



Sept. 6, 2018

MD Anderson
Children's Cancer Hospital
FY19 Annual Report



THE UNIVERSITY OF TEXAS
**MD Anderson
Cancer Center**
Children's Cancer Hospital®

Table of contents

Letter from division leaders	3
By the numbers	4
Expanding our capabilities	6
Adolescents and young adults	12
Energy balance	14
Accommodating more patients	17
Supporting our patients	18
Medical training	23
Research training	24
Advancing the science	25
Clinical trials	25
Spotlight on bench research	26
Acknowledgments	28



April 7, 2020



On the cover

Two-years-old at the time, Chenxi had fun at the Teddy Bear Clinic, an annual event conducted by our Support Services team that allows patients to switch roles for the day. The children perform exams, take vital signs, prescribe medications, and even do some procedures on Build-A-Bear teddy bears, provided by one of our generous donors, Bennett's Bears. Faculty, nurses, pharmacists, radiology technicians and others sign up months in advance each year to participate.



Richard Gorlick, M.D.
Division Head



Lisa Hafemeister, M.H.A., F.A.C.H.E.
Executive Director and
Division Administrator

The mission of the Children's Cancer Hospital is to bring hope for the future to pediatric patients and their families through clinical expertise, cutting-edge therapies, research, training tomorrow's health care professionals, and support programs for pediatric and young adult patients and their families. We are honored to present this annual report, highlighting some of our greatest achievements of fiscal year 2019 (FY19). *Please note: all photos published here were taken prior to COVID-19.*

During the COVID-19 pandemic, our faculty, staff and the patients we serve have adjusted to our new environment. We have learned to work differently, by providing compassionate virtual care options to patients, videoconferencing for divisional meetings, and by employing social distancing precautions while on campus to keep our patients, employees and community safe. Our extraordinary teamwork has continued to demonstrate our core values through compassionate acts of kindness, such as the inspiring chalk art for frontline workers displayed on our building entrance sidewalk. We are truly #OneMDAnderson. Together we are taking a stepwise, informed approach to safely recover our operations.

This report describes the sociodemographic landscape of our patients. It is amazing how many children we have been able to reach across Texas and around the world. We describe one-of-a-kind treatments and clinical trials, like the use of stem cell therapy to treat aggressive pediatric cancers; innovative multidisciplinary research, such as energy balance studies investigating the scientific link between physical activity, nutrition, and cancer; and how we are leading cancer care for unique populations like adolescents and young adults.

With the rapidly evolving health insurance system, navigating the health care system has become increasingly difficult for some families. We share the thoughtful steps we have taken to facilitate access to care for as many children as possible through the introduction of a patient navigator.

As a comprehensive cancer center, our care extends beyond medical services. We outline the many supportive programs the Children's Cancer Hospital uniquely offers. From camps to a comprehensive in-hospital accredited school, our supportive care and integrative medicine program delivers palliative care and symptom management through traditional and holistic treatment, as well as alternative options throughout the cancer continuum.

Our laboratories and investigators are committed to discovering breakthroughs in pediatric cancer detection, treatment and prevention of recurrence. We are training the next generation of pediatric oncologists, physician-scientists and translational researchers to accelerate our efforts. Hand in hand with our growth in expertise and specialized services provided, we have expanded our hospital to include a new Adolescent and Young Adult (AYA) clinic and are making plans to create a state-of-the-art pediatric gym.

Lastly, and most importantly, none of our accomplishments would be possible without the continued support of our donors, the Children's Cancer Hospital Advisory Group, and the commitment from our faculty and staff to deliver service excellence. Thank you for your dedication. Please enjoy this report, and we look forward to working together with you to become the premier pediatric cancer center in the world.

Sincerely,

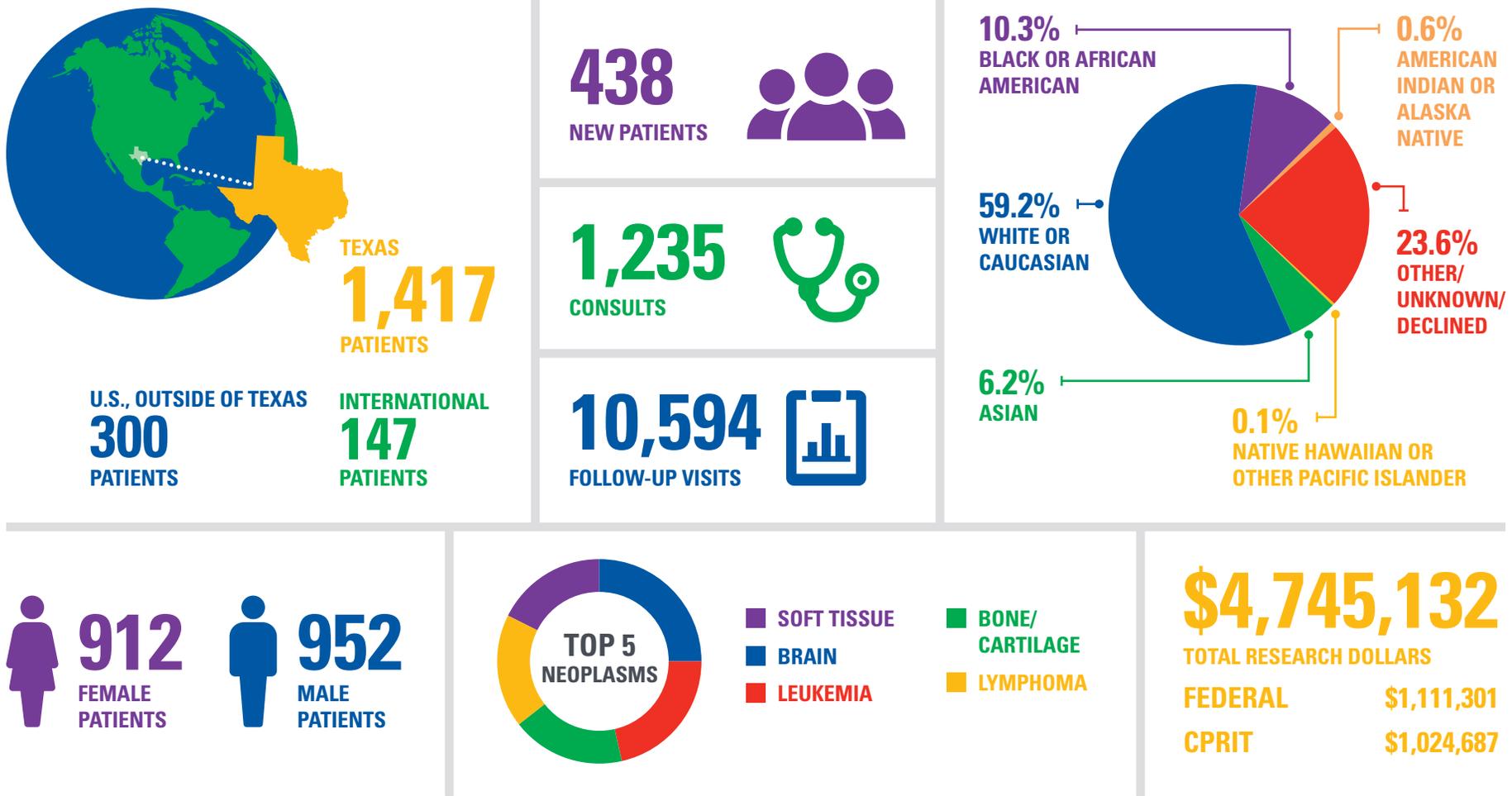
Richard Gorlick, M.D.
Division Head

Lisa Hafemeister, M.H.A., F.A.C.H.E.
Executive Director and Division Administrator

By the numbers

Clinical activity and gross patient revenue has significantly improved by more than 25% in the last two years due to growth in many of the clinical areas. Creation of the Adolescent and Young Adult program, growth in Stem Cell Therapy, Neurofibromatosis and Supportive Care services, as well as an increase of infusion services in the outpatient

Pediatric Ambulatory Treatment Center account for the most growth. The Pediatric inpatient service has developed extensively and has more than doubled in size over the course of the last three years, mostly attributed to an enhanced Stem Cell Therapy program and Supportive Care services.



In the news

Clinical care, research, camps and patient programs at the Children’s Cancer Hospital were in the spotlight in FY19. Whether highlighted in MD Anderson’s publications, or in stories produced by local or national media outlets, people heard a lot about us.

