

hopkinschildren's

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THE JOHNS HOPKINS CHILDREN'S CENTER MAGAZINE

sofia's story

An unprecedented breakthrough for children with spinal muscular atrophy?

Treating the Chronically Critically Ill Child
Complex Care Consult Service takes on the challenges

Pediatrics and the Prize
The evolution of pediatrician scientists



Navigating the Storm

Margaret Moon, M.D., M.P.H.
Chief Medical Officer

THIS IS NOT the first time we have faced a fierce storm threatening our patients and families, our faculty and staff members. This coronavirus pandemic, however, has been uniquely challenging, and has confounded epidemiologists, infectious disease specialists and other health professionals, including those who care for children. So, how have we responded since the virus arrived here in February? Through what we are known for — collaborative problem solving!

From the start, **Tina Cheng** and **David Hackam**, our Children's Center co-directors, opened the conversation with leaders across Johns Hopkins to ensure that we are sharing information and resources as best we can. Then, anticipating the multiple complex issues to address, within a week we established our Children's Center Incident Command Center. Pediatric nurse **Cathy Garger**, whose role as disaster coordinator gives her deep experience in incident command structures, quickly helped us understand what needed to be done. Similarly, pediatric emergency medicine physician **Amyna Hussein**, who has worked nationally in disaster relief preparedness, filled in the missing pieces to create an effective structure.

For this crisis, liaison to the infectious disease experts has been critical. Infectious disease specialists **Aaron Milstone** and **Anna Sick-Samuels** review our protocols to ensure that we optimize patient care while avoiding virus transmission. They also keep us fully informed about the emerging science regarding the poorly understood COVID-19 pandemic. Our pediatric hospital medicine team, led by **Eric Biondi**, hospital medicine director, along with **Lisa Fratino**, our nurse clinical operations lead, redesigned our bed management plans to create COVID-19 safe rooms for children.

For our ambulatory patients in the community, the challenge was how to balance social distancing with necessary care. **Barry Solomon**, **Megan Tschudy** and others at the Harriet Lane Clinic contacted our patients and their families through MyChart messaging, emails and telephone calls to ensure their health care needs, such as immunizations, were being met. Rather than waiting for our medically complex patients to call us, our team has been systematically calling them to be sure they have the medicines and supplies they



need, and that they are making appointments, either through telemedicine or in person. Pediatrician **Helen Hughes**, our telemedicine lead, has done a brilliant job of coordinating, teaching and encouraging faculty to adopt this practice. Consequently our telemedicine visits have increased dramatically. **Phil Spevak**, chief informatics officer, made sure our efforts to telecommunicate via MyChart kept up with our patients' needs. Along with senior business intelligence analyst **Muhammad Ismail**, Spevak has generated the data necessary to track telemedicine performance and outcomes.

Private pediatric practices and their patients have been supported as well, thanks to pediatrician **Rachel Thornton**'s coordination with the Maryland chapter of the American Academy of Pediatrics and our pediatric advisory group. Also, Thornton and child psychiatrist **Hal Kronsberg** have been educating parents how to talk about the coronavirus with their children to help lower their stress. Similarly, chaplain **Matt Norvell** has been a resource for our staff dealing with anxiety during this difficult time.

How did we pull all of this off? With such a rapidly evolving crisis, we had to establish well-ordered communications. To keep everyone informed, **Rebecca Trexler**, our patient- and family-centered care project administrator, sends out staff reports on all daily COVID-19 briefings, which helps staff members feel comfortable about voicing concerns and offering ideas. This is invaluable. If people are silent in a situation like this out of fear of being shut down, the risks increase dramatically and we fail. I am thrilled to see a culture of support, open discussion and honesty that has helped us avoid critical mistakes. Right now, that's incredibly important. 🌟



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Good News, Bad News?

So, a good news/bad news kind of year? Between pediatrician scientist **Gregg Semenza** being awarded the Nobel Prize in Physiology or Medicine and the escalation of the coronavirus into a global pandemic, one could easily make the case.

Indeed, we were thrilled when Gregg was honored for his discovery of how cells sense oxygen. He came to our department over 30 years ago, and early on emphasized the need to address the early antecedents of health. He decided to do so as a basic scientist who shed illuminating light on how cells behave around oxygen, which has implications for cancer, diabetes and heart disease, and how well patients heal from horrific burns and surgery for conditions like necrotizing enterocolitis. As a pediatric community, we should be incredibly proud that Gregg has won the Nobel Prize in Physiology or Medicine, only the third pediatrician in history to do so (page 24).

More good news — we've recruited pediatric cardiac surgeon **Bret Mettler** from Vanderbilt and dramatically re-energized and reorganized our Blalock-Taussig-Thomas Pediatric and Congenital Heart Center. Now within one collaborative structure we have the disciplines of pediatric cardiac critical care, anesthesia, cardiology and cardiac surgery — a natural progression in the place where congenital heart surgery came into existence in the United States with the first blue baby operation (page 34).

On the other end of the spectrum, our patients, families and staff are now facing the global coronavirus health crisis. Bad news, yes, but we're very impressed with our staff's speedy mobilization to address this challenge. Remarkable how many people have stepped up to think of solutions to manage what we're facing.

One individual who has long navigated us through such difficult times is administrator **Ted Chambers**, the constant here in the Children's Center and at Johns Hopkins over the last four decades. He was very integral in creating and constructing The Charlotte R. Bloomberg Children's Center building, and he put in place so many of the ingredients that fill it, the programs, policies and resources that people needed. His priority was singular and that was taking care of kids — he was pretty amazing at advocacy and making sure we got that right (page 3). The bad news is Ted is leaving; the good news is we, our patients and families, have gained so much from his warmth and wisdom. Thank you and enjoy this issue.

Tina Cheng, M.D., M.P.H.
David Hackam, M.D., Ph.D.
Co-Directors, Johns Hopkins Children's Center



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Shannon Ciconte
*Interim Director,
Marketing & Communications*

Gary Logan
Editor

Amanda Leininger
Executive Assistant Editor

Helen Grafton
Assistant Editor

Julia McMillan, M.D.
Cozumel Pruette, M.D.
Medical Editors

Karen Blum
Christen Brownlee
Julie Dubin
Mat Edelson
Michael E. Newman
Karen Nitkin
Allison Smith
Contributing Writers

Rachel Sweeney
Graphic Designer

Keith Weller
Photography

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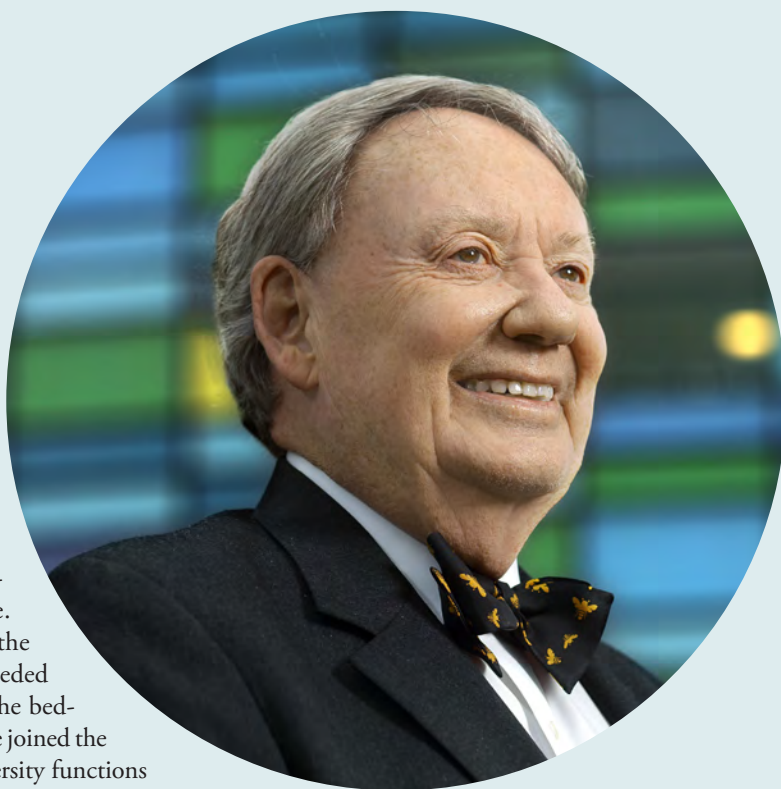
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For more information
To read more on the clinical services and programs covered in *Hopkins Children's*, visit hopkinschildrens.org.

How you can help
Call 410-361-6493

Ted Chambers, Beyond the Bowtie

The longtime administrator reflects on his role models over his four decades of service.



One cannot avoid asking, “Why the bowtie?”

Two bowtie-aficionados, Edward Halle and Barton Childs, inspired me to wear a bowtie. Ed, who was an administrator, did not have a background in health care, but he was the smartest, most financially acute person I ever met. Barton was a pediatrician and a geneticist who was extraordinarily generous in his willingness to share knowledge. I admired him so much because he never really stopped working and contributing to the institution. Those he inspired, people like Dave Nichols and George Dover, followed his lead.

How so?

Barton’s mantra was medicine had become overly routinized and data-driven — residents and new physicians were losing their ability to use their eyes and ears, their instincts, to treat patients. You could hear in the tone of a mother’s voice whether this was serious or not, yet that skill was being lost. Dave Nichols (former vice dean for education) took that insight and transformed the whole medical school curriculum. He had learned medicine by learning about organ systems, but he didn’t interact with patients. He turned that around so that medical students saw patients as they were learning about systems — that way, you never lost the humanity of patient care.

Other influences?

Robert Heysell (former president of the hospital) was probably the most inspirational person I ever met. A truly transcendent thinker, he argued that the management of patient care needed to be

as close to the bedside as possible.

That also meant the administration needed to be as close to the bedside as possible. He joined the hospital and university functions into one entity, which became the Department of Pediatrics.

How would you distinguish the faculty here?

The faculty here, perhaps more than any other place in the country, are geared toward teaching. That openness to information and knowledge — for a number of the administrators like me — was what kept us here. The Children’s Center is a very stimulating environment.

But they also take excellent care of patients and do groundbreaking research.

Yes, and that’s deeply embedded in the culture. Look at the whole triple-threat promotion system here. Every other place in the country has multiple tracks, with this whole decision tree of where you could fit yourself. Hopkins said ‘no,’ and stuck to it for many, many years — you had to be good at everything, which shaped who would survive and become our faculty.

What memories or messages will you take with you?

Many, but I remember Arnold Patz in ophthalmology, who was a very shy, unassuming individual. It was kind of magical that he would go on to win the Lasker Award for recognizing that high doses of oxygen were causing blindness in pre-

mature infants. Then there’s George Dover, who, as a pediatric hematologist, came up with today’s gold standard treatment for the painful crises of sickle cell disease without ever having a lab to himself, which is unheard of in academic medicine. It has been such an honor to be around such incredible minds and truly committed and remarkable people.

That’s it?

Well, I always tell this story of Bob Heysell when I had an opportunity to be administrator of the Department of Neurosciences. I was on the fence and walked into his office with this dilemma: Should I stay, or take the job? He cut me off: “Ted, you’re taking yourself far too seriously. Nobody really cares about what your next step is from a career standpoint. Hopkins was great before we arrived, and Hopkins will be great when we leave. The only thing you have to worry about is being a steward of the time you have here.” So, that’s pretty much how I’ve lived my life for the last 40 years. Do the best that you can with what you’ve got, and try to be loyal to Hopkins because it’s bigger and better than any one person or group of people. 🍀



sofia's story



Will gene-targeting
therapies offer
unprecedented outcomes
for children born with the
severest forms of spinal
muscular atrophy?

By Gary Logan



On the morning of Friday, Aug. 23, 2019, a newborn lies in her father's lap at Johns Hopkins Home Care Group in Dundalk, Maryland.

Image on opposite page shows then four-week old Sofia at her gene-therapy infusion with parents Brooke and Ryan Summer. At right, pediatric neurologist Tom Crawford tests Sofia's reflexes at her monthly clinic visit as her parents look on.



Her curious eyes

peer up at the sea of rainbow scrubs and the wide smiles of nurses and staff around her. “Sofia, Sofia,” they say, wanting her attention as an infusion technician at her side pushes a blue button on a panel, releasing a revolutionary new therapy intravenously through a vessel in her arm and triggering applause. Sofia’s face remains pink and animated, but her arms beneath a turquoise blanket are stone still at her side, her legs seemingly lifeless, signs of the neuromuscular disease that began afflicting her before her birth.

Sofia has spinal muscular atrophy (SMA), which affects 1 in 10,000 infants and results in progressive loss of mobility,

respiratory complications, muscle weakness and atrophy, as well as challenges swallowing and eating. SMA is also the leading genetic cause of infant death worldwide. Sofia has the most lethal form of the disease, SMA type 1, which claims the lives of most patients by age 2. However, on this day, at this moment, this therapy, clinical trials had shown, could dramatically change the course of the disease for Sofia and patients like her.

“We had been talking about this for months — everybody was talking about it — to have everything ready for a baby like Sofia,” says her pediatric neurologist, **Tom Crawford**. Over the past year or two, he explains, the team felt they

were at the cusp of their first gene transfer therapy for patients with SMA and now they were there. “Everyone knew their role and what they were going to do. Everybody knew this was a pivotal moment.”

What was behind this pivotal moment, this revolutionary new treatment for SMA? It is actually one of three novel gene-targeting treatments recently approved by the U.S. Food and Drug Administration (FDA). Over the past four decades, with no cure for SMA in sight, Crawford had worked with families and patients to promote their development and identity in the face of the disease. Rather than shelter and isolate these young patients, he encouraged parents to push them out into the world, no easy chore. At the same time, Crawford continued his efforts to find a cure through participating in clinical trials, though research gains were modest — until the FDA’s approval of the gene-targeting therapies. Then, everything changed.

The therapies, while not a cure, demonstrated new measurable motor milestones, such as head control, sitting,

“She’s grabbing my beard with her right hand. I said to myself, ‘Wow, that’s pretty cool.’ On her back, she’s lifting her legs straight up more, too.”

— RYAN SUMMER, SOFIA’S DAD



crawling and even standing for infants like Sofia with SMA type 1. In a Phase I clinical trial of 15 patients up to 6 months of age who received the same therapy Sofia received, at the study cutoff all the children were at least 20 months old and none required permanent mechanical ventilation. In comparison, only 8% of patients in a historical group survived to the same age without permanent mechanical ventilation. Seven children in the high-dose group were completely independent of ventilator assistance; 11 patients had achieved and retained the ability to swallow independently and four were able to feed orally.

Among the 12 infants who received the high dose, 11 were able to achieve head control. Nine were able to roll at least 180 degrees from the back to both the left and right and could sit unaided for at least 30 seconds, something normally never seen in babies with SMA type 1.

For Crawford and his patients, the sun had broken through the clouds. “For newborns with SMA, the change in their

course of disease is life-changing,” says Crawford. “Patients were able to feed themselves and get in and out of their wheelchairs by themselves, compared with certain death.”

How do the therapies work? SMA is caused by a mutation in the survival of the motor neuron 1 (SMN1) gene, which encodes a protein that motor neurons need to survive. The mutation prevents this gene from producing that protein. Every patient with SMA, however, retains at least one copy of SMN1 — the most severe patients tend to have two copies, milder patients up to four copies — which contain some protein. Two of the gene-targeting therapies are designed to splice and hijack a copy to create a molecule that can be translated into normal protein in the SMN1 gene.

The therapy Sofia received, on the other hand, works by delivering a working copy of the SMN1 gene to motor neurons in SMA patients, in effect replacing the missing or nonworking protein. In the

simplest terms, Crawford

suggests, rather than fixing a flat tire via the splicing approach, you are swapping it with a spare.

“You’re restoring normal function by introducing a functioning copy of the SMA gene,” Crawford explains. “The science is miraculous.”

