

Pedi Press

A Quarterly Publication of the Department of Pediatrics
Baylor College of Medicine

Volume 8, Issue 4

Fall 2019





Pedi Press



A Quarterly Publication of the Department of Pediatrics

Volume 8, Issue 4

Fall 2019

Pedi Press is a quarterly publication of the Department of Pediatrics, Baylor College of Medicine, Houston, Texas.

Dr. Mark Kline, Editor-in-Chief
Dr. B. Lee Ligon, Managing Editor/Graphics Design
Dr. Gordon Schutze, Consulting Editor
Julie O'Brien Anderson, Copy and Content Editor

Next Deadline
MARCH 5, 2020

Articles and other items should be submitted to Dr. Lee Ligon at bligon@bcm.edu.

Pedi Press

A Quarterly Publication of the Department of Pediatrics

Volume 8, Issue 4

Fall 2019

In this issue:

Feature Articles

Chairman's Report – Faculty Meeting	4
Reflections and Observations on 20 Years of Transitioning Patients: Where Do We go From Here?	6

Section Events

Adolescent & Sports Medicine	10
Cardiology	11
Critical Care	12
Developmental	14
Diabetes & Endocrinology	16
Neonatology	18
Palliative Care	19
Psychology	21
Center for Epidemiology & Population Health	22
Center for Research, Innovation & Scholarship	23
Medical Humanities Group	24

Faculty, Fellows & Staff Features

Dr. Kaplan Receives the PIDS Distinguished Physician Award	26
Dr. Hotez Honored by ASTMH	27
Faculty Recognized by American Association of Pediatrics	27
Seven Pediatric Faculty Members Elected to APS	28
Dr. Hotez Featured in AMA Magazine	28
Dr. Gill Delivers Commencement Address at TWU	29
Dr. Schwarzwald Leaves to Assume New Career Focus	29
Dr. Cuevas named 2019 STAT Wunderkind	29
Department Awardees Announced at Faculty Meeting	30
Faculty and Fellows Selected for Leadership Alliance	30
Faculty briefs...	31

Research

Pilot Study Will Focus on C1z Nephropathy as Diagnosis	32
Study to Evaluate Use of Combined Agents for Treating Malignancies	33
Screening and Referrals for Pediatric Patients with Autism Spectrum Disorder Explored	33
Study Identifies Cases Likely to Respond Poorly to Treatment	34
Vaccine for Schistosomiasis Found Safe	35
Study to Evaluate a New Intervention for Young Adults with Type 1 Diabetes	35

Upcoming Events

36

FEATURE STORIES

Chairman Presents Department Summary for 2019

New Leadership Appointments

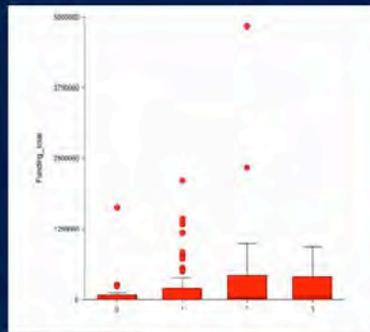
- Joan Shook, M.D., Executive Vice-Chair
- Tammy Kang, M.D., Executive Vice-Chair
- Lara Shekerdemian, M.D., Head, Department of Pediatric Medicine (TCH)
- Peter Ermis, M.D., Head, Department of Adult Medicine (TCH)

APPOINTMENTS

Dr. Mark Kline, Chairman, recognized four new leadership appointments: two Executive Vice-Chairs and two new Heads of Departments at Texas Children's Hospital.

Research Advancement Program

- 143 faculty participants
- \$36.4 million in new funding since 2015
- \$200K invested by the Department to date
- ROI – 182:1



RESEARCH

Research advancement also was impressive with 143 faculty participants and \$36.4 million in new funding since 2015.

U.S. Applicants to the Pediatric Residency Program



PEDIATRIC RESIDENCY PROGRAM

The Pediatric Residency Program had an unprecedented number of applicants for 2020, a total of 1085. The total represents an increase of 73 from the previous year and more than twice the number ten years ago.

All slides courtesy of Julie O'Brien

Other exciting news presented at the faculty meeting included the safety report from Texas Children's hospital, which has not had an SSE-related death in more than 655 days.

Serious Safety Events at Texas Children's

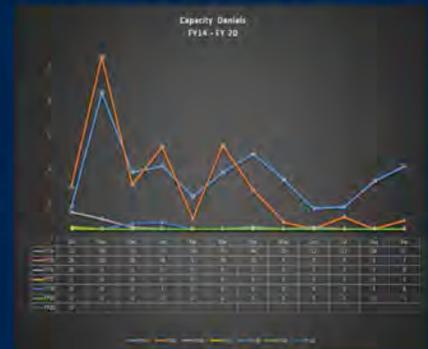
- SSEs are a reflection of an institution's culture and deviations from standards of care
- Despite dramatic growth in patient volumes, SSEs have decreased markedly, from 42 events and 7 deaths in FY12 to just five events and no deaths in FY19
- We have not had an SSE-related death in 655 days

Further, Texas Children's Hospital ended the 2019 fiscal year without have a single capacity-related denial.

Texas Children's Completed FY19 Without a Single Capacity-Related Denial

Days Since Last Capacity Denial

- Acute care, 1642
- NICU, 1479
- PICU, 643



Outstanding Clinicians, 2018-2019



Jenny Despotovic,
DO
Hem-Onc



Heather Dickerson,
MD
Cardiology



Karen Evankovich, PhD
Psychology



Terry Armstrong,
CPNP
Hem-Onc



Emily Lawrence, DO
Cardiology



Javier Chinen, MD, PhD
Allergy Immunology

Dr. Shekerdemian, Vice-Chair for Clinical Affairs announced the Outstanding Clinicians for 2018-2019. She explained the criteria as being

- full-time BCM faculty for 5 or more years
 - physician or APP
- assistant or associate professor
 - 50% or more clinical time
 - nominated by peers

Reflections and Observations on 20 Years of Transitioning Patients: Where Do We Go From Here?



"Here we are gathered again, in this place of hope, and sharing and being inspired. I am privileged to have walked these 20 years with you, especially with the youth and young adults who have shown courage beyond what I knew, who have not let their illnesses define them, who have pushed the limits. And I look forward to what we can do over the next 20 years. Thank you.

*-- Opening remarks by Dr. Albert Hergenroeder, Program Director
20th Chronic Illness and Disability Conference: Transition from Pediatric to Adult-Based Care*



On October 24 - 25, 2019, Texas Children's Hospital, Baylor College of Medicine, and the Robbins Foundation hosted the 20th Chronic Illness and Disability Conference: Transition from Pediatric to Adult-Based Care. **Dr. Albert Hergenroeder**, Professor and Chief of Adolescent and Sports Medicine, is the Program Director and gave a keynote address, during which he both reflected on where we have been and observed the challenges that lie ahead, with regard to transitioning adolescents to adult-based healthcare. The great strides that have been made over the decades in pediatrics have brought with them additional concerns and responsibilities, namely that with children living longer with their chronic diseases, both pediatricians and adult clinicians must now confront how to help these children transition from dependency to independency with regard to their healthcare and the quality of life.

Reflections on the Past

Enthusiasm for transitioning patients from pediatric to adult-based care in the early 2000s was minimal. Representative comments from Service Chiefs included: "Parents don't want to leave [pediatric care]." "Even if we continue to see them for the medical specialty needs, we would like them to have an adult PCP." "I'm worried that patients who have a serious disease will be lost to follow-up." Referrals were also inadequate: even though 70% of services at TCH reported they routinely referred the patient to an adult physician, only 45% were familiar with specific adult physicians to help with transition; only one chief reported having a protocol for transition, yet 85% of the services reported wanting help with transition. Clearly, the need for education was paramount.

Attendance at the annual transition conference has grown from fewer than 100 to mid-200s, all live attendance. Beginning in 2013, the conference was broadcast, expanding the number of attendees exponentially. For instance, in 2016, the off-site attendance soared to greater than 400. Higher numbers also were noted in 2017 and 2018. Between 2000 – 2019, conference speakers from 26 states in in the United States and from international sites, including Switzerland, the United Kingdom, and Canada, have spoken. Support for the live broadcast of the conference has been supported by the Association of University Centers on Disabilities since 2013 in addition to support from BCM/TCH and The Robbins Foundation.