PUBLICATIONS



15

PRINT
(newspaper/
magazine/trade)

MEDIA STORIES



120

BROADCAST*
(television and radio)

* CNN aired Prom Party Palooza on 89 affiliates, reaching approximately **1.88 million viewers**



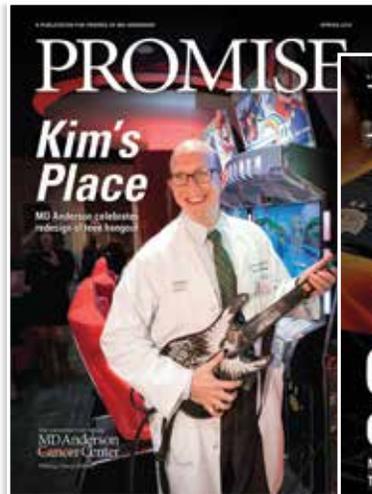
136

**ONLINE
STORIES***

* Several stories appeared in multiple online outlets

19

HEADLINES IN MD ANDERSON PUBLICATIONS*



* Four covers of *Promise Magazine* — we were on the cover every quarter

Our Stem Cell Transplantation and Cellular Therapy Program

The Children's Cancer Hospital is at the forefront of clinical practice and research discoveries in Pediatric Stem Cell Transplantation and Cellular Therapy (SCT-CT). Our program is led by internationally recognized, highly published experts who are dedicated to improving patient outcomes worldwide. We offer clinical trials for cutting-edge chemotherapies, biologic agents, and immunotherapies, some of which are not available anywhere else in the country for these age groups.

Kris Mahadeo, M.D., M.P.H., associate professor of Pediatrics and section chief, leads the program. He partners with **Joan O'Hanlon Curry, M.S., R.N., A.P.R.N., C.P.N.P., C.P.O.N.**, administrative director of Pediatric Clinical Services and president of the Association of Pediatric Hematology/Oncology Nurses (APHON), to coordinate a dream team of physicians, advanced practice providers, clinical nurses, transplantation coordinators and pharmacists caring for patients with leukemia, lymphoma, solid tumors as well as some genetic diseases and immune deficiencies.



Kris Mahadeo, M.D., M.P.H.
Associate Professor of Pediatrics
and Section Chief



Demetrios Petropoulos, M.D.
Professor of Pediatrics



Sajad Khazal, M.B. Ch.B.
Assistant Professor of Pediatrics



Priti Tewari, M.D.
Associate Professor of Pediatrics



**Joan O'Hanlon Curry,
M.S., R.N., A.P.R.N., C.P.N.P., C.P.O.N.**
Administrative Director of Pediatric Clinical Services

Expanding donor and eligibility options for pediatric patients

Our transplantation ensemble of experts can manage the most complex patients using all currently available donor sources — matched sibling, matched unrelated, umbilical cord blood, expanded cord blood, and haplo-identical (half-matched relatives) donors. We offer personalized regimens that balance a patient’s disease status and their co-morbid conditions, with the option of novel reduced-intensity transplant protocols when appropriate. This allows virtually every patient with a transplant indication to proceed. More information about all of the study numbers listed in bold is available at www.mdanderson.org/CCHclinicaltrials.

Stem cell transplant clinical studies:

- Phase II pilot trial to estimate survival after a non-total body irradiation (TBI) based conditioning regimen in patients diagnosed with B-acute lymphoblastic leukemia (ALL) who are pre-allogeneic hematopoietic cell transplantation (HCT) next-generation-sequence (NGS) minimal residual disease (MRD) negative (PBMTC ONC1701). **2018-0964**
- Phase II Head Start4: newly diagnosed children (<10 y/o) with medulloblastoma and other CNS embryonal tumors. **2018-0840**
- Phase I/II study of gemcitabine/clofarabine/busulfan and allogeneic transplantation for aggressive lymphomas. **2012-0506**
- Prospective registry of pediatric cellular therapy patients at risk for endothelial dysfunction, sinusoidal obstruction syndrome and/or multi-organ dysfunction syndrome (MODS). **PA19-0758**
- Prospective multi-center study to understand the contribution of vascular endothelial dysfunction towards morbidity and mortality among children and adolescents/young adults (AYA) undergoing HCT/CT. **PA19-0756**
- Additionally, we are the only site to offer these post-stem cell transplantation maintenance studies for leukemia patients:
 - Phase II study of blinatumomab maintenance following allogeneic hematopoietic cell transplantation for patients with acute lymphoblastic leukemia. **2015-0576**
 - Phase II study of venetoclax in combination with azacitidine in the post-transplant setting for acute myeloid leukemia, T cell acute lymphoblastic leukemia, and mixed phenotype acute leukemia. **2019-0353**

Chimeric antigen receptor T cells and other immune effector cell therapies

Chimeric antigen receptor (CAR) T cell therapy is an immunotherapy that alters a patient's own T cells, making them better able to recognize and attack cancer cells, while leaving healthy cells alone. T cells are harvested from the patient, just like in stem cell transplantation, but are re-engineered in a lab before being returned to the patient who has undergone high-dose chemotherapy and/or radiation. These and other Immune Effector Cell (IEC) protocols are located under the Foundation for the Accreditation of Cellular Therapy (FACT)-certified IEC center. It is an integral component of the MD Anderson CARTOX Program. We believe these trials will have superior outcomes and significantly less toxicities.

While exciting response rates and long-lasting remissions have emerged from clinical trials of CAR T cells in patients with B cell malignancies, the option is not available for everyone. Some patients do not have enough T cells to be eligible for this alternative therapy or they cannot forgo treatment for the time it will take to generate enough of these cells. To get around these barriers, MD Anderson investigators are turning to natural

killer (NK) cells to expand the use of CAR-directed therapy in patients with B cell malignancies. Early results of a trial examining cord-blood derived CAR-engineered NK (CAR NK) cells, study **2016-0641**, are promising.

Moreover, our program offers an array of cytotoxic lymphocytes (CTLs) engineered to fight against infections which may be commonly seen among immune-compromised patients and/or patients with post-transplant lymphoproliferative disorder — following stem cell transplantation or solid organ transplantation. We also offer mesenchymal stem cells which are increasingly recognized as an important tool in regenerative medicine for patients with graft-versus-host-disease (GVHD), and there are emerging clinical trials for rescue of organ failure after cytotoxic therapies.

Our SCT-CT faculty published the first comprehensive management guidelines for pediatric patients receiving this therapy in the August 8, 2018 *Nature Reviews*. Key aspects of these recommendations were adopted by the American Society for Transplantation and Cellular Therapy (ASTCT).

CAR T and CAR natural killer (CAR NK) possibilities

CAR T therapy has many benefits for select hematologic cancers.

.....
A Phase I/II study at MD Anderson is investigating another way to get around limitations of CAR T therapy.

.....
CAR NK cells offer an off-the-shelf approach with no need to collect cells from a patient, shorter manufacture time and so far, less toxic side effects such as cytokine release syndrome.

Chimeric Antigen Receptor (CAR) Therapy

- Phase I/II dose escalation study of umbilical cord blood-derived CAR-Engineered NK Cells in conjunction with lymphodepleting chemotherapy in patients with relapsed/refractory b-lymphoid malignancies. **2016-0641**
- Phase I/II multi-center study evaluating the safety and efficacy of KTE-X19 in pediatric and adolescent subjects with relapsed/refractory bs-precursor acute lymphoblastic leukemia or relapsed/refractory b-cell non-Hodgkin Lymphoma (ZUMA-4). **2016-0316**
- Managed access program (MAP) to provide access to CTL019, for acute lymphoblastic leukemia (ALL) or diffuse large b-cell lymphoma (DLBCL) patients with out of specification leukapheresis product and/or manufactured tisagenlecleucel out of specification for commercial release. **2018-0744**

Cytotoxic Lymphocytes (CTLs)

- Expanded access protocol for tacelecleucel for patients with Epstein-Barr virus-associated viremia or malignancies for whom there are no appropriate alternative therapies. **2019-0079**
- Phase III multicenter, open-label, trial of ATA129 for allogeneic hematopoietic cell transplant subjects with Epstein-Barr Virus-associated post-transplant lymphoproliferative disease after failure of rituximab (MATCH Study) (Atara 301). **2017-0769**
- Phase III multicenter, open label trial of ATA129 for solid organ transplant subjects with Epstein-Barr Virus-associated post-transplant lymphoproliferative disease after failure of rituximab or rituximab and chemotherapy (ALLELE Study) (Atara 302). **2017-0771**

- Phase II study of most closely HLA-matched allogeneic CMV specific cytotoxic T-lymphocytes (CTL) to treat CMV infection after hematopoietic stem cell transplantation (HSCT). **2013-0657**
- Phase II study assessing the effect of BK specific CTL lines generated by ex vivo expansion in patients with BK virus infection and JC virus infection. **2014-0279**
- Phase I study of the administration of off-the-shelf, expanded, most closely HLA matched, third party adenovirus specific T cells for therapy of adenovirus related disease in immunocompromised patients. **2017-0350**

Other Immune Effector Cell Therapies

- Phase I study of ex-vivo expanded allogeneic NK cells for the treatment of pediatric solid tumors. **2017-0085**
- Phase II clinical study to assess efficacy and safety of LN-145 (Autologous Centrally Manufactured Tumor Infiltrating Lymphocytes) across multiple tumor types. **2017-0672**
- Anti-viral central memory CD8 veto cells in haploidentical hematopoietic stem cell transplantation. **2018-0221**

Sharing knowledge and changing clinical standards of excellence

Pediatric SCT-CT leaders convened an international panel of multidisciplinary experts in February 2019 to evaluate the current standards and to create the first international guidelines for the diagnosis, severity grading and treatment of sinusoidal obstructive syndrome, a potentially life-threatening complication among children and young adults. The recommendations were later published in the Dec. 6, 2019 issue of *The Lancet Haematology*.

