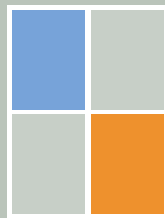


ANNUAL
REPORT
2017

team #endcancer

collaboration and exploration
lead to breakthroughs in cancer care



THE UNIVERSITY OF TEXAS

MDAnderson
~~Cancer~~ Center

Making Cancer History®

Mission

The mission of The University of Texas MD Anderson Cancer Center is to eliminate cancer in Texas, the nation and the world through outstanding programs that integrate patient care, research and prevention, and through education for undergraduate and graduate students, trainees, professionals, employees and the public.

Vision

We shall be the premier cancer center in the world, based on the excellence of our people, our research-driven patient care and our science.

We are Making Cancer History®.

Core values

Caring

By our words and actions, we create a caring environment for everyone.

Integrity

We work together to merit the trust of our colleagues and those we serve.

Discovery

We embrace creativity and seek new knowledge.

Join our community



MD Anderson Cancer Center



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Six months ago, Hurricane Harvey crashed into the Gulf Coast of Texas and carved a record-setting path of destruction through the Houston area.

Tragically, many lives were lost, more than 200,000 homes were flooded and 39,000 people were forced to evacuate. In the end, the storm resulted in damages totaling \$180 billion.

But it didn't stop the people who live here. During the storm, they assisted federal forces, local police, and fire and rescue personnel in rescuing thousands from the floodwaters. They volunteered their time and resources to shelter, clothe and feed those left with nothing. When the waters receded, they stepped up to help their neighbors recover by moving debris and water-logged furniture to the curb, and tearing out ruined drywall, carpet and flooring. They've helped rebuild homes and lives.

I hadn't yet arrived in Houston when Harvey hit, but Dr. Marshall Hicks and former colleagues shared with me stories of the heroic efforts to keep patients and the institution safe.

Having spent 20 years as a cancer surgeon, researcher, professor and hospital administrator at MD Anderson, I wasn't surprised to hear of the solidarity and sacrifice shown by our amazing employees. But I was extremely impressed and proud.

Texans learned a lot from what has happened. The region has rebounded and it is undergoing a transformation.

The same is true for the nation's top-ranked cancer center. The past year has been one filled with transition, and we've learned many things. My personal process of learning, unlearning and re-learning has been underway as I visit many areas of the institution. As a team we are learning together and improving. Our culture is changing. And we are transforming this beloved institution.

I continued to follow MD Anderson's progress over the past few years as I served as president and chief executive officer of the University Health Network in Toronto. And I continued to be inspired by the excellence of the institution's people, the world-class care provided to patients, the groundbreaking research conducted by leaders in our field, the innovative approaches to cancer prevention that is saving lives, and the outstanding educational opportunities being offered to our future leaders.

In the pages of this report, you'll read about some of the institution's highlights from the past year. They're quite impressive.

MD Anderson's first full-time president, R. Lee Clark, M.D., believed working together to provide comprehensive multidisciplinary care was the best way to make significant gains against cancer. His vision continues today, not only in our approach to treating patients, but also in our team-science research, outstanding education and training resources, and our all-encompassing strategy for cancer prevention.

The reality is there is no team like MD Anderson. And there is no group of people I would rather work with as we join forces with our patients and their loved ones in our fight to end cancer. It is together that we are Making Cancer History®. We are team #endcancer!



A handwritten signature in black ink that reads "Peter Pisters". The signature is fluid and cursive, with the first name "Peter" and last name "Pisters" clearly legible.

Peter WT Pisters, M.D.
President

It really has been a remarkable year – full of challenges, changes, and accomplishments. And, we witnessed new levels of determination and strength as our teams remained dedicated to our mission of eliminating cancer.

As an institution, we came to a point of reflection: Were we going to hang on to the status quo, or were we going to embrace the opportunity to change and evolve? The response was overwhelming: we couldn't wait. Our employees wanted to transform our beloved organization – together. They recognized that this would make us a stronger MD Anderson moving forward, and ultimately enable our next president to take us to even greater heights.



We laid out three priorities to guide us through this transformation: turn the tide financially, connect our people to our decisions, and build a team-based culture at all levels of the organization.

The people of MD Anderson were the key element that helped us on this journey. Our leaders empowered our teams, allowing them to be a part of the solution. By pushing decisions into the organization, we created accountability for shared success and increased engagement by letting our teams solve problems themselves. Through frequent communication and a focus on transparency, we actively shared what was happening at all levels of the organization so our employees could connect their efforts to the bigger picture. Finally, we identified near-term priorities on which to focus our energy, allowing us to make meaningful progress toward our shared goals.

This culture change was validated during the events of Hurricane Harvey. Teamwork, empowerment, and local decision-making were evident as our teams quickly adapted to an evolving situation. Our employees demonstrated just how resilient they are, working together to safely care for our patients and for each other. During and after the events of Hurricane Harvey, we saw a tremendous outpouring of support from across Texas and the nation – a testament to the cherished role our employees play and the strength of MD Anderson's reputation as a leader in cancer care.

The accomplishments of this year are due to our strengths – the excellence of our people, our culture of caring and teamwork, and our commitment to our mission. These traits are the bedrock of who we are at MD Anderson. As we begin a new chapter in our history, these strengths will remain our foundation and help us continue to make a difference in the lives of cancer patients – both today and tomorrow.

It was the privilege of a lifetime to serve MD Anderson as president ad interim, and I look forward to seeing us thrive as we move forward as one team – one MD Anderson.

A handwritten signature in black ink that reads "Marshall E. Hicks". The signature is written in a cursive, slightly stylized font.

Marshall E. Hicks, M.D.
Division Head, Diagnostic Imaging

Outstanding leadership during MD Anderson's first 76 years

Peter WT Pisters, M.D., became president of MD Anderson in December 2017. He's the fifth person to fill that role full time in the institution's rich 76-year history.

Through their leadership, these men took MD Anderson from a staff of one to the world's foremost center focused on cancer research, patient care, prevention and education.



Ronald DePinho, M.D.
President, 2011-2017

Ronald DePinho, M.D., MD Anderson's fourth full-time president, is internationally recognized for basic and translational research in cancer, aging and age-associated degenerative disorders.

During his tenure, he launched MD Anderson's ambitious Moon Shots Program™.

DePinho continues his research in the department of Cancer Biology.

John Mendelsohn, M.D.
President, 1996-2011

During his presidency, John Mendelsohn, M.D., saw the institution more than double in size and launched MD Anderson's largest fundraising campaign, Making Cancer History®: The Campaign to Transform Cancer Care.

Mendelsohn remains on the MD Anderson faculty as the co-director of the Sheikh Khalifa Bin Zayed Al Nahyan Institute for Personalized Cancer Therapy.

Charles A. LeMaistre, M.D.
President, 1978-1996

Charles A. "Mickey" LeMaistre, M.D., took the reins at MD Anderson after serving seven years as chancellor of The University of Texas System.

As one of the physicians on the first U.S. Surgeon General's Advisory Committee on Smoking and Health, which in 1964 issued its landmark report identifying cigarettes as a major health hazard, LeMaistre was dedicated to cancer prevention.

He established the cancer prevention program at MD Anderson, developing it into an international model of research and service initiatives that advanced the science and application of cancer prevention.

LeMaistre passed away Jan. 28, 2017, at the age of 92.

R. Lee Clark, M.D.
Director, 1946-1968
President, 1968-1978

Though his official title changed, R. Lee Clark, M.D., served as a chief administrator of a University of Texas System institution longer than anyone in the system's history.

During his 32-year tenure, Clark was an early advocate of the team approach to treating cancer patients. After being designated one of the first three comprehensive cancer centers in the U.S. under the National Cancer Act of 1971, MD Anderson was the prototype for comprehensive cancer facilities throughout the world.

A total team effort

In the past year, teamwork has played a large part in the lives of those who call the Houston area home. Its power shone bright during the dark days of Hurricane Harvey, as many made their way through the floodwaters to help bring stranded strangers to safety. Others worked tirelessly alongside fellow volunteers to make sure those forced from their homes and into shelters had dry clothes to wear and something good to eat.

At MD Anderson, doctors, nurses and staff showed the same sacrifice and dedication to patients and each other during and after the storm. More than 1,000 employees remained on-site to ensure care was continuous for approximately 530 inpatients, along with some 300 of the patients' family members. Approximately 35% of the institution's employees were severely impacted by the storm. To help them cope and recover, hospital leaders set up resources such as on-site child care, free rideshare service and financial assistance for eligible employees displaced from their homes.

MD Anderson President Peter WT Pisters, M.D., had been named the sole finalist to lead the cancer center the day before Harvey hit. As he watched the destruction unfold and the resilience with which people responded, he was reminded of what a special place MD Anderson is. "In good times and in bad, people make the difference, and in the face of this epic storm, MD Anderson's people did more than that," he noted. "They, along with their friends and neighbors across the region, helped restore hope."

Less than two months later, the city came together to cheer on the Astros during the team's thrilling run to the franchise's first World Series championship. Many of the players said they were inspired by the strength and humanity shown by so many during and after Harvey.

At MD Anderson, teamwork is at the heart of every effort made to treat patients, improve care and prevention, conduct research that leads to a better understanding of the disease, and educate tomorrow's cancer experts. Multidisciplinary care. Team-science approach. Interdisciplinary thinking. The Moon Shots Program™. Strategic business alliances. MD Anderson Cancer Network®. These are all examples of how doctors, scientists, specialists and staff at MD Anderson are working together to end cancer. This year's annual report underscores the power and importance of collaboration at the institution. That isn't unique to 2017, but it is a big part of what makes the nation's leading cancer center so special.



Teamwork leads to Innovative Treatments



A Colorectal Cancer Moon Shot team has developed a personalized vaccine to expose treatment-resistant tumors to immune attack

Jerry “JT” Burk was facing some tough odds. His colorectal cancer had metastasized and spread to his lungs, and the side effects of his chemotherapy treatment were keeping him from traveling and doing many of the things he enjoys most. But an innovative clinical trial designed by a team of MD Anderson doctors and scientists has helped the 66-year-old architect get his life back. The combination trial double-teams Burk’s disease by pairing a customized vaccine that teaches his immune system to target his cancer together with an immunotherapy drug that enables his T cells to continue the attack.

Burk joined the study, which opened in late 2016, on the recommendation of his doctor, Scott Kopetz, M.D., Ph.D., a professor of Gastrointestinal Medical Oncology and co-leader of the Colorectal Cancer Moon Shot™.

The game plan: Extensive analysis of tumors from Burk and other patients enrolled in the study reveals 10 mutations unique to each person. Principal investigator Michael Overman, M.D., associate professor of Gastrointestinal Medical Oncology, and Greg Lizée, Ph.D., associate professor of Melanoma Medical Oncology, then create a vaccine against those targets. At first, they were only looking into the vaccine’s ability to trigger an immune response, but this past September, a drug named Keytruda was added to the regimen. Essentially, the drug frees the immune system to attack tumors by blocking PD1, a protein on T cells that shuts down immune response. Read more about the clinical trial on Page 13.

CLOCKWISE FROM TOP LEFT:

THE PRINCIPAL INVESTIGATOR
Michael Overman, M.D., associate professor of Gastrointestinal Medical Oncology

THE PHYSICIAN ASSISTANT
Jamie Faber

THE SCIENTIST
Greg Lizée, Ph.D., associate professor of Melanoma Medical Oncology

THE DOCTOR
Scott Kopetz, M.D., Ph.D., a professor of Gastrointestinal Medical Oncology and co-leader of the Colorectal Cancer Moon Shot



Jerry “JT” Burk received a tailor-made vaccine that conditions his immune system to attack his cancer.

photo by Wyatt McSpadden



Ahndiep P. Tran is one of several radiation therapists who will use the MR-linac to deliver precise MRI-guided treatments to patients.

Radiation in real time

MR-linac merges MRI technology with a linear accelerator, allowing doctors to treat tumors with greater precision

By Ronda Wendler photos by Nick de la Torre

Up. Down. Sideways and around.

When people breathe, there's movement inside their bodies.

Organs, tissues and even cancer patients' tumors move around all the time," says Geoffrey Ibbott, Ph.D., professor of Radiation Physics at MD Anderson. "It's not unusual for a tumor to shift an inch or slightly more while a patient breathes in and out."

In the past, this movement has confounded doctors' ability to pinpoint a tumor's exact location before zapping it with high-energy beams of cancer-killing radiation.

But a new device being tested at MD Anderson promises to overcome that obstacle.

The MR-linac, as it is called, merges a magnetic resonance imaging (MRI) machine and a linear accelerator into a single device. The MRI machine relays high-quality, real-time images of tumors as they're blasted with radiation beams from the linear accelerator.

MRI machines and linear accelerators have been used in cancer therapy for years. What's new is the two are now one.

"For the first time, this new technology lets us see where the radiation dose is being delivered, as it's being delivered," Ibbott explains. "The live images keep the radiation directly on target during the treatment."

If a tumor moves out of the targeted radiation area when a patient inhales, the MR-linac automatically turns off the radiation beam. When the patient exhales and the tumor returns to its original position, radiation automatically resumes. This on-off treatment spares healthy tissue and allows doctors to deliver a more powerful dose of radiation.

"We used to treat the entire area where the tumor might travel during a normal breathing cycle," he explains. "But with this new technology, radiation is delivered only when the tumor is located where it should be."

Doctors watch treatment sessions on a video monitor. If what they see causes them to tweak the patient's treatment plan, the MR-linac can refine the target and reconfigure the radiation dose in less than two minutes, all while the patient is on the table. Ibbott calls this "radiation on the fly."

"Tailoring radiation to patients' tumors in real time – it's the ultimate in personalized medicine," he says.

THE ROLE OF EDUCATION

Medical dosimetrists work closely with the radiation oncology team — radiation oncologists, medical physicists, and radiation therapists — to create customized radiation treatment plans to target cancer while sparing normal tissue. The Program in Medical Dosimetry at MD Anderson's School of Health Professions trains students with the professional skills of dose calculation, treatment design and quality assurance through intensive classroom and clinical education. In FY17, there were 39 students enrolled in the program, which graduated 19 this past August.



THE MR-LINAC TEAM

From left:

Geoffrey Ibbott, Ph.D., professor of Radiation Physics, supervises all of the physics, dosimetry, commissioning and safety aspects of the MR-linac program.

Hannah Lee, Ph.D., a postdoctoral fellow in Radiation Physics, works with Ibbott to provide clinical medical physics services, including quality assurance procedures, commissioning and calibration of the device.

Jihong Wang, Ph.D., professor of Radiation Physics, is a medical physicist who supervises Radiation Physics' MRI safety program and is an expert in assessing and improving image quality.

Scan, plan and treat

In traditional radiation therapy, doctors complete what's called a "simulation" one week before treatment begins. The patient lies on a table in the treatment position while imaging scans identify the tumor's exact location and characteristics. Tiny ink marks are placed on the patient's skin to guide radiation beams during the upcoming session.

The radiation oncologist spends about a week working with medical physicists and dosimetrists (see sidebar) who create an individualized treatment plan, including radiation dose and number of treatments based on the tumor's size, location and type.

"All the while we're assuming that nothing is changing during this week prior to treatment," Ibbott says. "But in fact, things are likely changing."

While doctors are crafting a treatment plan, the patient's tumor may be growing, especially if it's aggressive. Conversely, it may be shrinking if chemotherapy or other anti-tumor treatments are working. Shifts in the patient's body weight, or fullness or emptiness in the stomach, bladder or bowels may also reposition a tumor.

"With the MR-linac, we can lock onto the tumor during radiation beam delivery," Ibbott says, "even if it's moving and changing shape, size or location before our eyes."

This solves a longstanding, unmet need, he says, by allowing doctors to clearly see the tumor during treatment rather than relying on pre-treatment images.

Magnets and metal

Before the MR-linac's debut, scientists wouldn't dare place an MRI machine near a linear accelerator.

"That's because MRI technology uses a powerful magnet to produce high-quality images," Ibbott explains. "The strong magnetic field would pose an obvious safety hazard in the presence of an all-metal linear accelerator, with parts that could fly across the room at a dangerous speed toward the magnet, harming everything in their path. Also, the magnetic field would disturb the operation of the linear accelerator and degrade tumor images. Magnets and metal don't mix."

The MR-linac's developers created a simple but elegant workaround to bypass this obstacle. Here's how Ibbott explains it:

"Think of the MRI machine as a hollow paper-towel tube, with a magnetic metal coil wrapping around it. The patient slides inside the tube where imaging takes place."

The MR-linac splits the metal coil in half, creating a gap in the middle where the patient is positioned. Radiation passes through this de-magnetized "safety zone" and images are created without distortion.

As an extra precaution, the linear accelerator portion of the MR-linac is shielded with proprietary technology that allows it to operate unaffected by the MRI machine's magnetic field.

"These adjustments enable these two machines that previously opposed each other to now work together for the benefit of patients," Ibbott says.

Leading the way

MD Anderson is the first clinical site in the world to install the MR-linac, other than the University Medical Center in Utrecht, The Netherlands, where it was designed with input from MD Anderson and several other institutions. Today, seven cancer centers worldwide have MR-linacs in place.

The technology has not yet been approved by the Food and Drug Administration. It's too new, says Clifton David Fuller, M.D., Ph.D., who is teaming with Ibbott to conduct a clinical trial of the MR-linac at MD Anderson. Fuller, an associate professor of Radiation Oncology, supervises the patient treatment arm of the trial, while Ibbott directs the technical aspects including equipment installation and operation, calculating radiation dosages, and ensuring safety precautions are in place.

Data gathered at MD Anderson and other trial sites will provide the machine's manufacturer, Elekta, with information needed to pursue FDA approval.

"Half of all cancer patients undergo radiation treatment," Fuller says. "All could potentially benefit from this game-changing technology."

Unraveling a complex disease with a simple blood draw

Liquid biopsies are an increasing area of focus for MD Anderson researchers and caregivers

By Ron Gilmore

Blood tests are routine and simple ways to diagnose a number of medical conditions, but until recently, such simplicity hasn't applied to a complex disease like cancer.

Researchers and clinicians at MD Anderson are studying and implementing uses for liquid biopsies – blood tests that detect telltale proteins in the blood that signal the presence of cancer. Liquid biopsies may one day lead to catching cancer early, when it's easiest to treat.

Take melanoma for example. Anthony Lucci, M.D., professor of Breast Surgical Oncology and Surgical Oncology, is known for his work in using circulating tumor cells (CTCs) to detect early-stage breast cancer. CTCs are primary tumor cells that have shed into the blood or lymphatic system and can lead to additional tumor growth or metastasis.

Lucci led the first and largest study in this field, with results published in *Lancet Oncology* back in 2012. Since then, he has discovered that the same CTCs found in breast cancer patients also are present in melanoma. In November, he presented finding that revealed a connection between CTCs and relapse in stage IV melanoma patients. This discovery points to liquid biopsy's potential to predict which patients have a high risk of disease progression.

“Optimal management of stage IV melanoma patients remains a challenge since – in spite of promising emerging therapies – many patients develop disease resistance,” says Lucci. “This study, designed to determine if CTCs are associated with relapse, detected CTCs in approximately 40% of advanced-stage melanoma patients.”

Lucci's group also is studying the significance of CTCs in earlier stages of melanoma, and hopes to have data available in 2018.

CTCs are just one type of liquid biopsy being studied at MD Anderson. Others include work with:

- Cell-free tumor DNA (ctDNA) – derived from dying tumor cells that release small pieces of DNA into the bloodstream or other body fluids
- Blood plasma – including proteins, antibodies and metabolites
- Exosomes – tiny virus-sized particles released by cancer cells that contain DNA, RNA and other proteins

In the past year, MD Anderson entered into a multi-year collaboration with Guardant Health, a major player in liquid biopsy development. The partnership tailors Guardant's technology to fit MD Anderson's patient population.