Responses from patients, parents, and healthcare providers have shown the personal impact of the conference on their lives, with comments such as “You saved my son’s life.” One patient noted that she left her first conference energized to meet with legislators, and then returned in 2008; since 2010, she has attended every conference in the years since. This patient enthusiastically mentioned that she was subsequently interviewed and hired by the Texas MCH Title V program and able to return to work for the first time in years. Another attendee shared that her professional care was improved, giving her a “toolbox of resources to educate parents on how to prepare themselves and their child/young adult with special healthcare needs to transition into adult healthcare.” So was her personal life, with new skills to transition her own special-needs daughter into being more independent. Evaluations reflected the amazing impact the conference had on them: 98% of participants at the live conference would recommend the conference to others; 98% of the learning objectives of the conference were achieved, and 100% of the broadcast audience reported gaining knowledge and skills.

Changes During the Past 20 Years

Pediatric care is not what it was 20 years ago. Diseases that once took patients’ lives in childhood have been so well addressed that patients now often live longer, into adulthood and even into mid-life. To deal with the new issues, major professional organizations began to issue statements or guidelines regarding the need for improved healthcare transition from pediatric to adult-based care: the Society for Adolescent Medicine (SAHM) issued a statement in 2000, the AAP issued consensus statement in 2002, the AHA

Diagnosis	Survival/Life expectancy
Childhood cancers	46% of survivors are 20 to 40 years old; 18% of survivors are over age 40
Spina Bifida	80% reach adulthood
Congenital heart disease	85% reach adulthood; adults with CHD>1,000,000
Cystic fibrosis	50% are over age 18
Down syndrome	Life expectancy 55
Sickle cell disease	Life expectancy 55
Hemophilia	Life expectancy 60

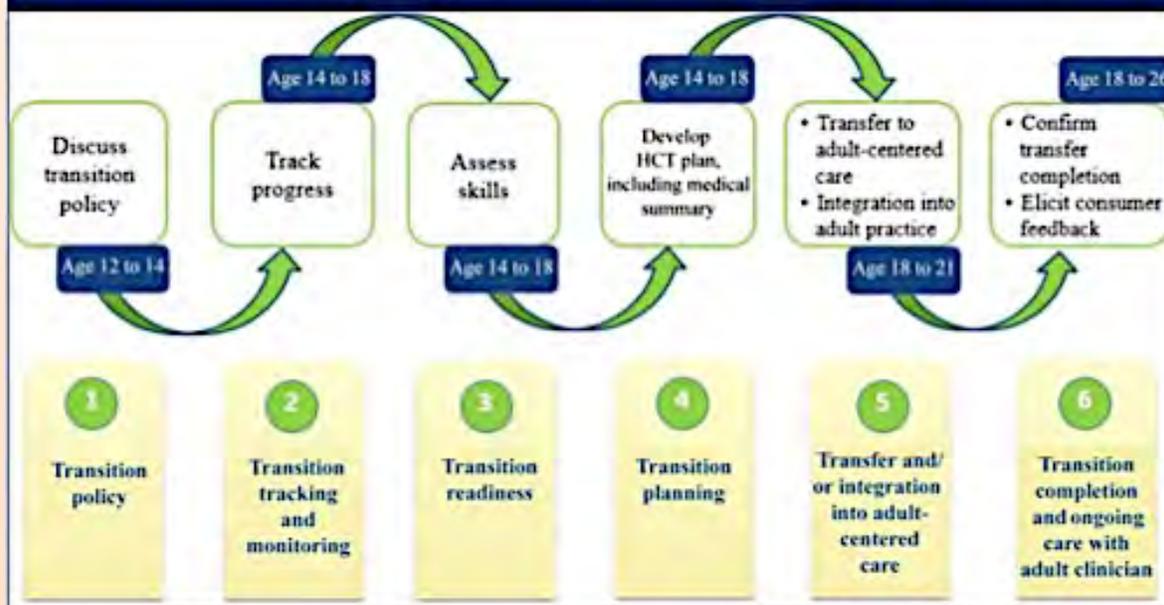
Source: Chang W. Transitioning the Chronic Ill Child to Adult Care, August 26, 2011

issued a scientific statement in 2011, the ADA issued its position statement in 2011, and the Child Neurology Foundation issued a statement in 2016. Adult providers’ “comfort levels” for treating these new populations were found to be at least 90% comfortable with healthy young adults, hypertension, and asthma; approximately 50% comfortable with behavioral and mental health conditions; and 20% or less comfortable with autism, cystic fibrosis, metabolic and chromosomal disorders, and technology-dependent issues.

In 2016, the American College of Physicians issued a pediatric-to-adult-care transitions initiative. The purpose was to develop disease-specific tools to assist physicians in transitioning young adults with chronic conditions into adult care settings, in collaboration with Got Transition, SAHM, and the Society of General Internal Medicine and more than 20 other participating organizations. On their website now are tools for eight specialties. Also, family advocacy groups have been formed and the importance of creating medical homes has been recognized.

Healthcare transition (HCT) still has hurdles, however, and time and money are required to implement successful programs. Certain actions have been reported to be beneficial: having the patient meet the adult provider before being transferred; having joint pediatrician-adult medicine clinics; providing transition coordinators; offering after-hours phone support; and having enhanced follow-up after transition. But what is needed in future research is improved research design and an ecological approach to research: including the patient, family, policy makers, adult and pediatric healthcare providers.

6 Core Elements from Got Transition



From the Got Transition National Research Center

Specific challenges that have been identified include having a definition of successful HCT; predicting successful HCT; dealing with loss to follow-up; providing professional training; having a policy to improve the interface among the educational, health, and vocational systems; and obtaining research funding. Defining successful HCT will need to include achieving optimal quality of life from the perspectives of the patients and their families.

Encouragement to Forge Ahead

Despite the numerous challenges, the future looks hopeful. In addition to important relationships that have been forged between BCM/TCH and other institutions and foundations are the exciting testimonies of patients such as that given by Isabel Stezel Bynres, who shared her story publicly for the first time at the conference. She and her twin sister both had cystic fibrosis. She shared the importance of the support they had from the family, that they were encouraged to go to college, and that both married. She also described how they both had lung transplants and that she survived but her sister did not. She has documented her story in a book called *The Power of Two: A Twin Triumph over Cystic Fibrosis*. Then there's Beth Sufian, a practicing attorney in Houston who also has cystic fibrosis and is a strong supporter of individuals with cystic fibrosis and has dedicated her professional care to advocating for others with cystic fibrosis. She is also Director of the

CF Legal Information Hotline and of the CF Social Security Project. Add to the list of such heroes as Jake Olson, who was born with retinoblastoma (cancer of the retina) and has been blind since the age of 12, but served as a backup snapper as a sophomore in 2017 and snapped an extra point attempt in the season opener against Western Michigan. He is a motivational speaker and has co-authored two books about overcoming adversity. Also, there is Ali Stoker, an actor and singer who suffered a spinal cord injury in a car accident at the age of 2 years. Nonetheless, she went on to fulfill her dream of being an actor. In 2009, she was the first actor to use a wheelchair to earn a degree, in Fine Arts, from New York University Tisch Drama Department. She subsequently was the first actor to use a wheelchair for mobility to appear on a Broadway stage and to be nominated for and win a Tony Award. And a last example is Kodi Lee, a singer-songwriter and pianist who was born with optic nerve hypoplasia, causing him to become legally blind, and later was diagnosed with autism at an early age; he also suffers from Addison's disease. None of these hindrances stopped him, however, and in 2019 he auditioned for the 14th season of America's Got Talent and in the end was declared the season's winner.

The future looks brighter than ever, inspired by individuals who would not allow their disabilities to define them.



DEPARTMENT NEWS SECTION EVENTS

Event Provides Evening of Fun - Free from Stigma & Challenges



On October 26, 2019, the **Section of Diabetes & Endocrinology** hosted a Halloween party of "EPIC" proportions. See page 16 for more information and photos.