The recommendations support universal adoption of pediatric diagnostic guidelines for sinusoidal obstruction syndrome — also known as hepatic veno-occlusive disease — proposed by the European Society for Blood and Marrow Transplantation (EBMT), and provide direction for implementing standardized practices across the globe. The specialists also proposed supplemental use of age-appropriate organ-specific toxicity criteria for severity grading and provide prophylaxis and treatment considerations among pediatric patients.

Commitment to superior patient outcomes and quality assurance

We are committed to achieving superior quality outcomes for every transplant patient, all of our SCT-CT specialists are FACT inspectors, and most hold leadership roles in FACT committees related to clinical standards, quality management and education.

Demetrios Petropoulos, M.D., professor of Pediatrics, serves as the Patient Safety and Quality Officer for Pediatrics. He supports the development and implementation of institutional safety and quality plans and leads practice-changing initiatives for the reliable delivery of high-quality and safe pediatric patient care.

Kaylyn continues to explore life following CAR T therapy and stem cell transplantation

Fourteen-year-old Kaylyn White has experienced a lot in her young life, including becoming one of the first pediatric patients at MD Anderson approved to receive CAR T therapy for refractory B-cell ALL. Bone and joint pain, random nosebleeds, weight loss, and fatigue led to community doctor visits and lab tests that found her white blood cell count was extremely high. The Harris County resident was rushed to MD Anderson's emergency center, where she was diagnosed. Kaylyn received chemotherapy but was later found to be resistant. "We know this lack of response to chemo translates into a poor prognosis," said **Sajad Khazal, M.B Ch.B.**, assistant professor and transplantation specialist.

The family said yes to tisagenlecleucel, known as CAR T, a recent groundbreaking therapeutic option that is considered a major advance for treating patients 25 and younger for the disease that challenged Kaylyn. While Kaylyn's cells were harvested — which typically takes four to six weeks — she remained in the hospital and continued to receive chemotherapy so her cancer would not spread. Just 30 days after infusion with her amped-up T cells, tests showed she had no evidence of disease, and her leukemia was in complete remission.

"It was a blessing to be able to have access to this procedure," said Lesley White, Kaylyn's mother. "This therapy did its job and the doctors helped save our daughter's life." "The teenager later received a leukemia remission consolidation with allogeneic hematopoietic stem cell transplantation to lower her risk of future relapse," said Khazal.

Kaylyn is now enjoying every day as best as she can and has participated in many Children's Cancer Hospital events, including the 2019 Celebrity Chef Cooking Demo, where she learned to make mushroom sliders with Chef Jehn Ngo, a local culinary expert.



Thanks to the stem cell transplantation performed by **Sajad Jawad Khazal, M.B. Ch.B.**, Kaylyn White can continue to experience fun and engaging activities. Shown above, she is polishing her culinary skills at our Celebrity Chef Cooking Demo during Childhood Cancer Awareness Month.

Renovations for 2020 planned following AYA program launch



Andrew Livingston, M.D., M.S.
Assistant Professor of Sarcoma Medical Oncology and Pediatrics



Michael Roth, M.D.
Associate Professor of Pediatrics



Wendy E. Griffith, B.S.W., M.S.W.
Program Manager, Pedi-Adolescent and Young Adult



Nerissa Maldonado
Program Coordinator, Pedi-Adolescent and Young Adult

The launch of the Adolescent and Young Adult (AYA) Oncology Program was celebrated in style in June 2018 with a big party on campus. The institution welcomed the service to improve the quality of life for MD Anderson patients between the ages of 15 and 39. With an outpatient multidisciplinary clinic and robust programmatic offerings, including the Young Adult Advisory Council and Young Adult Support Group, the program is one of the largest

of its kind in Texas. It offers a multidisciplinary approach to comprehensively assessing the needs of young people who are working toward independence and learning how to negotiate relationships, school, or those first professional jobs. Program co-directors are **Michael Roth, M.D.**, associate professor of Pediatrics and **Andrew Livingston, M.D., M.S.**, assistant professor of Sarcoma Medical Oncology and Pediatrics.

Our AYA program can help in four major areas on a primarily outpatient basis:

- Counseling for psychosocial and supportive care such as educational and vocational assessments, resource linkage, and counseling with a social worker and/or psychologist to help with self-esteem, emotions, and body image
- Counseling for fertility preservation to discuss family building options. In some cases, our program is able to cover the costs of fertility preservation, thanks to philanthropic funds
- Genetic counseling and testing that provides information about the risk of developing future cancers and hereditary factors which can help patients make family planning decisions

- Assistance with survivorship challenges, such as surveillance for long-term treatment side effects, counseling about nutrition and healthy lifestyle behaviors, neurocognitive testing and guidance following treatment, and help with facilitating the transition between pediatric to adult survivorship services when appropriate

Comprehensive assessment



- Symptom control
- Fertility
- Nutrition
- Late effects
- Behavioral health
- Body image
- Emotional health
- Exercise
- Cancer prevention
- Relationships and intimacy
- Careers
- Risky behaviors
- Spirituality
- Cancer genetics

Steady growth

From June 2018 to the end of August 2019, more than 500 unique patients utilized the AYA Clinic. Appointments are held at the Robin Bush Child and Adolescent Center, but due to growth and expansion of services, renovations were necessary. Two drivers of growth were institutional promotion of the program, and a pilot launched in three clinics — Brain and Spine Center, Gastrointestinal (GI) Center, and Lymphoma/Myeloma Center — to refer this demographic of patients to our program. To improve patient experience, the division launched a collaborative effort to repurpose space within the Child and Adolescent Center, allowing AYA patients to have their own entrance and waiting area. Program leaders also:

- Collaborated with MD Anderson’s Cancer180 to host the annual Young Adult Survivorship Conference.
- Expanded the reach of the AYA Scholarship Program and selected 39 recipients to receive support for the 2019–2020 school year and held a ceremony to celebrate the awardees.
- Worked closely with MD Anderson’s Camps and Special Events to host the first annual Young Adult Wellness Weekend, held at an Austin area resort, thanks to a donation from philanthropist and jewelry business owner Kendra Scott.

Looking ahead

To accommodate increasing demand in the coming year, the AYA program will open more clinic appointment slots and onboard a nurse navigator to assist with outreach and patient engagement. Program leaders are working to cover the costs for fertility preservation for more patients and expanding the Scholarship Program for cancer survivors.

...and the survey says

Feedback from our patient surveys has been overwhelmingly positive as 100% rated their experience as excellent or good, 100% said they would recommend the clinic to their peers, and 93% reported the AYA Clinic addressed issues that had not been previously addressed. One survey respondent wrote,

“

It was really nice to talk to a team that addressed me as a whole person — mentally and physically, rather than only my physical health. It was great to be connected with people and resources that could be helpful in the future. Everyone was very genuine and helpful.

”

says respondent



July 18, 2019

Mario stands with **Drs. Michael Roth** (left) and **Andrew Livingston**, proudly showing off his certificate as an AYA Scholarship recipient in July 2019.



April 1, 2019

Christine Randolph leads a dance exercise class at the first Young Adult Wellness Weekend.

Balance for life



Keri Schadler, Ph.D.
Assistant Professor of
Pediatrics



Karen Moody, M.D., M.S.
Associate Professor of
Pediatrics

Energy Balance research is the study of activities that generate energy (i.e. eating calories) or utilize energy (i.e. physical activity). Led by **Keri Schadler, Ph.D.**, assistant professor, and **Karen Moody, M.D., M.S.**, associate professor, the Energy Balance program utilizes novel methods to answer important questions at all stages, including:

- How does diet, nutrition, physical activity and exercise affect cancer risk, treatment effectiveness, and survivorship in pediatric patients?
- Does exercise make more chemotherapy get into the tumor?
- Does early nutritional support for bone marrow transplant patients reduce the chance of being admitted to the ICU?
- Can an eHealth exercise-based lifestyle intervention be used to prevent functional decline during and after cancer treatment?