Carolyn Hall, Ph.D., assistant professor of Breast Surgery-Research, and Anthony Lucci, M.D., professor of Breast Surgical Oncology and Surgical Oncology, are using circulating tumor cells to detect cancer. photo by Adolfo Chavez III

MD Anderson is working closely with Guardant to expand a highly specialized CLIA-accredited lab (CLIA labs comply with federal standards known as the Clinical Laboratory Improvement Amendments that regulate all clinical laboratory testing on humans), and pre-CLIA labs, where Guardant's digital sequencing technology will be used to create disease-specific assays that will be transferred for use in the CLIA-accredited lab to help detect cancers early and guide treatment.

“Liquid biopsies are far less invasive than traditional biopsies. This benefits our patients and provides significantly enhanced analysis of samples,” says Stanley Hamilton, M.D., who heads MD Anderson's Pathology and Laboratory Medicine division and the clinical lab component of the Guardant collaboration.

Other leaders of MD Anderson's liquid biopsy efforts include:

- Scott Kopetz, M.D., Ph.D., associate professor of GI Medical Oncology, is studying tiny traces of cancer called minimal residual disease (MRD) to guide treatment of colorectal cancer, as well as monitoring MRD to assess disease recurrence.
- Ignacio Wistuba, M.D., chair of Translational Molecular Pathology, and Raja Luthra, Ph.D., professor of Hematopathology, are working on pre-CLIA development of liquid biopsy approaches to streamline their transfer to the CLIA lab for potential use for multiple cancers.
- Samir Hanash, M.D., Ph.D., professor of Clinical Cancer Prevention, heads a five-year multi-institutional study launched three years ago to develop a liquid biopsy for the early detection of lung cancer. His study, which uses blood plasma components, involves taking blood samples from up to 30,000 heavy smokers for evaluation.
- Anirban Maitra, M.B.B.S., professor of Translational Molecular Pathology and scientific director of the Sheikh Ahmed Center for Pancreatic Cancer Research, is exploring the link between exosomes and pancreatic cancer. He's conducted a study to learn how pancreatic cancer patients will respond to particular therapies and what factors are involved in the disease's recurrence.
- Raghu Kalluri, M.D., Ph.D., chair of Cancer Biology, is studying exosomes and, in particular, a protein encoded by the gene glypican that is present on cancer exosomes. He believes exosomes may have potential as a liquid biopsy for the early detection of pancreatic cancer, as well as a way to deliver therapy.



Architect Jerry "JT" Burk joined a very unique clinical trial after his colorectal cancer spread to his lungs. photo by Wyatt McSpadden

Customized vaccines deliver extremely personalized therapy

A Colorectal Cancer Moon Shot team is exposing treatment-resistant tumors to an immune attack

By Scott Merville

Immunotherapy has taken hold as an effective treatment for a variety of advanced cancers, but so far, colorectal cancer has stubbornly resisted.

However, research led by MD Anderson shows that resistance could be struck down. The recent international clinical trial helped expose a weak spot that leaves about 5% of colorectal tumors receptive to immune attack. (see the related story on Page 14)

Now, a team in the Colorectal Cancer Moon Shot™ of MD Anderson's Moon Shots Program™ is expanding on this discovery to benefit even more patients.

Michael Overman, M.D., associate professor of Gastrointestinal Medical Oncology, led a Phase I clinical trial testing whether a special form of vaccination can recruit the immune response that's missing in most patients.

INNOVATIVE TREATMENT FROM THE COLORECTAL CANCER MOON SHOT

“Each patient is different, so we're developing a personalized vaccine based on specific targets we identify on a patient's tumor,” Overman says. “The initial question was ‘can we do this in a feasible amount of time for our patients?’ The pilot study showed we could.”

Overman and colleagues are combining these tailored vaccines with an approved drug that protects and boosts immune response to see if the one-two punch can help more colorectal cancer patients.

At first, analyzing a patient's surgically removed tumor and developing a vaccine against 10 specific targets on the tumor took nine months, says Greg Lizée, Ph.D., associate professor of Melanoma Medical Oncology, whose research focuses on the immune system. Now the time from surgery to vaccine treatment is three months and falling.

The trial opened in November 2016. By late summer, 17 patients with metastatic colon cancer had received customized vaccines. Several had their tumors shrink or remain stable temporarily, says Scott Kopetz, M.D., Ph.D., a professor of Gastrointestinal Medical Oncology and co-leader of the moon shot.

In September, the team added to the vaccine regimen a drug called Keytruda that supports the immune system's attack on tumors.

The rationale for the combination trial is that the vaccine will prime white blood cells called T cells to find and attack the targets on a patient's tumor, and Keytruda will keep the response going by blocking an off switch on those T cells that can shut down immune response.



A genetic flaw allows immunotherapy to help certain patients with colorectal cancer

A practice-changing clinical trial conceived and led by MD Anderson investigators showed that metastatic colorectal cancer patients with a specific genetic defect respond well to a common cancer immunotherapy drug.

About 5% of patients with colorectal cancer that has spread to other organs have a flaw in the genes that repair their tumors' cancer-causing DNA damage. Their tumors harbor an increasing number of DNA mutations, which attract the attention of immune system T cells that hunt down and destroy cancer cells.

The findings suggested that immunotherapy, a treatment in which the immune system is used to attack cancer cells, could help these patients, says Michael Overman, M.D., associate professor of Gastrointestinal Medical Oncology.

"Traditional chemotherapy and targeted therapies have little effect for patients with these tumors," he says, "so hopes for immunotherapy's effectiveness were high."

Overman and Scott Kopetz, M.D., professor of Gastrointestinal Medical Oncology, recommended a clinical trial for these patients to Bristol-Myers Squibb, the company that makes the immunotherapy drug nivolumab, known commercially as Opdivo.

The international trial that resulted showed tumors shrank in 23 of the 74 patients enrolled. In the other 51, disease progression halted for at least 12 weeks.

"That level of response and disease control is unheard of in these heavily pretreated patients, outside of frontline treatment," Overman says.

The Food and Drug Administration (FDA) approved nivolumab as second-line therapy for these patients this past July, and clinical trials are underway to test the drug as frontline treatment. The FDA also approved another immunotherapy drug, Merck's pembrolizumab, known commercially as Keytruda, for patients with tumors harboring this defect regardless of cancer type. Based on early results, the National Cancer Center Network recommended in November the testing of all metastatic colorectal cancer patients for the genetic defect.

Getting his life back

For Jerry "JT" Burk, a 66-year-old architect from Houston and one of Kopetz's patients, the trial came along when his colorectal cancer had spread to his lungs and he was almost out of options. Chemotherapy slowed the disease, but its side effects were taking an increasing toll on his quality of life.

"Getting off chemo and going on the trial in November (2016), I felt almost normal," Burk says. "I could do things like travel, because my immune system was no longer compromised (by the chemo). There were few side effects, and tumor growth slowed."

In March, his tumor growth increased, and he stepped off the trial and onto chemotherapy again.

"You have to think long and hard when you've been on chemo so long and then you back off of it and get your life back," Burk says. "It's a hard decision. But it's a bridge, and I'm hopeful."

The bridge worked, and in September, Burk moved on to the second phase of the immunotherapy trial.

Burk is doing well, Kopetz says, but it's too early to report overall results for the seven patients who had customized vaccines developed initially and have since proceeded to the combination trial.

How vaccines work

Some vaccines prevent cancer by destroying invading toxins or foreign substances that cause infection. For example, vaccines for the hepatitis B virus prevent liver cancer and vaccines for the human papillomavirus prevent cervical, anal and some throat cancers.

Lizée and colleagues are developing a different type of vaccine called a therapeutic vaccine, which triggers an attack against established cancer. Earlier versions of such vaccines largely failed because they targeted single invading substances that were not mutated, so T cells that attack invaders often ignored them, he says.

The team identifies "neoantigens" – targets that are unique to the tumor, using an exhaustive process made possible by recent technological advances.

Ten such targets are found for each patient. A vaccine against all 10 neoantigens is developed and given on a set schedule during the clinical trial, along with Keytruda.

Of the first 100 neoantigens identified for 10 patients, 98 were unique to the individual patients.

"Every patient is his own universe," Lizée says. "This might be the ultimate in personalized therapy."

CAR T-cell therapy shows significant remission rates for those with aggressive B-cell lymphoma

A study involving the recently approved CD19-targeting chimeric antigen receptor (CAR) T-cell therapy shows that 42% of patients with aggressive large B-cell lymphoma remained in remission at 15 months following treatment with axi-cel.

The study, named ZUMA-1, also reported measurable responses in 82% of patients and complete responses in 54%. Fifty-six percent were alive at 15 months following therapy, with some remaining cancer free two years post-treatment.

The findings were reported in the Dec. 10 online issue of *The New England Journal of Medicine*, and presented at the American Society of Hematology's (ASH) Annual Meeting and Exposition this past December. They resulted from a 22-institution study led by Sattva Neelapu, M.D., professor of Lymphoma and Myeloma at MD Anderson, and Frederick Locke, M.D., vice chair and associate member of the Department of Blood and Marrow Transplant and Cellular Immunotherapy at Moffitt Cancer Center.

"With the FDA's recent approval of this therapy, we believe this is a major advance in the treatment of patients with relapsed or refractory large B-cell lymphoma, and is likely to save or prolong lives of many patients," says Neelapu. "This study demonstrated that axi-cel provides remarkable improvement in outcomes over existing therapies for these patients who have no curative options."

MD ANDERSON-LED CLINICAL TRIAL

Scope: 22 institutions

Treatment: CD19-targeting CAR T-cell therapy

Results: 42% of patients with aggressive large B-cell lymphoma remained in remission after 15 months

In CAR T therapy, a person's own T cells – disease-fighting immune cells – are removed and sent to a lab where they are genetically re-engineered to produce chimeric antigen receptors (CARs) on their surface. CARs are proteins that allow T cells to recognize cancer.

The CAR T cells are then multiplied in the laboratory until there are millions. Next, they're sent to the hospital and infused back into the patient's bloodstream. These "attacker" cells not only recognize and kill cancer cells, but they may remain in the body long after the infusion has been completed and guard against cancer's recurrence.

The study, which began in April 2015, administered axi-cel to 108 patients who had failed prior chemotherapy and autologous stem cell transplantation. In some cases, the patients who had received chemotherapy were too far progressed to undergo stem cell transplantation and were placed on the trial following chemotherapy.

"This is the first FDA-approved gene therapy to treat adult lymphoma," says Locke. "Many patients' lymphoma tumors melted away within a month. The long-term follow-up results of the ZUMA-1 trial show that axi-cel remissions can last for years, and these are patients who did not respond to chemotherapy."



Emily Dumler's non-Hodgkin's lymphoma was put into remission by CAR T-cell therapy. photo by Adolfo Chavez III

Proud to be a clinical trial trailblazer

In 2013, Emily Dumler was diagnosed with a type of non-Hodgkin's lymphoma called diffuse large B-cell lymphoma. After chemotherapy and an autologous stem cell transplant failed to stop her cancer, she qualified for the final spot in Sattva Neelapu's study of CAR T-cell therapy for lymphoma.

"I felt grateful just to have another option – a really viable one that my doctors were excited about," Dumler says. "And for the first time in a long time, I actually felt hopeful."

Dumler was only the second patient at MD Anderson and the third in the world to receive CAR T cells to treat non-Hodgkin's lymphoma on the multicenter clinical trial. She battled what is known as cytokine release syndrome and neurological side effects, both common to CAR T-cell therapy, but quickly recovered. A month after her infusion, tests showed her cancer was in complete remission. Two and a half years later, she remains cancer free.

The experience not only changed Dumler's life, it changed the way she views clinical trials.

"Before I was on a clinical trial, I felt sorry for patients participating in them. I considered clinical trials a last-ditch effort," Dumler says. "But knowing that I was a trailblazer in this area makes me feel so proud. Clinical trials are the future of medicine. And they really are how new treatments become available."

5 years in, the Moon Shots Program is primed for continued progress

Discoveries are being turned into patient care, thanks to a unique infrastructure and team-science approach

It's been five years since MD Anderson launched its Moon Shots Program™, a collaborative effort to more quickly turn scientific discoveries into clinical advances that save patients' lives.

Established in the fall of 2012, the program already has yielded notable discoveries across the spectrum of cancer care, including prevention, early detection and treatment, and has inspired philanthropic support totaling more than \$453.5 million.

“The Moon Shots Program is an extraordinary platform for team-based science that has inspired donor support for transformative research that otherwise may not have been funded,” says Peter WT Pisters, M.D., incoming president of MD Anderson. “We have an obligation to lead in cancer prevention and control while working to accelerate improvements in patient outcomes – all of which is possible through the Moon Shots Program and MD Anderson’s commitment to Making Cancer History®.”

First inspired by MD Anderson’s fourth full-time president Ronald DePinho, M.D., the program established focused, multidisciplinary teams of clinicians and researchers to forge comprehensive approaches to improving the lives of patients and reducing cancer mortality. Each component of the program undergoes regular internal and external peer-review to prioritize and direct ongoing efforts, focusing on those most likely to have significant, rapid impact for patients.

Beginning with six Moon Shots™, the program was expanded in 2015 for a total of 13 disease-focused initiatives. The Moon Shots Program also established 10 platforms that provide unique expertise, technical support and novel infrastructure to support the program’s team-science approach and accelerate the translation of data and discoveries for patients’ benefit.

MD Anderson’s Moon Shots Program also served as an inspiration for the national Cancer Moonshot, which works toward the same goal, combining innovation and collaboration to make therapies available to more patients on a national scale. Two members of MD Anderson’s faculty serve on the Blue Ribbon Advisory panel to the national Cancer Moonshot, providing guidance and recommendations to the national effort.

Novel therapeutic approaches highlight program achievements

“Our singular vision of improving patient care has catalyzed our teams toward novel discoveries that, quite simply, would not have occurred without such focus,” says Giulio Draetta, M.D., Ph.D., co-leader of the Moon Shots Program, senior vice president, Discovery and Platforms and chief academic officer ad interim. “In five years, we have made notable advances for patients – most of which would not have been possible without the Moon Shots Program.”



“Progress against cancer has come in small steps, but the time is ripe to lengthen our strides, pick up the pace and gather ourselves to make a giant leap for patients.”

— Ronald DePinho, M.D.,
former president of MD Anderson,
during the 2012 announcement of
the Moon Shots Program

Some of those accomplishments include:

- The Institute for Applied Cancer Science (IACS), a moon shots platform, has advanced a novel drug from discovery to clinical trials for patients with acute myeloid leukemia (AML) in collaboration with the Myelodysplastic Syndromes and Acute Myeloid Leukemia Moon Shot™. The drug, which disrupts energy production in cancer cells, is now being tested in clinical trials in patients with solid tumors. The entire development pipeline, from laboratory discovery through clinical trials, has been managed exclusively by the Moon Shots Program, which made possible the accelerated translation to the patient care setting in fewer than five years. IACS is advancing multiple novel drugs toward the clinic, with five new compounds expected to enter clinical trials in 2018.
- The Lung Cancer Moon Shot™ has identified and resurrected an abandoned targeted therapy, poziotinib, for treating a rare group of lung cancer patients with specific treatment-resistant mutations. These patients, who previously had no effective treatment options, are seeing significant response rates in current Phase II clinical trials. The pre-clinical discovery, testing, and current clinical trials were catalyzed by multidisciplinary efforts and platform support with the goal of addressing this unmet need in lung cancer patients. (Read more on Page 21)
- The Melanoma Moon Shot™ has opened clinical trials to evaluate pre-surgical treatment for high-risk melanoma patients who otherwise would undergo surgery. Pre-surgical therapy is a standard practice in other cancers, such as breast, but previously wasn't feasible in melanoma because of a lack of active therapies. These trials will advance insights into the best approaches to treating patients after surgery. Additionally, using moon shots platforms, deep analyses of patient samples from these trials are being carried out to better understand why treatments work for some, but not all melanoma patients, and guide new trial development.

Since 2012, the Moon Shots Program has launched new approaches in surgery, targeted therapies, drug combinations and more.

Innovation, collaboration and the institution's global reach have been key to the enormous impact being made on the lives of cancer patients. As the program's research engines, the moon shots platforms provide scientific expertise and specialized infrastructure to support the program.

Here are some highlights of the progress made so far ...

13
moon shots

> \$453.5 million in total philanthropic support

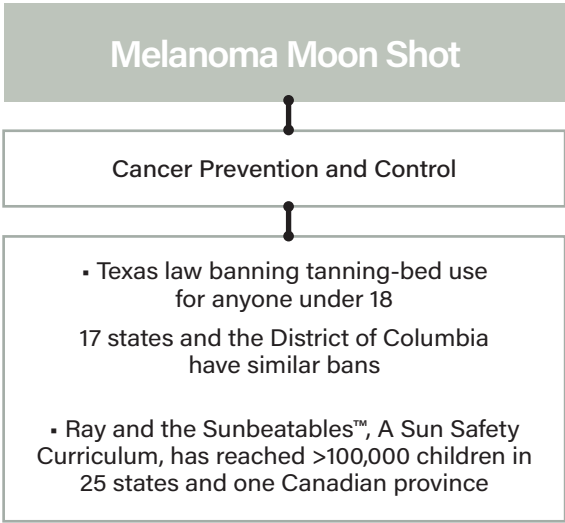
>15
clinical trials

for ovarian cancer as a result of the Ovarian Cancer Moon Shot

>100
clinical trials
that benefit from the immunotherapy platform

150
clinical studies
at MD Anderson that are being accelerated as a result of the Moon Shots Program

10
research platforms



Solid foundation for increasing impact

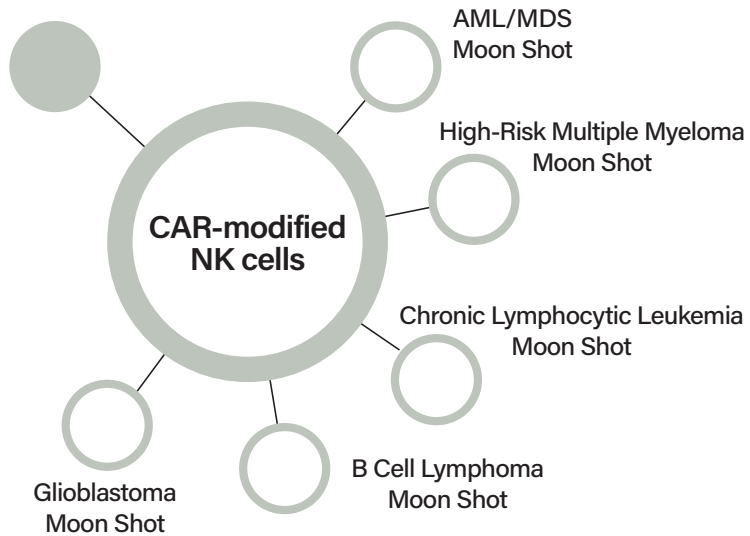
“We have made great strides in five years, but perhaps the most important achievement is the foundation upon which current and future program discoveries will be made and the lives that will be saved,” says Andy Futreal, Ph.D., chair of Genomic Medicine and co-leader of the Moon Shots Program. “With this infrastructure, our tremendous teams of scientists, together with collaborators here and abroad, are learning more each day from the very individuals we remain committed to helping – our patients.”

The research platforms, which work across the moon shots, continue to move therapies to the clinic and evaluate patient data to refine clinical strategies, ensuring patients receive the best care specific to their cancer.