What’s not miraculous is the ease of getting these therapies to patients — and as early as possible. For decades, patients went undiagnosed for months, (in some cases years), until physical symptoms, follow-up exams and lab tests confirmed SMA, a disease that begins its manifestations in utero. Maryland’s inclusion in May 2019 of adding SMA to its newborn screening panel, which prompted a call to Sofia’s pediatrician and Crawford during her first week of life, helped immensely.

“In this disease, motor neurons are dying over time. The kids we’ve treated at six months are not going to die but they had a fair amount of damage before they received the drug,” says Crawford. “If, on the other hand, I can do an infusion at two weeks, there’s very little damage, and I am stopping the process of motor neuron degeneration.”

Early detection, however, is not the only factor affecting outcomes. The cost of the new gene targeting therapies — up to \$2 million — and obtaining insurance coverage, institutional approval and coordination to provide the infusion, is a complicated process, which can also cause delays.

“We worked with people across the organization, from leadership and

“In a child with SMA1, especially one who had shown such a precipitous decline, this is extraordinary. It’s clearly working. Every case is different but this is so amazing from what used to happen before gene therapies.”

— TOM CRAWFORD, PEDIATRIC NEUROLOGIST



Since her infusion, Sofia's parents have measured her growth weekly using their own blanket algorithm.

administration, getting everyone to understand the whole process, the complexities involving treatment for these very small babies," says Crawford. "Then there's connecting families to additional financial resources, like grants and foundations, to help pick up the costs. Otherwise, patient families see the sticker price and abandon the drug. This is uncharted territory — no one here has written a purchase order for a \$2 million drug."

Crawford and his team, however, pulled it off, partly by collaborating with the Johns Hopkins Home Care group to hold the infusion procedure at their facility in nearby Dundalk to lower the costs associated with a hospital-based treatment. Still, Sofia was treated four weeks after her birth and, during the time between her diagnosis and treatment, she had started to decline. When Crawford saw her the first time on August 8, her arms were stuck on her side and she could not roll her head side-to-side, signs that Sofia was on the more severe end of the SMA1 spectrum.

There is variation in how well individ-

ual patients respond to the gene therapy, but the most significant determining factor, Crawford reiterates, is when they receive it. So, how did Sofia react to the therapy?

On Aug. 31 in the neurology clinic, a week and a day after her infusion, Sofia was moving her arms: the first inkling of improvement. On her next weekly follow-up appointment, she was "moving her arms a bit more," noted Crawford. "This is incontrovertible."

At her mid-September check-up, Crawford reported that Sofia scored higher on the assessment scale of infant movement. Then, on Sept. 24, Sofia's parents shared a video of Sofia moving both arms, bringing her hands up to her mouth, holding her head up and making facial expressions. Moreover, she was crying loudly, the single most critical measure of the strength of breathing muscles for babies with SMA.

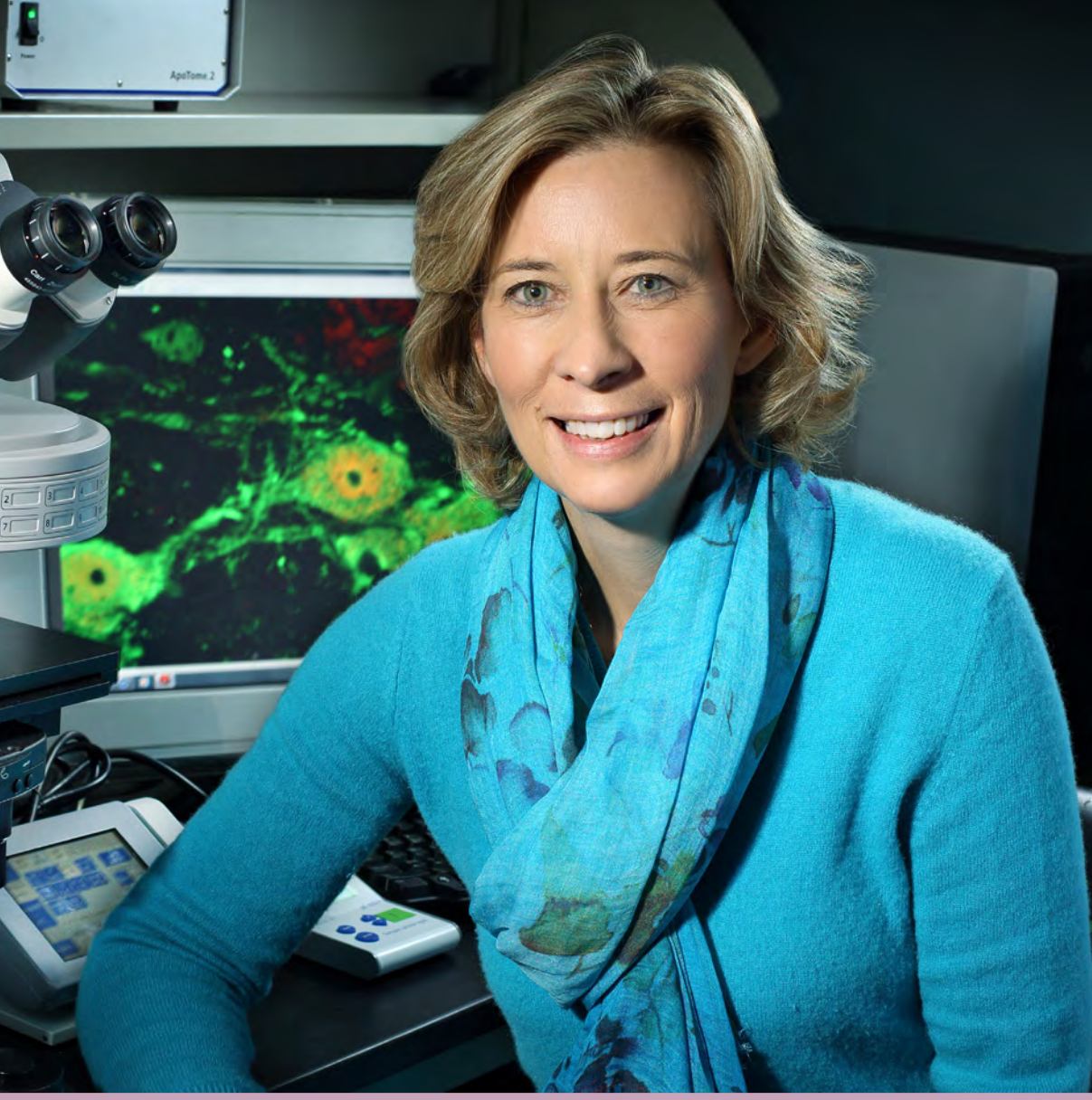
"If the breathing muscles are too weak, patients cannot cough and clear their lungs, which is the most common cause of death among these children," Crawford explains.

Pediatric physical therapist **Meghan Moore** heard the same thing and more: "When I saw Sofia, I said, 'Oh my God, she's so much louder.' Everyone is like, okay, we got it. I wish we had a decibel tool to track it."

Moore's newfound enthusiasm matched Crawford's. Before the gene therapies were developed, patients tended to decline, despite physical therapists' best efforts. Indeed, some neurologists felt physical therapy was simply hitting patients' limited reserves, which appeared not to be the case with Sofia.

"We're seeing through her abs and diaphragm, her ability to take a bigger breath and get more air in and out," says Moore. "This was the first time we've seen such improvement in an infant with SMA1. Yeah, it's pretty cool, super exciting because she's doing really well."

Indeed, with the added-value benefits of physical therapy Moore and colleagues foresaw for Sofia, they developed a new algorithm with more frequent visits to maximize her potential. Throughout the fall of 2019, the combination of the gene therapy and physical therapy appeared



Among neuroscientist Charlotte Sumner's goals is understanding why SMA progresses so quickly during the first weeks of life.

to be paying off. At Sofia's mid-October appointment with Crawford, Sofia's dad, Ryan, noted that her legs were still moving and getting thicker. Also, she had doubled her birth weight, at that point 11 lbs. 5 ounces.

Crawford's response: "Oh my God, you're a bigger baby!"

By early December, on a return visit, more signs of improved mobility had emerged. "There's more movement from the elbow and now she's starting to reach straight up," Ryan said. "She's grabbing my beard with her right hand. I said to myself, 'Wow, that's pretty cool.' On her back, she's lifting her legs straight up more, too."

Sofia had been lifting her head up more, too, but sluggishly. For babies with SMA1, that's an extremely chal-

lenging task because of their weak back and neck muscles. Ryan took it in stride: "All the changes are really slow but they're still happening."

Perhaps more meaningful for Ryan and Brooke than any recorded milestones, however, may have been the question from a nurse from an outside hospital when they brought Sofia in with cold symptoms: "Why would you bring a baby to a hospital with a simple cold?"

When Ryan explained that they were being extra vigilant because of Sofia's condition, the nurse replied, "What condition?"

Another sign? When Sofia's pediatrician started to check her ears at a checkup, the baby reached up to stop him. Such instances, Ryan says, put a wide smile on his face.

Crawford's assessment at the end of 2019? These were all early signs that Sofia was re-establishing the course of normal infancy: "In a child with SMA1, especially one who had shown such a precipitous decline, this is extraordinary. It's clearly working. Every case is different but this is so amazing from what used to happen before gene therapies."

Would Sofia continue to achieve such milestones in the new year — even walk — unheard of in a child with SMA1? Finding the answers to such questions through studies of the gene-targeting therapies is the work of clinicians and researchers like Crawford, including neurologists **Charlotte Sumner, Jessica Nance** and **Matthew Elrick**.

In the Lab

In 2001, following doctoral training at the National Institute of Neurological Disorders and Stroke, where she says she “stumbled upon SMA,” Charlotte Sumner arrived at Johns Hopkins’ East Baltimore medical campus for a clinical neuromuscular fellowship. Why did she choose Johns Hopkins?

“It’s hard to find a place that is strong in both clinical neuromuscular disease and neuroscience,” she says. In other words, she wanted to treat patients with diseases like SMA but also study the underlying science to advance treatments. Neuromuscular disorders were a bit neglected by neurologists, she believed, and thus more fertile for new research discoveries.

“Neuromuscular disease, while not getting the attention that central nervous system diseases get, is satisfying because we can often make clear diagnoses based on the evaluations we can do,” says Sumner. “Also, there’s been an explosion of genetic understanding, which has allowed us to molecularly delineate the diseases, and with that molecular delineation come the promise of treatment. This is really what’s gotten me so excited about it.”

Among Sumner’s current pursuits is understanding why SMA progresses so quickly during the first weeks of life. What are the underlying mechanisms at play that could help her and other researchers come up with new combination treatments to improve their efficacy? The challenge, she explains, is that motor neuron cells live in the spinal cord, which does not allow sampling in living patients.

“So, it’s very difficult for us to know how much of the drug is getting to motor neurons and how well that cell is

responding to the drug,” says Sumner. “Is the SMN induction even happening in those cells, and happening adequately? How well are these drugs actually working in individual patients? We have very little insight into that now.”

One answer, she notes, is the development of biomarkers to allow monitoring of disease activity in living patients, a collaborative research initiative Sumner and Crawford are conducting with industry partners. Among those biomarkers is a neuronal skeletal protein called neurofilament, which is released into the blood when motor neurons degenerate.

“With modern technologies, we can measure and monitor neurofilament and determine whether it’s going down, as we hope,” says Sumner. “If not, in this era of three gene targeting therapies we can think about adding something else in.”

Would such biomarkers also offer predictive or prognostic value for patients, as well, influencing treatment?

“That’s the idea,” says Sumner. “We don’t quite know yet, but that’s the hope.”

In a related protocol for those infants who unfortunately, do not survive, Sumner and Crawford perform expedited autopsies to retrieve tissues to study how SMN expression varies and changes over time. They’ve found that expression tends to be very high in utero and then begins to decline postnatally, which raised the question of potential treatments in utero.

Sumner and Crawford are looking into it, noting that one of the new gene targeting therapies is permeable to the central nervous system and may safely cross the placental barrier as a treatment for the fetus. Mouse studies have been

underway.

“When we treat mice in utero, they do better than when we wait to treat postnatally,” says Sumner. “I don’t think that would be necessary for all SMA patients, but potentially for some cases that are very severe.”

Sumner has already had conversations with maternal-fetal physicians at Johns Hopkins who have caught wind of gene therapy successes for SMA patients and are thinking of ways to offer treatments in utero. Pediatric neurologist Jessica Nance is on a similar track to pave the way for earlier gene-targeting interventions. She’s reaching out to genetic counselors and physicians in maternal fetal medicine to promote ideas across sites. The stakes, she says, are high.

“Even if you treat a child a week or two out, if they’re symptomatic, they’re still not going to be on a typical developmental trajectory. They may walk, but they may not walk on time or as vigorously as a child who does not have SMA,” says Nance, who was attracted to neuromuscular disease because it calls for “a special kind of sleuthing” to develop novel treatments. “We think we lose pretty quickly a fair number of motor neurons that we can rescue at the beginning of the disease, especially in the more severe types.”

Mat Elrick, now in his first year on faculty as a pediatric neurologist, expects that newborn screening for SMA will become increasingly sophisticated beyond the current panel of metabolic tests. In utero diagnosis of SMA is a possibility, too, he notes.

“Potentially, with sequencing the entire genome, the whole panel will be supplanted with genetic testing,” says Elrick. “The question is: Does it make



“Not only did the gene therapy stop the decline of Sofia’s disease but she got quite a bit better. It’s not a 100 percent cure, but it is a dramatic improvement.”

— MAT ELRICK, NEUROLOGIST

sense to push it to a prenatal test — and is that feasible?”

One thing’s for certain: Elrick agrees with his colleagues that this new era is a game changer. Prior to the gene-targeting therapies, patients were destined to die early or survive but were dependent on a tracheostomy and a g-tube, trapped in bodies they could not move, facing a very difficult and brief life. Sofia is proof, notes Elrick: “Not only did the gene therapy stop the decline of Sofia’s disease but she got quite a bit better. It’s not a 100 percent cure, but it is a dramatic improvement.”

Sumner, with an eye on the horizon, agrees: “SMA will be completely different now. We are now going to be able to treat a much larger proportion of patients with disease-modifying drugs. We will have many, many individuals living with SMA into adulthood.”

Still, many questions remain in both

the clinic and lab. There may be long-term toxicities associated with the genetic therapies; questions about their effects long term; whether they would be fully sustained over a lifetime. Will patients hit a plateau and decline? How would a child’s growth influence the outcome of a one-hour infusion?

“It’s kind of the wild, ‘Wild West’ right now — we’re all sort of watching and waiting,” says Sumner. “It’s remarkable how little toxicity there’s been so far, but there’s a lot to do.”

What keeps them going? Whether a deeply experienced veteran like Crawford, a mid-career scientist like Sumner, young research-minded clinician researchers like Nance and Elrick, they are all intensely tied to their patients and looking around the next corner for answers. Perhaps they did not find their niche in medicine so much as their niche found them.

“I found the way patients dealt with disability very inspiring,” says Elrick, who also studies the emerging polio-like neuromuscular disease acute flaccid myelitis in the lab.

Adds Nance, “I’m comfortable with relentless progressive disease and making lemonade,” a euphemism for facing adversity.

Nance and her colleagues do so in ways that reveal conviction: It’s apparent in all who play a role caring for these young patients, greeting them and parents in clinic with warm smiles; building relationships; treating them like members of their own family; and creating a caring medical home model for these fragile infants and anxious families, including Sofia and her parents.



Brooke playfully cues Sofia to bend her torso and lift her arms, physical therapy movements designed to increase her back and neck strength.

As this issue of the magazine went to press, Brooke reported that Sofia can now sit unassisted for up to one minute and roll from her back to her tummy, physical movements she was previously unable to attain. “The fact that she is able to have enough head control to sit unassisted is remarkable, and her ability to roll shows her continued strength and development combined with that curiosity she has always had,” says pediatric physical therapist Meghan Moore.