Co-provided by:



20th Annual Transition Conference Held in October



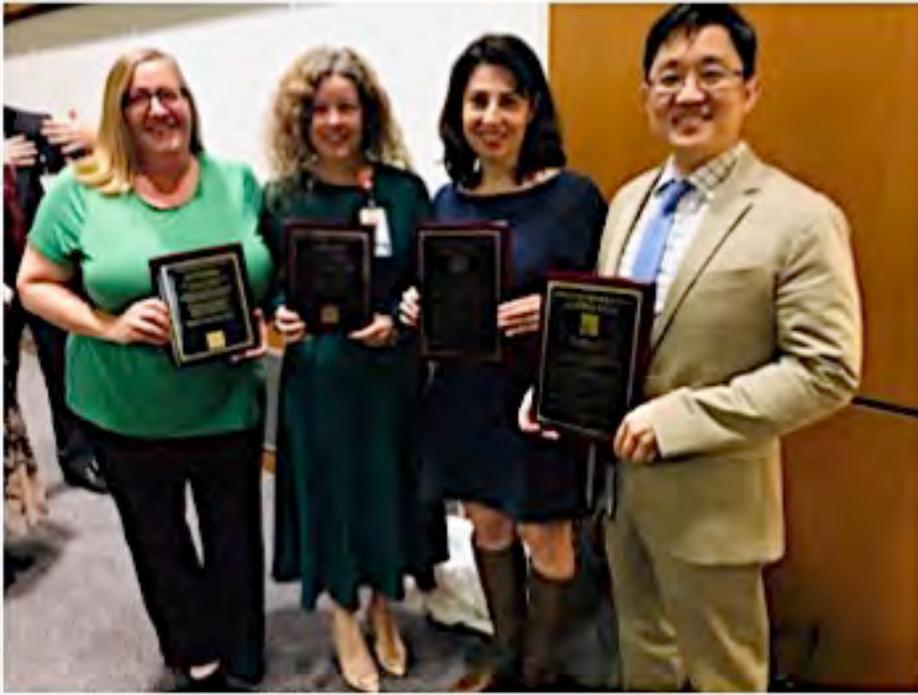
Texas Children's Hospital and Baylor College of Medicine along with the Robbins Foundation hosted the 20th Chronic Illness and Disability Conference: Transition from Pediatric to Adult-Based Care on October 24 and 25, 2019. **Dr. Albert Hergenroeder** is the Program Director.

The Conference targeted physicians in internal medicine, family practice, pediatrics, psychiatry, and physiatry; other healthcare providers including psychologists, social workers, nurses, dietitians, case managers, counselors, and primary care providers; and youth and young adults who have a chronic illness or disability and their parents or guardians. Because of increased awareness of transitioning to adult-based care, clinicians are required to update their knowledge of the changing strategies for integrating emerging adult-based care into practice.

Skill preparation and planning for healthcare transition for children and youth with special needs is known to be inadequate. Fewer than 50 percent of families nationwide indicate that their children with special healthcare needs have received the services necessary to make appropriate transitions to adult healthcare, work, and independence. One of the major hurdles to receiving this care is the lack of a workforce, including physicians, nurses, social workers, mental health providers and all who provide services to youth and young adults with special healthcare needs trained to provide the services necessary to make the healthcare transition. There is a strong need for physicians to have the knowledge and skills to provide the services needed to facilitate a successful transition from pediatric to adult-based care and services.

For all participants, there was a discussion of the legal issues involved in healthcare transition and successful healthcare transition programs and a review of the past 20 years of this conference and the field of healthcare transition. There also were small group sessions to meet and talk with faculty to exchange ideas among participants, and to share knowledge and information about how best to plan for a successful transition to adulthood.

Faculty Receive Special Recognition



l-r: Drs. Heather Dickerson, Emily Lawrence, Keila Lopez, and Jeff Kim

Outstanding Clinician award - **Drs. Heather Dickerson** and **Emily Lawrence** received the distinct honor of being awarded “Outstanding Clinician Award” at the Pediatrics Faculty Meeting.

Research Mentorship award - **Drs. Jeff Kim** and **Keila Lopez** are the recipients of 2019 Research Mentorship Awards in the Department of Pediatrics. Pediatric research, clinical postdoctoral fellows, and their peers and colleagues nominated the faculty members for their dedication and excellence in research mentorship.

Dr. Henri Justino has been elected to APS membership beginning January 1, 2020. The American Pediatric Society is the most prestigious academic pediatric organization in North America. Members are elected by a vote of the APS Council. Membership “is reserved for individuals residing in the United States or Canada who have distinguished themselves as child health leaders, teachers, scholars, policymakers and/or clinicians and whose important contributions are recognized nationally or internationally.”



Dr. Justin Zachariah has been awarded an R01 research grant from the National Institutes of Health-National Heart, Lung, and Blood Institute. The grant is titled "Causal Mechanisms in Adolescent Arterial Stiffness" in the amount of \$2,001,613.00 over 5 years. Dr. Zachariah's R01 grant advances his previous Career Development Award (K23) focusing on vascular properties in adolescents as a harbinger of future cardiovascular disease.





2019 Summit of the Americas Hosted by Section



The Pediatric Critical Care Summit of the Americas 2019 that took place November 7-10, was a unique event hosted by the Section of Critical Care. It was a joint meeting blending two pediatric critical care organizations: the U.S. based 26th Pediatric Critical Care Colloquium (PCCC) and the Latin America-based 15th Latin American Congress of Pediatric Intensive Care (SLACIP). The theme of the PCCC was “PICU: from global care to global health,” and the theme of the SLACIP was “PICU liberation, integrated multidisciplinary care, survival, and the technology-dependent child. As the conference was a Pan-American conference, all sessions were live-dually-translated into either Spanish or English. The goal for the joint conference was to create a forum for active discussions of the science and art of caring for critically ill children.



Plenary Speakers

Drs. Jesus Lopez-Herce (Spain) and Jefferson Piva (Brazil): *Intensive Care in the Americas in 2019: Forging a New Doctor*

Dr. Mark Kline (BCM/TCH): *Global/International Outreach in Pediatric Medicine*

Dr. Paul Checchia (BCM/TCH): *Pediatric Cardiac Care in 2019*

Drs. Bettina Von Dessauer (Chile) and **Fernando Stein** (BCM/TCH): *The Human Cost of ICU Survival*

Dr. Joseph Carcillo (University of Pittsburgh): *Inflammation, Sepsis, and Organ Failure: Where are We Going?*

In the end, there were more than 500 registrants from more than 15 countries; 7 pre-conference workshops; a tour of the TCH Legacy Tower for more than 100 participants; 3 full days of symposia; a bachata dance workshop; and Fiesta on Saturday night with approximately 200 attendees.



Co-chairs were
Drs. Renan Orellana
and **Nick Ettinger**.



Goals

Pediatric Critical Care Summit of the Americas 2019

- Support, encourage and promote excellence in clinical care and research in the field of pediatric critical care across the Americas.
- Encourage collegial relationships between US, Central American and Latin American pediatric intensivists and the greater inter-professional team.
- Promote basic, translational, and clinical research related to critically ill children both while acutely ill and after discharge from the ICU.
- Promote and disseminate related quality improvement and patient safety materials through established and developing communication technologies.

Event Showcases Guidance for National Spina Bifida Care



The Spina Bifida Association has updated the spina bifida–healthcare guidelines. Therefore, nationally and internationally, we now have guidance and the opportunity to care for all individuals as whole persons as they transition across the continuum of care, holistically approaching them in their language and cultural context. Globally, since 2000 there has been a 49% increase in immigration; thus, linguistically–congruous study of quality of life has enlarging relevance not only locally, but also abroad.”

Dr. Jonathan Castillo, Director
Spina Bifida Program at Texas Children’s Hospital.

*By Jonathan Porter Castillo, MD, MPH, Director of the
Spina Bifida Program at Texas Children’s Hospital*

The Meyer Center for Developmental Pediatrics at Texas Children’s Hospital, Baylor College of Medicine, and the Spina Bifida Association recently hosted the Houston Spina Bifida Education Day for patients, families, and caregivers. The event showcased newly established, evidence-based national guidelines for the care of individuals living with spina bifida. This is the first time the guidelines, written in part by TCH and BCM clinicians and researchers, were shared with such a broad audience. Families from all over Texas and other states attended the event. In addition to unveiling the new guidelines, experts from TCH presented talks covering a range of health issues faced by individuals living with spina bifida from infancy to adulthood.

The Guidelines call for clinicians and investigators to take a whole-patient approach to the care of this condition. From this perspective, Quality of Life in individuals with spina bifida is mediated by a wide-range of interrelated factors. In order to better serve this vulnerable population as they transition across the lifespan, multilingual condition-specific quality-of-life measures need to be further developed and implemented among Latino individuals with spina bifida, especially those who are recent immigrants, according to findings of a study concurrently conducted at BCM.



The Spina Bifida Team at Baylor College of Medicine / Texas Children's Hospital

Dr. Jonathan Castillo, Director of the Program at TCH, explained, "In the US, 25% of children live in immigrant families, and of the estimated 55 million Latinos, nearly three of every four speaks Spanish at home while remaining the fastest-growing demographic group. Even so, there is a lack of research on quality of life and *social determinants of health* among those living with spina bifida. At the same time, in spite of Congress's NIH Revitalization Act of 1993, calling for efforts to ensure that minority groups are included in representative numbers in research studies, few health-related quality of life studies have documented inclusion of Spanish-speaking Latinos."

