of this study represent an amazing advancement in our understanding of inherited prostate cancer. It is our hope that this information will provide new opportunities for diagnosis, prevention, and treatment of prostate cancer in patients with a strong family history of the disease," said Dr. Carpten, who also is Director of TGen's Integrated Cancer Genomics Division and a co-investigator on the study.

# Concerts Benefit TGEN DIABETES RESEARCH

**W**aylon Jennings fans from across the country gathered this summer for the first nationwide series of Waylon Birthday Bashes benefiting TGen.

And they plan to do it again this coming June.

The parties were touted by country music stars Keith Urban, Kris Kristofferson, Billy Ray Cyrus and Jamey Johnson, urging potentially millions of Waylon fans to come together and support TGen's diabetes research.

Urban, Kristofferson, Cyrus and Johnson — all members of the Waylon Fund National Honorary Committee for Diabetes Research — called on their followers to help TGen quickly translate laboratory discoveries into new ways to diagnose and treat diabetes, which affects nearly 26 million Americans.

“What’s most incredible to me over the past year since we launched The Waylon Fund, is how this grassroots effort has continued to spread across the country,” said Dean Ballard, the TGen Foundation’s Assistant Director of Development. “Waylon Birthday Bashes were created by the people who knew Waylon, family members and fans. Most organizers have celebrated Waylon’s birthday on June 15 for years. It was just a matter of connecting with them.”

In honor of Outlaw Country’s greatest hero, bashes benefitting TGen were held this year in Detroit, New York, Houston, Seattle, Nashville, and Whiteface, Texas, near Waylon’s his hometown of Littlefield. They featured both local and national acts, including: Waylon’s son, Shooter Jennings; Waylon’s brother, Tommy Jennings; Waylon’s co-producer and drummer, Richie Albright; and the Rowdy Johnson Band.

In TGen’s home state of Arizona, a Birthday Bash was held June 17 at the Yucca Tap Room in Tempe, featuring “DJ Dana” Armstrong, founder of Valley Fever Country Night. It also included DJ

Johnny Volume and several live-music acts: Ray Lawrence Jr., Tony Martinez, James Parks, Steve Larson, Hans Olson, Junction 10, and J.P. Harris and The Tough Choices.

“We have a built-in Waylon fan base here who are happy to support a progressive diabetes research fund in his name,” Armstrong said.

This coming summer, bashes are being added in: Los Angeles; Las Vegas; Corpus Christi, Texas; Bliss, Idaho; Cashmere, Wash.; and Tucson.

“It looks like we’ll easily triple the amount of venues by June,” Ballard predicted.

Working with Waylon’s widow, Jessi Colter, and their son, Shooter, TGen established The Waylon Fund in October 2011 with the goal of helping TGen scientists challenge conventional ideas and discover novel ways of attacking diabetes.

Waylon, who died in 2002 at age 64 from complications of diabetes, lived much of his storied career in Arizona, and is buried in the Phoenix suburb of Mesa.

TGen’s research into diabetes will allow physicians to more accurately predict who has the greatest risk of developing both Type 1 and Type 2 diabetes, while also developing better strategies for identifying and managing many aspects of the disease.

Dr. Johanna DiStefano, Director of TGen’s Diabetes, Cardiovascular & Metabolic Diseases Division, leads a team of scientists working in five areas of research: diabetes and heart disease, diabetic nephropathy, diabetes and obesity, diabetes and liver disease, and diabetes and individualized treatment strategies.

For more information about The Waylon Fund, please visit [www.thewaylonfund.org](http://www.thewaylonfund.org).



# CYCLE FOR THE CURE

Community Leaders Recruit Riders to Raise Funds and Awareness to Battle Breast & Ovarian Cancer

Seeking an end to breast and ovarian cancer, Cycle for the Cure's second year drew more than 150 indoor cyclists, participating in a 2-hour, heart-pumping stationary-bicycle ride at three exclusive Phoenix-area clubs.

Cycle for the Cure on Sept. 30 surpassed \$93,000 — more than double the amount raised in its inaugural year. Following simultaneous spins at Camelback Village Racquet & Health Club, Gainey Village Health Club & Spa,

and Studio 360, participants and friends celebrated with food, music and fun at the event's post-ride party hosted by Camelback Village.

Plans are to expand the event in 2013, attracting a growing number of riders dedicated to eliminating women's cancers. Though treatments have improved, nearly 40,000 American women will die this year from breast cancer, and 15,000 will succumb to ovarian cancer.

Heading this year's pack were event co-chairs Robyn DeBell and Vicki Vaughn, who were inspired to lead Cycle for the Cure because of the vision of TGen President and Scientific Director Dr. Jeffrey Trent.