A New Year

On the morning of Jan. 18, 2020, a week shy of the six-month anniversary of Sofia’s infusion, Ryan noted that she was progressing more slowly now, in contrast to the rapid, encouraging signs he and Brooke saw in Sofia’s initial response to the infusion. She was still lifting her arms and hands up high, increasing her back and neck strength, but her legs were not showing the increasing mobility her parents hoped to see.

“Right now the concern is her leg movement,” or lack of it, Ryan explained in the living room of the family’s Odenton, Maryland townhouse. “Dr. Crawford said her leg reflexes were gone, but our pediatrician felt them. Maybe it’s a

testament to her recovery?”

“She doesn’t move her legs up to her chest, but she can lift them off the ground,” added Brooke.

To demonstrate Sofia’s leg strength, Brooke grabbed her hands and pulled her up. Sofia was standing, albeit with some assistance. Brooke then lightened her grip and Sofia stood tall.

“That’s all her, not me,” Brooke said.

Other positive signs? In her high chair, her mother noted, Sofia holds her own bottle, grabs a spoon and brings soft solid foods like avocado, kale and sweet potato food to her mouth. She had grown longer — adding an inch — and heavier, reaching the 50th percentile. Cognitively, she appeared to suffer no deficits.

“She’s very aware,” said Brooke. “She said ‘Mama’ at three months.”

“Sofia is so curious about what’s going on,” added Ryan. “She’s really responsive.”

Another remarkable achievement they point to is her hair.

“The majority of my day with her is spent on hair control — it’s really wild; there’s nothing you can do about that mullet,” Brooke said, pointing to the long strands stretching down Sofia’s back.

Observers can see they enjoy their time with Sofia, as does she with them. She reveals a puckered smile in their interactions, leaving her parents beside themselves. One moment they cannot get enough of her, overjoyed to have her in their life; the next moment they are working with her on at-home physical therapy exercises to continue her progress and, hopefully, ward off potential deficits that come with SMA1.

“She keeps surprising us,” says Ryan. “Maybe she will walk one day.” 🍀

putting the **SPOTLIGHT** on pediatric chronically **CRITICALLY ILL PATIENTS**

Facing traditional challenges, the Complex Care Consult Service aims to uncover the big-picture issues that often go overlooked in treating these patients.

By Mat Edelson





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A public health emergency can hide in plain sight, even in top academic children's centers. Consider patients with pediatric chronic critical illness (PCCI). These are kids and their families who are hospitalized for weeks, months or even years. If these children survive — as is increasingly the case — many become “frequent fliers,” repeat emergency room visitors, cycling through as intensive care unit admissions, to step-down and rehabilitation units, and discharged with alarming regularity.

Historically, medicine has viewed these patients as uncommon outliers in a system designed to seek cures and permanent solutions. That gold standard, that model of “get them better and send them home for good,” can work even in serious cases, such as a baby born with congenital heart disease. Between early medical management and surgery, such a baby should eventually lead a normal life.

That model, however, generally does not work for PCCI cases. We're talking about a long-term inpatient child with a medically complex issue with no cure.

If the child is fortunate (relatively speaking), they have a well-studied condition such as cystic fibrosis, with a dedicated team that coordinates care among many CF-familiar specialists. Often, however, the diagnosis is a mystery, or an orphan genetic syndrome, with little known regarding treatments or long-term prognosis. Coordinated care teams rarely exist for these children; thus, their medical management is often haphazard. These difficult and sometimes tragic cases have traditionally been seen by providers as one-offs among the pediatric population, and certainly are not business-as-usual.

Or are they?

The few studies done in the 2000s generally focused on pediatric intensive care unit patients, leading many providers to



“Our goal is to always remind our doctors — and ourselves — to see and let Timmy be a kid first, and not define him by his condition.”

— Michelle Morrison, with her son, Timmy

assume that the PICU is the only place in the hospital such cases would be found, which turned out to be critically incomplete intel.

A 2019 Johns Hopkins study shed alarming light on the breadth and depth of what has now become the PCCI crisis. Conducted in conjunction with the multi-center PCCI Collaboration, researchers at the University of Rochester, NY and Johns Hopkins Children's Center took a single-day snapshot of six pediatric tertiary medical centers. It found that 41 percent of children outside the PICU and NICU setting (as in, on the general wards) also met the criteria for pediatric chronic critical illness.

Their needs, just as with their NICU and PICU counterparts, are overwhelming the traditional system of pediatric clinical care. In return, the system — never designed to fully address these patients' long-term situation — is placing a crushing emotional and financial burden on these children and their caregivers.

Even within the walls of the Johns Hopkins Children's Center, doctors and nurses managing certain PCCI cases wonder aloud whether they are doing more harm than good. They also freely admit that the entrenched system of conventional staffing, communication and financial commitment and reimbursement for these patients needs to adapt — and fast.

But how?

Premature infants — the children believed to make up the majority of PCCI cases — were once a rarity.

Technology and pharmaceuticals have changed all that. By 2015, some 80 percent of babies born at 26 weeks survived. Studies found that some 20 percent of those children went on to lead normal lives. Another 34 percent had "mild" disabilities; minor cognitive and/or visual impairments. But nearly half experienced moderate (24 percent) to severe (22 percent) disabilities. These latter cases

often have no formal diagnosis. They can also require life-long care, frequent hospitalizations, cadres of specialists and therapists, dozens of regular outpatient checkups, and families turning their homes into permanent mini-ICUs.

"These families are suctioning their children, setting up tube feedings, managing the ventilator and physical therapy, (and) providing very, very high levels of medical care in the home," says critical care neonatologist **Renee Boss**.

In a vacuum, or as words on a page,

"If you feel like our system doesn't let us do the right thing, it's very painful; it has a huge impact on everybody, but especially nurses. They suffer a lot of moral distress."

— **Maggie Moon**

this can suggest a seamless transition, with numerous medical professionals working hand in glove with parents, families and extended community caregivers toward a common goal: What is best for the child?

Reality is far messier, often a never-ending communications snafu across the entire chain of care. The norm is this: Doctors never trained, nor given proper time to consult across multiple specialties, truly involve families in the long-term ramifications of treatment decisions. Then again, most doctors were never required as students, residents, fellows or attending physicians to spend a minute in a patient's home. So they really have no first-hand awareness of how disruptive it is for parents and caregivers to be asked to turn their domicile into an ersatz hospital.

Consider also the front-line warriors in the process; NICU, PICU and general ward nurses. The emotional toll of caring for PCCI children for 12 hours a day, weeks on end, and often seeing little to no improvement can be devastating. It's actually fairly common for nurses to request removal from such cases.

"If you feel like our system doesn't let us do the right thing, it's very painful; it has a huge impact on everybody, but es-



Maggie Moon

pecially nurses. They suffer a lot of moral distress,” says Johns Hopkins Children’s Center’s Chief Medical Officer **Maggie Moon**.

While nurses and doctors get mandated time away from these cases, for inpatients and families, there’s no escape from an experience that’s been described by parents as akin to living in a war zone.

If that sounds traumatic, ponder the other severe stressors PCCI parents experience. There’s the shock of seeing one’s child tethered to life-sustaining equipment. Some parents may feel guilty, believing that their genetics, environment and/or lifestyle choices somehow contributed to their child’s situation. Some providers wonder whether emotionally frayed parents can competently give informed consent for medical care, no matter how much information is provided.

When one attempts to unravel all the issues involved in these booming PCCI cases — clinical, ethical, financial, psychological, legal and administrative — it’s clear that ad hoc solutions no longer suffice. What’s needed, hospital administrators say, are well-funded, system-wide initiatives supported from top to bottom. Those take time, effort, and, perhaps most importantly for administrators, quality data showing improved outcomes and positive bottom-line impact. But maybe, just maybe, the vanguard for such change within the Children’s Center is finally in place.

IMPLEMENTING A TEAM APPROACH

Imagine running the military without the Joint Chiefs of Staff issuing expert guidance. At the least, the situation among the rank-and-file would be chaotic, redundant and wasteful; at worst, downright dangerous.

And yet, that’s the way complex PCCI cases are often handled — and not just at Johns Hopkins. Re: that aforementioned 2019 report showing the exploding number of PCCI cases? It found that just one of the six hospitals surveyed had a dedicated PCCI complex care team — physicians, nurses and other providers extensively experienced with such cases, tasked with supervising and coordinating the extensive care and support needs for patients and family.

Two of the study’s authors are the Children’s Center’s hospitalists **Eric Biondi** and **Chris Grybauskas**. Both men know that, absent a complex care team, PCCI patients suffer discontinuity of care: Whenever these children come into an ER and are admitted, they are assigned to the specialty team dealing with their specific acute issue (e.g., pulmonology for respiratory distress) and not a team familiar with their overall case.

Such random assignments virtually assure that advice is rarely sought (or is outright ignored) from parents intimately familiar with how their chronically ill kids best respond to certain treatments.

Whether it’s setting ventilator pressure, suctioning a tracheostomy tube, prescribing a certain medicine or drawing blood, providers understandably default to “the book.” However, adhering strictly to ICU traditions or standard-of-care guidelines sometimes yields substandard results.

“Some parents know [from home experience] that if you set the suctioning machine higher, to #8 instead of #7 [removing secretions more aggressively from the airway], the child will decompensate. But the ICU staff always uses #8. And what do you think happens?” asks Boss. “Maybe one way to reduce length of stay in the ICU is to follow the parent’s guidance.”

To ensure that PCCI parents are involved and outcomes are optimized, Biondi and Grybauskas formally introduced a Complex Care Consult Service last year. It’s hoped that eventually the service will be upgraded to a Complex Care team that completely oversees PCCI cases. Biondi, director of Pediatric Hospital Medicine, came to Johns Hopkins in 2017 after implementing the University of Rochester’s (NY) Complex Care Team.

“If you look at my career, it’s basically me getting angry about something on a Wednesday afternoon and wanting to fix that thing quickly,” says Biondi, who chairs the American Academy of Pediatrics VIP — the Value in Inpatient Pediatrics network. (The VIP is a national quality improvement initiative involving hundreds of pediatric sites.)

Biondi found a kindred spirit in Grybauskas. A Maryland native and medical director of 9 North, a general care unit, Grybauskas is kind, compassionate ... and blunt. “For me, treating these patients right is all about fairness and justice,” says the hospitalist. “Sometimes it’s not a popular thing that, in many of these cases, I’m trying to convince people that the old-fashioned way of delivering care is not up to par.”

“For me, treating these patients right is all about fairness and justice. Sometimes it’s not a popular thing that, in many of these cases, I’m trying to convince people that the old fashioned way of delivering care is not up to par.”

— **Chris Grybauskas**

Hospitalists Chris Grybauskas, left, and Eric Biondi, outside the Emergency Room, stress that when children with complex medical needs arrive at the ER they are assigned to the specialty team dealing with their specific acute issue and not a team familiar with their overall case.



Upping the ante is the medical limbo common to many PCCI cases. “You can be a very sick and complex child with cancer and have a fantastic cancer team devoted to you — resources, doctors, and health care professionals who are part of a well-oiled team,” notes Grybauskas. “But say you have a poorly studied rare chromosomal deletion that affects seven bodily systems? Then you’re seeing seven different Hopkins specialists. There is no well-oiled team, no one captaining the ship, no one working together in any formal sense. It’s just the luck of the draw that one family gets a cohesive experience and one doesn’t.”

Working with vital funding from the Children’s Center Innovation Grant Program, Biondi carved out 20 percent of Grybauskas’ clinical time to implement the consulting service. Grybauskas, in turn, recruited veteran nurse practitioner **Kathleen Naughton**, whose peers consider her exceptional at mining voluminous Epic data, intake charts, and

lengthy parent interviews to highlight crucial information. The result is a succinct synopsis that helps providers assess a new patient.

“What’s created is kind of like a portable medical summary,” says pediatric hospitalist and internist **Sara Mixter**, who works part time with the service. “When I first meet a patient, I don’t necessarily need a blow-by-blow of their entire medical history. I need to know almost intangible things — like when this child has an episode of hiccups (normally a mild and temporary issue). That’s always a sign that something is going wrong. These are things that, unless they’re clearly pointed out, a physician wouldn’t otherwise know.”

Naughton and Grybauskas strive to uncover big-picture issues that can go overlooked in crisis situations. On this day of daily rounds, they’re sitting at a PICU nursing station with longtime nurse practitioner **Beth Wieczorek**. Her patient is in the PICU with severe

malnutrition for the third time in a year.

“This should’ve been caught by the doctors the second time,” grumbles Grybauskas, pointing to a monitor showing the child’s vital stats during all three hospitalizations. The pattern is clear: significant, lifesaving weight gain while an inpatient; dramatic weight loss while at home.

Not content to let the cycle continue, the duo and Wieczorek drill down into the case. Who are the home-care providers? Parents? Nurses who accompany the child on the bus and at school? Previous experience suggests that offering additional feeding education could be the answer: Many home and school caregivers struggle with the nuances of using a permanent feeding tube, which this child has.

The consult service is all about asking the right questions and then, when possible, finding workable solutions. Since they opened for business, Grybauskas estimates that specialists have asked



The Complexities of Care at Home

“Our goal is to always remind our doctors — and ourselves — to see and let Timmy be a kid first, and not define him by his condition,” says Timmy Morrison’s mom, Michelle.

The family walks the talk. All it took was a little anthropomorphizing (and a top hat, mustache, and bow tie) and that scary ventilator in the corner of Timmy’s bedroom became his night-time buddy, “Kent the Vent.” There’s

Thanks to the Complex Care Consult Service, 9-year-old Timmy Morrison has experienced a marked decline in hospitalizations, allowing him to enjoy more time at home playing his violin.

Pediatric nurse practitioner Kathleen Naughton, right, has developed a portable medical summary, what internist Sara Mixter calls a succinct synopsis, to assess complex patients.

for 80 consults. Their service is gaining awareness across the Children's Center, augmented by a proactive approach: The pair reviews every inpatient admission to see which kids fall under the sometimes hard-to-recognize PCCI banner, as in — what constitutes a Complex Case? Should two specialty teams constantly be involved? Three? Five? No national guidelines exist.

They're also constantly touting their service with specialists and parents, including a talk Grybauskas gave recently to the Pediatric Family Advisory Council (PFAC). "This is a game changer for the families that have been able to take advantage of it," says PFAC member Michelle Morrison. Her 9-year-old son Timmy is a stable medically complex patient who goes to school, loves Legos and video games and has generally expe-



“What’s created is kind of like a portable medical summary. When I first meet a patient, I don’t necessarily need a blow-by-blow of their entire medical history. I need to know almost intangible things.”

— Sara Mixter

also “Fred” the emergency bag for car rides and, well ... you get the idea.

“Creating Kent was huge in convincing Timmy when he was 4 to sleep with that new machine,” says Michelle. Indeed, life for her and husband Mark with Timmy is one long improvisation. There’s no “book” for caring 24/7 for a complex child. Some, like Timmy, have a formal diagnosis (the genetic syndrome Opitz G/BBB). Many do not. Either way, these parents are dealing with a child with perhaps a dozen specialists working their case.

“If we did every therapy each specialist suggested, there wouldn’t be enough hours in the day. And he certainly wouldn’t be able to play as much with his friends,” says Michelle.

As parents realize the constant home care their children require, they find that doctors don’t have answers – but eventually they discover other similarly situated parents do, and gladly share advice on social media.

“I wish doctors and nurses would realize that early on and help us find some of those websites,” says Erin Welch, whose 14-year-old daughter Charlotte was born with, among many issues, microcephaly and hypotonia (low muscle tone). “She was 8-months-old before I found my first Internet support group and talked to other parents. They told me that because she has hypotonia, she’s not always going to feed when you want her to, and you just have to go with your gut. I wish someone had

told me that the first week.”