Around the globe, the number of immigrants, refugees, and internally displaced persons is escalating. While immigration is often a result of social determinants, including political discrimination, poverty, education, and work-related prospects, immigration itself can also be conceptualized as a *social determinant of health*. Through the National Spina Bifida Patient Registry (NSBPR), BCM investigators have begun to recognize existing disparities within growing minority populations affected by spina bifida. This conference was a multidisciplinary effort of a large team of providers who regularly care for this population. Their teamwork not only put into practice the care coordination for which the guidelines call, but also made the teaching from these guidelines understandable to the families in both English and Spanish, bringing evidence-based patient education where it is most needed.

Patients and Parents Enjoy a Special Halloween



The Section played host to some 350 individuals at the Houston Health Museum, where patients and families experienced a “frightening night of fun.”



The purpose of the event was to provide a venue where children and adolescents with diabetes could enjoy an evening of fun without having to deal with the challenges and stigma of their conditions.





Annual Learning Session on Quality Improvement Held

The T1D Exchange Quality Improvement (QI) Collaborative held its annual Learning Session at Texas Children's Hospital on November 12-13, 2019. The Collaborative brings together 14 clinics, situated across the United States and treating more than 28,000 individuals with type 1 diabetes.

Under the leadership of **Dr. Rona Sonabend**, Associate Professor and Section Chief, and **Dr. Daniel DeSalvo**, Director of Strategic Collaborative, and **Curtis Yee**, Practice Administrator, the Section has taken a leading role in the T1D exchange QI Collaborative.

The Collaborative relies on an embedded and systemic approach: individual providers are empowered to identify areas of unmet needs within their clinic. They make small changes in care that scale up through the Collaborative to create best practices, which then are shared among and implemented by members at other clinics. More than 90 total visitors were present for the 2-day Learning Session, which was described as the best Session in the history of the Collaborative. Guests received a warm Texas welcome and were amazed at the tremendous resources available at TCH. Ms. Renee Thompson, QI Project Analyst at TCH was instrumental in making the learning session a huge success.

Annual Golf Tournament Raises Funds for NICU



The RBC Wealth Management Bad Pants Open, which attracted more than 300 golfers in their “baddest pants,” raised more than \$440,00 for The TCH Newborn Center at the Clubs of Kingwood. The Open is an annual golf tournament now in its 22nd year. During that time, it has raised more than \$7 million to support continued innovation and excellence in the research, treatment and care of critically ill and premature infants in the neonatal intensive care unit at TCH. Proceeds from the tournament are used for nurse training and education, as well as hands-on bereavement care programs.

The Open brought in Dan Boeve for a long drive exhibition, and Beck’s Prime provided golfers a lunch on the course. The golfers also received complimentary pre-golf stretching provided by Reach Stretch Studios. Among the tournament’s major sponsors were RBC Wealth Management, Jacob White Construction, CenterPoint Energy, and Pam and Gary Whitlock.

A post-play awards ceremony dinner catered by A Fare Extraordinaire followed, during which players were awarded plates painted by TCH patients for both the best golf scores and the “baddest pants.” Kelly Jett, a NICU nurse TCH, received the NICU Nurse of the Year award, a special recognition awarded at each tournament. Eva and Philip Quartey, parents of Blessing Quartey, who was born at 24 weeks gestation weighing just over a pound and received care in the NICU at Texas Children’s Pavilion for Women, were honored at the event. Eva shared how the doctors, nurses and other support staff helped her family through their 136-day long NICU journey.

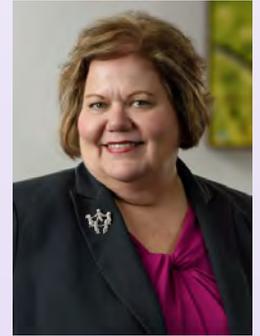
Each year, more than 2,500 critically ill and premature infants receive care in the TCH NICU. TCH has the nation’s largest NICU and is one of only two Level IV NICUs in the greater Houston area.

Section Makes Great Strides in Record Time

In only its third year at Texas Children's Hospital, the Section of Palliative Care has demonstrated itself as a national leader in the field of Pediatric Palliative Care. In addition to being one of very few pediatric palliative programs certified by The Joint Commission, hosting a top-tier physician fellowship for each of the past 3 years, and expanding palliative care services to all three Texas Children's Hospital campuses, the Section is becoming a leader in Research, Quality, and Global Outreach.



Dr. Jessica Casas (left), Assistant Professor and one of the palliative care physicians and leader of the Section's quality improvement committee, and **Joy Hesselgrave** (right), Assistant Clinical Director, have worked extensively with Amy Jeppesen, Senior Systems Analyst for Epic at TCH, and Leah Peters, Quality Improvement Specialist for TCH, to collaborate with other services and teams at Texas Children's to develop an End of Life Navigator in Epic, the electronic medical record.



The collaborative nature and thoroughness of the navigator development process culminated in a presentation at the annual Epic User Group Meeting in August 2019 (titled "Communication to the Max"), and the navigator has now been selected by Epic as a new clinical program, the first of its kind in pediatric palliative care. Epic selects clinical programs to undergo additional development and expansion in order to standardize best practices for workflow and documentation across all institutions using Epic across the country.

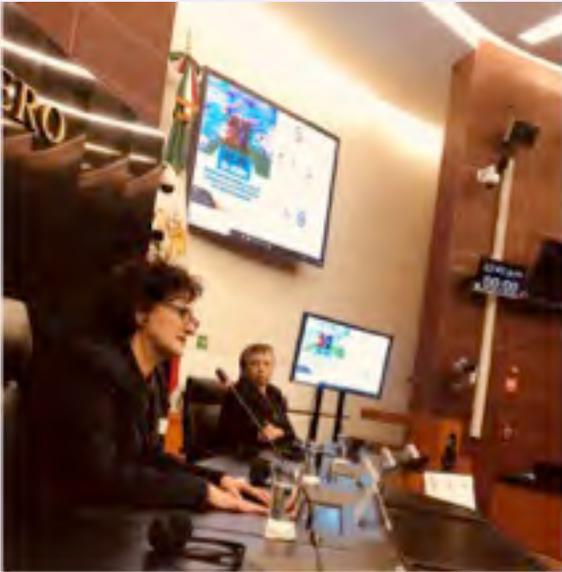
The Section is now participating in three NIH-funded, multi-center clinical trials for pediatric palliative care:

SHARE (Shared Data and Research) is a multi-site prospective cohort study developing a shared clinical database with pediatric palliative care patients and families. This database will include longitudinal data of patient- and parent-reported symptoms, parental affect and distress, parent-reported goals of care, and hospital costs. The Children's Hospital of Philadelphia is the primary coordinating site.

PRISM (Promoting Resilience in Stress Management) is a multi-site randomized controlled trial studying quality of life in adolescents and young adults with advanced cancer. The manualized PRISM intervention consists of sessions about topics including stress management, goal-setting, cognitive reframing, and benefit-finding. Seattle Children's Hospital is the primary coordinating site.

PediQUEST RESPONSE (Pediatric Quality of Life and Evaluation of Symptoms Technology Response Intervention Study) is a multi-site randomized controlled trial studying quality of life in pediatric patients with advanced cancer. The PediQUEST RESPONSE intervention includes the early involvement of a palliative care team and provider feedback on electronic patient- and parent-reported symptoms. Dana-Farber Cancer Institute is the primary coordinating site.

Section Leaders Have International Impact



Left: Gina Santucci addresses a question.



Right: Young boy who asked to stand where the senators speak rather than staying in his wheelchair to give his speech. He wanted to make sure his voice was heard loud and clear!



Ms. Gina Santucci and **Dr. Regina Okhuysen-Cawley**, members of the Pediatric Advanced Care Team (PACT) at Texas Children's Hospital, participated in an international expert panel convened by the Mexican Senate on October 28 - 29, 2019. Ms. Santucci shared her journey from critical care bedside nurse to advanced practice nurse with leadership roles in clinical care, research, and education. Dr. Okhuysen-Cawley focused on the development of palliative care services throughout the world, with an emphasis on intra-institutional, local, and global networking and outreach. A summary of the recommendations regarding pediatric palliative education and practice made by national and invited speakers, including a terminally-ill child who is receiving hospice care, was presented to the senators for review at the conclusion of the two-day workshop.

Dr. Kaplow Keynote Speaker at Healthcare Conference



Dr. Julie Kaplow, Professor and Chief of Psychiatry, was the keynote speaker at the *U.S. News & World Report Healthcare of Tomorrow Conference* and spoke candidly with “CBS Sunday Morning” host Jane Pauley about how children deal with long-term trauma and grief. In the interview, Dr. Kaplow noted that “we know that if we don’t address trauma, these kids can go on to develop lots of major common outcomes later in life,” with adverse childhood experiences being related to higher rates of post traumatic stress disorder, suicide risk, substance misuse, and intergenerational transmission of trauma.