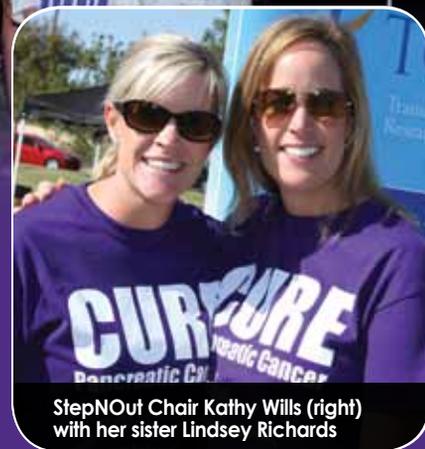
"Dr. Trent is a gifted scientist who is compassionate as well as brilliant. Personalized medical discoveries as a result of TGen's research are cutting edge," DeBell said. "Funding research is the best investment our community can make for our

medical health future."

DeBell and Vaughn are committed to TGen's translational model for personalized medicine, taking new laboratory discoveries quickly to clinical trials, where they help patients today.

"It is a pleasure to volunteer to help TGen," DeBell said. "Although I hope to never need to be treated by TGen, I know they are there for me, anytime, for any reason. That is peace of mind."





StepNOut Chair Kathy Wills (right) with her sister Lindsey Richards

# stepNout

RUN WALK DASH

## 1,000 Participants, 31 Teams Step Out to Support TGen Pancreatic Cancer Research

TGen's 5K stepNout annually draws nearly 1,000 runners and walkers to a family-style celebration in honor and memory of loved ones at Tempe's Kiwanis Park, raising nearly \$100,000 this year for research targeting pancreatic cancer, the nation's fourth leading cause of cancer death.

And like any important family event, it takes someone special to keep it organized.

The stepNout event chair, Kathy Wills, was inspired to volunteer by TGen Physician-In-Chief Dr. Daniel Von Hoff, who treated her late mother, Ann Yturri, for pancreatic cancer, which this year will kill more

than 37,000 Americans.

While this is her first year as event chair, Wills has been involved in stepNout since its inception, closely working with the event's longtime chair and friend, Rosanna Norman, who lost her husband to pancreatic cancer.

"I got involved with the TGen Foundation after my mom died from pancreatic cancer in 2003, at age 60, 4 months after being diagnosed. I felt so hopeless watching her suffer, and I asked Dr. Von Hoff how I could get involved once she passed. He introduced me to TGen and to a group of individuals who were just

like me," Wills said.

Since its start in 2006, the first six stepNout events collectively raised more than \$500,000, supporting groundbreaking pancreatic cancer research, including a worldwide network of clinical trials — which can help patients today — organized through TGen's Pancreatic Cancer Research Team (PCRT).

The 7th annual stepNout on Nov. 4 put the event on track towards raising another \$500,000. The event was fueled by the enthusiasm and camaraderie of 31 teams, some made up of family and friends, and others organized

by corporate sponsors, including:

Phoenix NAP and Cadwell family, MidFirst Bank, and the Arizona Diamondbacks.

"It's been fun to see the event grow over time and see new people get involved in such a wonderful cause," Wills said. "I know TGen will find a way to cure this awful disease, and I think helping to fund their research sooner than later is critical. Pancreatic cancer has affected so many people close to me. I want nothing more than to wake up one day and know that anyone diagnosed with pancreatic cancer will be cured."



# KEY TO THE CURE

## Fashion Fights Back Against Women's Cancer

**F**or the third year, Saks Fifth Avenue's Key to the Cure fundraising in Phoenix was dedicated to TGen's breast and ovarian cancer research.

A blend of sophisticated fashion, food and philanthropy, Key to the Cure drew hundreds of enthusiastic supporters Oct. 19 to a runway model show at the Saks at Biltmore Fashion Park.

This year, in addition to donating 2 percent of sales made Oct. 18-21, Key to the Cure raised \$100,000 for TGen, the sole local beneficiary of the local Saks Fifth Avenue. Nationwide, Saks has donated more than \$34 million for cancer research and treatment since it began the charity events in 1999.

"The people of Saks Fifth Avenue have become true partners in TGen's efforts to find better treatments for women's cancers," said TGen Foundation President Michael Bassoff. "Empowered by their company's national commitment, employees and management of Saks at the Biltmore have not only contributed their own funding, but they have also enabled Saks shoppers to join in TGen's research efforts."

This year, breast and ovarian cancer combined will claim the lives of more than 55,000 American women.

Vicki Vaughn, who is just coming off her second year as event co-chair for TGen's Key to the Cure, sees the future of medicine at stake.

"This research is fundamental to the future of medicine by not only creating targeted, personalized treatments for those already affected, but in cancer prevention. When I first heard of TGen, I was excited to know this cutting-edge genetic research is going on right here in our community," said Vaughn, who also is co-chair of TGen's Cycle for the Cure, which raises funds for breast and ovarian cancer research.

Katie Mueller, Vaughn's co-chair of Key to the Cure, has a personal reason for backing the program.

"As a 3-year breast cancer survivor, I am committed to working with groups who wish to find a cure for breast cancer. Although I am lucky that I am a survivor, I know many women who are still fighting for their lives. I want a cure, and I believe that TGen will help to find one," Mueller said.

"I believe and trust in the TGen team. Having spent time with many of its leaders and scientists, I see a team dedicated towards a common goal: To improve treatment through personalized medicine, and ultimately to find cures for these diseases."



Key to the Cure Co-chairs  
Vicki Vaughn and Katie Mueller