Both Morrison and Welch say they’ve seen some improvements in communication and empathy from providers over the past decade, but it’s incremental when the big picture is considered: Finances, relationships, work schedules, personal time ... every aspect of a parent’s and family’s life is impacted when they care for a complex child at home. They love their children dearly, but their resiliency is stretched to the max, and they need help.

And they wonder whether the medical establishment, with all its life-giving technology, is truly hearing their pleas?

— ME 🍌

Palliative Care: Reimagining Expectations

Whether it's a parent, physician or nurse who's caring for a complex child, the best way for them to cope with inherent emotional challenges might be to adjust expectations. Sick children don't always improve. The hope for a so-called "normal" life at birth can quickly change. It's a daunting, uncharted landscape to navigate, but several Children's Center providers are trying to help.

"If my only goal was to get every one of these children out of the hospital, I would often fail. So I have to change my goals, my hopes," says palliative care neonatologist **Renee Boss**. She believes parents — even those in dire situations — can make such shifts with the help of palliative care.

"In the best-case scenario, palliative care becomes available prenatally," says Boss, who founded the Division of Neonatal-Perinatal Medicine's Palliative Care Working Group. "This can help the family who decides to continue the pregnancy even if there's a chance the baby will die soon after birth. It can help (everyone) — parents, siblings, grandparents — prepare and give them some control over the birth process by having everyone there, perhaps having an important religious or cultural ritual, and making all the memories that are possible with photographs and so on."

PICU-based palliative care team nurse practitioner **Emily Johnson** employs a similar focus-changing approach with nurses. She has seen many struggle with caring for children who aren't going home anytime soon, if at all.

"So I say to that nurse, 'If we're not going to achieve (discharge) right now, what can you do so you're feeling like you're doing something good for this kid?' Then they realize, 'Well,

I can make their room look cute,' 'I can put bows in the child's hair,' 'I can call their mom and give them an update,'" says Johnson. "So, being able to help them redefine their role as a nurse in this child's life is a strategy I employ, especially when I can see their resiliency is getting limited."

Redefining expectations requires a new lexicon and perhaps someone translating the needs of providers and parents to each other. That's a role **Alison Miles** sees herself playing. She has a unique dual background in pediatric critical care and hospice/palliative care medicine, so she's seen many complex cases from both sides.

"I think my most valuable lessons (as a physician) have been when I've been able to align myself with a family and their values, even if they differ from mine," says Miles. "I may have to come up with a care plan that the ICU staff won't agree with. The staff might say, 'This isn't what I would want: it wouldn't be my goal.' But if I can communicate to the staff that this is what the family wants for their child, that it comes from a good place and their sense of values, just that conversation can make things better." — ME 🌟



“He’s been out of the hospital for nearly three years. Now, Nancy Hutton has also done a lot. She’s prescribed him medications and functioned as a long-distance primary care doctor for a very complex kid.”

— **Chris Grybauskas**

rienced a marked decrease in hospitalizations. While Morrison and her husband Mark personally cobbled together Timmy’s team of a dozen specialists in Cincinnati, Baltimore, and Philadelphia, she sees the new Complex Care Consult Service as filling important gaps that constantly confront parents.

That includes the often-byzantine demands of insurance companies denying coverage. Charly Walker was at such an impasse. Her 8-year-old daughter Zoey, born with Aicardi syndrome, a rare genetic condition affecting the brain and other body systems, was being denied a \$100,000 bone-saving drug for a seemingly bizarre reason: Zoey’s insurance would cover only an outpatient infusion. Yet no clinic or hospital near her rural Pennsylvania home would administer the drug, fearing complications.

For two years, the little girl went without the medication. Her bones became brittle. Some broke. Enter Grybauskas. It took three days of work and numerous machinations, but in the end, he got the drug covered and administered in the Children’s Center. “That was a victory, but the bottom line was: this kid suffered fractures that the kid never should have suffered,” says Grybauskas. “To me, that’s infuriating.”

For her part, Walker was amazed. “I was like ‘Wow! He was able to accomplish (getting Zoey’s drug) within just a couple of days,’” says Walker, cautiously. “I just hope it isn’t an issue when she has to get the drug again in six months.”

Though the Complex Care Consult Service is new, Grybauskas says he’s

using other established Children’s Center programs as a model for growth. He points to Child Life and the Palliative Care Team as success stories: They started small, proved their worth across the Children’s Center, and steadily increased their budgets and staff.

One story is particularly instructive. It involves the Palliative Care Team’s Director **Nancy Hutton** and the type of dedication that inspires Grybauskas’ work. Several years ago, Hutton met a complexly ill child constantly cycling in and out of the Children’s Center. Hutton was convinced that the child’s family, with more physician involvement and reassurance, could handle most of the child’s emergent issues at home. Hutton visited the child’s home quarterly and talked to the family via phone and video chat both weekly and whenever a mini-crisis arose. The result?

“He’s been out of the hospital for nearly three years,” says Grybauskas. “Now, Nancy Hutton has also done a lot. She’s prescribed him medications and functioned as a long-distance primary care doctor for a very complex kid. I had seen this kid when he had been hospitalized (prior to the consult service’s creation). I worked really hard on his case and never thought he would have responded so beautifully to Nancy’s efforts, to her care. She’s really good. And this is also a testament to how simple solutions can be in a disjointed medical system, if you have that one line of communication to somebody who is really invested in you, that can smooth over a lot of other problems.”

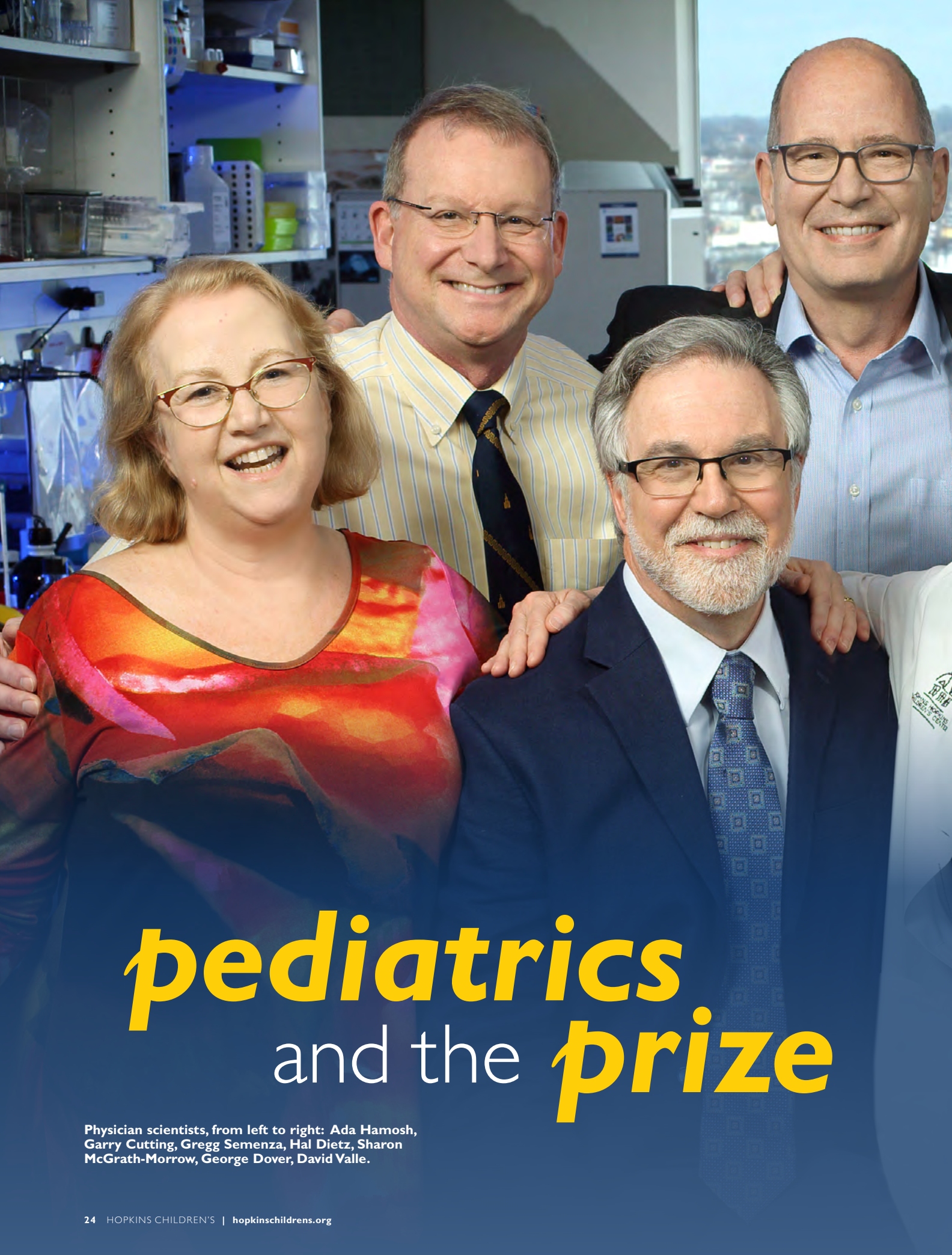
In addition to the support of his palliative care colleagues, other Johns Hopkins providers are buying in. Maggie Moon is proud of the consult service’s initial efforts, observing that, “They really acknowledge and embrace those patients’ complexities and help families with their experience of care so they’re not starting at zero again every time they come in.”

Still, there are long-term concerns. The consult service is available only during weekday business hours. That’s not likely to change anytime soon, according to several providers not directly involved with the service who spoke on background. They noted that full-fledged funding for similar concepts haven’t previously come through, and the institutional expectation is still that providers should volunteer their time to such teams and efforts.

Yet, despite this and other potential funding roadblocks — notably Maryland’s unique hospital reimbursement system and Medicare’s loophole that gives higher reimbursements to free-standing hospital specialists than to their Children’s Center counterparts — Grybauskas has faith that the bottom-line overseers get the big picture.

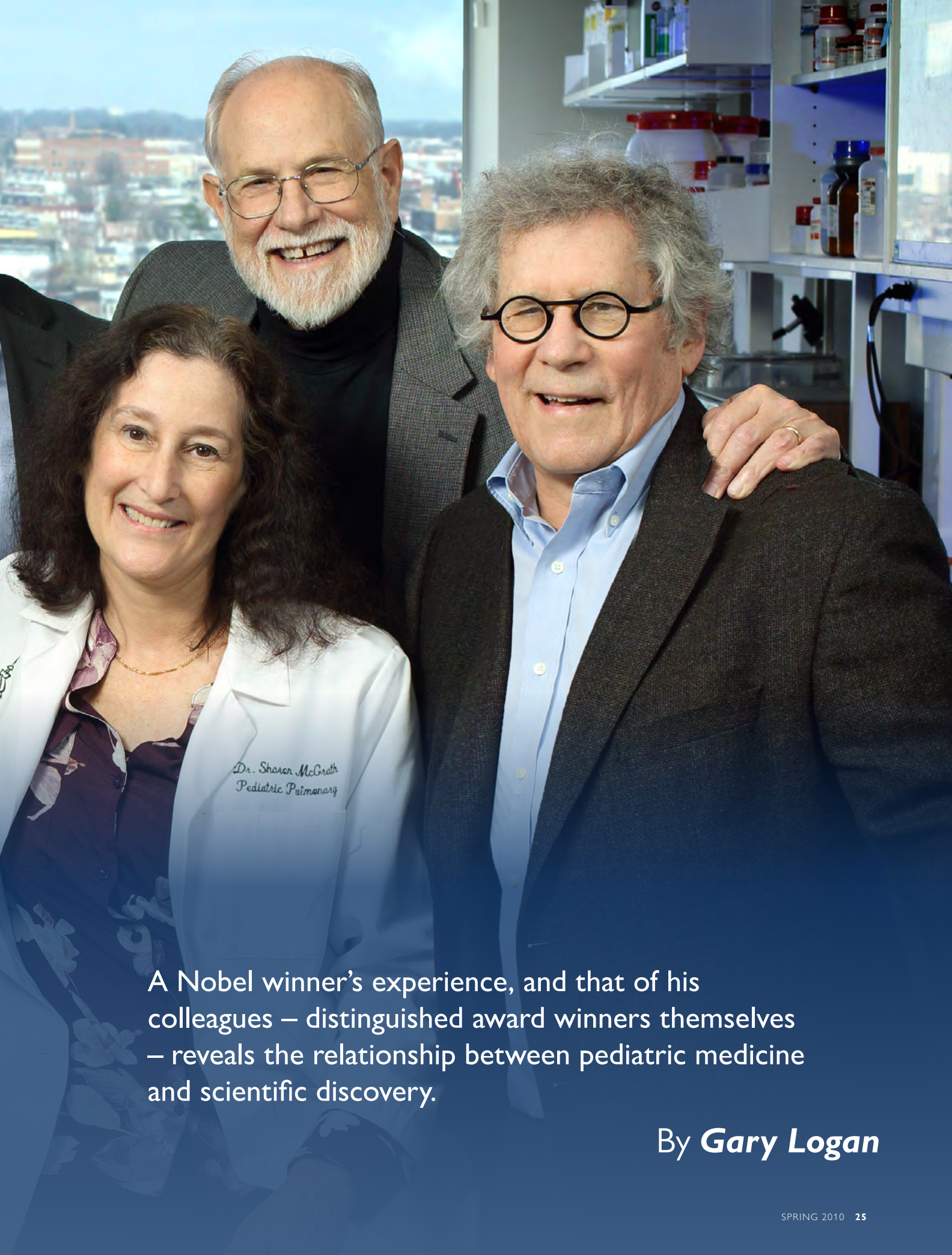
“I’ve had administrators say to me, straight up, ‘Chris, we don’t know how to fully fund a Complex Care Team.’ But they understand, even if we don’t have the data yet, that it makes sense that if we spend some money up front — even if it doesn’t generate revenue — these families should use less ICU days and better avoid the highest levels of hospital care. That’s where it’s most expensive to care for them.

“These administrators have told me... ‘This might not make money, but this is good medicine.’” 🍀



pediatrics and the *prize*

Physician scientists, from left to right: Ada Hamosh, Garry Cutting, Gregg Semenza, Hal Dietz, Sharon McGrath-Morrow, George Dover, David Valle.



A Nobel winner's experience, and that of his colleagues – distinguished award winners themselves – reveals the relationship between pediatric medicine and scientific discovery.

By **Gary Logan**

Physician-scientist Gregg Semenza

received the 2019 Nobel Prize in Physiology or Medicine for discovering how the body's cells sense and react to low oxygen levels. This discovery may lead to treatments for diseases such as cancer, diabetes and heart disease. He shares the award with William G. Kaelin Jr., of the Dana-Farber Cancer Institute, and Peter J. Ratcliffe of Oxford University.



Gregg Semenza circa 1995

Gregg Semenza, the 2019 winner of the Nobel Prize in Medicine or Physiology, recalls several early experiences that put him on a path to becoming a pediatrician and a physician scientist. There was his 3rd grade teacher, who chose not to embarrass him after he presented his first book report on a book he clearly had not read. That transformed Semenza into a voracious reader. Then there was his biology teacher who elegantly conveyed, and inspired in him, the beauty and thrill of scientific discovery. He was hooked. With her help he was able to enroll in a National Science Foundation summer program at the Boyce Thompson Institute, his first exposure to research and experiments. That led to a vision in his junior year at college to pursue a Ph.D. and a career in genetics. Then a friend's family had a child born with Down syndrome, at a time when he was in a lab focusing on human gene mapping and, in particular, chromosome 21. Children born with Down syndrome, he learned, have a third copy of that chromosome, called trisomy 21. Semenza then re-mapped his own path and added medical school, and later pediatrics, to his doctoral studies.