Section Welcomes New Faculty

The Section of Pediatric Psychology announced the addition of five new faculty members, who will increase access to Behavioral Health Services throughout the hospital:

Dr. Danielle Busby, Asst. Prof., will work primarily within the TAG Center and will focus on youth suicide in the context of trauma and loss. Eventually, she will also be involved in developing a depression / suicide prevention program within the Section

Dr. Sadiqa Cash, Asst. Prof., is in the Autism Center and will focus on evaluations for children with suspected autism spectrum disorder and other neurodevelopmental conditions.

Dr. Rachel Kentor, Asst. Prof., completed her fellowship in Psychology in September and will be working within the TAG Center, Cancer Center, and Palliative Care, focusing on bereaved youth as well as those who are facing end of life.

Dr. Claire Kirk, Asst. Prof., completed her fellowship with the TAG Center in August and will be working primarily within the TAG Center providing trauma-informed assessments and interventions for both inpatient and outpatient populations.

Dr. Tish MacDonald, Asst. Prof., will be working within the Neuropsychology Program with a specific focus on cultural neuropsychology services and will be partnering with Dr. Petra Duran to further develop and expand the Psychology’s Committee on Diversity and Inclusion.

Center Takes Look at Health Needs in Houston

On November 13, 2019, the Center for Epidemiology and Population Health gave a presentation on specific health needs in the Houston metroplex. Ryan Ramphul, Sr. Project Manager for TCH's Government Relations and Community Benefits, spoke on taking a spatial look at community health needs in Houston and the surrounding area.



Once every 3 years all not-for-profit hospitals in the US are required to do a Community Health Needs Assessment (CHNA) per section 501(r)(3) of the U.S. tax code. In the CHNA, hospitals are required to investigate the key health needs in the communities they serve through an extensive data gathering process that involves interviews, focus groups, and literature reviews. This year, we partnered with three other hospitals, Memorial Hermann, CHI St. Luke's, and Methodist on an initiative to interview 52 key stakeholders around the region about community health needs. We then supplemented this information with our own series of focus groups, conducted by graduate students at the University of Texas School of Public Health. In the end, we identified the following key community health needs, prioritized by our Community Benefits Workgroup:

1. Mental and Behavioral Health - alarming rates of anxiety and depression, as well as complex conditions such as schizophrenia and bipolar disorders; trauma associated with natural disasters, most recently Hurricane Harvey, also cause distress among community members.
2. Access to Care - despite Houston having a substantial healthcare infrastructure, many residents face challenges such as transportation, lack of insurance, cost, few providers, and difficulty navigating the healthcare system, all barriers to accessing medical care.
3. Social Determinants of Health - estimates suggest that as many as 19% of Texas children live in high poverty neighborhoods, which has been linked to reduced quality of education, safety, access to healthy food, outdoor recreation areas, and healthcare resources.
4. Maternal Health - maternal mortality rates in Texas doubled between 2010 and 2012, and lower income women were reported to be more likely to experience high-rise pregnancies and be affected by maternal health issues. Respondents noted that this population often delays accessing early prenatal care until approved for Medicaid coverage, which can take 45 to 60 days; are more likely to change providers in mid-pregnancy, thereby disrupting continuity of care; and generally have less access to basic gynecological services.
5. Obesity and Chronic Disease (Tie) - prevalence of obesity in greater Houston area is at an average of 27.9%, resulting from sedentary lifestyles, dietary habits, and food insecurity; lack of access to healthy food, due to lack of health food outlets and high cost of healthy foods, poses a substantial barrier to healthy behaviors for lower-income patients.
5. Environmental Health (Tie) - because of proximity to the Gulf of Mexico and flat topography, the Houston area is considered one of the most vulnerable regions in the U.S. for flooding; since Hurricane Harvey hit, an unequal level of recovery has occurred among residents, with those of lower socioeconomic status and minority populations experiencing longer periods of recovery and greater financial hardships as a result. Houston also ranks among the top most polluted regions in America, ranking 9th for ozone pollution and 17th for year-round particle pollution. Also the paucity of greenspace remains a critical issue.



TCH Educational Scholarship Awards

2020



IMPORTANT DATES

LOI Submission: February 3, 2020 (5:00 pm)

Grant Application Submission: April 6, 2020 (5:00 pm)

Notification to Award Recipients: June 1, 2020

CRIS@texaschildrens.org



Medical Humanities/Narrative Medicine Host Three Events

The first weekend in November, visiting faculty from the Columbia University Master's Program in Narrative Medicine visited Houston to support local leaders in helping grow local engagement and expertise in the field. On November 1, Dr. Danielle Spencer, Assistant Director of the master's program, presented "Narrative, Medicine, and Imagination" at the Department of Pediatrics Grand Rounds.

On Saturday, November 2, Dr. Spencer was joined by the Director of the program, Dr. Craig Irvine, and the two hosted the inaugural **Narrative Medicine Faculty Facilitator Training Workshop** at Baylor College of Medicine. Pictured above is Dr. Spencer giving the keynote address at the workshop.

The workshop was organized by a recent graduate of the Narrative Medicine's Master's Program, current BCM-MS-4 **Sally Huang**. With support from the BCM Office of Student Affairs, faculty from the TCH Medical Humanities Group, and the BCM Narrative Medicine Institute, the full-day workshop was designed to educate faculty members in the principles and practice of narrative medicine, and to provide a foundation upon which faculty members can start to lead narrative medicine small group workshops independently.

Among the more than 40 participants were Pediatrics faculty, including **Dr. Amanda Ruth** (PICU), **Dr. Audrea Burns** (Pediatrician-Scientist Training and Development Program), **Dr. Muralidhar Premkumar** (NICU), **Dr. Daniel Mahoney** (Palliative Care), **Dr. Marina Ma** (PM&R/Palliative), **Dr. Unoma Akamagwuna** (PM&R), **Dr. Grace Kao** (Pain Psychology), and Dr. Nancy Glass (ret. Anesthesia & Palliative Care).



The inaugural Narrative Medicine Faculty Facilitator Training Workshop drew an audience of more than 40 medical students, residents, and faculty, including many from the Department of Pediatrics

Several small groups of pediatric trainees, including the Primary Care Leaders Evaluating and Addressing Disparities (LEAD) track in the Pediatric Residency Program, the Global Child Health Resident Elective rotation, and the Palliative Medicine Fellowship Program, already are engaged in narrative medicine. Several Department faculty members also are engaged in a year-long narrative medicine curriculum for professional development and wellness. Following this training, Medical Humanities faculty hope to expand the educational and professional development activities offered to trainees and faculty in the Department.



Dr. Faiz Jiwani, internal medicine resident and graduate of the Columbia University Master's Program in Narrative Medicine (far left), led one of the small group discussions

On Saturday, November 7, the group hosted the beginning of the first IRB-approved narrative medicine research project for BCM pediatrics faculty.

Beginning in 2020, BCM will include narrative medicine education as a required part of the medical student curriculum.

For more information on how to become involved with medical humanities of narrative medicine education and scholarship, please email medicalhumanities@texaschildrens.org



DEPARTMENT NEWS FACULTY, FELLOWS & STAFF FEATURES

Dr. Kaplan Receives the PIDS Distinguished Physician Award

Dr. Sheldon L. Kaplan, Professor and Head of Infectious Diseases, received the **Distinguished Physician Award** from the **Pediatric Infectious Disease Society** at the annual meeting in October in Washington, D.C. The award is presented annually to a pediatrician who has an extensive and distinguished career in pediatric infectious diseases, marked by significant accomplishments and contributions in infectious diseases, including those as a clinician, educator, and/or investigator. The pediatrician's accomplishments and contributions are nationally and internationally recognized for their excellence and support the Society's mission to advance the knowledge of pediatric infectious diseases and its application to the care of children.

Dr. Kaplan's research focuses on infections in children caused by *Streptococcus pneumoniae*, *Staphylococcus aureus*, and *Neisseria meningitidis*, including evaluating new antibiotics for children with infections caused by antibiotic-resistant bacteria.



Dr. Hotez Honored by ASTMH

Dr. Hotez . . . has long been an inspiration to physicians and physician-scientists, especially those like me in his specialty of pediatric infectious diseases. His work in neglected tropical diseases is a career on its own."