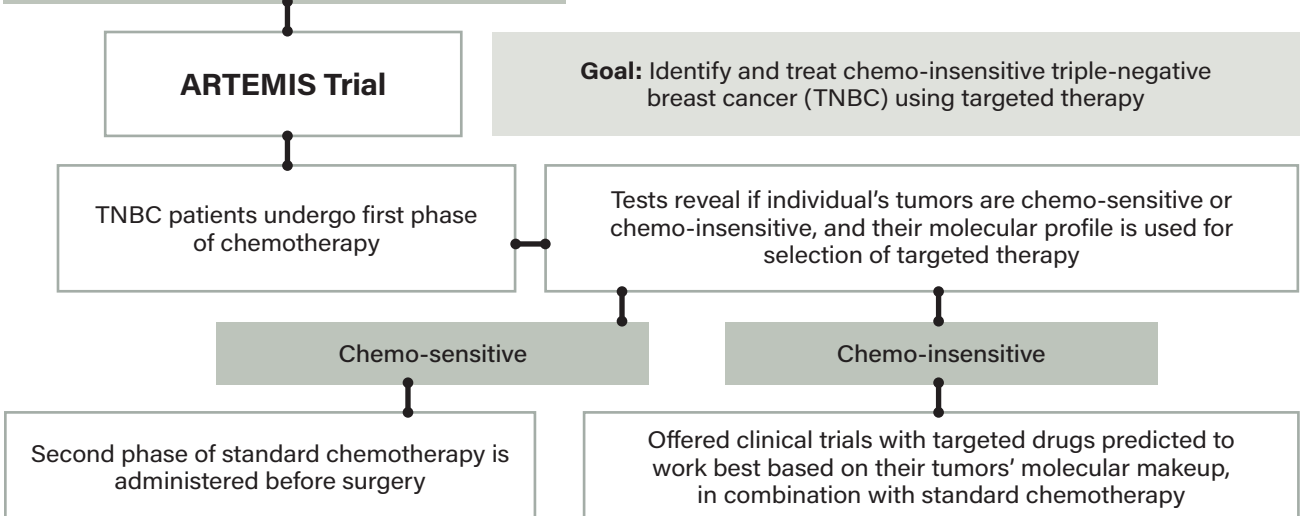
The APOLLO platform is performing large-scale analysis of patient samples over time, generating novel data to better understand how tumors develop resistance to certain treatments, making cancers more predictable and easier to treat. This platform is harnessing the power of big data and sharing information across disciplines to improve care for patients of all cancer types.

Adoptive Cell Therapy

The platform is genetically re-engineering the immune system’s natural killer (NK) cells to allow them to recognize and attack cancer. Five moon shots have current and planned clinical trials using these modified cells.



Breast Cancer Moon Shot



Next steps: Use what is learned to design more clinical trials, and discover the best drugs for each TNBC subtype

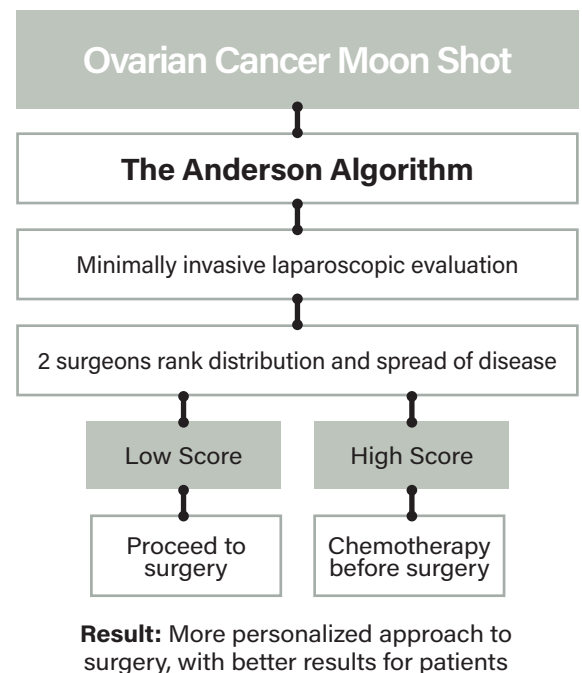
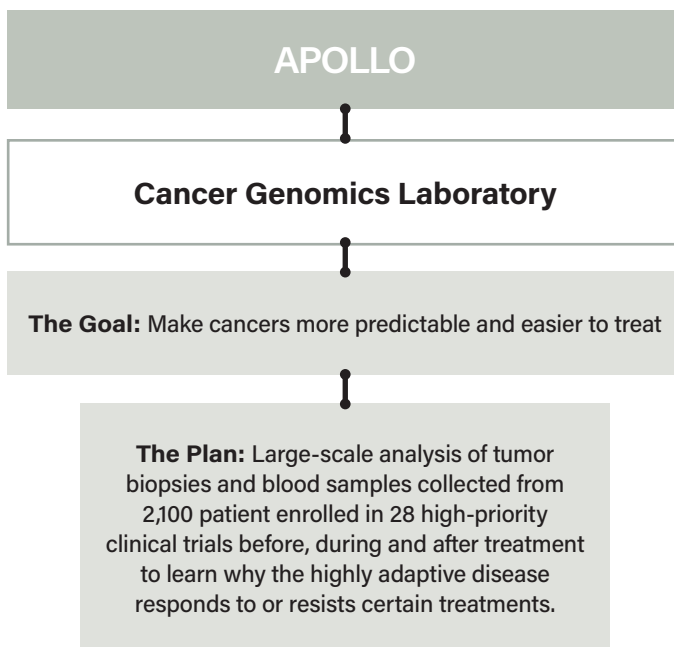
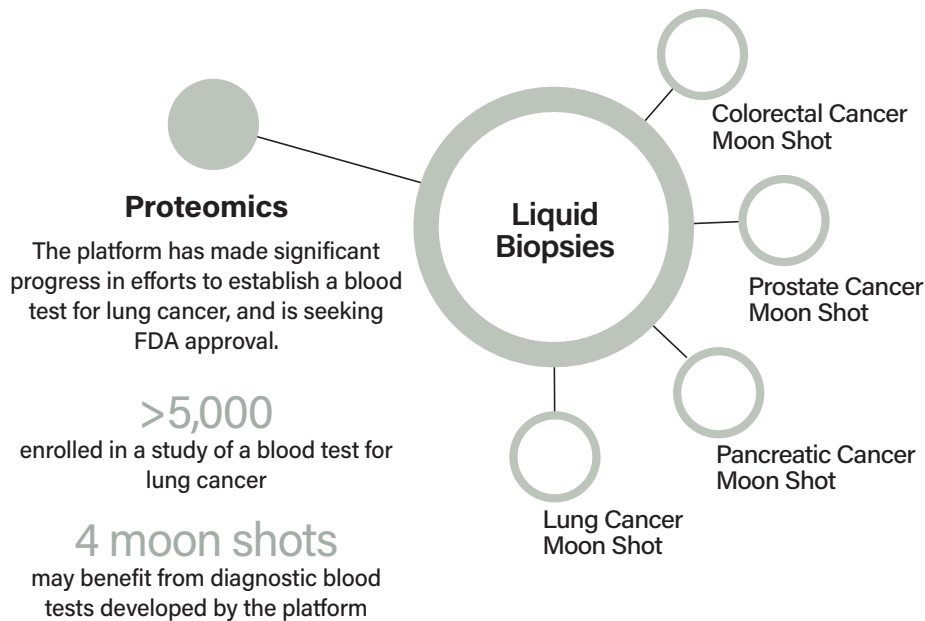
Additionally, the immunotherapy platform continues advancing immune-based therapies to make this game-changing treatment available to more patients.

Currently, there are more than 150 clinical studies at MD Anderson that are being accelerated as a result of the Moon Shots Program, investigating novel drug compounds as well as new approaches to improve the effectiveness of existing drugs.

The Moon Shots Program also is committed to advancing evidence-based cancer prevention and control practices, which have the potential to prevent up to 50% of cancers in future generations. The cancer prevention and control platform has established a range of targeted initiatives designed to advance early detection approaches, spread the use of actions known to reduce cancer risk, and improve access to screening and prevention services. Moon shots experts also have served as educational resources for legislators across the country on policies related to cancer prevention and control.

Pancreatic Cancer Moon Shot

- Assembling a panel of blood-borne molecules and proteins that act as a “liquid biopsy”
- Honing imaging algorithms that can pick out lesions in the pancreas missed by CT scans and MRIs, including cysts that are likely precursors of cancer
- Established a high-risk clinic to monitor those with a family history of the disease or genetic mutations that raise their risk





Teaming up to Explore and Discover



A repurposed targeted therapy is producing unprecedented response rates in patients with an advanced lung cancer that carries a highly treatment-resistant mutation

A team at MD Anderson led by John Heymach, M.D., Ph.D., chair of Thoracic/Head and Neck Clinical Oncology, has resurrected a failed drug and is using it to target a genetic mutation that drives a certain type of lung cancer.

The drug, called poziotinib, inhibits an insertion of exon 20 on the epidermal growth factor receptor gene, which is associated with non-small cell lung cancer. It currently is being tested in a clinical trial co-led by Heymach.

“We’ve had no effective drugs for these patients, who historically have progression-free survival of about two months, and a response rate of around 10 percent for other therapies,” Heymach says.

Discovery of this new option for a previously untreatable patient group came as a result of funding and support from MD Anderson’s Moon Shots Program™ and its Lung Cancer Moon Shot™.

Utilizing the Genomic Marker-Guided Therapy Initiative (GEMINI), which includes tumor samples and detailed clinical information on more than 4,000 lung cancer patients treated at MD Anderson since 2012, researcher Jacquelyne Robichaux, Ph.D., developed EGFR exon 20 cell lines as well as mouse models with cancer derived from patient tumors. She then tested a variety of EGFR inhibitors against them under the Lung Moon Shot’s drug repurposing program. Poziotinib proved promising.

Patient Deanna Brinkman was one of the first patients to enroll in the trial, and has seen that promise.

“On my first scan after poziotinib treatment, you could see there were bites taken out of the diseased tissue,” she says.

Read more about poziotinib and the clinical trial on Page 26.



Deanna Brinkman is enrolled in a clinical trial of poziotinib for non-small cell lung cancer.

photo by Wyatt McSpadden

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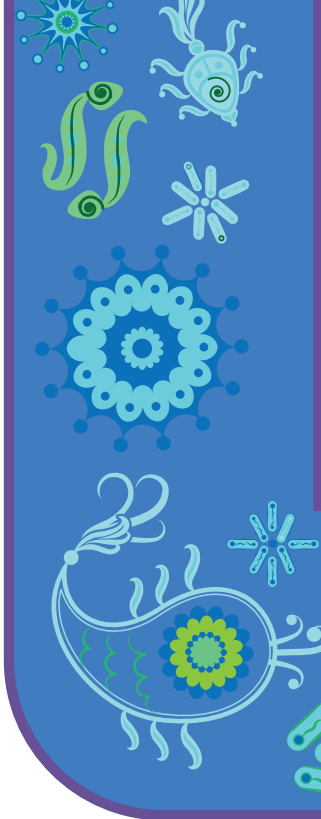
John Heymach, M.D., Ph.D., chair of Thoracic/Head and Neck Medical Oncology and co-leader of the Lung Cancer Moon Shot

Daveta Jenkins, clinical studies coordinator for Thoracic/Head and Neck Medical Oncology

Postdoctoral fellow Jacquelyne Robichaux, Ph.D., Thoracic/Head and Neck Medical Oncology-Research

Shuxing Zhang, Ph.D., PharmD, associate professor of Experimental Therapeutics





A gut reaction

By Scott Merville

Research ties the diversity of bacteria in the digestive tract to the impact of cancer immunotherapy

Bacteria and other microbes live in us by the trillions – not as agents of infectious disease but as vital allies with important roles in maintaining human health.

By most estimates, these useful invaders – collectively known as a person’s microbiome – outnumber the body’s own cells.

In recent years, scientists have begun to describe the distinctive features of this “microbiome” and to understand in greater detail its impact on essential processes such as digestion and immune response.

Jennifer Wargo, M.D., associate professor of Surgical Oncology, leads a team of researchers at MD Anderson uncovering a connection between the blend of bacteria found in late-stage melanoma patients’ digestive tracts and their response to a widely used immunotherapy drug that stimulates the immune system to fight off disease.

FUELED BY THE MELANOMA MOON SHOT

“Greater diversity of bacteria in the gut microbiome is associated with both a higher response rate to treatment and longer progression-free survival,” says Wargo.

The researchers studied fecal samples from 43 patients treated with immune checkpoint blockade drugs that unleash a block on the immune system so it can fight cancer. Their study indicated that certain characteristics of the patients’ microbiomes correlate with slower disease progression, while other qualities are associated with advancing the disease.

Findings revealed that patients with more varied types of bacteria in their digestive tracts had longer median progression-free survival, defined as the point in time when half the patients experienced progression of their disease.

“Greater diversity of bacteria in the gut microbiome is associated with both a higher response rate to treatment and longer progression-free survival. The microbiome appears to shape a patient’s response to cancer immunotherapy, which opens potential pathways to use the microbiome to assess a patient’s fitness for immunotherapy and to manipulate it to improve treatment,” says Wargo, who is also co-leader of the Melanoma Moon Shot™, part of MD Anderson’s Moon Shots Program™ to reduce cancer deaths by accelerating the development of therapies from scientific discoveries.

Research has shown that a person’s microbiome is unique and can be altered by diet, exercise, antibiotics, probiotics or through transplantation of fecal material. Treating the microbiome would open a completely new avenue of cancer treatment.



In November, MD Anderson signed an agreement with Seres Therapeutics, which makes live bacterial products, and the Parker Institute for Cancer Immunotherapy, to launch a clinical trial in 2018 that combines immunotherapy with treatment to improve the microbiome of

patients with stage 4 melanoma.

The clinical collaboration builds on Wargo's research, which was funded initially by the Melanoma Moon Shot.

In her current study, patients were treated with an immune checkpoint inhibitor, a class of drugs that blocks a protein named PD1 that prevents the immune system from attacking cancer. Immune checkpoint inhibitors release the block, which then frees T cells, the attack cells of the body, to locate and attack tumors.

"Anti-PD1 immunotherapy is effective for many, but not all, metastatic melanoma patients, and responses aren't always durable," Wargo says.

Researchers are seeking clues to understand these varied results to extend immunotherapy success to more patients.

"Evidence from lab and animal model research had previously indicated a relationship between solid tumors, immune response and the microbiome," says Vancheswaran Gopalakrishnan, Ph.D., a postdoctoral fellow in Wargo's lab. "Our study was the first of its type to look at the relationship between the microbiome and immunotherapy response in patients."

Specific bacterial types also had an apparent effect. Abundant *Faecalibacterium* was associated with longer progression-free survival and an abundance of *Bacteroidales* correlated with more rapid disease progression.

Patients with favorable microbiomes showed strong evidence of a more robust immune response. They had higher levels of immune cells circulating in the blood and penetrating the tumor, as well as lower levels of a variety of immune-suppressing cells.

To further investigate the link between the microbiome and response to therapy, Wargo and colleagues transplanted stool from patients into germ-free mice. Some of the patients had responded to immunotherapy drugs, and some had not. The mice that received transplants from responding patients had significantly reduced tumor growth, higher densities of beneficial immune T cells, and lower levels of immune suppressive cells. They also had better outcomes when treated with immune checkpoint blockade inhibitors.

Wargo cautions that there's still a lot to learn about the relationship between the microbiome and cancer treatment, and urges people not to attempt self-medication with probiotics and other methods to alter the diversity of their gut bacteria.

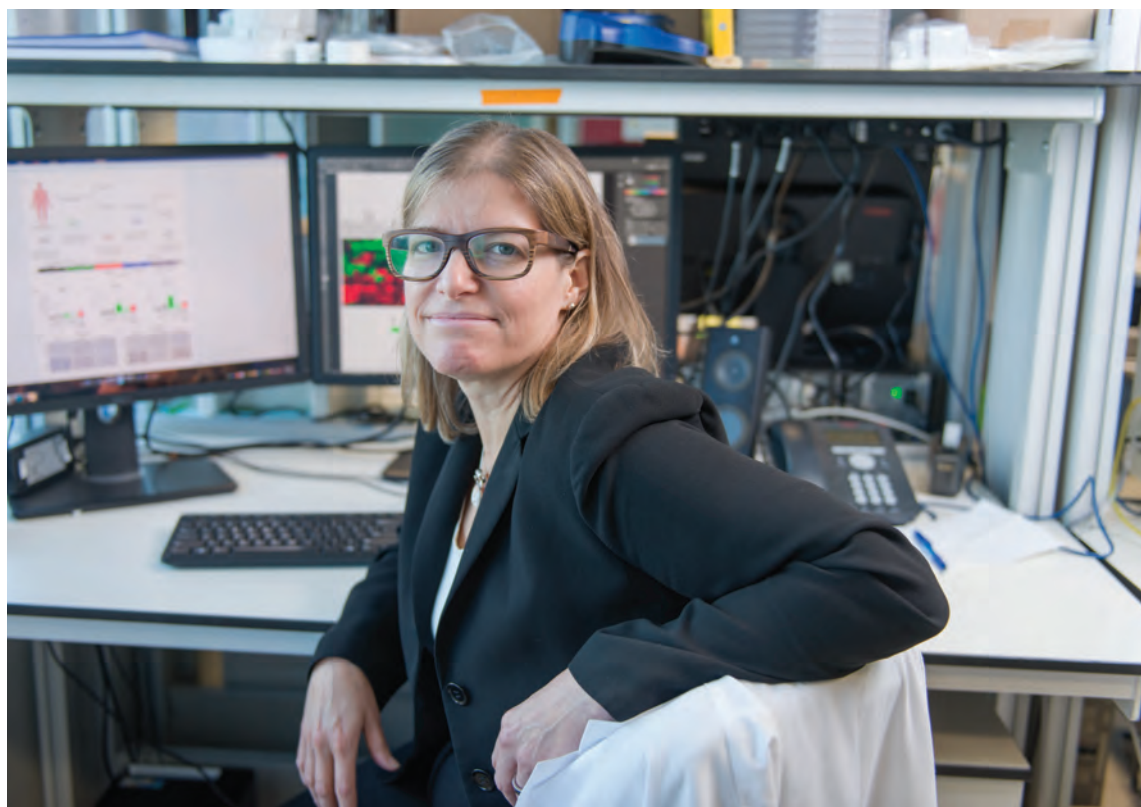
"They may not help their situation and could potentially harm themselves," she says. "Given that treating the microbiome would be a completely new approach, it will be best done in the context of a carefully monitored clinical trial."

Jennifer Wargo, M.D., associate professor of Surgical Oncology, leads a team of researchers uncovering a connection between the bacteria in late-stage melanoma patients' digestive tracts and their response to immunotherapy.
photo by Wyatt McSpadden

THE POWER OF DIVERSITY

Researchers took gut microbiome samples from 30 patients who responded to anti-PD1 immune checkpoint blockade drugs and 13 who did not respond. Their findings showed:

- A greater diversity of bacteria in the microbiomes of responders
- Increased abundance of the *Ruminococcaceae* family of bacteria within the *Clostridiales* order in responders
- Increased abundance of *Bacteroidales* in non-responders and a much lower diversity of bacteria





Padmanee Sharma, M.D., Ph.D., professor of Genitourinary Medical Oncology and Immunology

FDA grants accelerated approval of an immunotherapy drug for advanced bladder cancer

Bladder cancer patients with advanced disease have a new option for treatment after the Food and Drug Administration (FDA) approved a drug that launches an immune response against the disease.