“Our philosophy is that exercise and nutrition must be studied scientifically, using the same principles that are used to study pharmacologic interventions,” Schadler says. Thus, the foundation of the energy balance program is built upon work conducted in three basic science laboratories that use pre-clinical modeling to understand the molecular mechanisms by which energy balance impacts cancer biology, treatment efficacy and associated sequelae, and long-term survivorship.

Successes to date

Together, the pre-clinical and clinical research by the energy balance faculty led to nine peer-reviewed published research studies and almost \$1.5 million in external grant funding in 2019. Concurrent with research to ensure that scientifically sound interventions are developed, the energy balance team has developed several patient-centered programs to improve quality of life for our patients through exercise and nutrition. Exercise research and programming is expected to be further supported by the development of a pediatrics patient gym within our inpatient unit. **Maria Chang Swartz, Ph.D., M.P.H., R.D., L.D.**, a behavioral scientist, has joined our faculty to provide expertise in the psychosocial aspect of behavior change needed to successfully incorporate energy balance interventions as an adjuvant to cancer care.

The following support programs were created to accelerate energy balance initiatives:

Pro Fit: The Professionals and Fitness (Pro Fit) Program is a monthly event designed to lower the barriers for patients to stay physically active during treatment by making exercise fun in a safe environment. Local professional or college athletes join patients to play sports in our inpatient Pedi Dome. To date, we have hosted 12 Pro Fit events, including athletes such as Houston Dynamo soccer players, Olympian Yvonne Trevino, and the University of Houston basketball team.



Chef Adán Medrano uses his expertise to show patients Olivia Matovich (left) and Celeste Garcia how to make turkey chipotle tostadas.

Physical Education (P.E.) class: A weekly P.E. class is provided through our accredited school program, offering patients the chance to obtain P.E. credit while staying active and having fun.

Celebrity Chef Cooking Demo: Five local celebrity chefs cooked healthy versions of their popular recipes with the help of pediatric patient sous chefs. The event included tastings and education about healthy cooking.

Team Me: An incentive program was launched to encourage activity in bone marrow transplant patients, where participants earn points and prizes for being physically active during inpatient treatment.

Healthy cooking classes: Weekly dietitian-led classes are free to all patients through the use of the Ronald McDonald House Patient and Family Kitchen. Basic nutrition education and cooking skills through live demonstrations and food tastings are offered.

At The Table eCookbook: This online cookbook provides recipes searchable by ingredients or goals, such as weight gain or weight loss, and kid-friendly ideas to make healthy eating fun.

Research

Consistent with our goal of building an evidence-based energy balance program, our robust research environment includes four actively enrolling clinical trials and three basic science laboratories.

Clinical research

Energy Balance has launched multiple clinical studies to investigate the nutrition and exercise habits of our patients, methods to improve those habits, and outcomes associated with healthy nutrition and regular exercise.

Laboratory research



Schadler lab Keri Schadler, Ph.D.

The Schadler lab is a very successful research laboratory and among only a few in the nation studying the effect of exercise on the efficacy of chemotherapy against pediatric tumors.



Kleinerman lab Eugenie Kleinerman, M.D.

The Kleinerman lab is evaluating the use of exercise to protect against anthracycline-induced cardiac toxicity, focusing on novel mechanistic relationships between exercise and treatment-related toxicity.



Chandra lab Joya Chandra, Ph.D.

The Chandra lab performs innovative work using mouse models of pediatric leukemia to determine whether feasible changes in diet affect disease outcomes. Her group is also conducting clinical research on how to improve diet through cooking interventions involving families of pediatric cancer patients and survivors.

Peer-reviewed external funding

The seed funding investments of the division in supporting energy balance initiatives has led to almost \$1.5 million in external grant dollars, including \$900,000 from the Cancer Prevention Research Institute of Texas (CPRIT) for preclinical studies of the molecular mechanisms by which exercise affects tumor biology. This grant is awarded to only the top 9% of applicants, indicating the competitive nature of the science being done within our division. Similarly, the National Cancer Institute funded a proposal searching for blood biomarkers to suggest that exercise has induced vascular remodeling in cancer patients. Research examining the role of

energy balance in protecting against anthracycline-induced cardiac toxicity is funded by an Alex's Lemonade Stand Foundation Innovation Award.

Education

The Energy Balance and Integrative Medicine team is actively educating the next generation of energy balance and cancer researchers and clinical providers. In 2019, two clinical fellows, three Ph.D. students, and two postdoctoral fellows were trained in research on energy balance. These trainees are doing high-quality research, as evidenced by the selection of **Miriam Morrell, D.O.**, for a platform presentation at the Children's Oncology Group 2019 meeting,

an honor given to only four clinical fellows nationwide; the award of a CPRIT training fellowship for Ph.D. candidate **Hannah Savage**; an NIH R25 Award and a CPRIT Postdoctoral Fellowship to **Margaret Raber, Dr. P.H.**, as well as selection for the Citizen Scientist program, and numerous other oral and poster presentations by our trainees at national meetings.

Future directions

Our energy balance team will be conducting novel research combining exercise and nutrition interventions, a national study of exercise for bone cancer patients and, ultimately, incorporating evidence-based exercise and nutrition into standard of care.

CPRIT IIRA

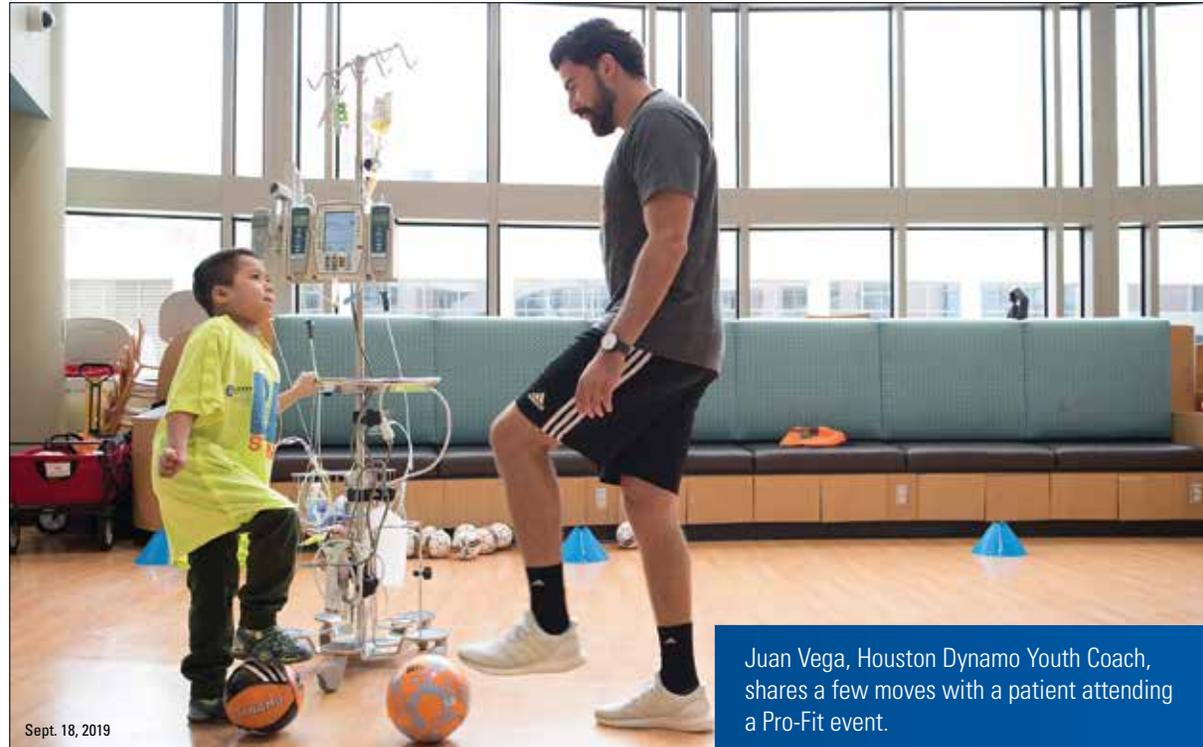
\$900,000 over 3 years; PI: Schadler

R21

\$250,000 over 2 years; PI: Schadler

ALSF

\$250,000 over 2 years; Co-Investigators: Kleinerman, Chandra, Schadler



Sept. 18, 2019

Juan Vega, Houston Dynamo Youth Coach, shares a few moves with a patient attending a Pro-Fit event.

Expanding our reach, changing spaces

Patient access

Changes in the health insurance industry have led to continual modifications in insurance plan coverage, treatment authorization requirements, and provider contracts. The complexity of the health insurance system has made it increasingly difficult for pediatric patients to access cancer treatments. A nurse navigator was welcomed into the operation in November 2018 to help our patients gain access.

Shana LeCounte, B.S.N., R.N., facilitates patient intake, coordinates care, and improves communication between patient families and interdisciplinary care providers. Her rich clinical background allows her to provide specialized information to families about disease-specific treatment guidelines, clinical trials and genetic/molecular testing.