-- Chandy C. John, MD, MS, FASTMH
President, ASTMH

Dr. Peter Hotez, Professor and Co-Head of Pediatric Tropical Medicine, was honored by the **American Society for Tropical Medicine & Hygiene** during the opening ceremony of the 2019 annual meeting for "his tireless work in promoting vaccines and refuting the anti-vaccine movement. A former President of the ASTMH, Dr. Hotez was recognized for his work in neglected tropical diseases and by President Chandy C. John for his vaccine advocacy, "which has been critical in highlighting the importance of vaccines to child health - and the cost of the misinformation spread by those who oppose vaccination - to the public. He has kept on with advocacy despite some deeply personal attacks on him and his work by those who spread misinformation about vaccines. We salute him for his integrity and his courage."

He also was invited to present the 2019 Fred L. Soper Lecture at the meeting. His lecture was entitled "Vaccine and Neglected Tropical Disease: Diplomacy in our Anthropocene Epoch"

Long an outspoken advocate for vaccines, Dr. Hotez described his personal experience with his daughter Rachel in a book published in 2018, *Vaccines Did Not Cause Rachel's Autism*.



Faculty Recognized by American Academy of Pediatrics



Dr. Fernando Stein, Professor, was the recipient of the **Distinguished Career Award** from the **American Association of Pediatrics**. Dr. Stein, a past-president of AAP, is a critical care pediatrician and serves as medical director of the progressive care unit at Texas Children's Hospital.

Dr. Susan Torrey, Associate Professor, received the **Michael Shannon Humanitarian Award**. She is associate director of the education division of the Global Hematology-Oncology Pediatric Excellence Program (Global HOPE), which develops pediatric hematology and oncology services and resources in Africa.



Seven Pediatric Faculty Members Elected to APS

The American Pediatric Society is the oldest (est. 1888) and most prestigious academic pediatric organization in North America. Members are elected by a vote of the APS Council. Membership “is reserved for individuals residing in the United States or Canada who have distinguished themselves as child health leaders, teachers, scholars, policymakers and/or clinicians and whose important contributions are recognized nationally or internationally.”

In each of the past four years, more new members of the APS have come from the Department of Pediatrics at Baylor College of Medicine and Texas Children’s Hospital than from any other medical school or hospital, representing between 10 and 15% of all of the new members elected in each of those years. This year is no exception. The following seven individuals have been elected to APS membership beginning January 1, 2020:

Alison Bertuch, M.D., Ph.D. (Hematology/Oncology)

Andrea Cruz, M.D. (Emergency Medicine/Infectious Diseases)

Henri Justino, M.D. (Cardiology)

Tammy Kang, M.D. (Palliative Care)

Julie Kaplow, Ph.D. (Psychology)

Anna Mandalakas, M.D., Ph.D. (Global Tuberculosis)

Bhagavatula Moorthy, Ph.D. (Neonatology)

Dr. Hotez Featured in AMA Magazine

Dr. Peter Hotez, Professor and Dean of the National School of Tropical Medicine, was featured on the front cover and in an article by Sara Berg by the American Medical Association magazine, *Moving Medicine*. The article relates Dr. Hotez’s experiences in his youth that led to his becoming a physician-scientist working on parasitic infections, personal experiences with his daughter’s autism, and his battle against the anti-vaccine group. Although anti-vaccination ideology has been prevalent since the early 1800s, it came into the limelight in 1998, with many parents refusing to vaccinate their children. The result was that in 2017 measles cases spiked in numerous countries where parents withheld their children from receiving vaccinations. That situation prompted him to write in 2018 about his daughter Rachel, a personal story published in his book, *Vaccines Did Not Cause Rachel’s Autism*. The reaction from the anti-vaccine community was vehement, with a group that “ganged up and flooded Amazon with over 100 one-star reviews of his book.” Speaking of his responsibility and that of other physician-scientists, Dr. Hotez commented, “We’re uniquely qualified to debunk misinformation and we now have to be willing to step outside of our laboratories and speak out on this because the American public is not hearing from our scientists.”



The article is available at

<https://app.svwps.com/americanmedicalassociation/ama/fall2019mag/opendistro.html>

Dr. Gill Delivers Commencement Address at TWU

Dr. Anne Gill, Professor and Assistant Dean of Interprofessional Education, delivered the commencement address for Texas Woman's University (Houston campus) graduation on December 15, 2019, at the Marriott Woodlands. More than 3000 graduating students, faculty, and family members attended. In addition to being a Professor of Pediatrics, Dr. Gill is in the Center for Medical Ethics and Health Policy and is a TWU alumnus.



Dr. Schwarzwald Leaves to Assume New Career Focus

I know that everyone will join me in thanking Heidi for 20 years of dedicated service and leadership in the Department of Pediatrics. Although we will miss her, we all can take pride in the good work she will do with Aetna on behalf of poor children and families statewide.

**-- Dr. Mark Kline, Chairman
Department of Pediatrics**

Dr. Mark Kline, Chairman announced that **Heidi Schwarzwald**, an important participant in BIPAI's early successes in pediatric HIV/AIDS in care, treatment, and health professional training in Romania and sub-Saharan Africa, was leaving Baylor College of Medicine to pursue a new career. Dr. Schwarzwald joined the faculty in 1999 and served in various leadership roles, most recently as Section Head and Associate Vice-Chair for Community Pediatrics. She served as Medical Director of Pediatrics for TCHP from 2012 until early 2019. In her new position, she will be Chief Medical Officer for Aetna Health's statewide Medicaid Managed Care Organization.



Dr. Cuevas named 2019 STAT Wunderkind

Dr. Cristina Poveda Cuevas, Research Assistant, has been named a 2019 STAT Wunderkind by STAT news, a national publication focused on finding and telling compelling stories about health, medicine, and scientific discovery. She is involved in screening and identifying suitable vaccine candidates using novel in-vitro techniques and assays. Her research has focused on the development of an effective and safe mRNA vaccine against Chagas disease. She developed and optimized a new and innovative high-throughput platform to accelerate the development and evaluation of a vaccine that could be used to both prevent and/or treat populations afflicted with Chagas.



[information extracted from TCM article by Dipali Pathak]

Department Awardees Announced at Faculty Meeting

Research Mentor Awardees

Keila Lopez (Assistant Professor)
Michelle Lopez (Assistant Professor)
Manish Shah (Associate Professor)
Yong Xu (Associate Professor)
Jeffrey Kim (Professor)
Robert Waterland (Professor)

Highest Impact Publication Awards

Ivan Chinn (first author publication): "Genetic and mechanistic diversity in pediatric hemophagocytic lymphohistiocytosis" in *Blood*
Peggy Goodell (senior author publication): "Mutant NPM1 Maintains the Leukemic State through HOX Expression" in *Cancer Cell*

Young Investigator Awards

Lisa Bouchier-Hayes (Assistant Professor)
Sonny Harpavat (Assistant Professor)
Maria Kim (Assistant Professor)
Krithika Lingappan (Assistant Professor)
Jennette Moreno (Assistant Professor)

Faculty and Fellows Selected for Leadership Alliance

Six pediatric fellows and junior faculty from Baylor College of Medicine/Texas Children's Hospital were selected and participated in the October 25, 2019, American Academy of Pediatrics (AAP) Young Physicians Leadership Alliance (YPLA), which is sponsored by the Section of Early Career Physicians (SOECP).

Those selected included Dr. Dustin M. Hipp from Critical Care Medicine; Dr. Shweta Parmekar from Neonatology; Drs. Robert W. Loar and Jolie Britt from Cardiology; Dr. Heather A. Haq from Hospital Medicine; and Dr. Liliana Morales-Perez from Emergency Medicine (not pictured). YPLA is a "3-year training program designed to develop leadership skills and establish a network of ambitious early career pediatricians and pediatric subspecialists. Through interactive forums and small group coaching, expert faculty guide participants through an exploration of their own leadership capacity, learning about leadership principles, behaviors, and tools that can help them achieve their personal and professional goals (<https://services.aap.org/en/community/aap-sections/early-career-physicians/young-physicians-leadership-alliance/>).



Faculty briefs

Dr. Marni Axelrad, Professor, was appointed to the American Psychological Associations Advisory Steering Committee for the Development of Clinical Practice Guidelines for a three-year term. The committee consists of nine nationally recognized psychologists overseeing clinical practice guideline development for the organization of more than 118,000 members.

Dr. Stacy Berg, Professor, presented “Medical Marijuana in the Healthcare Setting” at the Bioethics Grand Rounds at Methodist Hospital. The event was sponsored in part by BCM’s Center for Medical Ethics and Health Policy.

Dr. Maria Elena Bottazzi, Professor, was inducted into the National Academy of Sciences of Honduras during a ceremony held in Tegucigalpa, Honduras on September 24. Her plenary presentation entitled “Immunizations Against Neglected Tropical Diseases” highlighted the advances in vaccine development made at BCM and TCH.