"I decided to go for the M.D./Ph.D. at the University of Pennsylvania so I could also get clinical training to take care of patients with genetic disorders," he says. "Because many genetic diseases are manifest at birth," he adds, "the next logical course was pediatrics."

"Because many genetic diseases are manifest at birth, the next logical course was pediatrics."

– Gregg Semenza

That training Semenza found in Duke's pediatric residency program. His next stop was Johns Hopkins and a fellowship in pediatric genetics. Already he had gleaned insights into the influence of pediatric training and practice on genetic research. What other connections would he discover? Would his prize have alluded him had he not followed a pediatric path early on? Why not internal medicine? What is the relationship between pediatrics and scientific discovery — between treating patients and finding answers for them through basic science research in the lab? Is there one? Through the lens of Semenza's seasoned colleagues, there are many connections.

For **Dave Valle**, a faculty member when Semenza arrived at Hopkins — and who points to his junior high school

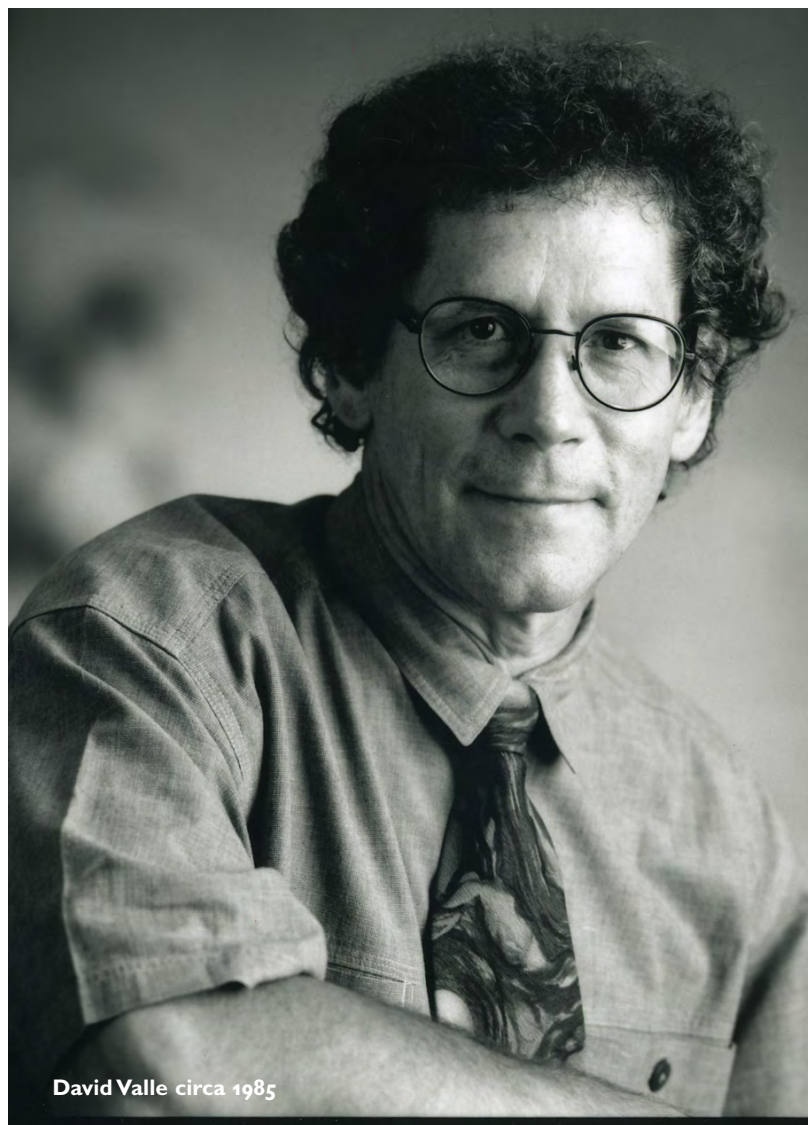
biology teacher as a major factor in his career — a significant pediatrician/basic scientist connection is the continuum of development. Valle, now director of the Department of Genetic Medicine, concedes that environment and the social and family context of the child affects a child's development, but its primary influence is biology and genetics. That means the opportunities to understand biology, human biology, genetics and pediatric health and disease are completely obvious in a young patient. In the simplest terms, Valle says, "A child is biology looking at you in the face."

That face may include one of more than 7,000 Mendelian disorders, almost all quite rare and caused by some abnormality in one particular gene, 90 percent of which present in the pediatric age range. That raises questions about the variants and the gene involved in the patient's particular problem.

"But the answer is right there in front of you, sitting in your office or exam room or on a mother's knee, saying 'c'mon doc, figure me out and help me out,'" says Valle.

Pediatric pulmonologist **Sharon McGrath-Morrow**, who began treating children with ataxia telangiectasia early in her career, agrees. "By being a clinician, I'm able to look at the patient and ask the question: What kind of therapies can we do to extend life, increase quality of life, slow disease progression? Can we borrow from other chronic lung diseases and start instituting interventions in this genetic disease, which we did and increased survival by about 8 years."

McGrath-Morrow explains that she and her colleagues were able to show high levels of pro-inflammatory cytokines in ataxia telangiectasia, from which they were able to obtain molecular signatures as potential biomarkers of the disease.



David Valle circa 1985

"The answer is right there in front of you, sitting in your office or exam room or on a mother's knee, saying 'c'mon doc, figure me out and help me out.' A child is biology looking at you in the face."

– Dave Valle

Garry Cutting circa early 1990's



“I just liked the idea of being able to [genetically] profile children with these diseases with new tools that really gave us an opportunity to change direction and make a difference for the child and the family.”

— Garry Cutting

“Then we can prognosticate and think about possible interventions mechanistically based on these biological signatures in the blood,” says McGrath-Morrow.

Semenza concurs: “I always say the human body is the most sensitive assay we have. There are subtle effects over long periods of time that will manifest themselves, particularly during development. You may not have an assay sensitive enough to see what this small change does to the protein — you change one amino acid in a protein with 800 amino acids — it may be difficult to demonstrate that effect in any kind of lab assay. But you have the patient staring you in the face.”

That interaction, Valle says, lends to the focus required of physician scientists, to ask thoughtful and clear questions so they can reasonably expect their experiments will lead to meaningful answers. The problem the pediatrician sees, he adds, tends to be more distinct than the problem the internist sees.

“In a child there may be associated complications but the particular problem is occurring in an individual who is otherwise healthy,” says Valle. “Whereas an adult patient has hypertension, diabetes, all this wear and tear, and it’s just not as clean and sharp of a situation.”

Also, adds geneticist **Garry Cutting**, the pediatrician can take advantage of the child’s plasticity to alter the disease path.

“An adult, I’m afraid to say, only has a certain ability to recover from certain insults,” says Cutting, “whereas with a child there’re things you can do, therapies you can consider for a child that can change lives.”

In medical school, he adds, his mentors were pediatric geneticists who also formed his thinking. Then a hematologist gave him an opportunity to work in his lab, which sharpened his vision.

“I just liked the idea of being able to profile children with these diseases with new tools that really gave us an opportunity to change direction and make a difference for the child and the family,” says Cutting. “It made me feel that’s where I had the greatest opportunity to make a difference.”

Creating a Collegial Environment

In ways, the canvas has been set for the Semenzas of the world, suggests Valle, awaiting the brush strokes that will render a motivated and talented trainee’s pursuits. That’s where mentors come in, Valle adds, creating and sustaining a collegial and collaborative environment seasoned with some serendipity. In Semenza’s case, it was what Valle calls “a powerful happenstance.”

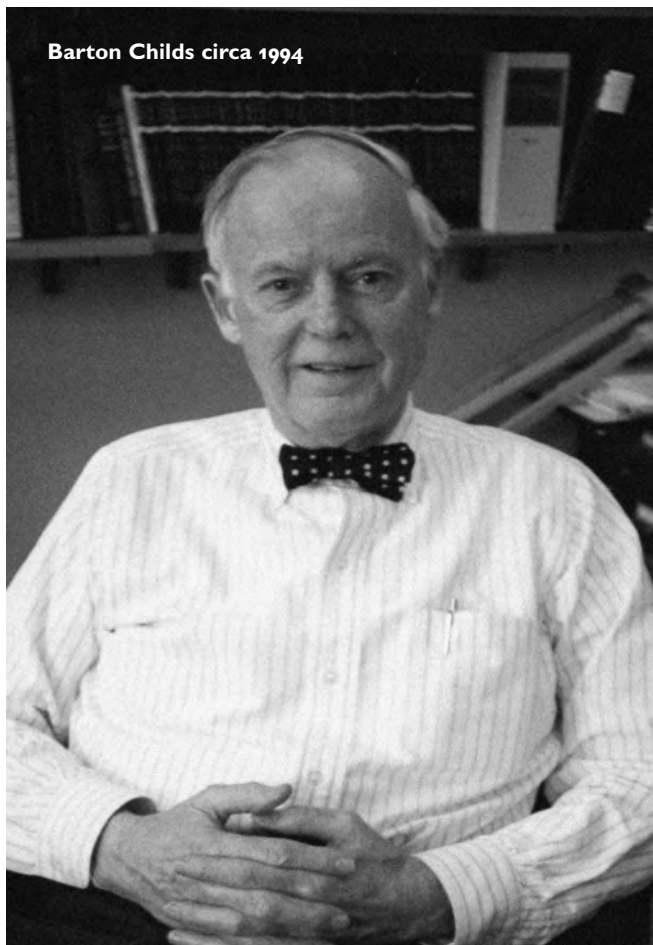
During the course of his Ph.D. work at Penn, Semenza became interested in thalassemia, an inherited blood disorder in which the body makes an inadequate amount of hemoglobin, the protein in red blood cells that carries oxygen. Indeed, it would become the subject of his thesis. He accepted a position as a genetics fellow at Hopkins because of the opportunity to work in the lab of pioneering geneticist **Haig Kazazian**, who was then at the cutting edge of understanding the molecular basis of thalassemia, and **Stylianos Antonarakis**, an associate professor of pediatrics.

“It was another case where Gregg’s career, through his own choices and some degree of serendipity, put him in the perfect place for what was really turning him on,” says Valle.

Then, something surprising happened. Their work on red blood cells prompted Semenza to ask a more fundamental question: What is hemoglobin trying to do? It’s trying to carry oxygen. So, then, how does the cell translate that information? At that moment he scientifically intuitively asked the question that would lead to decades of groundbreaking research on how cells sense oxygen and a Nobel Prize, but not one tied to the specific grant work of the lab at the time. Nonetheless, Kazazian and Antonarakis gave Semenza their blessing to pursue the answer.

“They did something that really good mentors do at a place that allowed it to happen,” says Valle. “What often happens,

Barton Childs circa 1994



the trainee comes into a lab and the PI says here's what we're working on and I want you to work on this. They did not do that. They said here's the general area we're interested in, why don't you think about this and come up with a question you think you would like to investigate. When you do it that way, the trainee is more inspired and more invested in the work."

In that environment, Semenza found the freedom to pursue answers to other questions that provoked him. Some of the questions were stimulated by morning rounds with **Saul Brusilow** in the Pediatric Clinical Research Unit (PCRU), where one third of the patients were afflicted with genetic disorders of metabolism. Inherited abnormalities of the urea cycle, which were nearly always fatal, was the focus of Brusilow's work.

"It was this classic physician-scientist mix of taking care of these patients, some of whom were very sick, and at the same time learning from these experiments of nature in a compassionate way," says Valle.

The questions and answers surfaced through the extremely collegial and collaborative environment Semenza found at Johns Hopkins, especially in pediatric genetics. How did that happen among physician scientists? In large part, through pediatrician **Barton Childs**.

After graduating from Johns Hopkins University School of Medicine in 1942, Childs quickly built a reputation as a superb pediatrician, who many faculty members had asked to care for their children. His interest in birth defects stimulated an inter-

est in genetics, a science new to medicine and one characterized as "professional death" for those who pursued it. So, in 1952-53, Childs pursued genetics training at the University College London and returned to begin his life's work of advocating for the role of genetics in all of medicine and not just pediatrics. In so doing, Child's model of a physician scientist — whose clinical insights and first-hand look at disease symptoms and human variation prompted important biological questions in the lab — had a magnetic effect.

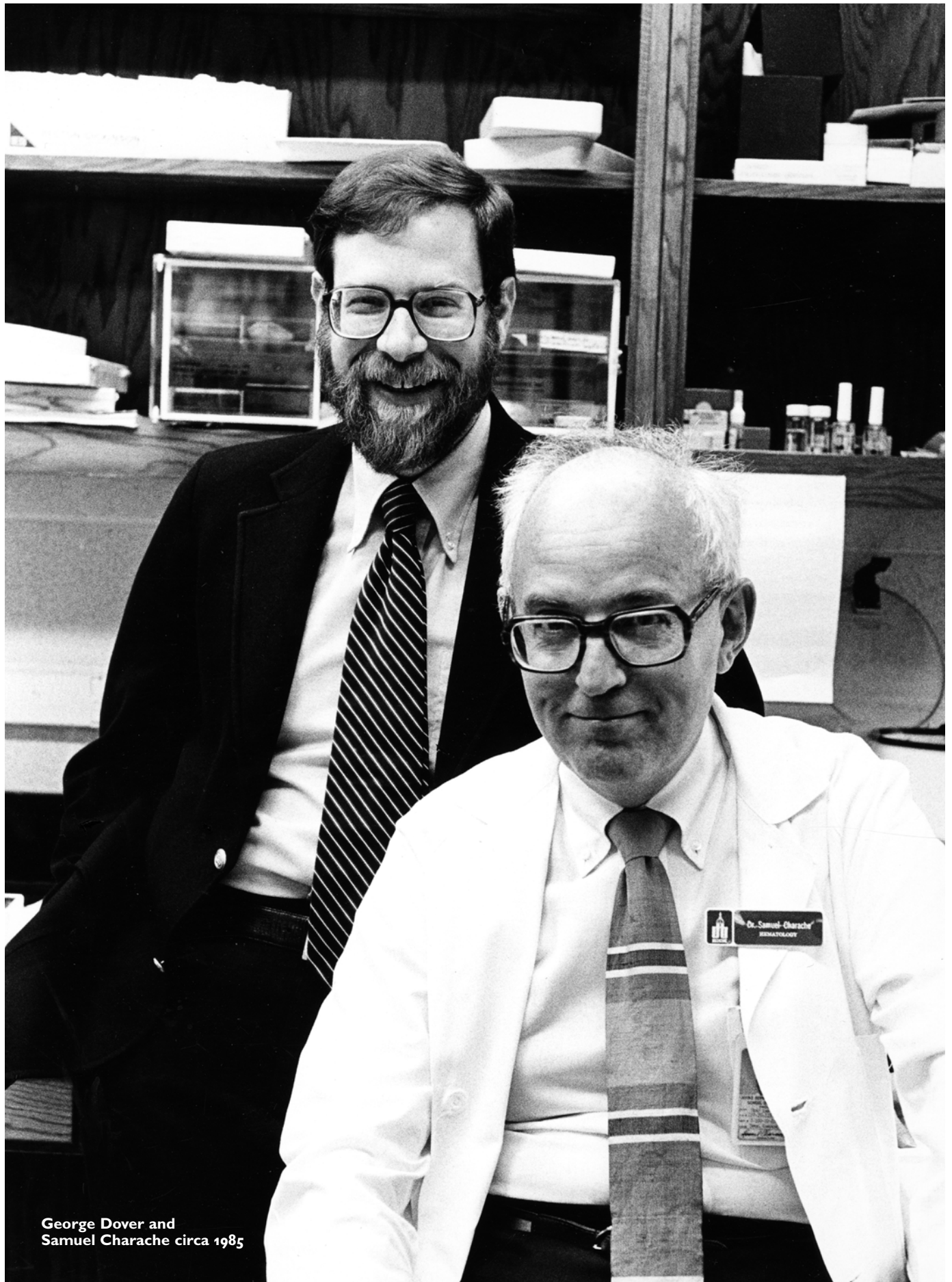
"What came out of that were people strongly attracted to Hopkins, people who understood the importance of the physician perspective in basic science and the importance of training," says **George Dover**, former director of Johns Hopkins Children's Center. "All of this was occurring in pediatrics at a time when there was no genetics department, when medical genetics didn't exist. The science behind this was in its very nascent phase, so no one really understood it very well. Barton's energy influenced everything — then it became a very exciting place."