Space renovations

Space is a finite resource and re-imagining how it is used to drive our mission is an exciting focus. Every square foot is imperative to what we do including the centers where we care for patients, basic science laboratories where new intelligence is unearthed, and staff workrooms and offices that sustain the operation. As the demand for clinical services increases and regulatory requirements change, the functional requirements of our space to treat patients, organize and innovate also change. Steering resources to the following two projects support current and future programmatic aims and enhance our overall care delivery model.

Child and Adolescent Center aesthetic renewal

Physical environments that support staff workflows and staging of resources make safe patient care and efficient clinical operations possible. That is why careful attention is being given to the aesthetic renewal of the Child and Adolescent Center where outpatient care is delivered. With this construction, emphasis has been placed on optimizing workrooms for team collaboration and creating a flow for patient care that matches the way we work now. The lobby, play area and public restrooms will receive a much-needed refresh to include contemporary furnishings. Our phasing plan will allow work to be completed incrementally so clinic operations are minimally disrupted.

AYA Clinic

Swift growth of the AYA patient population makes a compelling case to create a welcoming space just for them. Along with an age-appropriate lobby and furnishings, the AYA space will consist of three exam rooms enclosed by contemporary barn doors, a consult room, an area for patient vitals, and mobile computer workstations for staff. The lobby will be outfitted with modern furniture with charging station capabilities, digital check-in, a resource wall, and video monitors to provide educational loops for the patients.

With projections that the Children's Cancer Hospital will require further expansion, we are indebted to the large team of architects, designers and stakeholders collaborating with us to maximize use of existing space and to thoughtfully explore options to bring new design concepts to life.

Supportive Care and Integrative Medicine: Making a difference throughout the cancer continuum

Our Supportive Care Program responds to the needs of nearly 2,000 patients each year who come to us in various stages of disease progression. They require not just an arsenal of medical expertise, but also the type of clinical and supportive care capabilities aimed at improving their quality of life during and after therapy. The Children's Cancer Hospital has a big umbrella under which many types of supportive care mitigate pain and symptoms, provide psychosocial support to patients up to age 29, and when necessary, help transition patients to end-of-life care and offer bereavement support to families.

Karen Moody, M.D., M.S., associate professor of Pediatrics and director of our Pediatric Palliative and Supportive Oncology Program, oversees our integrative approach which blends evidence-based complementary and conventional therapies, and enables practitioners to support goal-setting and medical decision-making, interdisciplinary care-coordination, mind-body medicine, and nutritional counseling. Recent program successes include dramatically expanding patient access to integrative medicine, implementing a clinical pain algorithm for providers, creating and applying nursing guidelines for opioid administration, introducing new pharmacological agents, establishing interdisciplinary inpatient rounds, developing robust research, and implementing support programs for staff including Code Lavender to discuss the stresses of working in a challenging clinical environment.

Behavioral Pediatrics

Thanks to phenomenal advances in medicine, 80% of childhood cancer patients survive at least five years after treatment. However, some survivors experience secondary disease and/or treatment side effects that can be short-term or lifelong. Our Behavioral Pediatrics group helps patients respond to the emotional, behavioral or neurocognitive challenges that can occur.



Rhonda Robert, Ph.D., A.B.P.P.
Professor of Pediatrics

Rhonda Robert, Ph.D., A.B.P.P., professor, leads this group of experts who had more patient encounters in the past year than ever before. One reason for the increase was a new consensus among the division's medical oncologists and clinicians that, based on psycho-oncology literature, illness severity and risk for medical treatment side effects should play a chief role in consult requests. The group introduced an online screening tool inviting Adolescent and Young Adult (AYA) Oncology Program patients to answer questions about their psychosocial status, resulting in referrals for follow-up appointments with a care team member.

In keeping with MD Anderson's mission to educate the next generation, students from a third local university's psychology master's program were added to our ranks of trainees. These graduate-level students were supervised by Behavioral Pediatrics staff during patient encounters. This group performed several behavioral science projects, including one that focused on parental adjustment to childhood cancer because it has the potential to buffer kids' emotional reactions to traumatic events. Our faculty and staff are evaluating an intervention that helps parents cope with the diagnoses of life-threatening illnesses.



“
All through nursing school, my goal was to come back to MD Anderson so I could support kids who were like me.

”
Caitlyn Mortus, B.S.N., R.N.,
Clinical Nurse

Caitlyn Mortus, B.S.N., R.N., clinical nurse, Nursing G9, is one of many inpatient nurses who are dedicated to our patients. She joined MD Anderson in 2019. Becoming a nurse has been one of her goals since she walked the halls of the Children's Cancer Hospital as a patient. Mortus was diagnosed with Burkitt lymphoma when she was in the seventh grade. "The nurses were by my side most of the time," she remembers. "All through nursing school, my goal was to come back to MD Anderson so I could support kids who were like me." Having gone through therapy herself, she knows what the good days look like during treatment. "I can relate and be extra patient because I know some days just aren't fun," Mortus said, adding that being able to relate to her patients without having to say anything and knowing she's making a difference is why she ultimately became a nurse.

Cognitive tasks as an indicator of treatment side effects

Parker was diagnosed in June 2018 with Burkitt lymphoma, which often grows first and fast in the head and neck or the abdomen. The symptom that made Parker's parents bring him to their local Emergency Room (ER) was a constant bellyache. Doctors there thought it was constipation, which they treated him for, but the problem persisted forcing the family to return to the ER. A CT scan and upper GI endoscopy found the problem. Parker started treatment immediately and completed chemotherapy in December 2018. He has had no evidence of disease since and now returns to the Child and Adolescent Center for follow-up visits with **Michael Roth, M.D.**, associate professor, and the entire care team.

Behavioral Pediatrics meets with Parker because of neurocognitive risks associated with the chemotherapy regimen for Burkitt lymphoma. "Vincristine and methotrexate can cause problems with cognitive development and functional performance such as reduced attention and memory, information processing speed, and efficiency," said **Peter Stavinoha, Ph.D., A.B.P.P.**, neuropsychologist and professor. He added that these challenges tend to be higher in children treated at a younger age. While not every child will experience significant problems,



Grace Yang, M. Ed., L.P.C., a senior counselor in the Behavioral Pediatrics program, enjoys working with five-year-old Parker in our 'therapeutic playroom.' He is a very sociable boy who walked into the room and immediately found the dump trucks and dinosaurs. When Yang asked him to sit with her at a table and stack some cubes to make them look like the image in front of him, he was able to do so with no problem. This is an example of one of many types of neuropsychology tasks Yang and her colleagues perform while assessing patients during and after treatment.

the Children's Cancer Hospital provides regular surveillance for neurocognitive effects of treatment through childhood and transition to young adulthood.

Neuropsychological assessment involves direct cognitive testing with children, such as what Yang

is seen doing, or something more age appropriate for older patients. Yang and colleagues use evaluation results to provide guidance for educational planning, psychosocial support for adjustment issues, and longer-term planning and guidance for parents to optimize their child's long-term outcome and quality of life.

Support Programs

When a child has cancer, it affects everyone in the family. Easing that burden is what motivates the talented staff of Support Programs. Specialists in our Child, Adolescent and Young Adult Life Program (known as Child Life) are among those experts. Child Life assists patients by psychologically preparing them for tests, surgeries and procedures, and works with families to celebrate milestones that occur during hospitalizations, such as birthdays and graduations. When needed, these experts offer support for grief and bereavement. In the past year, Child Life expanded legacy projects to include voice and heartbeat recordings housed in stuffed animals, necklace charms made from artwork that patients created, and held a bereavement event called Camp Dandelion to engage families with writing activities and discussions about their 'new normal.' Child Life employees conducted the Texas Association of Child Life Professionals (TACLP) Annual Conference on campus for 200 colleagues and students, where they discussed trends and research in the field and how to increase the depth of compassionate support families need during their cancer journey.



March 25, 2019

Child Life



March 6, 2019

College and career fair for teens and young adults

School is a normal part of life for children, but when a cancer diagnosis and treatment interrupts that rite of passage, the academic and social impact can be devastating. Our in-hospital, accredited Pediatric Education and Creative Arts Program, also known as The School, represents the bridge to carry our patients over from missing out on learning opportunities to being on grade level or above when they return to the classroom following successful treatment. Masters-prepared educators work with inpatients at the bedside and in our classroom and serve as liaisons between patients and teachers from their local schools to assist patients with assignments and study skills. In the past year, the program welcomed an early childhood educator who focuses on patients between 2½ to 5 years old, and established a teaching schedule specifically for cellular therapy and intensive care patients who require additional infection control measures. The program added a successful college and career fair for teens and young adults and broadened its Google Expedition Passport Series, which uses virtual reality technology to introduce patients to the

far-away places they read about and see in the movies. School program staff have also shared their own best practices with colleagues around the country by presenting posters and giving invited lectures at The Health Educator and Academic Liaison Association (HEAL) Conference.