The FDA approval of the checkpoint inhibitor nivolumab, known commercially as Opdivo, was based on a Phase II multi-center clinical trial led by Padmanee Sharma, M.D., Ph.D., professor of Genitourinary Medical Oncology and Immunology at MD Anderson.

“Nearly 20% of patients had their tumors shrink substantially, with some complete responses, which is a significant improvement over other second-line options for these patients,” Sharma said.

Under its breakthrough therapy designation and priority review status, the FDA granted accelerated approval of nivolumab for patients with locally advanced urothelial carcinoma whose disease progresses during or after platinum-based chemotherapy treatment, which remains frontline standard of care.

Nivolumab unleashes an immune system attack on cancer by blocking activation of a protein called PD-1 on T cells, white blood cells that find and attack abnormal cells, viruses or bacteria that have specific targets. PD-1 acts as a brake, or checkpoint, to shut down activated T cells.

20%

Patients who had significant tumor reduction in response to nivolumab – some had complete response

The HPV vaccine's enormous potential to stop oral cancer

Researchers have found that the human papillomavirus (HPV) vaccine may reduce the rate of oral HPV infections in young adults by as much as 88%. However, given the vaccine's low rate of uptake in the U.S. – especially in males – the impact of the vaccine on oral HPV infections remains low.

This is the first study to explore the possible impact of HPV vaccination on oral HPV infections. Maura L. Gillison, M.D., Ph.D., professor of Thoracic/Head and Neck Medical Oncology at MD Anderson, presented the findings in advance of the American Society of Clinical Oncology's 2017 Annual Meeting in June. The study was conducted while Gillison was at The Ohio State University Comprehensive Cancer Center.

Studies have shown HPV is responsible for several cancer types in men and women, including cancers in the back of the throat in an area known as the oropharynx. According to the Centers for Disease Control and Prevention, HPV is linked with approximately 70% of oropharyngeal cancers, and incidence of the disease is rising dramatically in the U.S.

Unfortunately, explained Gillison, no clinical trials have prospectively evaluated whether the existing FDA-approved HPV vaccines will prevent oral infections that lead to the disease. Therefore, the vaccine isn't approved for the prevention of head and neck cancers. It is approved for the prevention of cervical, vulvar, vaginal and anal cancers in women and anal cancers in men.

The researchers analyzed data from 2011-2014 in which participants began to self-report if they had received one or more HPV vaccines. They reviewed responses from 2,627 young adults ages 18-33, and compared the prevalence of an oral HPV infection in those who received one more doses of the vaccine to those who did not.

In the cohort, the researchers evaluated the prevalence of HPV type 16, 8, 6 and 11 – the four types included in HPV vaccines before 2016.

They found that the prevalence of vaccine-type infections was far less common in individuals who had been vaccinated than not vaccinated. When comparing the two groups, the infection rates in the vaccinated group was about 88% lower than in those who hadn't been vaccinated.

88%

The percentage of oral infections related to HPV that could be prevented in young adults

A failed drug is showing unprecedented success against a previously impervious mutation

By Scott Merville

During her years of struggle with lung cancer, Deanna Brinkman has avidly researched her specific type of disease. So when an immunotherapy drug failed to stop it in late 2016 she understood the implications all too well.

“I called my oncologist,” she recalls. “We have a superb relationship. He knew that I knew what I was talking about, and I knew and understood what he said: There’s nothing for this.”

What remained at that point were clinical trials, preferably one with a drug that targeted the genetic mutation that drove her disease – an insertion of exon 20 on the epidermal growth factor receptor gene (EGFR).

**FUELED BY
THE LUNG CANCER
MOON SHOT**

That talk with her oncologist in Richmond, Virginia, where Brinkman lives, set off a chain of events that landed her at MD Anderson in January 2017. Here, a team led by John Heymach, M.D., Ph.D., chair of Thoracic/Head and Neck Clinical Oncology, had resurrected a failed EGFR drug with lab research indicating that it might be uniquely suited to attacking exon 20 non-small cell lung cancer.

When a clinical trial of the drug, called poziotinib, opened in March, Brinkman became one of the first to enroll.

“If you could see my scans, it’s absolutely astounding,” Brinkman says. “On my first scan after poziotinib treatment, you could see there were bites taken out of the diseased tissue. It was the most incredible thing, an amazing image. Nothing is growing now.”

By the end of 2017, her disease was still responding to treatment.

“We’ve had no effective drugs for these patients, who historically have progression-free survival of about two months, and a response rate of around 10 percent for other therapies,” Heymach says.

By October, eight of 11 patients – 73% – in the clinical trial had their tumors shrink by at least 30%, with some tumors shrinking by up to 50%.

“These highly encouraging early results are unprecedented for exon 20 insertion non-small cell lung cancer,” Heymach notes. By the end of the year, 30 patients from around the world had enrolled in the trial, which will take up to 50.

This new option for a previously untreatable patient group comes courtesy of MD Anderson’s Moon Shots Program™, launched in 2012 to accelerate the development of new approaches to cancer.

Heymach is co-leader of the Lung Cancer Moon Shot™, which has funded the effort from the beginning, first identifying poziotinib through its program to screen and repurpose existing drugs, then supporting the preclinical research to confirm the drug’s potential, and finally the clinical trial.

About 2% of non-small cell lung cancer patients in the United States (about 3,500 annually) have an EGFR exon 20 insertion, although the incidence of exon 20 insertion disease is much higher in Asia. Other tyrosine kinase inhibitors against EGFR have been approved by the Food and Drug Administration, but none have proved effective against exon 20.

Poziotinib had been tried and abandoned as a general EGFR inhibitor when Heymach’s team turned up evidence of its potential against exon 20.

Lung Cancer Moon Shot

GEMINI

(Genomic Marker-Guided Therapy Initiative)

The trial: Poziotinib for non-small cell lung cancer patients

The results: 73% had tumors shrink by 30%, with some shrinking by up to 50%.

The collaboration: Researchers used GEMINI, which includes tumor samples and clinical information from more than 4,000 lung cancer patients treated at MD Anderson, in discovering the drug’s effectiveness.



Postdoctoral fellow Jacquelyne Robichaux, Ph.D., tapped the moon shot's Genomic Marker-Guided Therapy Initiative (GEMINI), which includes tumor samples and detailed clinical information on more than 4,000 lung cancer patients treated at MD Anderson since 2012. She developed EGFR exon 20 cell lines as well as mouse models with cancer derived from patient tumors, and tested a variety of EGFR inhibitors against them under the Lung Moon Shot's drug repurposing program.

"Poziotinib is the only drug we've ever found that was dramatically better for exon 20 than it was for the classical EGFR mutation that everyone tests," Heymach says.

Working with Shuxing Zhang, PharmD, Ph.D., associate professor of Experimental Therapeutics, Robichaux and colleagues identified structural aspects of the drug that explain that divergent impact.

The team took its findings to Spectrum Pharmaceuticals, a Nevada-based biotechnology company that initially developed poziotinib, leading to rapid development of the Phase II clinical trial.

Heymach notes there is still much to learn about the drug and its long-term impact for patients.

Brinkman has coped with weight loss and a debilitating condition caused by the drug called paronychia – a painful inflammation of the tissue around fingernails and toenails.

But she focuses on what she considers the important things: being mentally alert and physically able, having wonderful friendships and being happily married.

"I'm interested, curious, always learning something new, including languages. I'm 76 now, and I'm going to learn Latin."



Deanna Brinkman and her husband, Ray, travel to MD Anderson from their home in Virginia for her treatment and check-ups. photo by Wyatt McSpadden

Protein identified as potential druggable target for pancreatic cancer

A protein known as arginine methyltransferase 1 (PRMT1) may be a potential therapeutic target for pancreatic ductal adenocarcinoma (PDAC), the most common type of pancreatic cancer, and one of the deadliest – only 10% of patients surviving five years. PRMT1 is involved in a number of genetic processes, including gene transcription, DNA repair and signaling.

"Our study has identified and validated for the first time an arginine methyltransferase as a novel genetic vulnerability in PDAC," said Giulio Draetta, M.D., Ph.D., professor of Genomic Medicine and director of Institute for Applied Cancer Science (IACS). "These findings strongly suggest a role for PRMT1 in PDAC development and illuminate a path toward the development of therapies for patients in desperate need of innovative solutions."

Various treatment regimens have failed to improve PDAC patient survival, driving the critical need for finding druggable targets essential for tumor maintenance. Draetta's team developed an in vivo platform called Patient-based In vivo Lethality to Optimize Treatment (PILOT), a technology enabling systemic identification of tumor vulnerabilities in patient-derived tumors. Through PILOT, they discovered novel epigenetic drivers in PDAC, including PRMT1 in tumors that harbor KRAS mutations on the background of p53. KRAS and p53 are genes often associated with cancer.

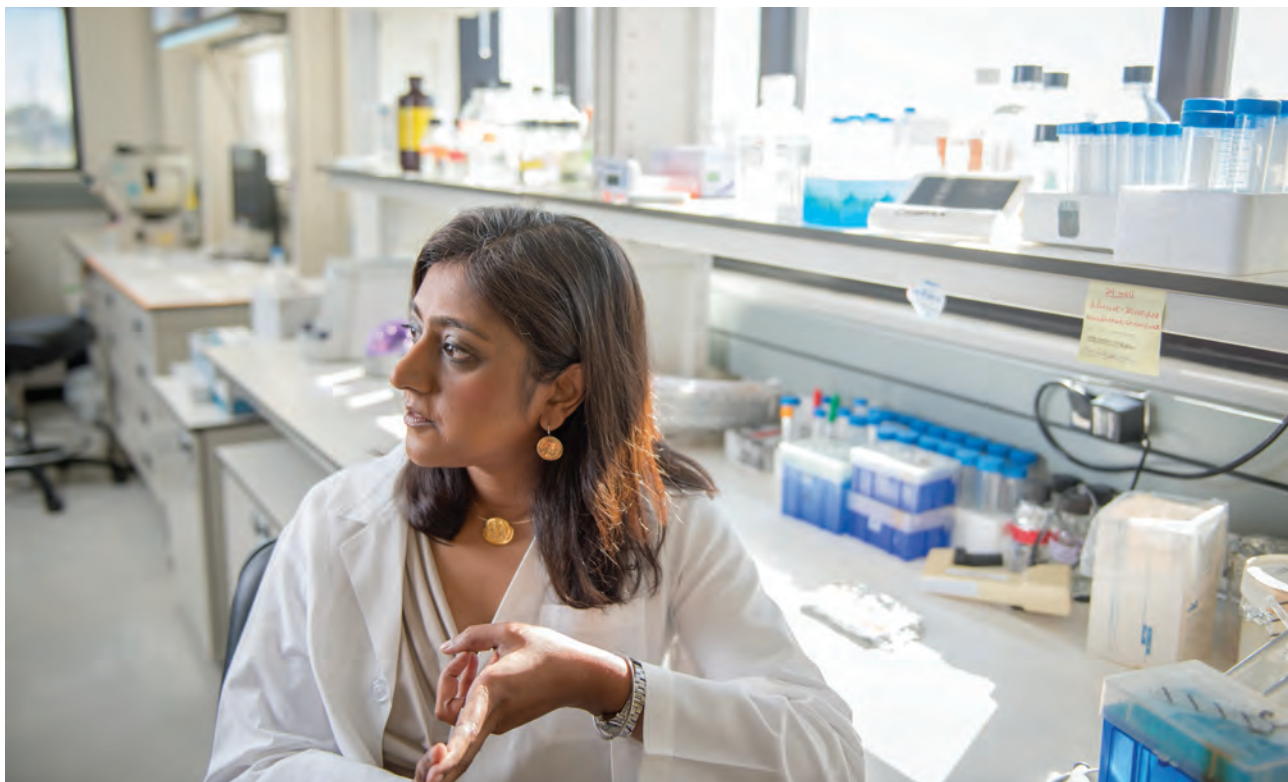
"Through this assessment of epigenetic regulators, we identified PRMT1 as a top scoring 'hit' in these patient-derived tumors," said Virginia Giuliani, Ph.D., a senior research scientist at IACS. "This novel dependency was subsequently validated in multiple patient-derived pancreas models."

PRMT1

A protein that research suggests plays a role in the development of pancreatic cancer.

Combination of diet and exercise improves outcomes for obese pediatric cancer patients

By Katrina Burton



Joya Chandra, Ph.D., worked with Eugenie Kleinerman, M.D., and Keri Schadler, Ph.D., on the study. photo by Wyatt McSpadden

Overweight pediatric cancer patients who eat a healthy diet and exercise regularly may improve outcomes and reduce treatment side effects that cause disease, according to a review study led by researchers at MD Anderson Children's Cancer Hospital.

Specifically, the findings showed chemotherapy may work better in these patients than in those whose eating and exercise habits are not as healthy.

PEDIATRIC CANCER RESEARCH

This discovery propels the need for more work to determine how energy balance – a combination of diet and exercise – can be implemented effectively during treatment to manage or treat obesity.

“The purpose of the review was to delineate between obesity reduction as a goal for energy balance interventions versus simply changing diet or adding exercise,” says Joya Chandra, Ph.D., associate professor of Pediatric Research and the study's lead author. “For example, our review confirmed modifying diet or adding moderate exercise can improve chemotherapy efficacy independent of weight loss.”

The researchers reviewed 67 studies including 32 novel clinical trials in pediatric patients, and data from a variety of cohorts with pediatric patients diagnosed with different cancers, including patients with acute lymphoblastic leukemia (ALL), rhabdomyosarcoma and brain tumors. The cellular mechanisms by which energy balance impacts tumor growth also were highlighted.

“What's exciting about this study is that it lays the groundwork for additional studies in a pediatric clinical setting that involves specific diet and exercise interventions,” says Keri Schadler, Ph.D., assistant professor of Pediatric Research, and co-author of the study.

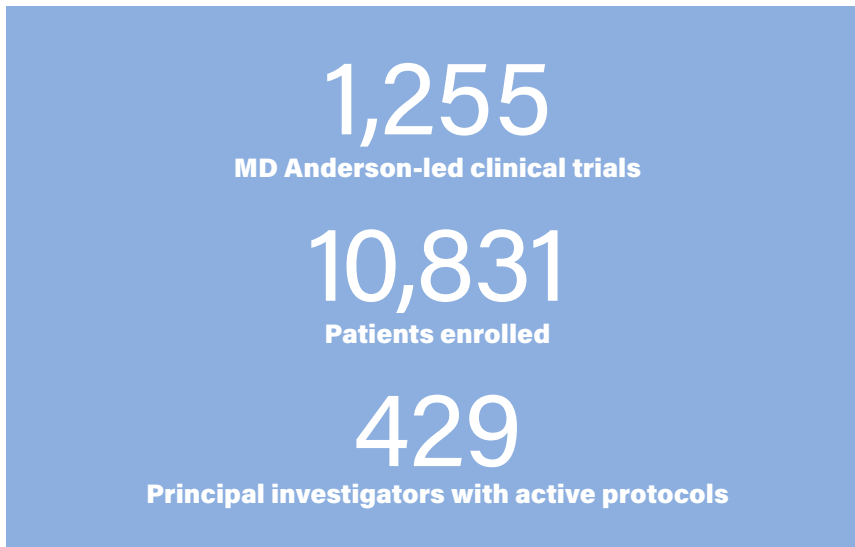
The relationship between diet and exercise and its positive effects on treatment outcomes in obese cancer patients has sparked interest for quite some time, but for pediatric patients, the research has been limited.

While healthy eating is encouraged during and after treatment, special diet interventions as part of treatment for pediatric patients are uncommon. Additionally, when it comes to physical activity, clinicians are cautious about administering an exercise regimen in a cancer care setting.

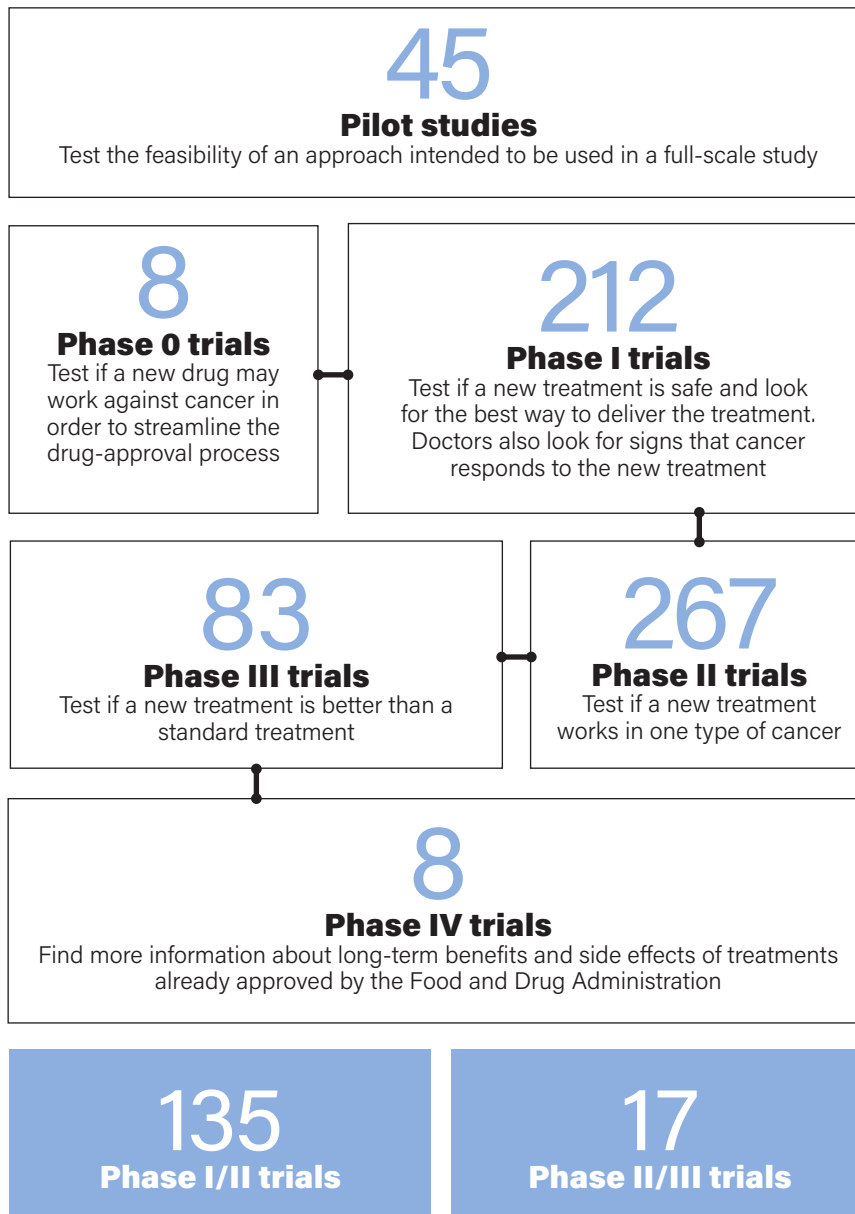
Chandra, who is co-director of MD Anderson's Center for Energy Balance in Cancer Prevention and Survivorship, is helping her Pediatric Research team and colleagues throughout the institution understand the connection between nutrition, physical activity, obesity and cancer.

“The results from our study gives credibility to the need for energy balance interventions in clinical settings to improve treatment outcomes for pediatric patients,” says Eugenie Kleinerman, M.D., professor of Pediatrics, and a co-author of the study.

Studies lead to understanding and breakthroughs



Clinical trials include:



Predicting a deadly leukemia that strikes after cancer treatment

Patients who've been successfully treated for breast, colon and other cancers can go on to develop an often-fatal form of leukemia, sometimes years after completion of treatment, due to a genetic mutation leading to secondary malignancies known as therapy-related myeloid neoplasms (t-MNs).