Indeed, Child's vision aligned with budding physician scientists, including **Barbara Migeon**, his first fellow who would start a medical training program for Ph.D.'s in genetics and go on to make significant contributions to the field, particularly in understanding X-chromosome linked diseases such as hemophilia. Another arrival was Kazazian, who would start a fellowship training program in genetics for physician scientists, and whose lab later on would be one of the first to use the polymerase chain reaction (PCR) to identify human disease mutations. Next up, Antonarakis, who with Kazazian published several important papers on the molecular bases of classical hemophilia due to the deficiency of clotting factor VIII.

Other genetics minded fellows and future luminaries included Cutting, whose lifelong focus has been on understand-

"The science behind this was in its very nascent phase, so no one really understood it very well. Barton's energy influenced everything — then it became a very exciting place."

— George Dover



George Dover and
Samuel Charache circa 1985

“You can talk to anyone about anything anytime. You want to talk to someone who is an expert in a certain disease, they’ll say ‘Let’s grab some coffee.’ You come and stay here because it’s an unbelievably bidirectional collegial environment.”

– Ada Hamosh



Ada Hamosh circa 2016

ing cystic fibrosis. His lab followed a large group of CF patients, nearly all who had the same genotype of the disease gene locus but varied widely in their clinical severity. This work allowed for the prediction of differences in the course of disease and response to therapy for individual patients. Then came **Hal Dietz**, whose groundbreaking studies on the molecular pathology of Marfan syndrome (MFS) led to the repurposing of an existing cancer drug to treat MFS. And, of course, Gregg Semenza and the seminal discovery of hypoxia-inducible factor 1, or HIF-1, and how its expression is important in many areas of biology and medicine, including cancer.

Led by Child’s influence, the Division of Pediatric Genetics emerged in the mid-1960s, merged with the Division of Medical Genetics under Kazazian in 1989, and morphed into the McKusick-Nathans Institute of Genetic Medicine (IGM) in 1999. Valle took over as director of the IGM in 2007; **Ada Hamosh** is clinical director of the Department of Genetic Medicine, of which two thirds of the patients are in the pediatric age range. Among other accomplishments, she is scientific director of Online Mendelian Inherited in Man, or OMIM®, a catalog of human genes and genetic disorders and traits cre-

ated by **Victor McKusick**. Citing her experience and those of her colleagues, the Hopkins trait she cites first for physician-scientist success is a collegial and collaborative nature, which she sees as a natural for pediatricians.

“In pediatrics, it’s about working as a team. Everyone is invested in fixing the child,” says Hamosh. “When you’re a pediatrician, you’re aligned with parents to care for the child. It’s a very, very different world than adult medicine.”

So, she finds that same collegiality in the lab world at Johns Hopkins?

“You can talk to anyone about anything anytime,” answers Hamosh. “If you have a piece of equipment and want to test it out in somebody’s lab, they will say, ‘Sure, c’mon over.’ If you don’t have a piece of equipment and cannot afford it, someone will collaborate with you to get this done. You want to talk to someone who is an expert in a certain disease, they’ll say ‘Let’s grab some coffee.’ You come and stay here because it’s an unbelievably bidirectional collegial environment.”

Pediatric pulmonologist McGrath-Morrow cited similar experiences with Dover, who along with Johns Hopkins’ **Samuel Charache** discovered that the cancer drug hydroxyurea

can boost fetal hemoglobin and decrease the excruciating painful sickling crises for patients with sickle cell disease. Early in McGrath-Morrow's lab studies of cystic fibrosis and neonatal lung disease as a junior faculty attending, she was developing mouse models to look at pathways dysregulated by different injuries. Dover asked her how the studies were going, noting that it's really important to find a clinical correlate to her work. Her answer was chronic lung disease of prematurity, which prompted him to suggest she start a clinic for those patients. She did, which led to a registry of 1,000 patients and outcomes she has been following longitudinally since the year 2000. Her most recent paper explored a possible link between bronchial pulmonary dysplasia and early adult onset chronic obstructive pulmonary disease.

"When he said that to me, it made absolute sense but I had never put it together," says McGrath-Morrow. "George was always my go-to person in having vision, being able to look at your work and get an idea what direction to go."

These invaluable go-to people tend not to go away, either, says Semenza, explaining in part the systemic nature of mentoring at Hopkins.

"When I came here there was a whole bunch of faculty who helped me and made a major contribution to my success, and now I feel a great need to repay that debt," says Semenza. "The way I pay it is by mentoring the junior people now. It's a positively reinforcing culture, that's the way things are here, it's always been this way."

What makes a physician scientist?

A collegial and collaborative environment, wise and supportive mentors, smart young trainees — doesn't that sound like a formula for producing successful physician scientists? What else is needed? One quality is the ability to focus, which Valle and others saw in Semenza early on.

"He was very focused, very sharp, and he asked very pungent questions," says Valle. "I tell our scientists in training, many of us can come up with questions that would be useful to know the answer. But there are few questions that, if you know the answer, will really move the field and move you to a new level of understanding. Gregg was able to do that."

"If in real estate it's location, location, location, in science it's focus, focus, focus," adds Cutting, who shared a lab with Semenza early on. "If you want to be successful, if you want to add to the knowledge base, you put out decent manuscripts, get funded for the work you're doing, and keep your eye on the

prize. Gregg exemplifies that laser-like focus."

What makes a physician scientist successful? Curiosity and persistence, says pediatric cardiologist **Anne Murphy**, citing Semenza's perseverance in searching for HIF through labor-intensive protein purification when traditional approaches failed. "I would have given up," she laughs.

An observant clinical eye, helps too, she adds, noting that a patient's deteriorating condition in the OR following bypass heart surgery prompted her research into the molecular basis of myocardial stunning, which may lead to sudden heart failure after open heart surgery. The work of her research team was named one of the top 10 research achievements for 1999 by the American Heart Association.

"There are clues all around us, we just need to look," says Murphy. "As Yogi Berra used to say, you can see a lot just by looking."

Being extraordinarily industrious, or producing the hard work, of course, is essential. Semenza says the goal is to be efficiently industrious, "not spending as many hours as possible at work, but getting as much done possible during that time at work. You do that is by learning to be efficient."

That reinforces the lab team to be more productive. Rather than having your staff stressed out over making their deadline because things were left to the last minute, they are ahead of the game. "Which is why a lot of the staff like working with me because I'm never breathing down their neck at one minute to midnight," says Semenza.

That in turn supports the work life balance, which physician scientists acknowledge is a necessary ingredient for success in science as well. As is spending some time at the end of each day to "think hard" about what you learned that day, says Valle. Allowing yourself to fail helps, too.

"Not every experiment works and you need time to figure out why it didn't work," says Dover.

Last but not least, passion and a vision.

"To be successful, you have to really want it, because it's hard," says McGrath-Morrow. "You can be a super smart person, but if you don't really want it, you're not going to do it. But you can't do it unless you have the environment and people who can help you — you can't do it on drive alone."

In that regard, concludes Cutting, "Gregg has been a nice exemplar. There are many of us who have had our own great successes and we're happy to pursue these paths."

Adds Dover, "Gregg Semenza, in my mind, has inherited and reached the epitome of what we all have felt since Barton Childs, what we as physician scientists wanted to accomplish — we wanted to make a difference. We wanted to straddle the clinic and the patient with the laboratory — we wanted to change medicine." 🌟

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pediatricrounds



Bret Mettler

A Compassionate Competitor at Heart

By Karen Blum

New Director of Pediatric Cardiac Surgery to serve as co-director of the Blalock-Taussig-Thomas Pediatric and Congenital Heart Center

BRET METTLER SAYS that throughout his youth, he maintained a passion for science and for sports. The two interests combined well for him in surgery.

“It’s that concept of competing with yourself and competing for outcomes,” says Mettler, who became director of the Division of Pediatric Cardiac Surgery and co-director of the Blalock-Taussig-Thomas Pediatric and Congenital Heart Center in January. “Every day is a new challenge, and it went along with the athletic prowess I had, wanting to win and succeed.”

Mettler comes to Johns Hopkins Children’s Center from Vanderbilt University in Nashville, where he spent nearly a decade as an assistant professor of cardiac surgery and director of pediatric cardiac transplantation and mechanical support for Vanderbilt’s Monroe Carell Jr. Children’s Hospital. He specializes in pediatric cardiac surgery, pediatric heart transplant, congenital heart diseases and thoracic surgery.

As routine practice, Mettler asks his patients and their parents to send him holiday cards so he can watch the children grow. By the time he left Nashville, he says, his walls were covered with 10 years’ worth of photos. He saw newborn patients become fourth graders.

“The best part of working with this population is knowing you’re changing a child’s life,” he says. “Parents expect to have 50 to 60 years with their children. Being able to give them that gift and being able to affect them and allow them to have a long life, I can think of nothing better in medicine.”

At Johns Hopkins, Mettler has both short- and long-term goals. First, he’ll help reorganize the heart center to bring together — under one collaborative structure — the disciplines of pediatric cardiac critical care, pediatric cardiac anesthesia, pediatric cardiology and pediatric cardiac surgery. Down the road, Mettler says, he wants to further develop the adult congenital heart center and the pediatric transplant and mechanical support programs, increase outreach to patients nationally and collaborate with other centers on multi-institutional studies of children with congenital heart disease. Only 100 or so cases of congenital heart disease occur in every 1 million children, Mettler says, so no center has enough patients to perform needed large-scale studies.

“Our goal is to provide a service line of care, where patients can call one phone number and have access to cardiology, ongoing studies, surgical therapies, postoperative care or outpatient care,” he says. He also wants to see “a dedicated hospital floor to put these patients on, with nursing staff who really understand congenital heart disease and the specific issues that go along with children who are affected.”

On the research side, Mettler has been interested in developing a type of artificial pulmonary valve using a patient’s own stem cells. The structure would grow and develop as the child ages, without the replacement operations necessary with current bioprosthetic valves or with valves transplanted from donors.

Mettler received his M.D. from the University of South Dakota. He completed residencies in general surgery at the University of Texas Health Science Center in San Antonio and the University of Michigan Medical Center, a research fellowship in cardiac tissue engineering at Boston Children’s Hospital, and residencies in cardiothoracic surgery at the University of Virginia Medical Center and in congenital cardiac surgery at Boston Children’s Hospital. 🌟

A 'Blue Baby' Returns

By Gary Logan

ON A RECENT August morning, **Hugh Michael Edenburn** walked into the atrium of Johns Hopkins Children's Center at his usual swift pace. When information receptionist Elyse Geber learned who the 76-year-old visitor was, however, she had to catch her breath.

"Oh my God," she greeted him, hand on her heart. "You're history."

On Oct. 2, 1945, Edenburn, then 2 years and 7 months, underwent the "blue baby" operation to repair a congenital heart defect that had taken the lives of thousands of infants until the groundbreaking surgery was developed and introduced at Johns Hopkins in 1944. As a former respiratory therapist, Geber knew all about tetralogy of Fallot, the deadly condition marked by a bluish or "cyanotic" tint to the child's skin due to the lack of blood flow to the lung. She also knew the history of the landmark surgery led by **Alfred Blalock**, of the essential diagnostic work by pediatric cardiologist **Helen Taussig** and of the critical laboratory studies by surgical technician **Vivien Thomas**. To have a patient in front of her three-quarters of a century later, Geber felt, was astonishing. Colleagues were equally thrilled.

"You are an example and a reminder of our past, of innovations in cardiac surgery and how pediatric cardiology came to be a specialty," said **Shelby Kutty**, co-director of the Blalock-Taussig-Thomas Pediatric and Congenital Heart Center at Johns Hopkins.

When Edenburn was a toddler in Waterloo, Iowa, his health was dramatically different. Without enough oxygen to reach his lungs, doctors informed his mother, he would be bedridden by 3 years old and dead by 5 or 6. Prepare yourself, they said.

Instead, after reading an article in *Collier's* magazine about Blalock's revo-

lutionary surgery, she prepared to take her son to Baltimore. He was a candidate for the procedure that provided a second chance at oxygenation by joining an artery leaving the heart to an artery leading to the lungs, a procedure that came to be known as the Blalock-Taussig shunt.

Edenburn remembers only a few of his first moments at Johns Hopkins: The front doors of the hospital, the towering Christ statue immediately past the doors and standing in his crib on the ward crying because he missed his mother. He recalls Taussig thumping her fingers on his chest before the surgery. He also remembers that, after the operation, he was able to breathe freely for the first time in his life.

As he got older, however, he had problems with his circulation and his breathing became more labored. Then, at the age of 18, events presented another challenge. While he was being treated for

injuries from an automobile accident, doctors diagnosed him with subacute bacterial endocarditis, an infection of the lining of the heart, and recommended an updated tetralogy of Fallot repair.

"Before the repair my friends were always waiting for me to catch up to them," Edenburn told surgeon-in-chief of The Johns Hopkins Hospital **Robert Higgins**. "After the surgery, I was waiting for them."

Now, he enjoys life in Cebu with his wife Epifania, whom he met in the Philippines and married in 1997. He eats mostly fish and avoids red meat, dairy and alcohol, except for an occasional glass of red wine. He has never smoked. So, why the return visit now, almost 70 years later?

"I wanted to see the Christ statue, the portrait of Vivien Thomas," Edenburn said. Then he looked down, borderline teary: "It's really emotional for me. I wanted to know if anyone knew about me, if they heard about me."

They had. "How's your heart today?" someone asked as he left.

He responded, "It's great." 🌟

Hugh Michael Edenburn, a historical figure himself, stops by Johns Hopkins heart history display.



Exploring Theranostics for Children

BEFORE ARRIVING at Johns Hopkins Children's Center in 2018, pediatric cardiologist **Shelby Kutty** built a reputation at the University of Nebraska Medical Center as one of the foremost authorities in multimodality cardiovascular imaging. It was there in 2007 that he connected with adult cardiologist Thomas Porter, who was investigating an emerging field of medicine called theranostics, which combines diagnostics and therapeutics via imaging such as ultrasound. Porter was applying theranostics as a therapy for stroke in adults — targeting ultrasound energy vibrations to break up clots in the brain — and not as a treatment for cardiac conditions in children. Nonetheless, Kutty was intrigued.

“His aim was to use transcranial ultrasound with a modified energy and contrast microbubbles to lyse clots in stroke situations,” explains Kutty. “The essential principle was he could see the ultrasound and contrast acting on the tissue, which really sparked my interest as an ultrasound imager with an interest in integrating and optimizing new technologies.”

“Theranostics is happening in cancer care and nanotechnology, but it is totally unexplored in the pediatric setting.”

While Kutty was exploring theranostic applications in children, he realized they are vulnerable to thrombus-forming conditions with limited therapeutic options, or therapies associated with serious side effects such as bleeding complications. Collaborating with Porter, he focused on complications of chronic indwelling central lines that are often used in pediatric patients. The two investigators found success in an acute thrombus pig model, which generated grants to study theranostic treatments to maintain the patency of central venous lines and intracardiac shunts, and also to treat femoral artery spasm. Young children who undergo heart catheterization experience needle trauma to the femoral artery, Kutty explains,



The vibration of microbubbles in the ultrasound field, Shelby Kutty notes, releases vasodilator substances like nitric oxide in the vessel wall, improving perfusion.

which tends to cause spasm and reduce distal perfusion.