Dr. Kimberly Davis, Asst. Prof., was selected as a weekly highlighted senator for the BCM Faculty Senate.

Dr. Amy Hair, Asst. Prof., was the winner of the Perinatal Research Society Annual Associate Member Paper Prize at the PRS meeting.

Dr. Kristyn Hoffman, Student, received first-tier mention for the Young Investigator Award from the American Society of Tropical Medicine & Hygiene for her abstract entitled “Kinetoplastida: Diagnosis, Treatment and Vaccine Development.”

Dr. Peter Hotez, Professor and Dean, was honored with the Ronald McDonald House Charities Medical Award of Excellence during a ceremony at the Field Museum of Natural History in Chicago. The award recognizes individuals for their leadership and contributions to improving the critical needs of children and their families throughout the world.

Dr. Krithika Lingappan, Asst. Prof.
-- received the Young Investigator Award from the Department of Pediatrics, BCM
-- was appointed to the Editorial Board of AAP’s *NeoReviews*.

Dr. Stephen Mack, Asst. Prof., received an Early Career Development Grant from the Pediatric Brain Tumor Foundation for his project, “Harnessing Viral Mimicry to Target H3K27M-Driven Pediatric Glioma. The \$300,000, 3-year grant supports novel research by young investigators.

Dr. Diane Nguyen, Asst. Prof. and formerly BIPAI Global Health Coordinator, was appointed as BIPAI Director, Global Programs.

Dr. Frank Placencia, Asst. Prof., received his Master’s Degree in Health Care Ethics.

Dr. David Poplack, Professor, was honored by the Children’s Cancer Cause with the \$10,000 Leonard M. Rosen Memorial Research Award in recognition and support of his life-saving work around the world.

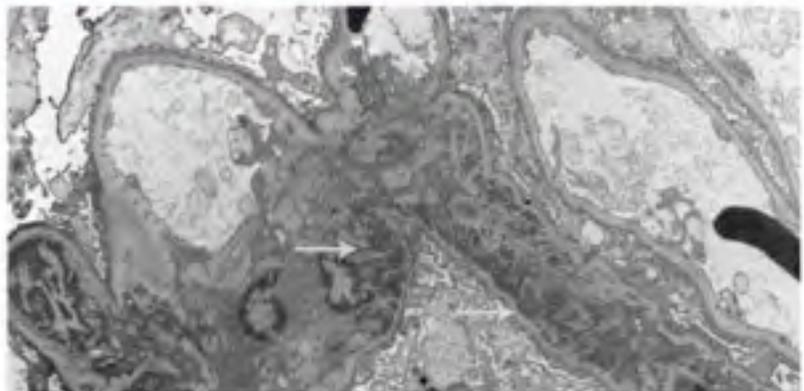
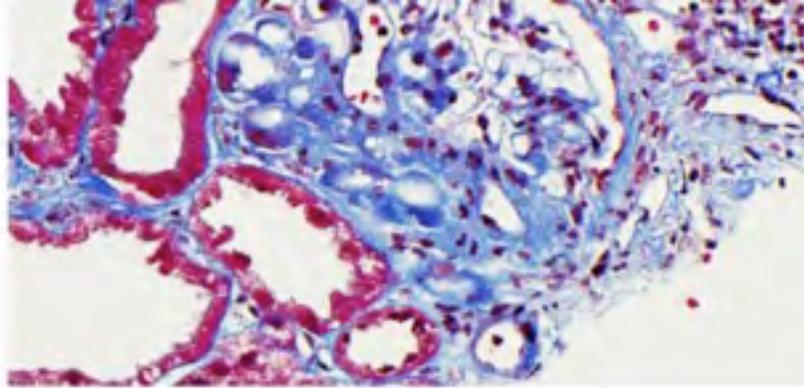
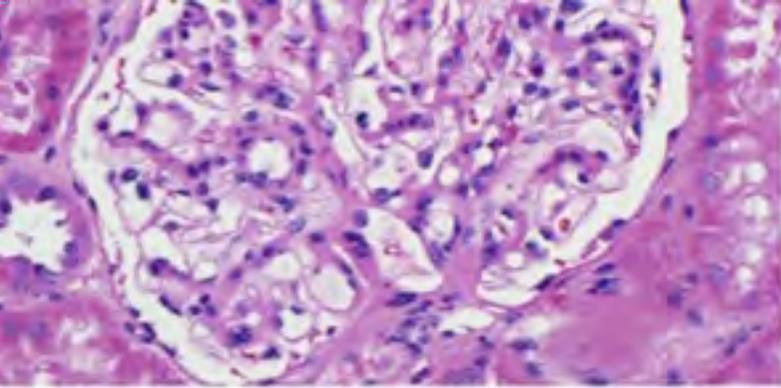
Dr. Wendy Rhoades, Asst. Prof., was selected as part of the 2019-2020 cohort of Doximity Fellows to write Op-Med pieces for Doximity, an online networking service for medical professionals. Doximity Fellows is a select group of inspired clinicians and leaders in medical news creation.

Dr. Anna Schrader, Instructor, was named the 2019 recipient of the Physician Assistant of the Year award from TCH. She demonstrates a commitment to patient and family centered care in her work with the multidisciplinary lung transplant team and is involved in education through mentoring students.

Dr. Michael Speer, Professor, was elected the Vice-Chair of the Texas Department of State Health Services Newborn Screening Advisory Committee.

Dr. Teri Turner, Professor, has been named President-elect of the Academic Pediatric Association, an organization dedicated to child health professionals engaged in research, advocacy, improvement science, and educational scholarship.

Dr. Leonard Weisman, Professor, retired after more than 25 years at BCM.



DEPARTMENT NEWS RESEARCH

Pilot Study Will Focus on C1q Nephropathy as Diagnosis

Dr. Scott Wenderfer, Associate Professor of Pediatrics, Renal Section, has received the 2020 NephCure-CureGN Pilot Project Award. This award provides research support for a peer-reviewed clinical study on the kidney disease C1q nephropathy.

Nephrotic syndrome (NS), a leading cause of kidney failure in adults and children, is classified by kidney biopsy, performed in patients who present with abnormal urinary protein excretion. Many patients with NS are resistant to medical therapy, and large gaps exist in our understanding of mechanisms of injury causing NS. Meeting this critical need should translate into better novel therapies. Initial reports linking isolated deposition of a protein called C1q to a distinct type of NS have been contentious. Due to its rarity, the relevance of C1q nephropathy as a diagnosis has been vigorously debated.



Now, the availability of 3,000 highly phenotyped patients with NS in two large research cohorts (NEPTUNE and CureGN) finally provides the opportunity to study the value of C1q nephropathy as a clinical diagnosis. The Cure Glomerulopathy Network (CureGN) is the largest prospective registry of patients with glomerular disease, involving 2400 patients across North America. NephCure International is a patient-oriented disease advocacy organization. Dr. Wenderfer will be leading a multicenter team including nephrologists, pathologists, epidemiologists, and pediatricians.

Study to Evaluate Use of Combined Agents for Treating Malignancies



Dr. Rayne H. Rouce, Asst. Prof. and Assoc. Director of Community Engagement, BCM, will collaborate with researchers from the BCM Center for Cell and Gene Therapy and The Methodist Hospital System in a study entitled “Autologous T-Cells Expressing a Second Generation CAR for Treatment of T-Cell Malignancies Expressing CD5 Antigen (Magenta).” She and colleagues plan to combine two different ways of fighting disease: antibodies, proteins that protect the body from bacterial and other disease, and T cells, special infection-fighting blood cells that can kill other cells including tumor cells.

In other studies, researchers have taken T cells from a patient’s blood, grown them in laboratory, and then put them back into the patient. The antibody that will be used in this study is anti-CD5, which first came from mice that had developed immunity to human leukemia. The antibody sticks to T-cell leukemia or lymphoma cells. CD5 antibodies have been used to treat patients with T-cell leukemia and lymphoma. For this study the anti-CD5 was changed to that, instead of floating free in the blood, it is joined to the T-cells to form a chimeric receptor. T cells have been shown to work better if proteins that stimulate T cells are added. One such is CD28, which makes the cells grow better and last longer in the body, thereby providing the cells a better chance of killing the leukemia or lymphoma cells.

Dr. Rouce and her team will attach the CD5 chimeric receptor with CD28 added to the patient’s T-cells and then test how long the cells last. The CD5 chimeric receptor T cells with CD28 are investigational products not approved by the U.S. Food and Drug Administration.