A study led by Andy Futreal, Ph.D., chair of Genomic Medicine, and Koichi Takahashi, M.D., assistant professor of Leukemia and Genomic Medicine, revealed pre-leukemic mutations, called clonal hematopoiesis, may predict whether patients develop t-MNs. Clonal hematopoiesis appears to function as a biomarker for patients who develop t-MNs, a leukemia recognized for its extremely poor prognosis.

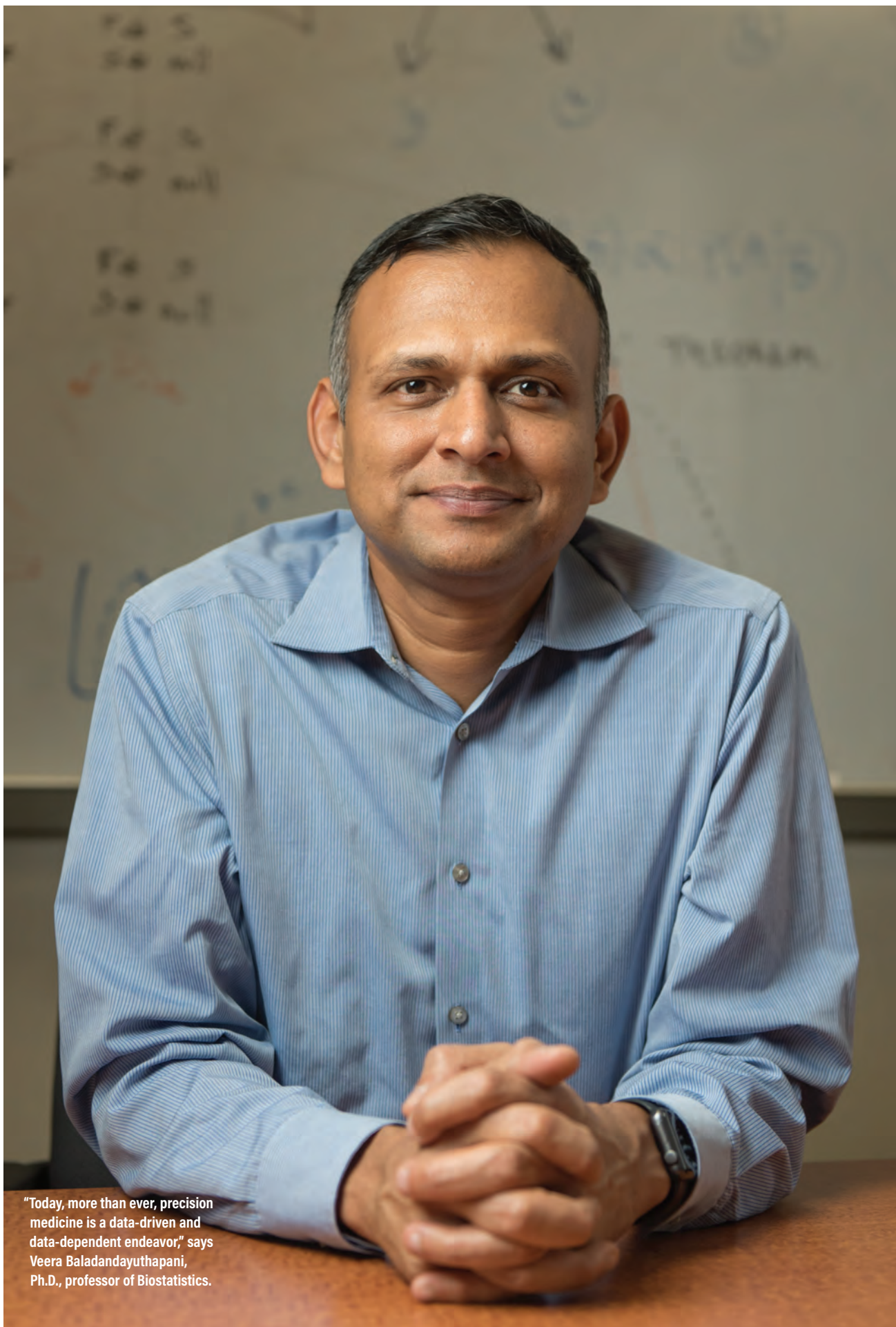
Being able to detect t-MNs earlier is crucial given that the disease usually occurs three to eight years following chemotherapy and/or radiation therapy.

Futreal's team studied 14 patients with t-MNs and found traces of pre-leukemic mutations or clonal hematopoiesis in 10. To determine if pre-leukemic mutations could reliably predict whether the patients would develop leukemia, the researchers compared prevalence of pre-leukemic mutations in the 14 patients with 54 patients who did not develop t-MNs after therapy.

"We found that prevalence of pre-leukemic mutations was significantly higher in patients who developed t-MNs (71%) versus those who did not (26%)," says Futreal. "Based on these findings, we believe pre-leukemic mutations may function as a new biomarker that would predict t-MNs development."

CLONAL HEMATOPOIESIS:

A genetic mutation found in 71% of patients who developed therapy-related myeloid neoplasms, an often-fatal leukemia that occurs in about 5% of cancer patients treated with chemotherapy and/or radiation therapy.



"Today, more than ever, precision medicine is a data-driven and data-dependent endeavor," says Veera Baladandayuthapani, Ph.D., professor of Biostatistics.

Meet the architects who design treatment-advancing clinical trials

By Ron Gilmore photos by Wyatt McSpadden

A cancer patient who is enrolled in a clinical trial that very well may save their life gets to know their oncologist or specialist, their nurses, and the many members of the care staff they see and speak with during their visits.

But they aren't likely to meet an important group of people that plays a crucial role in designing that life-saving study, ensuring the proper information is included and analyzed. Most people are unaware that behind all successful clinical research are experts in biostatistics and bioinformatics.

"Biostatisticians and bioinformaticians are key players on the clinical research team, and an integral part of the clinical trial enterprise," says Prithviraj Bose, M.D., associate professor of Leukemia. "The entire validity of the results of a clinical trial becomes questionable if the trial is not adequately 'powered' to assess its endpoints."

This often involves complex calculations of study sample size and taking into account the many factors related to disease treatment, the drugs being tested and more, he adds.

Anirban Maitra, M.B.B.S., professor of Translational Molecular Pathology, agrees.

"No clinical trial can be designed without input from biostatistics. A poorly designed clinical trial is worse than no trial at all. We are keenly dependent on biostatisticians for ensuring that our clinical trials yield useful information for future generations of patients," says Maitra, who is the scientific director for the Sheikh Ahmed Pancreatic Cancer Research Center. "Bioinformatics is critical in deciphering the wealth of new genomic data. The bioinformatics team uses sophisticated algorithms to elucidate markers of response and resistance to therapy, as well as determine how patients can be stratified appropriately for the most relevant therapies under study."

Biostatisticians and bioinformaticians must have a high level of understanding of the research question being asked, and design the trial accordingly with the information available, setting reasonable expectations for a new treatment based on input from the clinical investigator.

Someone who knows a thing or two about the importance of data collection is Michael Andreeff, M.D., Ph.D., professor of Leukemia, who was a pioneer in the development of flow cytometry, a method for counting and sorting cells that is commonly used in today's clinical trials.

"In clinical studies, we are looking for genetic patterns that aren't obvious. It is simply too much information and too complex," says Andreeff. "With 20,000 genes, how is it even possible to understand what you must know in researching disease? Bioinformaticians are able to help us with that understanding through analysis of large databases such as The Cancer Genome Atlas, to see if data correlates with other studies."

Deluged with data

The accessibility to information that has come with the growth of the Internet has been a boon for many industries and endeavors, and it's been especially important for clinical trials. But the enormous amount of data available is too much for doctors hoping to find reliable treatments or even cures for cancer to take in. There is simply too much coming at them at a high rate of speed.

How do biostatistics, bioinformatics and computational biology benefit patients?

In a nutshell, experts in these quantitative sciences collaborate with clinical and basic science researchers by developing statistical and mathematical models that are used in the design and analysis of clinical trials. Their knowledge of quantitative sciences also is used to help research in genomics, proteomics, radiotherapy, molecular and cell biology, computer-assisted diagnoses and image analysis.

Enter biostatistics and bioinformatics.

“Modern medicine has generated unprecedented amounts of data. A combination of clinical, environmental and public health information, proliferation of associated genomic data, and increasingly complex digital information from sources such as electronic health records, social media, mobile health and imaging,” says Veera Baladandayuthapani, Ph.D., professor of Biostatistics. “Biostatistics maximizes access to, and usability of, such data to enhance, improve and inform decision-making, and is one of the pillars of cancer research. Today, more than ever, precision medicine is a data-driven and data-dependent endeavor.”

While biostatisticians help ensure a clinical trial is designed for success, bioinformaticians translate massive databases of cancer-related information that was virtually unknowable just two decades ago when the ambitious Human Genome Project. The effort, which began in 1990 and ended in 2003, mapped out the intricate details of every human gene, revolutionizing how clinical trials were designed, and what they were able to accomplish.

Genomic Explosion

For scientists like Han Liang, Ph.D., associate professor and deputy chair of Bioinformatics and Computational Biology, the Human Genome Project ignited a passion for big data that continues to this day.

“I was so fascinated by the fact that we could know each detail of the genome and use this information to understand our evolutionary journey of millions of years, and cure diseases for future generations,” says Liang. “Genomics has revolutionized modern medicine over the last decade, especially for cancers. As a result, bioinformatics is one of the fastest moving fields. New data, technology and analytic tools have emerged at an incredible speed, which excites me and makes me want to dig into them. I feel so lucky to be a bioinformatician in the era of ‘big data.’”

Work by John Weinstein, M.D., Ph.D., chair of Bioinformatics and Computational Biology, was critical to the creation of The Cancer Genome Atlas (TCGA), an ambitious project funded by the National Cancer Institute and the National Human Genome Research that has provided a wealth of genomic data related to cancer. His early work spearheading the comprehensive molecular profiling of 60 cancer cell lines presaged what became TCGA. The Human Genome Project, TCGA and other titanic scientific undertakings paved the way for collaboration among clinical researchers and today’s biostatisticians and bioinformaticians. Those projects have provided novel approaches to clinical trial design and the development of potential new therapies.

“Today we have a plethora of innovative and creative opportunities that range from early detection, biomarker discoveries and adaptive clinical trial designs,” says Baladandayuthapani. “Cancer is one of the most advanced and well-characterized disease systems. Certainly studies today must be data-driven.”

No clinical trial is successfully completed without a host of participants with their own individual expertise. It truly takes a team to create a cure from the seemingly endless mounds of interwoven data available today.

“I have worked with multiple clinical and science groups at MD Anderson that cut across multiple cancer types,” says Baladandayuthapani. “One of the things that drew me into this field was the ability to connect with a broad range of scientists and find diverse, high-impact problems to work on. This modern era of big data has presented considerable challenges and opportunities that will keep us occupied for years to come.”

“Biostatisticians are vital because they help us separate truth from fiction, offer unbiased information, and are able to answer in-depth questions a clinical investigator wants to ask.”

— Michael Andreeff, M.D., Ph.D.,
professor of Leukemia





CAR-equipped natural killer cells target blood cancers

Immune cells with a general knack for recognizing and killing many types of infected or abnormal cells also can be engineered to hunt down cells with specific targets on them to treat cancer, researchers at MD Anderson reported in the journal *Leukemia*.

The team's preclinical research shows that natural killer (NK) cells derived from donated umbilical cords can be modified to seek and destroy some types of leukemia and lymphoma. Genetic engineering also boosts the NK cells' persistence and embeds a suicide gene that allows the modified cells to be shut down if they cause a severe inflammatory response.

A first-in-human Phase I/II clinical trial of these cord-blood-derived, chimeric antigen receptor-equipped natural killer cells opened at MD Anderson in June for patients with relapsed or resistant chronic lymphocytic leukemia acute lymphocytic leukemia, or non-Hodgkin lymphoma. All are cancers of the B cells, another white blood cell involved in immune response.

The chimeric antigen receptor (CAR) – so-called because it's added to the cells – targets CD19, a surface protein found on B cells.

MD Anderson's Moon Shots Program™ funds the clinical trial, which is led by Katy Rezvani, M.D., Ph.D., and Elizabeth Shpall, M.D., both professors of Stem Cell Transplantation and Cell Therapy.

CD19

A protein found on the surface of malignant and normal B cells. Genetically enhanced natural killer cells target the protein and kill the cell.

Nivolumab plus chemotherapy improves leukemia patients' survival

Combining the immunotherapy drug nivolumab with standard chemotherapy more than doubled response rates and improved overall survival in patients with acute myeloid leukemia (AML), according to MD Anderson researchers.

The results were from a Phase IB/II ongoing study that paired nivolumab with azacitidine (AZA) in patients who had previously demonstrated poor complete response and overall survival.

“The combination of AZA and nivolumab showed a response rate of 34 percent, which compares favorably to a historic response rate of 12 to 15 percent with AZA alone,” says Naval Daver, M.D., assistant professor of Leukemia.

The study, which followed 51 patients with an average age of 69, showed median overall survival for those treated with combination was 9.3 months, compared with an historic median of 4.7 months for patients treated with AZA alone.

Daver says longer follow-up is necessary to confirm the overall survival benefit of the approach.



49.5%

Increase in median overall survival rate for patients treated with combination of nivolumab and chemotherapy



Teaming up to Strengthen and Support



When a rare cancer diagnosis took a young man and his family by surprise, they found the medical and emotional care they needed at MD Anderson Children's Cancer Hospital

Greg Giraldo loves to compete. Whether on the football field, the basketball court or the cross country course, the 13-year-old athlete is determined to succeed. That drive gave Greg an advantage three years ago when a CT scan revealed a tumor behind his right eye. Pediatric oncologist Winston Huh, M.D., diagnosed him with rhabdomyosarcoma, and had to break the news to Greg and his family.

Though surprised at first by the diagnosis, Greg soon was ready to begin a tough year and a half of treatment. Huh, nurse practitioner Beatriz Rozo and other members of the care team at MD Anderson Children's Cancer Hospital saw Greg through 52 weeks of chemotherapy. That was followed by 25 weeks of precision treatment guided by radiation oncologist Mary Frances McAleer, M.D., Ph.D., at MD Anderson Proton Therapy Center. Throughout it all, the Giraldo family was able to stay close by Greg's side. He and his two brothers spent lots of time bonding and participating in a number of events and activities at the Children's Hospital. And the three enjoyed a week of summer camp at MD Anderson's Camp Star Trails. They also found support from MD Anderson staff who helped the family learn to cope with the psychosocial aspects of cancer, cancer treatment and all Greg was going through. That included taking part in a research study led by pediatric psychologist Martha Askins, Ph.D. Today, Greg is doing well. He'll be a high school freshman next fall, and still sees his MD Anderson team when he returns for follow-up visits three times a year. Read more of his story on Page 39.



Greg Giraldo was diagnosed with a rare cancer called rhabdomyosarcoma.
photos by Wyatt McSpadden

CLOCKWISE FROM TOP LEFT:

The Children's Cancer Hospital team that managed Greg's care included his doctor, pediatric oncologist Winston Huh, M.D., nurse practitioner Beatriz Rozo, pediatric psychologist Martha Askins, Ph.D., and radiation oncologist Mary Frances McAleer, M.D., Ph.D.

A cancer hospital where progress in Alzheimer's treatment is being made

By MD Anderson staff

Cancer and Alzheimer's both are age-related diseases, but people who get one are less likely to get the other, says MD Anderson scientist Jim Ray, Ph.D.

"It's true – people who have Alzheimer's are less likely to develop cancer, and people who've had cancer are less likely to get Alzheimer's," says Ray, who heads Neuroscience Research at MD Anderson's Institute for Applied Cancer Science.

Ray and colleagues think cancer and Alzheimer's are opposite ends of the aging spectrum.

"In cancer, cells that are supposed to die won't; and in Alzheimer's, cells that are supposed to live don't. Thinking about these diseases this way creates a tremendous opportunity for both fields," he says.

Ray leads the Neurodegeneration Consortium, a multidisciplinary team of researchers from MD Anderson, Baylor College of Medicine and the Massachusetts Institute of Technology. The consortium was launched in 2012 with a \$25 million gift from the Robert A. and Renee E. Belfer Family Foundation to better understand the underlying biology of Alzheimer's disease and turn that knowledge into effective therapies for patients. The development of new, beneficial therapies has been rare, Ray says, but he's inspired by the cancer care successes he's seen at MD Anderson.

"It's been 14 years since an Alzheimer's drug was approved by the Food and Drug Administration, and even at that, the drugs we have don't slow the disease," he says. "In contrast, new cancer drugs are being approved on what seems like a monthly basis. Being at MD Anderson gives those of us conducting Alzheimer's research an important opportunity to learn from that success."

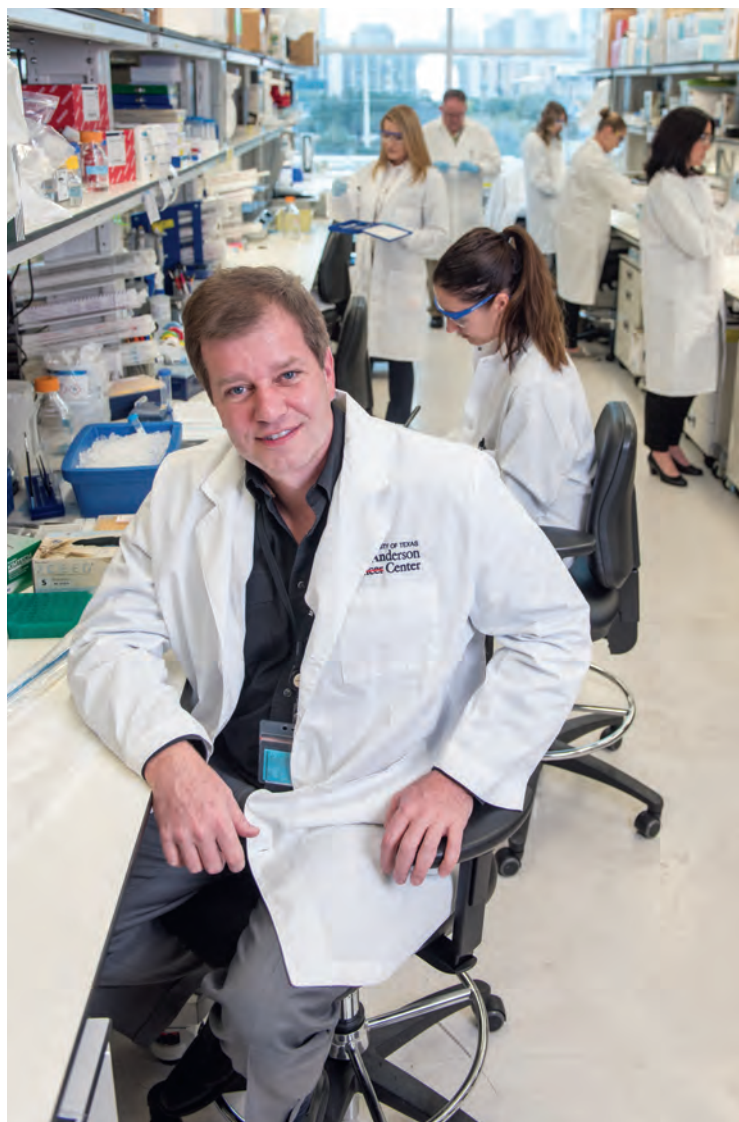
One way that cancer research may help Alzheimer's patients is through the study of chemobrain, a common side effect of chemotherapy that causes problems with thinking and short-term memory.

"It's projected there will be more cancer survivors with chemobrain than Alzheimer's patients in a few years," Ray says. "We have to find ways to promote healthy brain aging and protect the nervous system from the damaging effects of chemotherapy."