The Camps and Special Events Program is in charge of delivering fun experiences for patients and their siblings. The indoor camps feature arts and crafts, robot games, singing and dancing, access to an on-campus petting zoo and even adaptive canoeing. The away camps held during the summer allow small kids and teens the opportunity to have fun with peers who understand what they have been through. Camp Star Trails takes place at a no-barrier offsite campground in Burton, Texas, and offers an array of activities, including swimming, zip-lining, outdoor games, healthy cooking sessions, nature walks, horseback riding and cabin accommodations for children of all abilities. Camp AOK for teens 15–18 years of age takes place in Magnolia, Texas and includes a variety of activities throughout the week.



June 17, 2019

Camp Star Trails

Supporting our patients

Four Seasons Hotel Houston hosted the newest experience — Camp H-Town — and turned many of its rooms into camp-style cabin accommodations. This camp incorporates field trips for patients and siblings to iconic local establishments and visits from professional athletes and special guests. The biggest bash of the year was the Annual Prom Party Palooza, which included year-round planning and execution from more than 80 volunteers across the institution. Prom offered three parties in one evening — something fun at the Pedi Dome for the younger kids, the big dance for the teens and young adults on the 24th floor Observation Deck, and a circus-inspired theme for parents and guests. Almost everything was donated — including the delicious food and party favor bags. MD Anderson collaborated with sponsors Peli Peli Restaurants, NACE Houston Chapter, and The Sunshine Kids Foundation to make this event happen. Muzzie's, a retailer in Vintage Park,

contributed more than 100 new dresses in 2019 to make the prom a fashion event for the young ladies. Other donors were SJP by Sarah Jessica Parker with beautiful shoes, Something Blue and Dillard's with dresses, Men's Wearhouse with tuxedos, and a host of local restaurants and businesses. The annual prom garnered the highest number of local and national news coverage hits to date. Camps and Special Events also coordinated a variety of occasions throughout the year including a collaborative event with the PGA TOUR Wives Association and awareness events during Childhood Cancer Awareness Month.

The mission of the Arts in Medicine (AIM) Program is to connect patients and their families to visual arts, music, theater, and dance. These activities give patients a sense of empowerment, reduce anxiety and pain, and help them build a community among families going through a similar journey. In the past year, a certified music therapist joined the group and



Arts in Medicine (AIM) Program

often partners with Child Life staff to enhance patient experiences. The therapist also is involved in research with **Karen Moody, M.D., M.S.**, associate professor, enrolling patients between 3 and 8 years old in the PINPOINT Study (**2018-1066**) to determine how active music engagement (AME) reduces emotional distress. Other program accomplishments include initiating the Postal Art Exchange, which connected pediatric patients and families with artists from around the world through their artwork. AIM received great reviews of its ceiling tile project which invited the kids to use stencils and paint to create a new look for ceiling tiles that were later placed above the area where the 'end of treatment' bells hang — six at each end of the hall. This will be an ongoing project. Additionally, AIM sparked a relationship with Da Camera of Houston, a jazz and chamber music ensemble company, to perform bedside in inpatient rooms, as well as regular visits with other groups — the Houston Symphony, the Houston Grand Opera, the Houston Ballet, and the Books Alive! Theater Program.



Annual Prom Party Palooza

Training the next generation



June 13, 2019

(l-r) **Nelda Itzep, M.D.**, **Grace Nehme, M.D.**, **Sheetal Phadnis, M.D.**, and **Brianna Murphy, D.O.**

The Pediatric Hematology/Oncology Fellowship Program prepares the next generation of medical oncologists and hematologists who want to care for small children, teens, and young adults diagnosed with cancer. The program accepts four candidates a year for a three-year odyssey which provides an excellent foundation in clinical care and scholarly work.

Under the supervision of renowned faculty from the Children’s Cancer Hospital, MD Anderson, and several UT Health System partners, our fellows diagnose and prescribe treatments for patients with complex blood and solid tumor cancers. They also train in benign hematological disorders. At the end of the program, our trainees are well-prepared to take the exam to earn certification from the American Board of Pediatrics in hematology and/or oncology.

Priti Tewari, M.D., a stem cell transplantation specialist, serves as Program Director. She works with a Program Evaluation Committee to audit components of the curriculum to determine areas that may be enhanced for optimal trainee instruction. In the last year, the fellowship program expanded based on feedback from former trainees. New additions included procedure blocks, a rotation focused on AYA, and a Fellows Research Showcase in which trainees learned how to improve research presentations and public speaking skills. Tewari also works with a Clinical Competency Committee to appraise the knowledge base and milestones our fellows are achieving.

In 2019, four physicians received certificates of completion. **Nelda Itzep, M.D.**, elected to remain for one more year to focus on palliative and supportive care training. **Grace Nehme, M.D.**, is working as a pediatric hematology/oncology specialist in Dubai, United Arab Emirates (UAE); **Sheetal Phadnis, M.D.**, went to New York University for a year of training in pediatric brain tumors; and **Brianna Murphy, D.O.**, is working at Memorial Sloan-Kettering in New York providing general oncology care to pediatric patients. Following graduation, another year is available through the Pediatric Advanced Hematology/Oncology Fellowship Program. With faculty mentorship, the fellows are able to gain more clinical expertise in one of five subspecialties while developing data they can use to establish themselves as independent researchers. The five tracks are Stem Cell Transplantation and Cellular Therapy, Leukemia/Lymphoma, Non-Neural Solid Tumors, Neuro-Oncology, or Palliative Care. The division had two advanced fellows in FY 2019. **Mira Kohorst, M.D.**, focuses on stem cell transplantation and cellular therapy, while her colleague, **Nelda Itzep, M.D.**, focuses on palliative care.

Educating tomorrow's scientists

Training Program

(duration, number of students enrolled)

Postdoctoral fellows

3 to 5 years
10 postdoctoral fellows

Graduate students

5½ years
Nine graduate students

Summer students

10 weeks
10 summer students

The Pediatrics Research Training Program offers a range of intensive educational training opportunities to help future scientists enhance their critical thinking skills as investigators, learn more about the biology of pediatric cancers, help them design their basic research and preclinical studies, and develop therapies. Just as becoming a medical doctor takes at least 14 years, becoming a bench scientist capable of performing independent research requires many years of preparation.

Our graduate students rotate through all of the Children's Cancer Hospital laboratories, interact with principal investigators, work on assigned projects, and participate in some of the daily activities of running a laboratory. Upon graduation, they apply for postdoctoral fellowships in areas they want to pursue as careers. Graduate and postdoctoral fellows write papers and give presentations before their peers. The trainees have many opportunities to present to lay and peer audiences because they may need to share knowledge with potential stakeholders, including donors and grant application appraisers, who will need to understand why their science is worthy of financial support. Some go

on to hold faculty positions and start their own independent research programs in academic settings, while others work for the federal government or pharmaceutical companies pursuing specific cancer research. Yet, other trainees may choose to stay on as senior scientists in university laboratories because they enjoy the academic environment but do not want to run their own labs.

Several research trainees had an outstanding fiscal year. Many gave first-author poster presentations at society meetings, including the American Association of Immunology, American Association for Cancer Research, and the International Society for Pediatric Neuro-Oncology. Research trainees also published first-author papers on complex topics in highly regarded publications, such as *Cancer Letters* in 2018, *Clinical Cancer Research* in 2019, the *Journal of Pediatric Hematology and Oncology* in 2018 and *Supportive Care in Cancer* in 2019. The accomplishments of medical and research trainees were applauded at a special Trainee Recognition Day held at MD Anderson's Hickey Auditorium in June 2019.



Trainee Recognition Day

Our laboratories

Our laboratories are conducting cutting-edge research to tackle pediatric cancers from all angles. Multidisciplinary teams of researchers are using state-of-the-art methodologies to investigate the genetic makeup of common childhood cancers, develop novel immunotherapies, and test the efficacy of these new treatments.



Chandra Lab
Joya Chandra, Ph.D.

Understanding the biology of pediatric leukemias and brain tumors
Defining roles for diet and exercise in pediatric cancer treatment and survivorship



Gorlick Lab
Richard Gorlick, M.D.

Identifying novel targets in osteosarcoma
Assessing efficacy of novel anti-cancer agents in osteosarcoma models



Li Lab
Shulin Li, Ph.D.

Identifying immune regulators in interaction with genetic mutations to promote cancer cell stemness and progression in pediatric-related tumors
Discovering novel approaches of immune therapy and diagnosis



Gopalakrishnan Lab
Vidya Gopalakrishnan, Ph.D.

Understanding how pediatric brain tumors develop and using this information to develop therapies



Kleinerman Lab
Eugenie S. Kleinerman, M.D.