“We created models of femoral artery injury and then targeted ultrasound contrast-therapy to the injured site. The vibration of microbubbles in the pressure fluctuations of the ultrasound field releases vasodilator substances like nitric oxide in the vessel wall,” says Kutty. “Then we monitored distal pulse and perfusion, and found that the perfusion improved.”

In another collaboration, with cardiologist Jonathan Lindner of Oregon Health & Science University in Portland, Kutty plans to participate in a trial using theranostic therapy for limb ischemia to help alleviate the acute pain crises in patients with sickle cell disease. The vascular occlusion of red blood cells triggering the crises, Kutty notes, is an ideal target for theranostics.

“By applying theranostic energy, you are improving blood flow and relieving the painful crises in the vessels where sickle crisis is occurring,” says Kutty.

Indeed, Kutty is exploring other clinical trials, including targeted theranostic therapy for young adults presenting in the emergency department (ED) with vascular pain — the therapy would reduce the need for morphine. He adds that given the accessibility and portability of ultrasound, its noninvasive nature, and its use by ED physicians, intensivists and obstetricians, among others, make it a universally applicable tool. Its applications as a theranostics tool may have just scratched the surface, especially for children.

“Theranostics is happening in cancer care and nanotechnology, but it is totally unexplored in the pediatric setting,” says Kutty. “We have a wide open field in which it can be applied in cancer treatments for children.” — GL 🍌

1,000 Bladder Exstrophies and Counting

TREATING 1,000 PATIENTS over almost four decades would be no big deal for almost any surgeon. But when the patients' condition is among the rarest and most complex of birth defects — bladder exstrophy, in which the bladder develops outside the fetus — and there are only a handful of surgeons in this country who treat it, the record is staggering.

“Initially, once our service was established at Johns Hopkins Children’s Center some 36 years ago, referrals took off like a rocket ship because these children and their parents did not have anywhere else to go,” says pediatric urologic surgeon **John Gearhart**.

The benefits of bladder exstrophy surgery include urinary control, improved appearance of the genitals, reduced problems with future sexual functioning, and prevention of kidney and other infections. But due to the complexity of the congenital defect, Gearhart explains, finding such specialized surgeons and a multidisciplinary team equipped and ex-

perienced to treat and manage these patients has been an exhausting hit-or-miss exercise by parents. Through the growth of parents groups, internet chat rooms and websites around the world, however, families have found their way to Gearhart, pediatric orthopedic surgeon **Paul Sponseller**, pediatric urologist **Heather Di Carlo** and the multi-disciplinary team dedicated to treating these complex patients.

In addition to surgeons experienced in surgical reconstruction with 3D MRI guidance, the team includes pediatric pain medicine specialists, pediatric nurses trained in caring for patients with bladder exstrophy, physical and behavioral therapists to help patients manage voiding issues, and a child psychologist. To help educate families and keep them updated on advances in evidence-based treatments for bladder exstrophy, Gearhart and the team hold educational forums and an annual reunion for patients and their families. — *GL* 🍌

Collaboration Conquers a Rare Cyst

A DERMOID CYST is an odd anomaly that seems harmless. It is a collection of normal tissue, such as hair follicles and sweat glands, formed from cells trapped in the spinal cord or brain during development. This remnant of tissue is then found in a part of the body where it should not be found, typically the midline face with the nose the most common location. It is a sly master of disguise, too, often mistaken for a blackhead that won’t go away. This cyst is generally as benign as a blackhead, but it may grow on a years-long journey through the base of the skull and even into the brain. Then it becomes potentially lethal.

“The problem is anytime there’s an opening from the outside of the body to the inside, it becomes a portal of entry for infection,” says pediatric neurosurgeon **Alan Cohen**. “There can be up to a 20 percent risk of developing meningitis or an abscess in the brain.”

Cohen is describing the recent case of a young girl who underwent a six-hour surgery that he and pediatric plastic reconstructive surgeon **Rick Redett** performed as surgeons on the multidisciplinary Pediatric Cranial Reconstruction Center team at Johns Hopkins. Because this was a very rare (one in 30,000) dermoid cyst that found its way through the nasal septum, the skull, the dura covering the brain and potentially the brain itself, both specialists were needed.

The two surgeons then meticulously excised the cyst and laid tissue down into the track to block any potential bacteria, preventing development of meningitis or a brain abscess. After a three-night stay in the hospital, the girl returned home.

“She healed up pretty well,” says Cohen. “She looks great.” — *GL* 🍌



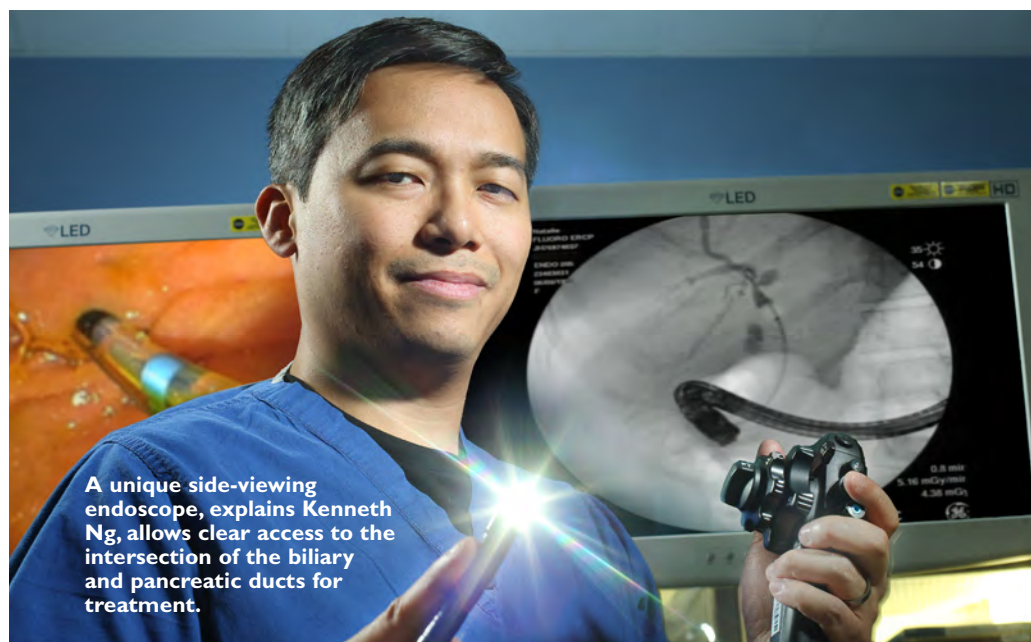
John Gearhart with pediatric urologic surgeon Heather Di Carlo.

New Endoscopic Tools for Children

ENDOSCOPIC Retrograde Cholangiopancreatography (ERCP) is commonly used in treating biliary and pancreatic disorders in adults but rarely used in children, partly because the technology is expensive and the volume of pediatric patients much lower. Pediatric gastroenterologist **Kenneth Ng** is leading efforts at Johns Hopkins Children's Center to change that practice and improve care for children with conditions like biliary and pancreatic duct stones and cancerous and noncancerous tumors.

"In the adult world, many centers have the expertise to use this technology, but in the pediatric world it's actually quite rare," says Ng. "I feel it's very important to bring that expertise to our pediatric patients."

In ERCP, Ng explains, an index-finger thick endoscope is placed through the mouth and into the stomach and the first part of the small intestine. There, in the duodenum, a cannula is passed through the endoscope into a small opening where dye is injected and X-rays



A unique side-viewing endoscope, explains Kenneth Ng, allows clear access to the intersection of the biliary and pancreatic ducts for treatment.

taken to study the ducts of the pancreas and liver. While traditional endoscopes with a forward viewing camera have generally played a diagnostic role, ERCP's innovative sideways-viewing cameras that enhance visualization of and access to the intersection of bile and pancreatic ducts, now allow gastroenterologists to treat detected disorders as well.

"Essentially, ERCP is a technique that uses a unique side-viewing duodenoscope to both examine and treat diseases of the biliary and pancreatic ducts — essentially you're driving sideways," says Ng. "In patients with suspected or

known pancreatic disease, ERCP will help determine the need for surgery or the best type of surgical procedure to be performed," adds Ng.

Ng is also introducing other new tech tools to the pediatric GI endoscopy program, including rotational clips to improve bleeding control, and EndoFLIP, a balloon-catheter system designed to measure GI track characteristics.

"It's kind of a fancy ruler," says Ng, "that allows us to know the before and after of our actions and helps guide us to optimize care." — *GL* 🍌

Connecting Young Patients to Psychiatric Care

By Karen Nitkin

A NEW TELEMEDICINE program at Johns Hopkins Bayview Medical Center is making life easier for pediatric patients with serious, persistent mental health issues, their families and their clinicians.

"The use of telemedicine has facilitated psychiatric visits for patients who had previously gone months before they could coordinate a way to come to the clinic," says **Hal Kronsberg**, child psychiatrist for the Johns Hopkins Child Mobile Treatment Program.

The program serves about 90 patients, ages 4 to 24, at a time. They have conditions such as schizophrenia or bipolar disorder, and did not have success with outpatient or school-based treatments.

"It supports the kids, but also the families," says Kronsberg. "Some kids may go in and out of foster care or experience homelessness, and we continue to see them despite these disruptions."

Here's how it works: Kronsberg and the patient set the appointment time.

When therapists visit patients in the community, they bring a tablet with the Polycom app, which provides a secure connection. Polycom accesses the cameras on the devices so Kronsberg and the patients can see each other.

Before the program launched in October 2018, patients had three ways to connect with Kronsberg: They could travel to the Johns Hopkins Bayview offices, a therapist could pick them up, or Kronsberg could travel to the patient — limiting the number of patients he can see. Telemedicine adds a fourth way, says **Tawny Epperson**, clinical supervisor for child mobile treatment services: "Children and teenagers are very comfortable talking through the iPad." 🍌

An EMU for Children

PEDIATRIC NEUROLOGIST Sarah Kelley recalls the complex case of a child diagnosed with epilepsy, and the cause of his seizures not known. What started out as periodic shaking of his right leg evolved into violent, minute long episodes — up to 60 each day — on his entire right side. The good news was the patient's epilepsy could be treated surgically, but only if the lesion in his brain triggering the seizures could be located. Conventional MRI had revealed the culprit and a higher-resolution MRI scan confirmed its precise location at the bottom of a deep fold in the cortex of the brain. The lesion, however, was a few millimeters away from the area in the brain responsible for motor movement, posing a serious surgical risk for the patient. Intracranial monitoring in the epilepsy monitoring unit (EMU) helped reduce that risk, and subcortical motor brain mapping was used to ensure vulnerable areas were not injured. Immediately after surgery, the patient was

able to speak, and 24 hours later, he gave his family a high-five with his right arm. Two weeks later, he was walking.

Kelley cites the case as an illustration of the human and technological resources that Johns Hopkins pediatric neurologists/epileptologists, neuroradiologists and neurosurgeons, among others, can bring to bear in diagnosing and treating young patients suffering from seizures. However, she adds, one fly in the ointment was that the one EMU at The Johns Hopkins Hospital served all age groups. That meant children went through seizure monitoring, in some cases for up to a week, with adults in neighboring beds and not all pediatric services readily available. This all changed in January this year when a new EMU for pediatric patients only opened in The Charlotte R. Bloomberg Children's Center building.

"We now have an epilepsy monitoring unit staffed 24/7 with pediatric-trained neurologists, neurosurgeons and nurses,

epilepsy specialists and technicians," says Kelley, director of the new unit.

The pediatric EMU is a four-bed unit with state-of-the-art technology, including high-definition cameras and wireless monitoring in two of the rooms, allowing patients to walk freely. Other services such as pediatric respiratory therapy, pediatric home care and child life services, are available to patients and their families.

"Our youngest patients will now have easy access to all the resources of a children's hospital," says Kelley. "We'll be able to build capacity and bring more children in more quickly for the care they need."

The goal of epilepsy monitoring is to determine if the child is having seizures and, if so, their frequency and location. Patients undergo electroencephalography (EEG), with electrodes placed on the scalp, to record the brain's electrical activity. In some cases, it's already known that the patient has seizures and the EEG is performed to allow doctors to change the patient's medicine in a safe environment. Other times, the patient has failed a pharmacological treatment and the EEG results are used to determine if surgery is an option.

In some cases, intracranial monitoring — in which electrodes are placed in or on the brain in the operating room — is used to further localize the area of seizure onset. Both adult and pediatric EMU staff review all complex cases during their weekly epilepsy conference.

"That's where we get 15 epileptologists in adult medicine and pediatrics together to think about the details of each case to determine by consensus the best course of action for each patient," says Kelley. — GL 🍌



Pediatric neurologist Sarah Kelley, middle, and child life specialist Crissie Traugott with a patient in Johns Hopkins Children's Center's epilepsy monitoring unit.

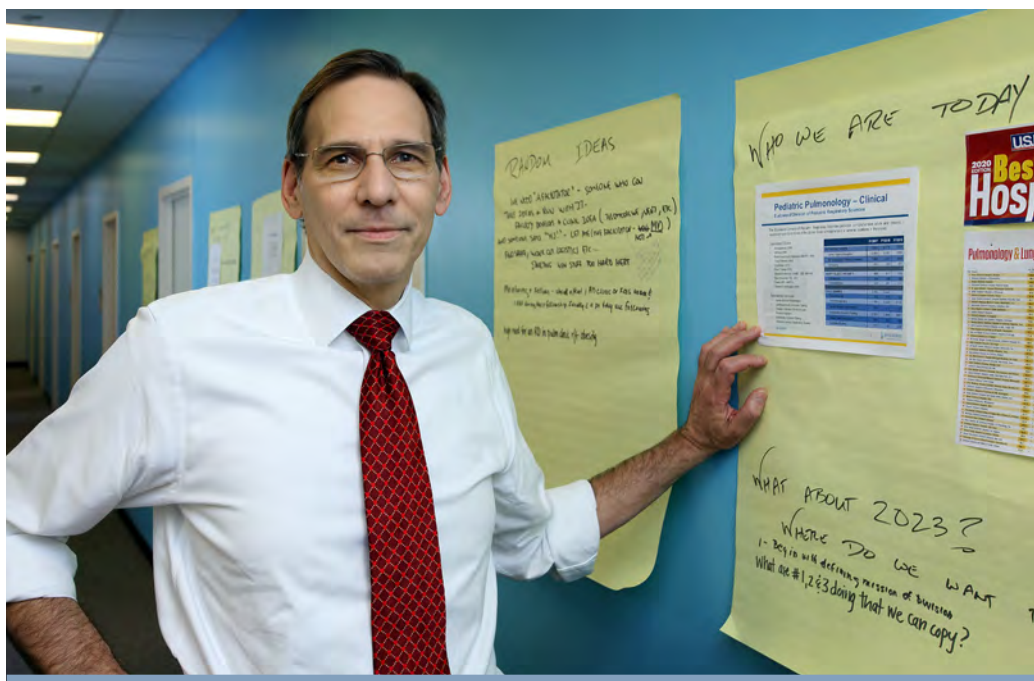
Peter Mogayzel, Advancing CF Care and Research

GIANT YELLOW Post-It Notes line the hallway outside faculty offices in the pediatric pulmonary division with messages to consider like, “Is this our goal?” It’s a strategic planning day, and **Peter Mogayzel**, director of the Eudowood Division of Respiratory Sciences since January 2019, is ready to get to work.

“We’re trying to position ourselves to be the destination of choice for treating pediatric pulmonary disease in the region,” he says. “We are working to enhance our basic science and clinical research, and foster our fellowship program. We’re not trying to be the biggest practice on the East Coast — we’re trying to be one that’s providing the best possible care and one seen as a referral center for children with complex needs.”