The collaborative nature of the Neurodegeneration Consortium promises to speed up such discoveries by sharing promising findings across its three institutions.

And continued support is helping to fund the work. The Belfer Foundation followed up its initial gift with another \$3.5 million in 2015. And in 2016, the M.D. Anderson Foundation has given \$500,000 to the consortium. In addition, support from MD Anderson donors has totaled more than \$25 million.

MD Anderson donors have given more than \$25 million to support the Neurodegeneration Consortium



Jim Ray, Ph.D., leads a multidisciplinary team of researchers that is developing more effective treatments for Alzheimer's disease.

photo by Wyatt McSpadden

Preventing melanoma, one tanning bed ban at a time

MD Anderson's work to make indoor tanning illegal for minors in Texas helps inspire a similar ban in Poland

Texas and Poland are miles apart, but cancer prevention advocates and experts from both places worked closely together to reshape health policy and reduce early melanoma risk in young people.

Jeffrey Gershenwald, M.D., professor of Surgical Oncology, and Waldemar Priebe, M.D., professor of Experimental Therapeutics, partnered with several colleagues in Poland in a multidisciplinary effort that significantly contributed to national legislation that restricts indoor tanning use for minors under 18. The new law also requires proof of age before using a solarium – as tanning beds are known there – and limits advertising of the services.

Strong evidence suggests that using a tanning bed during adolescence or young adulthood can increase the risk of melanoma by 75%.

THE MELANOMA MOON SHOT AND THE CANCER PREVENTION AND CONTROL PLATFORM ARE STOPPING CANCER

“We worked with melanoma colleagues in Poland and requested data from their cancer registry. Remarkably, we learned that between 2005 and 2015, melanoma rates nearly doubled in women under age 45,” says Gershenwald, medical director of the Ben Love/El Paso Corporation Melanoma and Skin Center at MD Anderson. “From there, we shared significant scientific evidence about the dangers of indoor tanning

and the potential impact of public policies aimed at reducing skin cancer risk in youth with the Polish health ministry and other governmental officials and presidential staff.”

The MD Anderson team was recognized for this collaboration by the Chancellery of the President of the Republic of Poland, and in November 2017, a bill prohibiting the use of tanning beds by minors was signed into law and will be enacted nationwide in Poland in early 2018.

The Polish law is based on similar legislation that was passed in Texas in 2013. MD Anderson surgical and medical oncologists and behavioral scientists from the Melanoma Moon Shot™, the Governmental Relations department, and the cancer prevention and control platform were instrumental in that accomplishment. They joined advocacy organizations from across the state to educate legislators about the dangers of tanning beds. They're also partnering with the American Cancer Society Cancer Action Network, a nonprofit advocacy affiliate of the American Cancer Society which promotes evidence-based policies at all levels of government to improve access to care and quality of life for cancer patients, additional cancer prevention and early detection programs, and increased tobacco regulation.

“Through this partnership, we continue to share lessons learned about the dangers of indoor tanning and engage colleagues and key stakeholders in other states that are considering similar indoor tanning legislation,” Gershenwald says.

Currently, 17 states, including Texas, and the District of Columbia have similar laws prohibiting access to tanning beds for minors, and there are early indicators that this legislation and an ongoing public health campaign are changing behavior.

The Centers for Disease Control and Prevention has reported a significant drop in the use of indoor tanning devices by teenagers in recent years, says Gershenwald, who is a co-leader of the Melanoma Moon Shot.

“If no youth currently aged 14 or younger ever practiced indoor tanning in the U.S. during their lifetime, the CDC estimates more than 200,000 cases of melanoma would be prevented, saving nearly \$1.1 billion in treatment costs and more than 458,000 years of life,” Gershenwald says.



Jeffrey Gershenwald, M.D., professor of Surgical Oncology, is a co-leader of the Melanoma Moon Shot. photo by Wyatt McSpadden

Progress in preventing skin cancer

Findings from an MD Anderson study released in 2016 showed that 81% of indoor tanning facilities complied with the Texas ban on indoor tanning for those under the age of 18.

In 2014, the Food and Drug Administration reclassified indoor tanning devices, moving them from low-risk Class I medical devices up to Class II, which means they're associated with moderate to high risk. It also required tanning products used in salons to carry a visible “black box” warning that reads, “Attention: This sunlamp product should not be used on persons under the age of 18 years.”

And in 2015, the FDA proposed a nationwide ban on the use of indoor tanning beds by anyone under the age of 18.



MD Anderson doctors, nurses and staff launch a full-court press to stop a young athlete's cancer

By Katrina Burton photo by Wyatt McSpadden

Playing basketball, football and running cross country this school year was not what Luz Giraldo envisioned for her son Greg, when he was diagnosed with cancer three years ago. In fact, she and her husband, are just grateful that Greg is here today.

“Participating in normal activities is a bonus,” says Giraldo. “Greg was such an active and healthy child who never got sick.”

Greg was only 10 years old when he felt burning pain in his right eye. He thought the pain was the result of a classmate’s jacket hitting him in the eye during gym class, but the continued pain alerted his mom that something was wrong. A CT scan revealed a tumor behind his eye, and a biopsy confirmed it was cancer.

Giraldo recalls the shock her family felt when Greg’s doctor, Winston Huh, M.D., a pediatric oncologist at MD Anderson Children’s Cancer Hospital, told them Greg had orbital rhabdomyosarcoma, a rare childhood cancer of the eye socket.

“We never suspected cancer,” she said.

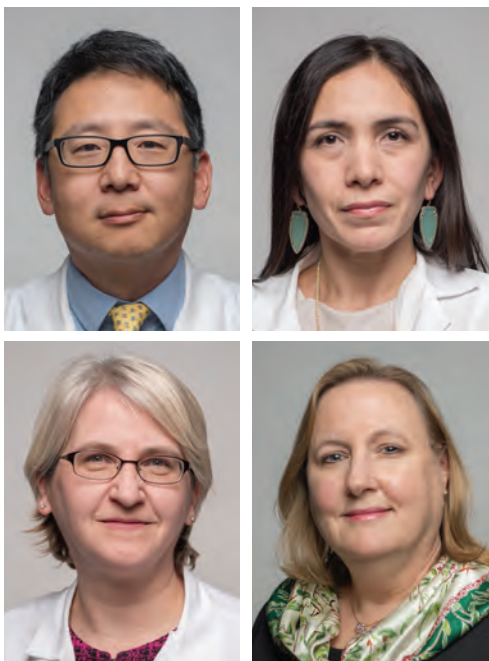
Greg went through a grueling 52-week regimen of chemotherapy, and another 25 weeks of radiation therapy. During treatment, he and his two brothers attended MD Anderson’s Camp Star Trails – a weeklong camp for MD Anderson patients and siblings, hosted annually at Camp For All in Burton, Texas.

“This was great bonding time for the boys,” says Giraldo. “They had so much fun at camp. Greg was relieved to know that there were other kids going through the same thing he was going through.”

Giraldo says her son’s cancer journey has been challenging for the entire family, but credits MD Anderson with providing much-needed support.

“Greg’s brothers were always invited to the same hospital events and activities he was. MD Anderson made sure they were entertained and occupied during Greg’s chemotherapy and radiation treatments.”

Today at age 13, Greg shows no evidence of cancer. He wears eyeglasses to help with vision problems caused by the disease, but he doesn’t let that get in the way of sports. He comes back to MD Anderson every four months for follow-up care, and he and his brothers continue to participate in hospital activities and events.



Family connections

Students learn to explain genetic influences on diseases

By Ina Fried photos by Wyatt McSpadden

The patient had been diagnosed with a genetic disorder many years ago in his home country, but he'd never been told anything about it.

“When I saw him, I was able to explain the condition – familial adenomatous polyposis – to him and to explain that it was causing him to be at high risk for colon cancer,” says Annelise Pace, a second-year student in the genetic counseling master’s degree program at The University of Texas MD Anderson Cancer Center UTHealth Graduate School of Biomedical Sciences.

EDUCATING AND TRAINING TOMORROW'S ONCOLOGY EXPERTS

“I was also able to explain what that condition meant for his children and to help him figure out when it would be appropriate to order genetic testing for the children,” says Pace, who conducts genitourinary cancer research at MD Anderson.. “He was so grateful to finally have this condition explained, because he’s been confused about it for so many years.”

Making decisions

Helping people understand complex medical information in a way that helps them make decisions is the goal of genetic counseling.

“An essential component of a genetic counseling session is taking a thorough family history and doing a risk assessment for conditions in this family that might have a hereditary component,” says Claire Singletary, director of the Graduate School’s genetic counseling program. “We look at whether there are genetic tests for the likely hereditary conditions. We help the family work through whether the tests would provide them with useful information.”

According to the National Institutes of Health, more than 2,000 genetic tests are available. A genetic test can confirm or rule out a suspected genetic disorder, such as Duchenne muscular dystrophy, or can help determine a person’s risk of developing or passing on a disease, such as cancer. Inherited genetic mutations play a major role in about 5 to 10% of all cancers. Researchers have associated mutations in specific genes with more than 50 hereditary cancer syndromes, which predispose individuals to developing certain cancers.

Test results may help in planning for the expected course of a disease or in choosing among treatment options. Faced with the same test results, different circumstances may lead patients to make different decisions.

For example, Pace’s faculty research advisor, Ashley Woodson, tells of counseling members of a family in which several young women with breast cancer tested positive for a hereditary form of the disease.

“These were women in their 20s and 30s, and some were not married or thinking about future children at that point, while others had finished having their families,” says Woodson, a 2011 graduate of the program. “Testing positive for a hereditary form of breast cancer certainly forces young women to make decisions about future family planning before they typically would have. These genes can increase the risk of another breast cancer or ovarian cancer and so they have to talk through all of that.”

Annelise Pace is a second-year student in the genetic counseling master’s degree program at The University of Texas MD Anderson Cancer Center UTHealth Graduate School of Biomedical Sciences.

LEADERS IN LEARNING

MD Anderson is committed to research-driven cancer care and comprehensive educational programs to enable the next generation of cancer fighters.

7,000+
trainees

83
accredited graduate medical education programs

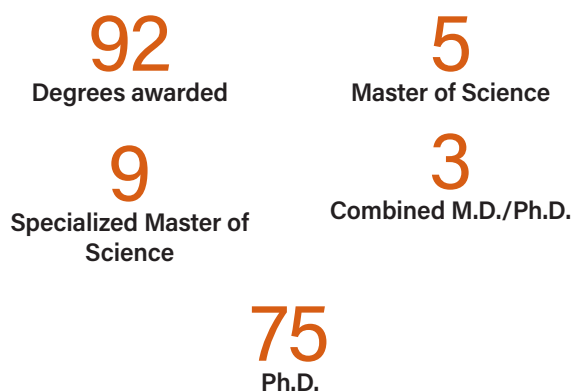
10:1
School of Health Professions’ student to faculty ratio



In collaboration with the UT Health Science Center at Houston, MD Anderson awards M.S., Ph.D. and M.D./Ph.D. degrees at the MD Anderson Cancer Center UTHealth Graduate School of Biomedical Sciences.

MD Anderson's School of Health Professions awards degrees in 10 bachelor's programs and two master's programs in allied health disciplines.

GRADUATE SCHOOL OF BIOMEDICAL SCIENCES



SCHOOL OF HEALTH PROFESSIONS



All students are trained in pediatric medical genetics, prenatal and cancer genetic counseling. The accreditation agency for genetic counselors requires students to have participated in at least 50 counseling sessions. Students in the program average 150 to 200 sessions.

“The Texas Medical Center is unmatched on the depth and breadth of cases that we see here,” says Singletary, who specializes in prenatal counseling. “And Houston being the most diverse big city in the country, is a fantastic place to intersect with people from all different parts of the world and all different walks of life and cultural backgrounds.

“Genetics is expanding into all areas of medical care, such as cardiology and neurology,” Singletary says. “One day personalized genetic medicine is probably going to be a part of how all our health is taken care of.”

Claire Singletary, director of the genetic counseling master's degree program

Depending on their specific circumstances, some of the women might decide to have preventive mastectomies, while others might decide to be screened more frequently.

Knowledge and skills

Helping patients sort through questions surrounding genetic testing requires both scientific knowledge and psychosocial skills. Successful program applicants average above a 3.5 GPA and GRE graduate school entry exam scores around the 70th percentile or better. Prospective students are encouraged to shadow a genetic counselor and to do advocacy work for people with disabilities, staff a crisis hotline, or participate in other programs that give them experience working with individuals going through a difficult time.

One of about 40 accredited two-year master's degree programs in the United States and Canada, the school's program receives more than 150 applications annually. About 40 applicants are selected for interviews. From those, eight to 10 students are admitted. Each receives a scholarship to partially offset the cost of tuition.

“We've received tremendous support from UTHealth and MD Anderson, and we've been fortunate to continue to grow our class size,” Singletary says. “Nationally, there are more genetic counseling jobs than there are genetic counselors graduating on an annual basis. The field is trying very hard to meet that demand.”

Growing field

When the program was founded in 1989, there were three genetic counselors in Houston. Now there are nearly 100, thanks to this program. While 30-45% of admitted students are Texans, about 70% stay in Texas to practice after graduation.

The program is rigorous, but faculty and student mentors help with the transition to a new city, to graduate school life and later to preparing to defend a research thesis and navigating the world of jobs.

Fellowship allows already elite nurses to specialize in cancer care

By Ronda Wendler

As a child in Long Island, New York, Kathryn Mazzarella was fascinated by her mother's tales of life as an oncology nurse.

"Hearing about how she connected with patients and their families inspired me," says Mazzarella, who years later chose the same path. First, she earned a bachelor's degree, then landed a job in the Leukemia and Lymphoma unit at a prominent New York City hospital while completing a master's degree in nursing.

"I was fortunate to begin my career in a high-risk specialty that required clinical precision and human compassion," she says. "It challenged me to expand my knowledge about various forms of cancer."

Last year, while attending the Oncology Nursing Society's annual conference, Mazzarella learned about MD Anderson's Post Graduate Fellowship in Oncology Nursing. The program provides advanced practice nurses – those with master's or doctorate degrees and three years of clinical experience – with exposure to all major areas of oncology nursing, from prevention to palliative care, and everything in-between. Mazzarella applied and was accepted.

"The fellowship is for those who want to achieve oncology nursing excellence through a channel other than the traditional on-the-job training apprenticeship model," says Joyce Dains, Dr.PH, professor and chair ad interim of Nursing.

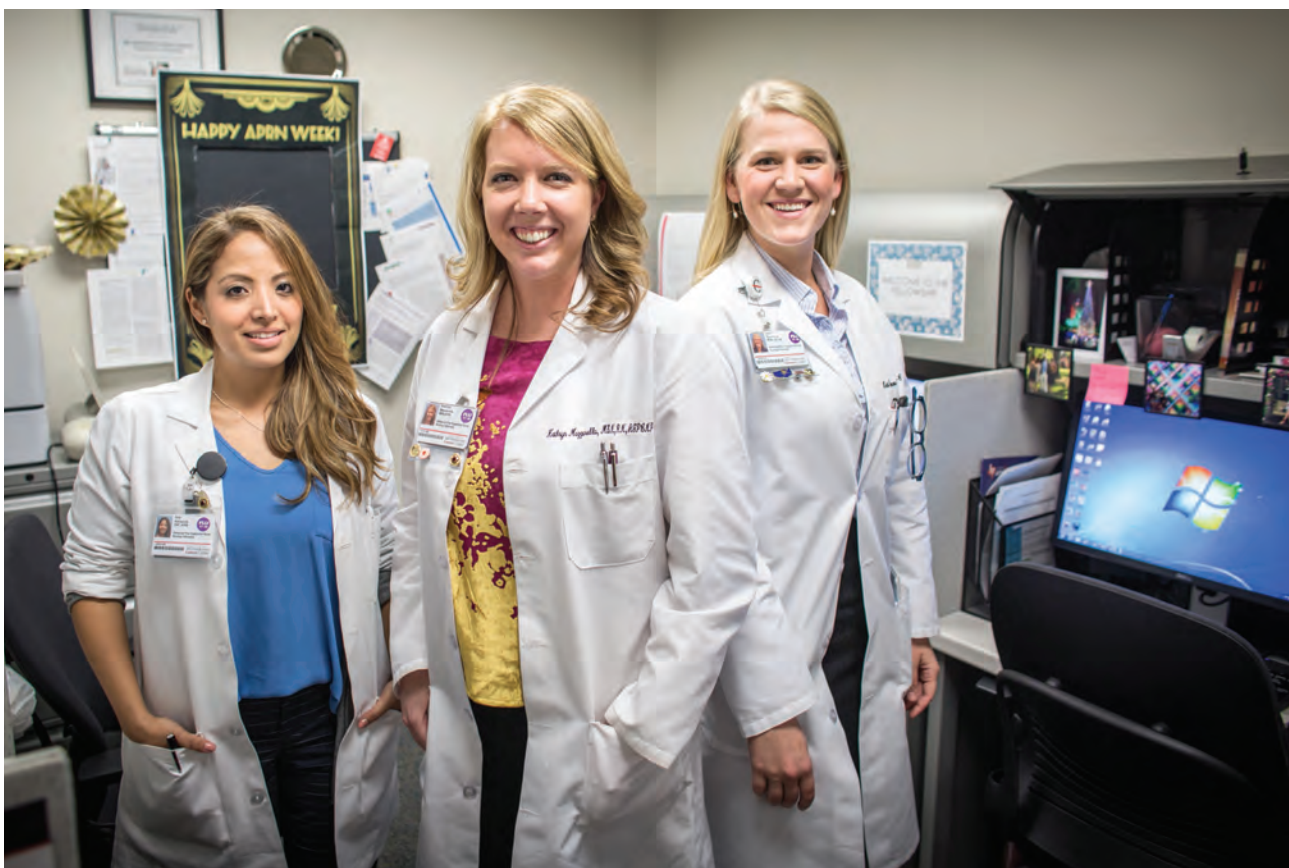
Dains helped launch the program 12 years ago. At the time, it was the first of its kind in the country. Today, 10 such programs exist. Most are modeled after MD Anderson's pioneering fellowship.

Not just a disease

Like Mazzarella, Ana Adriaola began her career in a hospital oncology unit. Unlike Mazzarella, she's the first nurse in her family.

MD Anderson's nursing program holds the American Nurses Credentialing Center's Magnet Nursing Services Recognition status, which recognizes health care organizations for quality patient care, nursing excellence and innovations in professional nursing practice.

There are
3,353
registered nurses
working at
MD Anderson



From left: Ana Adriaola, Kathryn Mazzarella and Heidi Simmons are advanced practice nurses in MD Anderson's Post Graduate Fellowship in Oncology Nursing. photo by Nick de la Torre

“I was working as a hospital secretary in an oncology unit when I saw what nurses do and how they contribute to the team,” she says. “I loved the fast-paced nature of the job and knew it was for me.”

Adriazola continued working while attending nursing school. After earning a bachelor’s degree, she signed on as a staff nurse in the same unit where she’d been a secretary.

“One of the first patients I met was only three months older than me,” she recalls. “He had acute lymphocytic leukemia and was in and out of the hospital for treatment. This guy went through a lot – pain, infections, three bone marrow transplants and two relapses. All the while, he kept smiling and remained positive.

“He and all cancer patients remind me each day that frankly, I have it easy,” she says.

As Adriazola’s passion for nursing grew, she earned a doctorate degree in nursing practice, then entered MD Anderson’s Post Graduate Fellowship in Oncology Nursing to gain a greater insight into how the disease impacts patients.