Doxorubicin-induced cardiotoxicity: Defining mechanisms and identifying preventive interventions
Developing a novel Dendritic Cell Vaccine combined with checkpoint inhibition for osteosarcoma



Schadler Lab
Keri L. Schadler, Ph.D.

Studying tumor vascular biology and the utilization of exercise as a therapeutic adjuvant

Clinical trial top enrollers

Clinical trial enrollment increased by 11% in 2019 compared to the previous year. This is a credit to our Clinical Research Team's expanded focus on cellular therapies and the increase of innovative studies from the Children's Oncology Group. Many of our trials are only offered at MD Anderson or at limited sites in the United States.

We were successful in working with principal investigators throughout MD Anderson, the Institutional Review Board, and pharmaceutical companies sponsoring drug agents to achieve approval to lower the age of participation to 16 years and, in some cases, even younger in trials that had previously only accepted adult patients. Some of our top-enrolling clinical trials include those from transplantation and cellular therapy (see pages 7–9).



Shulin Li, Ph.D.
Professor of Pediatrics



Amy Heimberger, M.D.
Professor of
Neurosurgery



Eugenie Kleinerman, M.D.
Professor of Pediatrics



Stephanie Watowich, Ph.D.
Professor of Immunology



Nancy Gordon, M.D.
Assistant Professor of
Pediatrics



Najat Daw, M.D.
Professor of Pediatrics

The Children’s Cancer Hospital has many clinicians on the team, but how much do you know about the outstanding laboratory scientists whose work drives clinical trials and other patient care initiatives? We are putting a spotlight on faculty members whose work you need to know about.

Shulin Li, Ph.D., professor of Pediatrics, has received patents in the United States, Europe, China, and Japan for a tool that uses a small amount of blood to identify traces of cancer cells that have escaped treatment. The “Specific Detection Tool for Mesenchymal and Epithelial-Mesenchymal Transformed Circulating Tumor Cells (CTCs),” was commercialized globally for research application through The University of Texas System, with MD Anderson retaining the rights for clinical application. Known as the seeds of metastases, CTCs make treatment less effective and spread disease to distant organs. This tool was developed primarily for pediatric patients because CTC assays designed for adult tumors — by detecting epithelial markers such as EpCAM and cytokeratins — are ineffective in detecting CTCs from non-epithelial tumors, such as neuroblastomas and certain sarcomas that are diagnosed in children. Li received a Pediatric Cancer Research Foundation (PCRF) grant and an NIH R01 award in 2017 and 2018, respectively, to support this effort.

The key for CTC capture is based on the discovery that all highly malignant tumor cell surfaces are present with cell surface vimentin (CSV), which is present inside of the normal mesenchymal cells. Extending this discovery, Li’s team has invented

a first and second generation of tumor-targeted IL12 immune therapy, and one of them received a U.S. patent through support from multiple mechanisms. Of note, the second generation of tumor-targeted IL12 therapy is able to eliminate a large portion of human osteosarcomas in mice. This effort will move to clinical trial soon.

Li’s body of work also includes an investigation into what could become the next big thing in immunotherapy. He and MD Anderson colleague **Amy Heimberger, M.D.**, professor of Neurosurgery, and others are studying an immune checkpoint (such as PD1, CD39, CD73) regulator that appears to dictate glioblastoma (GBM) progression by shutting down immune surveillance. This indicates a potential new area of clinical application. Li published the team’s preclinical findings as senior author in the Jan. 25, 2019 issue of *Nature Communications*. Therapy targeting this molecule is underway as a patent. You might ask, “How did the scientists reach this point?” Several years ago, Li and his lab used a method of electric pulse to shock squamous cell carcinoma and sarcoma cells to make them more susceptible to immune therapy when they discovered other candidate genes related to immune resistance using gene expression arrays. One of those other candidate genes was fibrinogen-like protein 2 (FGL2), known to suppress the immune system and highly expressed in GBM. Li and Heimberger had a mutual interest in the subject and began to work together. A researcher in the study found that FGL2 protein present in malignant tumors prevents dendritic cells — crucial to launching any immune response — from

activating T cells that seek and destroy targeted invaders. In Li's lab, researchers demonstrated that by inactivating or 'knocking out' the FGL2 protein in a tumor, they can eliminate tumor (both lung tumor and GBM tumor) progression in mice in the presence of the intact immune systems. Understanding this type of expression is key to discovering causes of GBM progression. Li, who received a \$1.83 million R01 award in 2016 to support this investigation, is actively working with Heimberger to develop therapeutic strategies to target the protein.

In addition to the highly competitive R01, Li is also principal investigator for a U01 grant with peers at MD Anderson, George Washington University, Johns Hopkins University, and University of Hawaii. The goal of "Pathway Specific Functional Biomarkers for the Early Detection of Liver Cancer," is to implement a multi-institutional framework to collect the highest quality biospecimens from patients with various well-defined liver pathologies and conduct specific biomarker validation studies for early detection of HCC and risk stratification of patients with cirrhosis.

Eugenie Kleinerman, M.D., professor of Pediatrics, is internationally recognized for her scientific and clinical expertise in sarcomas, particularly osteosarcoma (OS) metastases to the lungs. She served as Division Head of Pediatrics from 2001–2015, and in fact, was the first woman at MD Anderson to serve in that capacity. Kleinerman pioneered the use of a unique immunotherapy agent, liposome-encapsulated MTP-PE, for children with unresponsive relapsed osteosarcoma lung metastases. Her Phase II clinical trials conducted at MD Anderson demonstrated that liposomal

MTP-PE therapy activated the tumoricidal properties of macrophages, prolonged disease-free survival and could be combined with chemotherapy. The success of these trials led to a national Phase III trial sponsored by the Children's Oncology Group. Newly diagnosed osteosarcoma patients treated with liposomal MTP-PE plus chemotherapy had a 30% reduction in mortality rate compared with patients that received chemotherapy alone. This demonstration of efficacy led to the approval of liposomal MTP-PE by the European Medicine Agency and is now standard of care in numerous European countries.

In fiscal year 2019, Kleinerman won a \$100,000 grant from St. Baldrick's Foundation to support her research of a novel dendritic cell (DC) vaccine combined with the checkpoint inhibitor anti-PD1 for osteosarcoma therapy. The vaccine, developed by **Stephanie Watowich, Ph.D.**, professor of Immunology, and her lab team, has shown activity against melanoma. She and Kleinerman are working together to modify vaccine production to target osteosarcoma cells. DC vaccine therapies are a new area of focus in immunotherapy, showing activities in other cancers, but have never been tested against osteosarcoma. Kleinerman's lab is using a vaccine in pre-clinical mice models that are implanted with tumor cells at the same site where disease or metastases would be found in the original human host against a primary tumor and lung metastases. If the scientists can demonstrate the vaccine works here, Kleinerman will be able to make a case for clinical trials for patients with relapsed and metastatic osteosarcoma disease, as well as for patients who are chemo-resistant to front-line therapies.

Additionally, Kleinerman was awarded a \$250,000 award from Alex's Lemonade Stand Foundation to support her study of exercise strategies for decreasing doxorubicin-induced cardiotoxicity in childhood sarcoma patients and long-term survivors. She and colleagues on the Energy Balance team surmise that specific exercise regimens, initiated during or after treatment with the chemotherapy drug, will decrease the possibility of acute cardiac damage and promote rapid recovery, thereby reducing late cardiac effects and improving cardiac health in survivors. Preclinical work is still ongoing to identify biomarkers that would confirm the success of the intervention in each patient.

Another Kleinerman success this past fiscal year includes pre-clinical studies with **Nancy Gordon, M.D.**, assistant professor of Pediatrics, that led to a Phase I trial of gemcitabine as an inhalation therapy for patients 12–50 years old with lung metastases from osteosarcoma or other tumors and no proven survival-extending options. The **2015-0720** protocol, led by **Najat Daw, M.D.**, professor of Pediatrics, was approved because many patients with lung metastases receive systemic chemotherapy, which has significant side effects and, in this population, limited survival benefits. To reduce side effects and improve outcomes, the aerosolized chemotherapy directly to the lungs is a good option to investigate.



Sept. 26, 2019



April 27, 2019

All donations matter.
The cumulative total amount of donations less than \$50,000 received in FY19 was an amazing **\$1,317,838.**



Sept. 21, 2019



Oct. 28, 2019

From the bottom of our hearts, thank you.

So much of what sets the Children’s Cancer Hospital apart is made possible because of generous donors. A community of individuals, civic groups, philanthropists, and corporations have committed to helping our cause. Donor support allows us to investigate ideas that are becoming breakthroughs and provide psychosocial activities for our patients and families to deal with one of the most challenging diagnoses they will face in their lifetimes.