Screening and Referrals for Pediatric Patients with Autism Spectrum Disorder



A collaborative study by researchers at BCM and Children’s Hospital Colorado exploring different providers’ diagnostic and referral practices for children who failed the M-CHAT at the 18- or 24-month visits was published recently in *Pediatrics*. The M-CHAT (Modified Checklist for Autism in Toddlers) is a popular screening tool for identifying toddlers with autism spectrum disorder (ASD); it has a standardized questionnaire that screens for behaviors that toddlers who have ASD might be expected to exhibit.

According to Dr. Robin Goin-Kochel, Assoc. Prof. and senior author on the article, a child who scores 3 or higher is considered to have failed the screening; a score of 3-7 places a child in the “gray zone,” and providers are encouraged to do a follow-up interview. The interview helps clarify the behaviors that the child “failed,” such as failing to respond to his/her name or preferring isolated play; it also helps to improve the specificity of the M-CHAT.

The study focused on children who failed the M-CHAT at 18 and/or 24 months, and researchers reviewed their records over the course of 2 to 4 years, during which they examined the types of referrals that were made, whether the families completed the referrals, and what diagnoses were documented in the medical record. Despite the high rates of screening that were found, only 31 percent of providers placed referrals for autism-specialists after the failed screens, and only approximately half of the families that received referrals completed them.

The researchers concluded that screening for ASD alone does not translate into earlier diagnoses of ASD or of children at a younger age. They emphasized the importance of providers acting on failed screens and of families following through on referrals if children are to begin appropriate interventions such as applied behavior analysis (ABA).

Study Identifies Cases Likely to Respond Poorly to Treatment

"We've reached the limits of intensive chemotherapy for improving survival in ALL – adding higher doses or additional drugs increases side effects and often doesn't improve response . . . This study is important in identifying a potential alternative approach to attacking leukemia cells in patients with high-risk disease."

--Dr. Karen R. Rabin

"This research is a demonstration of the power of multidisciplinary collaborations. By bringing together individuals with expertise in epidemiology, pediatric oncology, and leukemia biology, we have made an important step towards identifying a biomarker of treatment response for children diagnosed with ALL, as well as a potential therapeutic target."

--Dr. Philip J. Lupo

Researchers at Baylor College of Medicine and the Texas Children's Cancer Center recently reported their findings that profiling the bone marrow "metabolome," the constellation of small molecules produced by metabolism in bone marrow, at the time of diagnosis allows to identify patients most likely to respond poorly to treatment.

Once their profiling revealed that metabolites involved in pathways of the central carbon metabolism were more abundant in patients who presented with minimal residual disease after treatment than in patients who cleared the disease, these researchers tested in the laboratory the effects of disrupting central carbon metabolism on ALL cells.

"We found that drugs that interfere with central carbon metabolism had an anti-leukemic effect both in ALL cell lines and in pediatric patient samples" and that the "effect was observed with dose ranges that had previously been shown to be achievable and well-tolerated in patients with other forms of cancer."

--Dr. Jeremy M. Schraw



The research was performed in the attempt to identify cases of patients who survive pediatric acute lymphoblastic leukemia (ALL) but are most prone to adverse results of therapy.

In a retrospective study, Dr. Jeremy M. Schraw, postdoctoral associate, and his colleagues, including **Drs. Karin Rabin** and **Philip J. Lupo**, both Associate Professors in pediatric hematology and oncology, looked for potential biomarkers in the bone marrow. They hypothesized that the metabolome reflects the patients biological characteristics. No previous research has profiled the metabolites produced in the bone marrow to identify high-risk patients with ALL.

The researchers' findings are important because they highlight the potential value of the metabolome as a means to identify high-risk ALL patients and plan more effective treatments. Metabolite profiling may reveal potential Achilles' heels of other conditions as well, such as particular metabolic pathways like central carbon metabolism in ALL, which might suggest potential new treatment strategies.

Other authors who contributed to this work include Jacob J. Junco, Austin L. Brown and Michael E. Scheurer.

Financial support for this study was provided by a Pediatric Pilot Award from the Baylor College of Medicine Department of Pediatrics, the Cancer Prevention and Research Institute of Texas (CPRIT RP160097), the American Society of Hematology Scholar Award, the Lynch family, the National Cancer Institute at the National Institutes of Health (K07 CA218362) and a Consortium Grant by St. Baldrick's Foundation with generous support from the Micaela's Army Foundation.

Vaccine for Schistosomiasis Found Safe

Texas Children's Hospital Center for Vaccine Development at Baylor College of Medicine and its consortium partners for the Schistosomiasis Vaccine Initiative, have published the results of the first human phase 1 clinical trial of the *Schistosoma mansoni* Tetraspanin-2 (Sm-TSP-2) vaccine. Authors on the article published in *Vaccine* included **Drs. Peter Hotez** and **Maria Elena Bottazzi**. Schistosomiasis, also known as snail fever and bilharzia, is a debilitating parasitic disease that infects hundreds of millions around the world and is considered one of the deadliest of the neglected tropical diseases. Symptoms include abdominal pain, bloody diarrhea, cough, malaise, and headache.



The researchers assessed the safety and immunogenicity of a candidate Sm vaccine in a phase 1, double-blind, dose-escalation trial in 72 healthy Sm-naïve individuals aged 18 – 50 years of age. They were randomized to receive 3 doses, 8 weeks apart, of saline placebo, or 10 µg, 30 µg, or 100 µg of recombinant Sm-Tetraspanin-2 vaccine formulated on aluminum hydroxide adjuvant (Sm-TSP-2/Al) with or without 5 µg of glucopyranosyl lipid A aqueous formulation (GLS-AF). They then assessed clinical and serologic responses for 1 year after administration of dose 3. This trial concluded that the vaccine was “safe and immunogenic” in the population previously naïve to *Schistosoma mansoni*. The authors highlight that the vaccine could “become a key technology and our best hope for eliminating this neglected tropical disease.”



Study to Evaluate a New Intervention for Young Adults with Type 1 Diabetes

Type 1 diabetes (T1D) management is challenging for young adults, who assume responsibility for self-management and transfer T1D care from the pediatric to adult healthcare system as they also manage typical developmental demands (e.g., social, financial, work/school, residential). Many young adults have extended (>6 months) gaps before follow-up in adult care, which increases their risk for poor health outcomes and serious health complications. Very few evidence-based programs support young adults with T1D during the transition period between pediatric and adult care. Novel, developmentally appropriate approaches are needed to help them negotiate a timely transition to adult care. **Dr. Marisa Hilliard** received a grant of \$1,303,531 from NIH to investigate ways to enhance the transition.



The goal of the proposed study is to evaluate a new intervention for young adults with T1D that maximizes diabetes-related social support by building on young adults' social support network and providing them access to relevant information about T1D care in adulthood. As they leave pediatric T1D care, young adults with T1D will be paired with a trained “Peer Mentor,” an experienced, slightly older young adult with T1D, who will meet with them in-person and have frequent (weekly, then monthly) contact for 12 months. During the contacts, the Peer Mentor will use strengths-based psychoeducational strategies to teach and model problem-solving and stress-management skills, encourage the young adult to seek support from their existing social support networks, identify new sources of diabetes-related support, help prioritize T1D self-care during the very demanding young adult period, share his/her transition experiences and strategies for successfully navigating the adult healthcare system, and help prepare for the first adult care visit.

The study will test intervention with 150 young adults randomly assigned to the intervention or usual care. Our main goal is to evaluate the intervention's impact on important T1D outcomes, such as glycemic control, time to adult care, treatment adherence, and psychosocial well-being. Clinic appointment information, questionnaires, adherence data, and glycemic control biomarkers will be collected at baseline and 6 and 12 months later. The ultimate goal of this research is to validate a supportive intervention that can facilitate T1D self-management and transfer of care during the difficult young adult years and promote optimal T1D health outcomes.

Upcoming Events . . .

**Ben and Margaret Love Foundation
Bobby Alford Award for Academic Clinical Professional**
January 31, 2020
Nominations Deadline

**Faculty Awards for Excellence in Patient Care Early Career
Star**
January 6, 2020
Portfolio Submissions Deadline
Master Clinician
February 25, 2020
Nominations Deadline

Soft Tissue Sarcoma Conference
February 6 - 7, 2020
txchconference@texaschildrens.org

**Norton Rose Fulbright Faculty Excellence Award
(Teaching & Evaluation, Education Leadership,
Educational Research, Enduring Educational Materials**
March 16, 2020
Portfolio Submissions Deadline

2020 TCH Educational Scholarship Awards
Tuesday, March 24, 2020
LoI Submission
February 3, 2020
Grant Application Submission
April 6, 2020 (5 pm)
Notification to Award Recipients
June 1, 2020

2020 Pediatric Research Symposium
Tuesday, March 24, 2020