“Oncology encompasses the entire person, their family, and their environment,” she explains. “You can’t treat cancer without knowing who is in the patient’s life supporting them, what they eat, what their financial status is, where they live, what they have access to. This is what draws me to oncology – the realization that cancer is not just a disease, but a person’s life.”

Highly competitive

MD Anderson’s Post Graduate Fellowship in Oncology Nursing is highly competitive, says Angela Bazzell, DNP, the program’s director. Only three to four fellows are accepted each year from a pool of 50 to 80 applicants from all over the country.

“Our fellows are top-tier,” says Bazzell, who graduated from the program in 2015. “Their academic and work references are impeccable. Their passion for nursing is palpable.”

The one-year program combines classroom learning with hands-on experience. Fellows attend grand rounds, travel to a national oncology conference, care for patients with supervision from an advanced practice provider or a physician, author an academic manuscript for publication in a peer-reviewed journal, and prepare to take the national Advanced Oncology Certified Nurse Practitioner exam that will earn them the highly regarded AOCNP credential.

At first, participants spend more time in the classroom than in the clinic. Before long, patient care takes a leading role. Last year’s fellows racked up 3,000 patient visits while rotating through about 20 departments. During the last half of the program, each fellow selects an area of specialization.

Heidi Simmons decided to focus on cell therapy for treatment of blood cancers like leukemia, lymphoma and myeloma, where healthy cells are infused into patients to replenish those damaged by cancer. Before joining the program this year, she was a nurse in MD Anderson’s inpatient Lymphoma service and completed the cancer center’s New Graduate Nurse Residency Program which helps newly graduated nurses move into the registered nurse role. Later, she worked in a bone marrow transplant unit at an Austin hospital.

“I’ve learned a great deal caring for patients with cancers of the blood,” says Simmons, who holds a master’s degree in nursing. “I’m looking forward to working with CAR T-cell patients, whose immune cells will be re-engineered to fight their tumors using this emerging kind of cell therapy.”

Simmons moved from Austin when she learned of her acceptance to the fellowship, while her husband remained behind to continue his job as a financial portfolio manager. They see each other on weekends when they explore new coffee shops and take their two dogs on outings.

In demand

Program participants typically receive about five job offers when the fellowship concludes, yet 80% choose to remain on staff at MD Anderson.

“They’re highly sought after, and demand is growing quickly,” says Dains.

A study by the American Academy of Medical Colleges predicts a shortage of 4,000 medical oncologists by 2020. This translates to 10 million visits by cancer patients that can’t be handled due to physician shortage.

“Our program is creating a pipeline of highly qualified oncology nurse practitioners who can help alleviate the shortage,” Dains says.

Attract, develop and retain the best cancer research nurses

By Mary Ann Hellinghausen

Suzanne Phillips enjoyed 26 years working for Dow Chemical Co., much of that time as a researcher in product development. It was a thrill to see packaging that her team had developed on the shelf of her local grocery store.

But a desire to directly impact people, specifically cancer patients, was calling her.

That aspiration led her to nursing school and, ultimately, to her work as a research nurse resident, learning how to help patients on clinical trials in our Lymphoma and Myeloma department.

Phillips is a participant in a new Research Nurse Residency Program – the first of its kind in the nation – launched at MD Anderson in October 2016. The program is open to new nursing graduates or nurses with less than one year of experience. They don't need to be embarking on second careers, but the program is drawing the interest of nurses like Phillips.

The goal of the program, modeled after our successful Clinical Nurse Residency Program, is to attract, develop and retain research nurses. The residency consists of a four-week orientation phase followed by a 12-month residency program within a department. It includes more than two weeks of classroom learning throughout the year.

Research nurses play a vital role for the 10,831 patients enrolled in MD Anderson's more than 1,250 clinical trials.

"Being a research nurse is a very hard job," says Lore Lagrone, administrative director, protocol research, Lymphoma and Myeloma. "It's like a big jigsaw puzzle. Your satisfaction comes from making all the pieces fit."

"The perception is that research nurses are 'wall huggers' because others see them waiting in the halls to talk with a patient or a doctor," Lagrone adds. "But you don't see all of the 40 gazillion things that have to get done on the other side."

Among those "gazillion things" are matching the right patient with the right study; ensuring proper patient consent; coordinating testing and appointments; evaluating how the patient is responding to the protocol; finding solutions to a patient's side effects; and documenting, documenting, documenting.

The residency program has attracted "exceptionally qualified" applicants, says Denise Erdman, clinical research supervisor, Radiation Oncology. Seasoned candidates pursuing second careers bring valuable attributes.

"The freshness that a new nursing graduate brings is exciting," Erdman says. "But the maturity and life experience of those seeking second careers are great traits also. They are eager learners, very motivated and proactive in seeking answers."

For Phillips, having a research background provides a comfort zone.

"For me as a researcher, this is a great fit. I've been in the lab testing things before, and you use a lot of the same tools and approaches. I understand how to put together a multidisciplinary team."

"But one of the joys of this job is developing a long-term relationship with patients and their families," Phillips says. "When they're so grateful to be here, that's so rewarding. You really are on the frontline of this patient's treatment."

This story originally appeared in Messenger, MD Anderson's quarterly publication for employees, volunteers, retirees and their families.



photo by F. Carter Smith

Be Well Baytown engages the community to prevent cancer

In an effort to improve public health and lower the risk of cancer, Baytown community organizations are partnering with MD Anderson and ExxonMobil to launch Be Well Baytown™.

It's estimated that more than half of all cancers can be prevented by diet, exercise, preventive care, UV radiation protection and tobacco control. Based on this evidence, Be Well Baytown's aim is to promote wellness and stop cancer before it starts by applying the knowledge and tools available today.

MD Anderson is teaming up with Baytown schools, workplaces, social service agencies and others to launch the initiative, which is made possible by a \$10 million gift from ExxonMobil. It is the most comprehensive community-driven health initiative focused on cancer prevention and control in the United States.

Be Well Baytown will provide the community with information, services and activities to promote healthy lifestyles. It plans to reach more than 70,000 residents and will include educational health events, community-wide walking clubs, and youth programming during and after school. More than 475,000 pounds of fresh fruits and vegetables will be delivered to individuals in need over three years.

"By joining forces at work, in classrooms, and all around our community to make our health a priority, we can improve the lives of our families and neighbors," says Dan Helgesen, ExxonMobil Baytown refinery manager.

Be Well Baytown is an initiative of MD Anderson's cancer prevention and control platform, which is part of the institution's Moon Shots Program™, an ambitious effort to reduce cancer deaths by more rapidly developing and implementing advances in prevention, early detection and treatment based on scientific discoveries.

Community organizations participating in Be Well Baytown include:

- Baytown/West Chambers County Economic Development Foundation
- Baytown Chamber of Commerce
- City of Baytown
- Communities In Schools of Baytown
- Goose Creek Consolidated ISD
- Harris County Public Health
- Harris Health System
- Hearts and Hands of Baytown, a ministry of Iglesia Cristo Viene
- Houston Methodist San Jacinto
- Lee College
- Love Network of Baytown
- Our Promise for West Baytown
- United Way of Greater Baytown Area & Chambers County
- YMCA of Greater Houston



photo by Wyatt McSpadden

Husband and wife volunteers know the little things make a big difference

Roland and Jane Moreau, Inpatient volunteers

When Jane Moreau was treated for melanoma at MD Anderson in 2001, she was so impressed by the care she received from doctors and staff that she wanted to do something to give back to the hospital.

So 10 years later, following her retirement from ExxonMobil, Jane began volunteering in the gift shop at the Albert B. and Margaret M. Alkek Hospital.

"I'm always inspired by patients' positive attitudes and courage in accepting their illness," she says. "It helps me to be a better person."

Impressed by the work his wife was doing, Roland Moreau was inspired to join Jane as a volunteer a few years later after his retirement, also from ExxonMobil. Since then, the couple has spent most of their time working as inpatient volunteers, doing what they can to make patients and caregivers more comfortable. Both also help each year with Children's Art Project Pop-Up Shops and the Christmas Day luncheon in Pediatrics.

Faith is a very important part of life for the Moreaus. Each week, Roland, who is a member of the Archdiocese of Galveston-Houston's Catholic Chaplain Corps, visits with Catholic patients. Both say they've always admired the level of faith and spiritual grounding exhibited by many of the patients, families, doctors and nurses at MD Anderson. This admiration led to their donation of a new tabernacle for the Freeman-Dunn Chapel in the Main Building.

Roland says the most enjoyable aspect of volunteering is knowing you can make a small, but important, difference in someone's life each day.

"Our visits with patients and families are always a wonderful learning opportunity and very humbling," Roland says. "If you can bring a smile to someone's face during a visit, that is its own reward."



This nurse, survivor and donor wants to open minds and hearts

By Ronda Wendler photo by Wyatt McSpadden

Melissa Gilhart has traveled the world for two decades, but don't call her a tourist.

Instead of flocking to the Eiffel Tower, Great Wall of China or other “must-see” attractions, Gilhart rents a bicycle or scooter and rides to small towns and rural villages to spend time with the locals.

“I want to absorb their culture, eat their food, and speak their language,” says Gilhart, an MD Anderson nurse. “People are fascinating. I learn so much by going off the beaten path.”

She's sheared wool on a New Zealand sheep ranch, visited an elementary school in Vietnam, and lived with a family in Mexico while learning to speak Spanish. Wherever she goes, Gilhart immerses herself in the communities she visits.

After visiting 55 countries on seven continents, she's come to a conclusion: people everywhere are essentially the same.

“Though our cultures, religious beliefs and languages may differ,” she says, “we're all the same in our shared humanity.”

Gilhart's fascination with people from all walks of life is one of the reasons she came to work at MD Anderson 17 years ago.

“The people who come here from all over the world inspire me,” says Gilhart, who, as a member of the institution's Nursing Resource Pool, works on various units depending on staffing needs. “Many make enormous personal and financial sacrifices to get here, yet they're so grateful to be at MD Anderson.”

Gilhart felt called to help. With assistance from MD Anderson's Development Office, she established a \$20,000 endowment benefiting international patients. For seven years, Gilhart deposited what she could into the endowment until last year it reached the \$20,000 goal amount. The Development Office is now investing the fund and it's growing, bit by bit. Gilhart also has tailored her will to continue supporting the endowment upon her death.

Cancer has impacted Gilhart's life not only as a caregiver, but also a patient. In 2008, she was diagnosed with breast cancer. When her prescribed medication caused severe hot flashes, Gilhart sought help from Meide Liu, M.D., who recently retired as the first acupuncturist to work at MD Anderson's Integrative Medicine Center.

The treatment worked and Gilhart named her philanthropic fund in honor of Liu. She expanded the fund to provide acupuncture not only to international patients, but to all MD Anderson patients who may benefit.

“Studies have shown that acupuncture can help control a number of cancer symptoms and treatment side effects, including nerve pain, fatigue, nausea, insomnia and dry mouth caused by radiation,” explains Gilhart, who's now in remission. “This 2,000-year-old branch of traditional Chinese medicine helped me through my cancer journey. I want others to have the same opportunity.”



photo by Nick de la Torre

Volunteers send patients in the right direction

Valdia Blair and Dan Neskora, Patient Navigators

After a 40-year career in Health Information Management at MD Anderson, Valdia Blair has found volunteer work to be a good fit during retirement. For the past 11 months, she's been helping patients, their friends and families, and hospital guests find their way to appointments and other locations.

She and Dan Neskora often are the first faces people see when they enter the lobby of the Main Building. The duo is very good at first impressions.

Neskora, a retired chemical engineer and seven-year volunteer, says his goal as a navigator is to "make the patient experience as pleasant as possible, and make the sometimes overwhelming size of MD Anderson seem a little smaller."

Blair and Neskora know firsthand about the patient experience – both are cancer survivors who were treated at the institution. They were inspired to volunteer by their own experience as patients.

"When I came to MD Anderson for a second opinion, I remember how my first encounter with a volunteer made me feel," Neskora says. "And I wanted to do the same for others."

What do you enjoy most about volunteering?

Blair: "Meeting the patients and their caregivers, and being available to help in any way I can during their time at MD Anderson."

Neskora: "Meeting and helping patients begin their cancer treatment journey, and then being remembered when they come back during and after their treatment. I've made many friends along the way."

Is there a particular experience that comes to mind when you think about your work as a volunteer?

Neskora: "Yes, one experience in particular stands out in my memory. A gentleman came in the front door, and I could tell immediately he wasn't feeling well.

He'd received cancer treatment elsewhere, then was referred by his physician to MD Anderson. However, the gentleman had no information on the referral. With the help of a staff member, we talked with the gentleman and he shared his diagnosis with us. With that information, I was able to escort him to the appropriate clinic. The following week I saw the gentleman again and he was feeling much better. I would see him every week for several months and each time he was doing better and better. He always thanked me for helping him that first day."

Blair: One day, an unaccompanied patient was dropped off at the R. Lee Clark Clinic. It turned out that his appointment was in another clinic, and he became visibly upset. I reassured him that I would get him to the Mays Clinic, where he needed to be. Once there, he asked if I could stay with him until the nurse called him back, and I did. While we waited, he shared a little about himself and his grandkids. When the nurse called him back, I helped him to the door. As I turned to walk away, he reached for my hand and placed a folded \$20 bill in it. When I told him I couldn't accept it, he asked me why, saying that I had gone above and beyond to help him. I told him, "This is why we're here!"

Of note: In addition to his work as a navigator, Neskora has been active in improving the treatment experience for patients, survivors and caregivers. He's the current chair of the myCancerConnection Steering Committee, which works with staff to develop and guide support programs for patients, survivors and caregivers. He also trains new volunteers to be navigators, and was a mentor to Blair.

Reaching beyond the clinic

Cancer Network broadens and deepens collaborations with members

MD Anderson Cancer Network® is benefiting patients and furthering the mission to end cancer not only through clinical programs, but also with collaborations that advance education, prevention and health policy.

Just as the network is growing and evolving, new outreach opportunities are emerging to serve patients and elevate the standard of care in communities across the nation. MD Anderson's cancer prevention and control platform, a component of the institution's Moon Shots Program™, is making the most of the natural fit with network members, many of which are large hospitals or hospital systems located across the nation.

“Our relationships with network members have matured, and we are now broadening and deepening the reach and impact through cancer control initiatives and policy collaborations within their health systems and local communities,” says Michael T. Walsh Jr., executive director of cancer prevention and control within the Moon Shots Program. “We are learning together about how to successfully advance our shared mission, as well as helping to level the playing field with programs that directly address quality of care, prevention and practice in a community.”

Alongside a suite of quality improvement initiatives targeting clinical achievements in cancer control standards of care, MD Anderson teams up with network members such as MD Anderson Cancer Center at Cooper, a fully operational and clinically integrated partner located in Camden, New Jersey. The cancer center shared information with lawmakers in that state debating legislation that ultimately raised the minimum age for buying tobacco products from 18 to 21 years old. With guidance from MD Anderson, which served as a clinical and scientific resource for a statewide coalition on the same issue in Texas, Cooper provided its expertise on teen smoking and the risks of tobacco to a grassroots and bipartisan legislative effort to raise the minimum age to buy tobacco by three years. In July 2017, then-Governor Chris Christie signed a bill that made New Jersey the third state in which minimum legal sale age of tobacco products was raised to 21, an important public health policy measure.

“Cooper was pleased to join with our partner, MD Anderson, in this effort to raise the purchasing age for tobacco products from 18 to 21 as one way to prevent the devastating, long-term health effects often experienced by those who begin smoking or using tobacco when they are young,” says Kevin O’Dowd, senior executive vice president and chief administrative officer at Cooper University Health Care.

Just a few months earlier, Oklahoma Governor Mary Fallin approved a bill prohibiting the use of ultraviolet (UV) tanning beds or other devices by those under 18. In 2015, MD Anderson collaborated with certified member St. John’s Health System, now part of Oklahoma Cancer Specialists and Research Institute, and the American Cancer Society Cancer Action Network, to promote skin cancer prevention in a public policy forum in Tulsa.

“Based on our successful collaborations with network members on a variety of initiatives, we want to continue to look for ways to improve public health beyond the walls of the clinic,” says Mark Moreno, vice president for Government Relations and co-leader of the cancer prevention and control platform. “The members of the network are natural teammates in this effort, given our shared mission to end cancer.”

TEAMING WITH OTHERS TO MAKE QUALITY CARE MORE ACCESSIBLE

MD Anderson Cancer Network® advances the institution’s mission of eliminating cancer by collaborating with community hospitals and health systems to improve the quality of care nationwide.

The network provides expertise ranging from quality assurance and best practices to full clinical integration to four membership types.

MD Anderson is the nation’s leading cancer center, and members benefit from the institution’s unparalleled commitment to cancer care, research, education and prevention, as well as access to evidence-based treatment protocols and its unique multidisciplinary approach to treating patients.

17

Certified members
in 14 states

7

Partners
in 5 states

3

Associates
in 3 countries

2

Affiliates
in 2 countries

>100 million people
now have access to an
MD Anderson location

>52%
of new cancer patients in the U.S.
have access to a partner or certified
Network member

Grassroots movement has grown into a very big source of comfort and support for patients

As a longtime MD Anderson volunteer and patient advocate, the late George Vietor realized what a big difference little things can make in the life of a patient.

In 1991, he spearheaded the creation of an endowment to fund a wide range of programs and amenities not traditionally supported by cancer center funds.

The Volunteer Endowment for Patient Support – VEPS for short – has granted many requests over the years for programs and resources that bring comfort and support to patients and their families, including blanket warmers in clinics, patient education materials and survivorship celebrations.

“People expect leading-edge medical care when they come to MD Anderson. They’re pleasantly surprised by the ‘extras’ that enhance the patient experience,” says Frances Snipes, a director in Volunteer Services and Merchandising, which administers VEPS.

Through donations and fundraising efforts, the endowment has grown to \$3.9 million and distributed more than \$3.1 million for patient amenities and programs to date.

Each quarter, the interest generated from the endowment is used to fund initiatives for patients.

A few of the projects funded in 2017 include:

- Young adult support group
- Patient and caregiver snack-chat social hour for lymphoma and myeloma inpatients
- Breast reconstruction awareness (BRA) workbook and education
- Inpatient holiday luncheon and gift distribution
- Chapel flowers to enhance prayer, worship and meditation
- Caregiver support programs
- Education binders for newly admitted hematology patients
- myCancerConnection cancer survivorship conference
- Lodging, transportation, meals and parking assistance for qualifying patients

VEPS began as a grassroots movement funded by cookbook sales, fashion shows and art auctions. The annual VEPS luncheon, held each spring, is now the endowment’s principal fundraiser.

This year’s luncheon co-chairs Rahul Agrawal, Caroline Brown and Rachel Cruz are cancer survivors who share their thoughts about supporting VEPS and volunteering at MD Anderson:

Rahul Agrawal

“During my time as a cancer patient at MD Anderson, I learned what a difference small comforts can make. The heated blankets were the best – everyone loved them. I’m pleased to now be supporting the program that provided funding for those blankets and so much more. Patients come here from all over the world to seek care. The little things help them feel closer to home.”

In addition to co-chairing the 2018 VEPS Luncheon, Agrawal provides one-on-one support to cancer patients through myCancerConnection, where newly diagnosed patients are connected with survivors who have “been there.” He is a myCancerConnection Steering Committee member and volunteers with myCancerConnection’s Cancer180 program for young adult survivors ages 18-39.



photo by Adolfo Chavez III



photo by Wyatt McSpadden

Caroline Brown

“When I was a patient at MD Anderson, I saw firsthand how important volunteers are to patients, caregivers and the medical staff. I decided the best way to give back to the cancer center that gave me so much was to volunteer. It’s a rewarding experience that I look forward to every week. Cancer is no easy feat – our patients’ strength, resilience and determination motivate me to volunteer.”