We would like to recognize the following individuals who made contributions in the past fiscal year that were \$50,000 and above and hope to earn your continued support.

More than \$500,000

7-Eleven/Stripes
 Energy Transfer LP
 Estate of Athena Loutsch
 James B. & Lois R. Archer Charitable Foundation
 The Living Legends Foundation, Inc.

\$101,000 – \$500,000

Addi’s Faith Foundation
 Ed Rachal Foundation
 Harry T. Mangurian, Jr. Foundation, Inc.
 Houston Tri Delta Philanthropies, Inc.
 Mr. Kelcy L. Warren
 Swim Across America
 The Energy Transfer/Sunoco Foundation
 The M. S. Doss Foundation, Inc.

\$50,000 – \$100,000

Bob Tallman’s Charities, Inc.
 Latattore, LLC
 LyondellBasell Industries
 Texas 4000 For Cancer
 The Harry and Estelle Soicher Foundation
 United Continental Holdings, Inc.

MD Anderson Cancer Center Board of Visitors’ Children’s Cancer Hospital Advisory Group

The Children’s Cancer Hospital Advisory Group is a vital component of the MD Anderson Cancer Center Board of Visitors (BOV). This leadership body is committed to helping us achieve our mission of eliminating cancer in children. Board programs emphasize private fund development, public relations, and outreach on behalf of the hospital. We are grateful for their dedication.

Mrs. Linda Mays McCaul, Chair
 Mr. & Mrs. James Archer
 Mr. Michael G. Bartolotta
 Ms. Michelle M. Bengue
 Mrs. Karen Comiskey
 Mrs. Anne P. Connally

Mrs. Nadine Craddick
 Mrs. Donna Culliver
 Mr. Timothy Culliver
 Mr. Jay Eisenberg
 Mrs. Elizabeth G. Epley
 Mr. Brendan J. Fikes

Mrs. Patsy Fourticq
 Ms. Barbara Hurwitz
 The Honorable Brenda L. Johnson
 Ms. Luci B. Johnson
 Mr. Andrew D. McCullough, Jr.
 Mrs. Pamela K. Onstead

Mrs. Joan Weingarten Schnitzer
 Mr. Sam L. Susser
 Mr. Mac Tichenor
 Mrs. Sheridan Williams
 Mrs. Kathryn Hall Wilson

Division of Pediatrics — Administrative leadership and faculty

ADMINISTRATIVE LEADERSHIP

Division Head and Department Chair
Richard Gorlick, M.D., Professor

Deputy Division Head, *Ad Interim*
Cynthia E. Herzog, M.D., Professor

Medical Director for Pediatric
 Children's Cancer Hospital and Center
 Medical Director for
 the Robin Bush Child and
 Adolescent Center
Douglas Harrison, M.D.,
 Associate Professor

Director, Pediatric Research
 Laboratory Program
Shulin Li, Ph.D., Professor

Director, Pediatric Clinical and
 Translational Research Program
Jonathan Gill, M.D.,
 Associate Professor

Executive Director and Division
 Administrator
Lisa Hafemeister, M.H.A., F.A.C.H.E.

Administrative Director,
 Pediatric Clinical Services
**Joan O'Hanlon Curry, M.S., R.N.,
 A.P.R.N., C.P.N.P., C.P.O.N.**

Associate Director, Pediatrics Finance,
 Pediatrics Administration
Michelle Gatliff, B.S.

Director, Pediatric Operations,
 Pediatrics-Support Programs
Kevin Long, M.B.A., F.A.C.H.E.

Department Administrator,
 Pediatrics-Research
Tajuanna Thibodeaux, M.B.A.

Associate Department Administrator,
 Pediatrics-Patient Care
Luis "Tony" Choy-Morga, M.B.A.

Project Director, Office of Division,
 Pediatrics Administration
Altrivice Revis, M.B.A.

CLINICAL FACULTY

**These faculty have secondary appointments
 in Pediatrics.*

Adolescent and Young Adult (AYA)
 Oncology/Survivorship

Michael Roth, M.D.,
 Associate Professor

Andrew Livingston, M.D., M.S.,
 Assistant Professor*

Brain and Spinal Tumors

Soumen Khatua, M.D.,
 Associate Professor

Zsila Sadighi, M.D.,
 Associate Professor

Wafik Zaky, M.B.B.CH.,
 Associate Professor

John Slopis, M.D., Professor*

Critical Care

Jose Antonio Cortes, M.D.,
 Assistant Professor

Linette Jeanna Ewing, D.O.,
 Assistant Professor

Rodrigo Mejia, M.D.,
 Professor

Shehla Razvi, M.D.,
 Assistant Professor

Endocrine

Steven Waguespack, M.D.,
 Professor*

Anita Ying, M.D., M.B.A.,
 Associate Professor*

Infectious Diseases

Natalie Daley Garnes, M.D., M.P.H.,
 Assistant Professor*

Integrative Medicine/
 Supportive Care

Kevin Madden, M.D.,
 Assistant Professor*

Karen Moody, M.D., M.S.,
 Associate Professor

Leukemia/Lymphoma

Michael E. Rytting, M.D.,
 Professor

Branko Cuglievan, M.D.,
 Assistant Professor

Cesar Augusto Nunez, M.D.,
 Associate Professor

Michael Roth, M.D.,
 Associate Professor

Robert Wells, M.D.,
 Adjunct Professor

Non-Neural Solid Tumors (NNST)

Najat Daw Bitar, M.D., Professor

Jonathan Gill, M.D.,
 Associate Professor

Richard Gorlick, M.D.,
 Professor

Douglas Harrison, M.D.,
 Associate Professor

**Fiorela Natali Hernandez
 Tejada, M.D.**,
 Assistant Professor

Cynthia E. Herzog, M.D.,
 Professor

Pooja Hingorani, M.B.B.S.,
 Associate Professor

Division of Pediatrics — Administrative leadership and faculty

Psychology

Martha Askins, Ph.D.,
Associate Professor

Rhonda Robert, Ph.D., A.B.P.P.,
Professor

Peter Stavinoha, Ph.D., A.B.P.P.,
Professor

Stem Cell Transplantation and Cellular Therapy/Immunology

Sajad Jawad Khazal, M.B. Ch.B.,
Assistant Professor

**Kris Michael Mahadeo, M.D.,
M.P.H.,** Associate Professor

Demetrios Petropoulos, M.D.,
Professor

Priti Tewari, M.D.,
Associate Professor

UTHEALTH SCIENCE CENTER ADJUNCT FACULTY

Cardiology

**Gurur Biliciler-Denktaş, M.D.,
F.A.C.C., F.A.S.E.,**
Ad Interim Co-Chief and
Associate Professor

Gastroenterology

J. M. Rhoads, M.D.,
Director and Professor

Nephrology

Rita D. Swinford, M.D.,
Director and Associate Professor

Pediatric Neurosurgery

David I. Sandberg, M.D.,
Chief and Professor

Pediatric Surgery

Kevin P. Lally, M.D., M.S., F.A.C.S.,
Chairman and Professor

Pulmonary Medicine

James M. Stark, M.D., Ph.D.,
Chief and Professor

RESEARCH FACULTY

Basic Research

Joya Chandra, Ph.D.,
Associate Professor

Donghang Cheng, Ph.D.,
Assistant Professor

Vidya Gopalakrishnan, Ph.D.,
Associate Professor

Nancy Gordon, M.D.,
Assistant Professor

Jiemiao Hu, Ph.D.,
Instructor

**Sankaranarayanan Kannan,
D. Phil.,** Instructor

Eugenie S. Kleinerman, M.D.,
Professor

Pappanaicken R. Kumar, Ph.D.,
Instructor

Shulin Li, Ph.D.,
Professor

Shinji Maegawa, Ph.D.,
Instructor

Amer M. Najjar, Ph.D.,
Assistant Professor

Keri L. Schadler, Ph.D.,
Assistant Professor

Shavali Shaik, Ph.D.,
Instructor

Harjeet Singh, Ph.D.,
Instructor

Rong-Hua Tao, Ph.D.,
Instructor

Hiroki Torikai, M.D.,
Instructor

Xin Zhou, Ph.D.,
Assistant Professor

Health Outcomes

**Maria Chang Swartz,
Ph.D., R.D., L.D.,**
Assistant Professor

Project team

The Annual Report team is pleased to highlight recent accomplishments that provide hope and comprehensive treatment options to our patients and their families. We appreciate colleagues who contributed content and ideas to make the publication a success.

Altrivice Revis, M.B.A.
Project Director

Cecilia Aguerre, Ph.D.
Program Manager

Maxsane Mitchell, B.S.
Program Manager

Katrina Burton, B.S.
Program Manager

Jasmine Raziuddin, B.F.A.
Senior Communications
Designer



April 27, 2019



July 25, 2019



June 18, 2019



June 18, 2019



June 16, 2019