Besides serving as a 2018 VEPS Luncheon co-chair, Brown is an inpatient volunteer in the evenings, and a member of MD Anderson’s Advance Team, a volunteer leadership board whose members initiate philanthropic and awareness events to advance the institution’s mission to eliminate cancer.

Rachel Cruz

“I know how overwhelming it can be to intellectually and emotionally process a cancer diagnosis, then to navigate your way through treatment. If I can educate new patients and caregivers about resources available to them, or make their cancer journey less daunting by sharing my experiences, I feel like I’ve made a contribution.”

Not only is Cruz serving as a 2018 VEPS Luncheon co-chair, but she also is an Advance Team member, myCancerConnection and Coffee Cart volunteer, and past chair of the myCancerConnection Steering Committee and the myCancerConnection Cancer Survivorship Conference.

Mulva Family Foundation gift funds melanoma and prostate research

The Mulva Family Foundation has given \$25 million to melanoma and prostate cancer research at MD Anderson under the direction of Patrick Hwu, M.D., chair of Melanoma Medical Oncology, and Christopher Logothetis, M.D., chair of Genitourinary Medical Oncology.

The gift is part of a \$75 million grant that includes \$50 million to create the Mulva Clinic for the Neurosciences at the Dell Medical School at The University of Texas at Austin.

Jim Mulva, chair of the MD Anderson Cancer Center Board of Visitors and a UT Austin graduate, is past chairman and CEO of ConocoPhillips. Miriam Mulva is a graduate of St. Norbert College near Green Bay, Wisconsin. The Mulvas are Texas residents and split their time between Austin and Green Bay.

The Mulva Family Foundation has been a consistent supporter of UT Austin and MD Anderson. Medicine, education, youth and the Catholic Church are its primary initiatives. The family’s substantial commitments to the university include \$60 million to the McCombs School of Business and the Cockrell School of Engineering and \$15 million to help build the new Liberal Arts Building. They have given more than \$5.6 million to MD Anderson. The Miriam and Jim Mulva Conference Center at MD Anderson is named in recognition of a \$5 million gift to the Miriam and Jim Mulva Fund for Melanoma Research.



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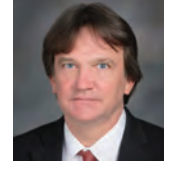
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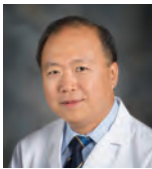
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* current as of March 1, 2018

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The MD Anderson Cancer Center Board of Visitors is an appointed board of people committed to helping MD Anderson achieve its mission of eliminating cancer. Board programs emphasize private fund development, public relations and outreach on behalf of the institution.



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Over \$1 million raised to 'Give Cancer the Boot'

More than 5,000 cancer patients and their family members, volunteers, friends, faculty members, staff and supporters turned out for MD Anderson's second annual Boot Walk to End Cancer® in November.

The 1.2 mile walk through the Texas Medical Center, which featured plenty of music, food and entertainment, raised more than \$1 million to support cancer research and programs at the cancer center.

The top employee fundraiser was Albert Koong, M.D., chair of Radiation Oncology, and the team that raised the most money was the IBC Wranglers. Breast Medical Oncology's Jie Willey, an administrative director of protocol research, and Angela Alexander, Ph.D., a clinical studies coordinator, led the Wranglers.



BOOT
WALK
to #endcancer®

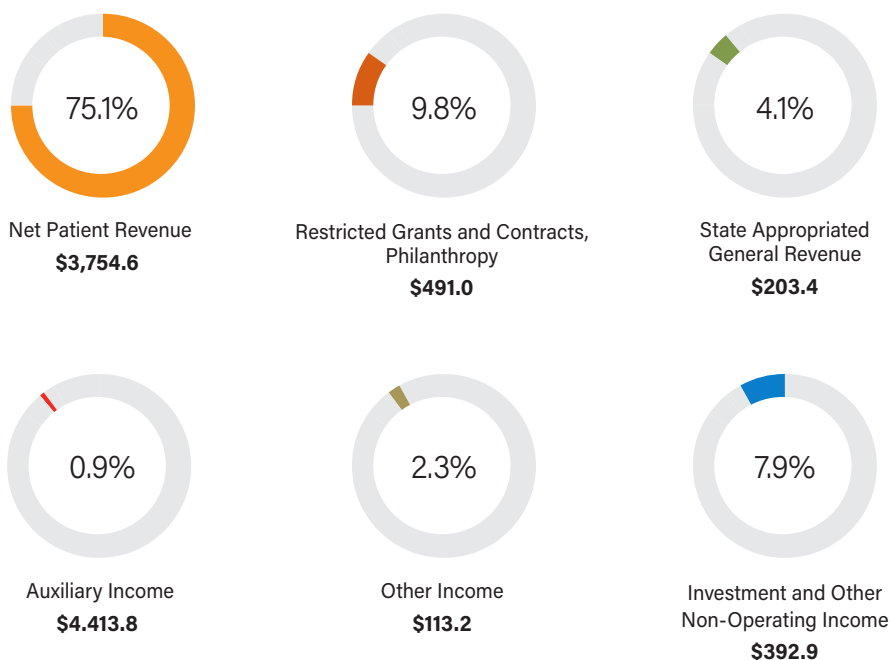
mdanderson.org/boot

Fiscal Year 2017
financial and statistical data
Sept. 1, 2016 - Aug. 31, 2017

Sources of revenue

	FY 2013	FY 2014	FY 2015	FY 2016	FY 2017
Patient Revenue					
Gross patient revenue (Includes inpatient, outpatient and professional services)	\$6,582,112,827	\$6,994,996,215	\$7,567,179,285	\$7,571,426,899	\$8,214,974,402
Deductions from gross patient revenue ¹	3,403,247,816	3,659,313,782	3,935,319,324	4,044,324,615	4,460,335,552
Net patient revenue	\$3,178,865,011	\$3,335,682,434	\$3,631,859,960	\$3,527,102,284	\$3,754,638,850
Other Revenue					
Restricted grants and contracts, philanthropy	\$505,144,559	\$421,761,275	\$402,702,183	\$466,883,217	\$491,038,777
State-appropriated general revenue	154,562,093	185,393,182	187,350,746	201,848,484	203,439,111
Auxiliary income ²	40,674,618	41,502,690	44,808,473	42,462,462	44,137,660
Other income ³	75,564,178	99,702,455	107,422,200	112,515,085	113,187,342
Investment and other non-operating income	180,428,432	328,881,907	121,624,475	129,632,830	392,901,020
TOTAL REVENUE	\$4,135,238,891	\$4,412,923,943	\$4,495,768,037	\$4,480,444,361	\$4,999,342,760

Sources of revenue (in millions)



¹ Amounts discounted from established rates as a result of agreements with third-party payors, including Medicare, Medicaid and insurance companies. Also includes deductions associated with indigent care and bad debt.

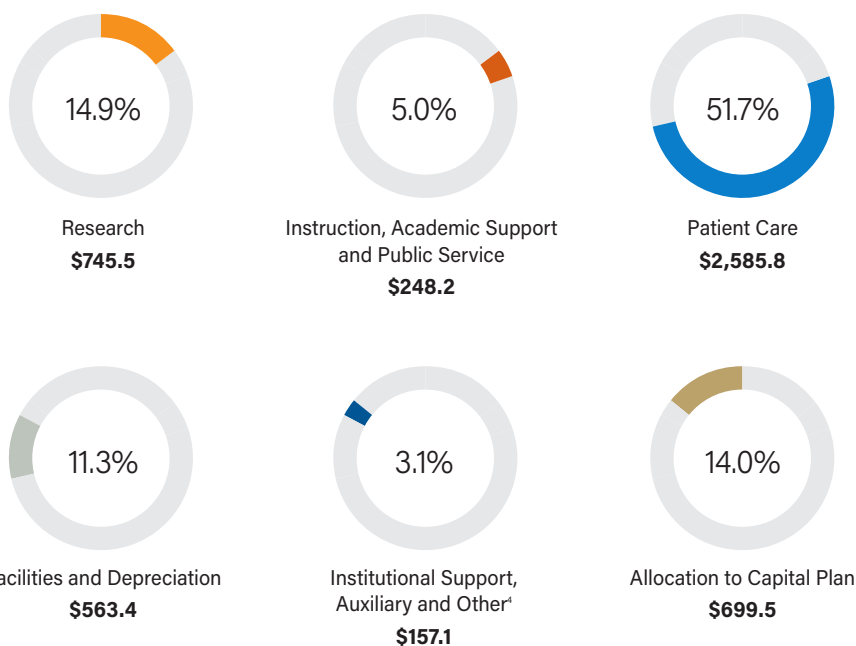
² Funds received from parking fees, valet services, dining facilities, hotel charges, gift shop sales and vending-machine sales

³ Includes tuition and student fees, Children's Art Project sales, management fees and other sources.

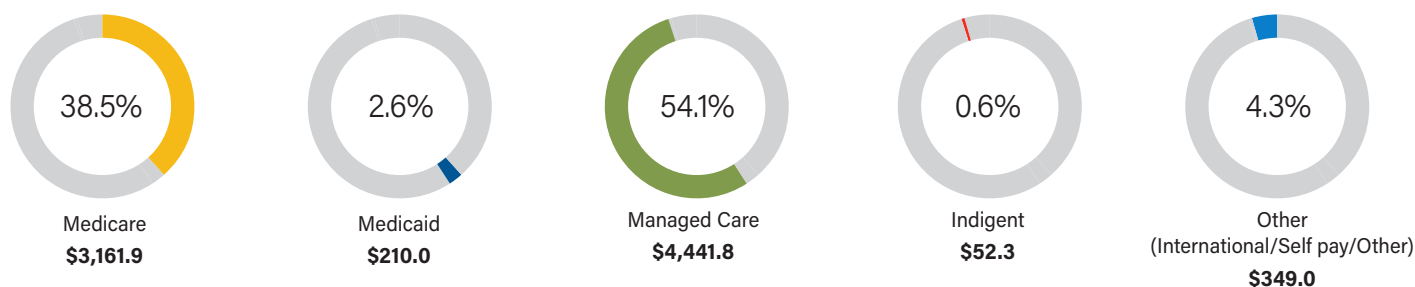
Uses of revenue

	FY 2013	FY 2014	FY 2015	FY 2016	FY 2017
Research	\$588,664,553	\$631,944,987	\$668,243,371	\$687,940,063	\$745,481,237
Instruction, academic support and public service	209,633,502	195,958,981	225,871,577	234,488,229	248,155,843
Patient care	2,013,554,826	2,055,617,566	2,369,972,993	2,642,145,329	2,585,835,231
Facilities and depreciation	471,935,938	486,793,306	508,973,014	550,277,895	563,364,679
Institutional support, auxiliary and other ⁴	305,390,616	312,865,408	155,828,553	158,060,132	157,051,220
Allocation to capital plan (For future projects to replace and improve facilities and technology)	546,059,455	729,743,695	566,878,529	207,532,714	699,454,551
TOTAL EXPENSES	\$4,135,238,891	\$4,412,923,943	\$4,495,768,037	\$4,480,444,361	\$4,999,342,760

Uses of revenue (in millions)



Gross revenue by payor classification (in millions)

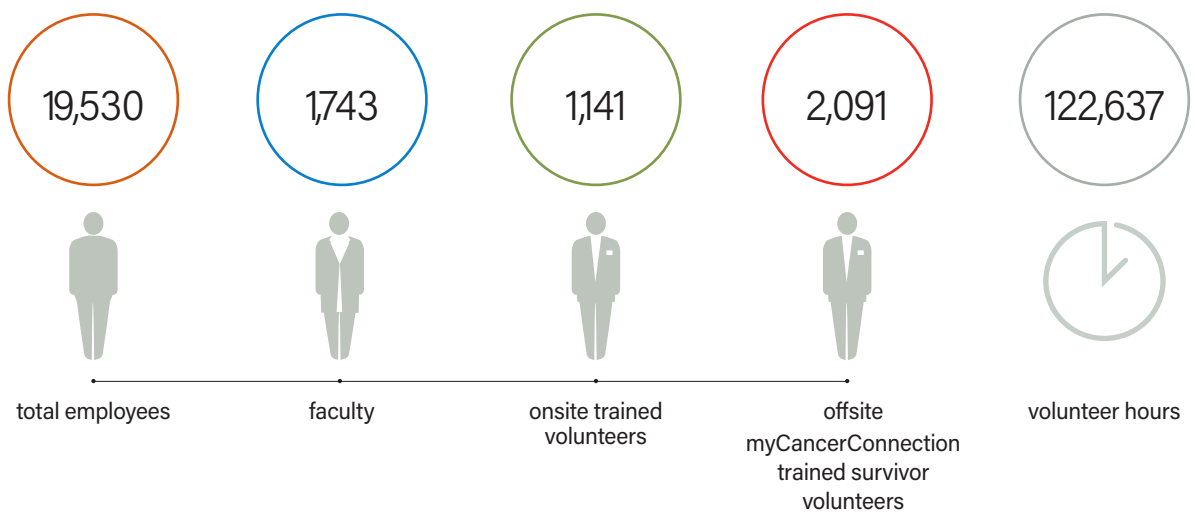


⁴ Includes support for parking, food and gift shop services, as well as general institutional support (e.g. information technology, human resources, administration, development activities, etc.)

Clinical profile

	FY 2013	FY 2014	FY 2015	FY 2016	FY 2017
Hospital admissions	27,905	27,761	28,167	27,391	28,793
Patient days	202,553	202,636	202,483	198,080	202,411
Average daily census	569	571	574	561	577
Average length of stay	7.3	7.3	7.2	7.2	7.0
Average number of inpatient beds	635	654	665	661	681
Outpatient clinic visits, treatments, procedures	1,338,706	1,363,008	1,440,684	1,404,329	1,441,403
Pathology/laboratory medicine procedures	11,718,405	12,005,766	12,334,917	12,073,679	12,700,333
Diagnostic imaging procedures	501,887	523,297	530,590	524,044	574,018
Surgery hours	70,221	69,506	69,987	67,936	70,460
Total active clinical protocols	1,065	1,101	1,197	1,202	1,255

Workforce



MD Anderson provided more than **\$287.3 million** in uncompensated care to Texans with cancer in FY17.*

**This figure includes unreimbursed costs of care for patients who either have no insurance or are underinsured, or whose care was not fully covered by government-sponsored health programs.*

Total philanthropic gift support by type

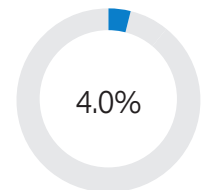
Cash gifts	Amount
Corporations	\$13,317,483
Foundations	\$22,691,342
Individuals	\$45,058,145
Organizations	\$3,722,662
Trusts and estates	\$10,484,353
Subtotal	\$95,273,985

Pledge gifts	Amount
Corporations	\$7,035,381
Foundations	\$32,241,584
Individuals	\$44,404,270
Organizations	\$17,080,395
Trusts and estates	\$47,528,052
Subtotal	\$148,289,682

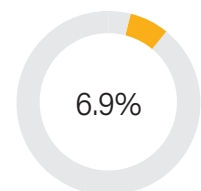
Gifts-in-kind	Amount
Corporations	\$193,006
Foundations	\$60
Individuals	\$22,028
Organizations	\$9,120
Subtotal	\$224,214

TOTAL	\$243,787,881
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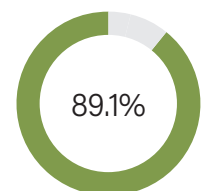
Total philanthropic gift support by purpose



Education/Prevention/Patient Assistance
\$9,751,515



Annual/Unrestricted/Undesignated¹
\$16,920,040



Research²
\$217,116,326

¹ These dollars fund institutional peer-reviewed research.

² Donor-targeted gifts to research conducted in all mission areas.

Sources of research expenditures

	FY 2013	FY 2014	FY 2015	FY 2016	FY 2017
External funding for research					
Federal grants, contracts	\$182,970,502	\$158,986,303	\$161,170,908	\$155,043,499	\$167,061,117
Private industry grants, contracts	65,579,036	75,307,463	81,076,353	155,043,499	127,758,909
Philanthropy, foundations	101,642,898	147,016,586	172,412,727	166,374,314	191,181,214
Total external funding	\$350,192,436	\$381,310,352	\$414,659,988	\$410,872,268	\$486,001,240
State funding allocated for research					
State-appropriated general revenue	\$11,776,785	\$13,636,669	\$13,658,113	\$14,991,640	\$15,021,736
Tobacco settlement receipts	5,837,249	11,175,016	10,227,690	12,188,092	13,143,222
CPRIT	24,262,525	25,072,890	32,049,453	40,227,040	53,292,732
Total state funding	\$41,876,559	\$49,884,575	\$55,935,256	\$67,406,772	\$81,457,690
Internal funding allocated for research					
Hospital operating margins	\$182,770,342	202,607,346	198,607,568	193,071,901	187,850,299
Institutional grants ¹	95,730,271	102,391,157	111,374,655	115,938,206	88,864,952
Total internal funding	\$278,500,613	\$304,998,503	\$309,982,223	\$309,010,107	\$276,715,251
TOTAL RESEARCH EXPENDITURES	\$670,569,608	\$736,193,430	\$780,577,467	\$787,289,147	\$844,174,182

Education profile

	FY 2013	FY 2014	FY 2015	FY 2016	FY 2017
Clinical residents, fellows	1,231	1,276	1,507	1,693	1,755
Research trainees	1,743	1,853	1,890	1,847	1,779
Observers, visitors, special programs	507	452	752	838	906
Nursing trainees	1,306	1,238	1,352	1,499	1,506
Student programs participants	1,396	1,204	817	810	806
School of Health Professions students	291	318	303	317	339
TOTAL TRAINEES	6,474	6,341	6,621	7,004	7,091

¹Philanthropic donations to the institution internally designated to support research and PRR funds internally allocated to support research activities.

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LOCATIONS

MD Anderson has Houston-area locations in the Texas Medical Center, Bay Area, Katy, Sugar Land, The Woodlands, Bellaire and West Houston (diagnostic imaging), Memorial City (surgery) and The Woman's Hospital of Texas (gynecologic oncology). MD Anderson physicians also provide cancer care to Harris County's underserved patients at Lyndon B. Johnson Hospital. In addition, there are two research campuses in Bastrop County, Texas. The institution also has developed a network of national and international locations.

MD ANDERSON CANCER NETWORK

www.mdanderson.org/cancernetwork

PARTNERS

- Banner MD Anderson Cancer Center (Gilbert, Arizona)
- MD Anderson Cancer Center at Cooper (Camden, New Jersey)
- Summit Medical Group MD Anderson Cancer Center (Berkeley Heights, New Jersey)
- Baptist MD Anderson Cancer Center (Jacksonville, Florida)
- Scripps MD Anderson Cancer Center (San Diego, Calif.)
- UT Health San Antonio MD Anderson Cancer Center
- UT Health Northeast's Cancer Treatment and Prevention Center (Tyler, Texas)

CERTIFIED MEMBERS

- 17 health systems and hospitals in 14 states

ASSOCIATES

- Hospital Israelita Albert Einstein (São Paulo)
- MD Anderson Cancer Center Madrid
- Vehbi Koc Foundation American Hospital (Istanbul)

AFFILIATES

- MD Anderson Radiation Treatment Center at American Hospital (Istanbul)
- Presbyterian MD Anderson Radiation Treatment Center (Albuquerque, New Mexico)

EDITOR

Andy Olin, *program director, Public Relations*

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For information on patient services at MD Anderson, call askMDAnderson at 877-632-6789, or log on to www.mdanderson.org/ask.

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