It’s the same careful consideration he has applied throughout his career. Mogayzel’s interest in medicine started when he was a high school student growing up in New England. He earned his M.D. and a Ph.D. in biochemistry from Boston University, then headed west to the University of Washington, Seattle, for a pediatrics internship and residency, where he first encountered a patient with cystic fibrosis (CF). He came to Johns Hopkins Children’s Center for a pediatric pulmonary and critical care fellowship in 1994 and made it his home.

Mogayzel has since become an internationally recognized expert in CF, having



headed the Johns Hopkins Cystic Fibrosis Center since 2002 and serving on the board of directors for the Cystic Fibrosis Foundation. He has taken on additional leadership roles over the years, serving as medical director of the pediatric lung transplantation program and the pediatric specialty clinics, and as one of the key drivers behind Johns Hopkins adoption of the Epic electronic health-records system.

Mogayzel “has worn many hats [at Johns Hopkins] and he’s worn them all very, very well,” says **Beryl Rosenstein**, professor emeritus of pediatrics and former director of the Johns Hopkins Cystic Fibrosis Clinic, who has worked with Mogayzel since his fellowship days. “He communicates well in a quiet manner, and is viewed by all as a leader. Patients love him and sing high praise for all of his efforts.”

The past year has been an exciting one for Mogayzel. The first CF patient he treated at Johns Hopkins turned 25; he

For inspiration, follow the “Yellow Post-It Path,” urges Peter Mogayzel.

gave a plenary talk on the evolution of CF care at the North American Cystic Fibrosis Conference in Nashville; and partnerships in clinical trials of Trikafta, the first triple combination therapy drug for patients with the most common CF mutation, which led to its approval by the U.S. Food and Drug Administration.

He is now working on additional studies of Trikafta and other modulator drugs — which help stop the downward trajectory of lung function commonly seen in CF — in infants. Mogayzel also has his eye on potential future gene therapies. One study is evaluating the promise of using an inhaled form of messenger RNA to help produce normal proteins in patients affected by CF, who have defects in the CFTR protein that typically helps maintain a balance of salt and water on the lung and other surfaces.

“The future for CF is really moving toward developing therapies for the last 10 percent of the population that don’t have one now, and then hopefully moving toward a cure,” Mogayzel says. “We think these drugs are going to reshape the way we care for patients with CF because we’re going to have a much healthier population of children, where we can move more to telemedicine and nonhospital-based care.” — KB 🍌

“The future for CF is really moving toward developing therapies for the last 10 percent of the population that don’t have one now, and then hopefully moving toward a cure.” — PETER MOGAYZEL

Rick Redett's Quest: Reach the Unreachable

THE ITCH Rick Redett cannot scratch enough these days may have had its genesis when he was a young Peace Corps volunteer in the Central African Republic, thanks to parents who instilled a strong sense of civic responsibility. There, with visions of becoming a wildlife biologist, he lived in a remote camp with two Byaka Pygmy families and learned first-hand their health challenges, including poor nutrition and high child mortality. Their tremendous health demands shifted his vision to medicine.

"I realized that there's really nothing more basic than somebody's health, and that I could make a bigger impact as somebody's physician," says Redett.

Wrapping up his Peace Corps stint, Redett pondered primary care or tropical medicine, perhaps infectious disease. Then, assessing the international health scene, he learned that most resources were targeting the medical needs while surgical needs were largely ignored. He related to the words of renowned medical missionary Paul Farmer, "Surgery is the neglected stepchild of global health."

Redett decided to forge a surgical path and today he is a pediatric plastic and reconstructive surgeon. That discipline, he stresses, is the "perfect specialty" to blend surgical interests with the surgical needs of children in developing countries.

"In our plastic and reconstructive surgery practice here, we do a lot of high-tech stuff, including microvascu-

lar and craniofacial surgery, but by far the majority of what I do here I can do anywhere," says Redett. "It's a great specialty to take to a developing country."

Indeed, over the past two decades Redett has traveled the world on medical missions with various groups, performing cleft lip and palate and burn reconstruction with surgical teams where the need is the greatest — places like Central and South America and sub-Saharan Africa. As beneficial as that work is, Redett says, after the teams leave the follow-up can be poor. In recent years, he has been rethinking the fly-in medical mission and exploring more-impactful ways to help solve the global surgery shortage. One path he took is as a visiting educator with ReSurge International, a global reconstructive surgery program that focuses on teaching.

"As a visiting educator, I go to Zimbabwe and Tanzania and do surgery with residents, general surgeons, and plastic surgeons there — the idea being that by the time I leave, they're going to know how to do that operation," says Redett. "Then I go back to that country over

and over again to establish a relationship with their surgeons."

Another practice that resonates for Redett is building capacity for surgery to happen in low- and middle-income countries. That means not just increasing the number of trained surgeons but also helping to build infrastructure.

Redett and like-minded global surgeons realize they are up against a huge financial and human resource problem that only unlimited resources will solve. So there's the itch, finding solutions for a seemingly unsolvable global public health problem — unmet surgery needs to the tune of 143 million more procedures needed in low- and medium-income countries.

Redett's answer is keep at it, define the problem, spend time in a country and determine the needs, bring them back to colleagues and potential supporters, partner with groups like ReSurge, build models and share what works.

Redett's quest suggests fixing global surgery is not so much an itch as a relentless passion. Regardless, he keeps scratching it. — GL 🍌





Expanding Ambulatory Access

Diabetes care in Bethesda among new outreach services

VLADIMIR NAZOR began his medical journey in 2015 in Las Vegas, where he was diagnosed with type I diabetes. It continued in Texas and then at Johns Hopkins Children's Center in Maryland after his parents moved there in August 2019. His parents' reaction?

"I'm impressed. We've been through a lot of clinics, lots of places in Texas and Las Vegas, and this place really seems to care," says his mom, Kimberly. "As soon as you walk through the door, everyone cares."

The door, however, was not to the hospital but to a remote pediatric subspecialty clinic in Bethesda, Maryland, just north of Washington, D.C., off the I-270 corridor, which was very accessible to the suburban Severn family. The parents learned through the medical community that Johns Hopkins pediatric endocrinologist **Sandra Salsberg** "came highly recommended." She and the clinic staff, the parents found, treated them like members of the care team.

"There's a lot of discussion back and forth," Kimberly says. "They're interested in our perspective as well."

The care includes diabetes nurse educators who address areas in which patients are struggling and provide supports to meet their needs. Diabetes, Salsberg explains, is a challenging disease that requires a lot of education and coordination with patients and their parents.

"We all work together to monitor the regimen, to make sure there are no complications and to manage dosing," she says. "We see them every three months but we expect changes as kids grow, and we don't expect our patients to wait for their visits to get in touch with us."

In a similar experience, Allycia Sindorf, mother of 3-year-old Mackenzie, and Lisa Hess, mother of 8-month-old Anderson, were thrilled with the care for their children. Both moms wondered, however, how they would manage the repetitive long commute to multiple Johns Hopkins pediatric subspecialists for the

ongoing follow-up care required for children with complex conditions. Sindorf lives in Stevensville on Maryland's rural Eastern Shore, 46 miles from the Children's Center, and Hess 90 miles south in California, Maryland. Then the two mothers learned that Johns Hopkins had opened a new remote location for its pediatric subspecialists on Bestgate Road in Annapolis, significantly less than their driving distance to Baltimore.

"This is big time more convenient — it takes 15 minutes to get here as opposed to 45 minutes to Baltimore," says Sindorf.

"Part of why I like going to Annapolis is seeing multiple disciplines like cardiology, GI (gastroenterology) and pulmonary without having to make long trips to Baltimore," adds Hess.

Shakeika Fields of Fort Washington, Maryland, near Washington, D.C., helped solve one of pediatric pulmonologist Lydia Kim's problems and one of her own, too. When Kim called and asked Fields if a sleep clinic in Howard County would shorten her commute to Johns Hopkins pediatric outpatient clinic for follow-up appointments for her daughter, 7 year-old Madison, Fields immediately replied, "Oh yeah, sure thing. You'll cut 30 minutes off our trip." For Kim, that meant another patient who would justify opening a community sleep clinic on the campus of Howard County General Hospital, a Johns Hopkins Medicine member hospital.

"While we've had a sleep lab at Howard County to conduct sleep studies, children with issues like sleep apnea would have to come into East Baltimore to be evaluated for one," says Kim. "Now we also have a sleep clinic that allows patients to be evaluated and followed up at one suburban location."

For more information, call 443-997-5437. — GL 🍌



At Madison's latest visit, pediatric pulmonologist Lydia Kim helped her find a comfortable fit for her CPAP, not an easy chore for a young child.



Shawn Ralston, left, and Emily Boss

Improving Surgical Care with a 1,000 Foot View

By Christen Brownlee

IMAGINE YOU'RE a parent whose child has just suffered an injury and landed in the local emergency room. Maybe the emergency room doctor there calls a surgical consult, and a surgeon then performs a procedure on your child without communicating it to you, preparing your child, or optimizing your child's pain control. Maybe your child needs surgery that night. You wonder if the entire care team knows how to take care of children? Who is putting your child to sleep, who will perform the surgery? Maybe a surgeon describes surgery to you and your family in terms you don't understand. Maybe a surgeon orders a CT scan or MRI, which was exhausting and scary for your child to undergo — and later at rounds you find out from a

different team that the test wasn't necessary. Or, maybe you don't hear anything on rounds at all, or ever during your child's hospitalization.

These are all problems that can affect patients and their families. However, says **Emily Boss**, chief surgical quality officer at the Johns Hopkins Children's Center, these types of issues can be addressed through systems-level change, where there is potential to sustain improvements in care for the whole of patients and families.

"It's hard to make quality and safety sound sexy," Boss says, "but its fundamentals are at the heart of providing the best care to our patients."

Since 2008, Boss has treated patients as a pediatric otolaryngologist-head and

neck surgeon, a job that not only lets her help individual patients but also gives her pivotal insight on what problems patients and families are facing in real time. In January 2019, she stepped into her new role as chief surgical quality officer, rising to the top of a nationwide search and now leading efforts to tackle quality and safety issues and improve healthcare delivery for children.

Every month, she and leaders from various pediatric care teams meet as members of the Children's Center Quality and Safety Council to discuss issues that have affected their patients — ranging from unclear directions for administering over-the-counter medications, to inefficient postoperative discharge procedures, to prevention of serious adverse

events during and after surgery. To better understand the scale and details of these problems, the team develops specific metrics to measure.

“There’s an old business adage that you can’t manage what you don’t measure,” Boss says. “A lot of quality and safety work is in developing and choosing the right measures so we can monitor problems, as well as their improvement after interventions.”

Once they have a handle on the particulars of quality and safety issue, Boss and her colleagues develop and implement solutions, collecting more metrics on the back end. One of their recent efforts focuses on children who receive appendectomy at Johns Hopkins, a rela-

tively common surgery that about 7% of people have in their lifetime, most commonly during childhood. National benchmarks showed more revisits to the emergency room after discharge compared to peer institutions.

To help improve this number, Boss and her colleagues developed a set of standards that helped surgeons adequately treat all patients at average risk of complications and identify patients who have an increased risk of complications, steps that could decrease revisit rates. They are currently measuring how these changes affect readmission rates to see whether there are ways to further improve care.

“I love taking care of patients and in-

“A lot of quality and safety work is creating the right measures so we can monitor problems, as well as their improvement.”

— EMILY BOSS

fluencing individuals’ quality of life, but quality and safety is exciting because through organizational changes, you can affect thousands of lives at once,” Boss says. “I’m proud to play a key role in helping our Children’s Center take that 1,000 foot view.” 🌟

Teaching the Science and Psychology of Change

By Julie Weingarden Dubin

WHEN Shawn Ralston presented at Pediatric Grand Rounds last November, she shared what she cares about most as a clinician and a researcher: Overtreatment. She delved into her favorite topic: De-implementation (divesting from ineffective and harmful medical practices). Because she views de-implementation ideas through the lens of cognitive psychology, she illustrated her points with another favorite subject: ‘80s movies scenes, like those in “Ferris Bueller’s Day Off” and “The Breakfast Club.”

Ralston joined Johns Hopkins Children’s Center last spring as Chief Medical Quality Officer. A hospitalist by training with 12 years as a hospitalist director, Ralston is Editor in Chief of the American Academy of Pediatrics (AAP) journal, *Hospital Pediatrics*, and has chaired AAP panels for bronchiolitis guidelines.

Her clinical work as a hospitalist and her research has focused on respiratory viral illnesses, with the goal of making care for acute viral bronchiolitis more evidence-based. This is difficult, she notes,

because it involves asking people to cease tests and treatments proven pointless.

“Unfortunately, there is not much that really helps children with viral lower respiratory tract infections such as RSV (Respiratory Syncytial Virus), so the nature of most quality improvement work for these diseases is around urging the discontinuation of unnecessary therapies,” Ralston says.

Ralston was attracted to Johns Hopkins due to the work done around prevention of harm to hospitalized patients by Marlene Miller, former head of the Division of Quality and Safety. Following Miller’s departure, the division has been split into two divisions — one covering medicine, led by Ralston, and the other surgery, directed by Emily Boss.

Johns Hopkins is well positioned to do academic quality improvement research, which is unusual for an emerging field, says Ralston.

“Pediatric hospital medicine is a new specialty and I’m helping Johns Hopkins build a fellowship in pediatric hospital medicine starting in 2021 or 2022,” says

Ralston. “We’re recruiting people who specifically want to train in academic quality improvement.”

Quality improvement is about making medicine better, which can mean safer, more effective or more efficient care depending on your perspective, Ralston says, but better almost always involves the idea of change. Ralston has shifted her interest to learning what motivates physicians to change their behavior.

“I am particularly interested in the psychology of abandoning formerly used tests and therapies as the medical evidence evolves,” says Ralston. “Fields like cognitive psychology are becoming necessary to master in order to really improve care in medicine.”

Sometimes, classic film buff Ralston concludes, it takes an iconic song like the Simple Minds’ “Don’t You Forget About Me” from “The Breakfast Club”—a film about teens in Saturday detention learning more about themselves than ever expected—to see the possibility of teamwork across very different groups within an institution. 🌟

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Johns Hopkins infectious disease specialist Aaron Milstone's newest research focuses on parents as reservoirs for NICU infections.

Prophylactic Treatment for Parents Reduces Babies' Risk of Infection

By Christen Brownlee

LONG BEFORE the coronavirus pandemic began, other infectious disease threats lurked in hospitals and continue to do so. That's why infectious disease specialist **Aaron Milstone** is vigilant about washing his hands as he sees patient after patient in the neonatal intensive care unit (NICU). He also regularly dons isolation gowns, masks, and gloves. His clinician colleagues — other doctors, nurses, trainees, and technicians — all take these precautions as well, steps they're rigorously taught in training to prevent the transfer of germs that can make their young patients sick. However, he says, there's one prominent group of frequent visitors to the NICU who do not meticulously undertake these safety measures: parents.

"We educate healthcare workers on infection prevention, track events, give them data and make action plans, but we're not even reminding parents about washing their hands," Milstone says. "We know parents are reservoirs about introducing infections to their children, but we don't usually treat parents with the same rigor that we do healthcare workers."

Staphylococcus aureus, a bacterial species that harmlessly affects about a third of most adults, can be devastating to babies with weak immune systems, he explains. Studies show that up to 3.7% of very low birthweight babies in NICUs get *S. aureus* infections during their stay, and up to a quarter of these young patients die.

But a new study led by Milstone and

published in the Jan. 28, 2020 *Journal of the American Medical Association*, shows how effective treating NICU parents can be for reducing their potential to unwittingly introduce infection. Employing a quick and painless intervention, providers in the Johns Hopkins NICU significantly cut infection rates of *S. aureus* transferred directly from parents.

Over a four-year period, from November 2014 to December 2018, the researchers screened parents whose babies were admitted to the Johns Hopkins NICU to see if they were carriers for *S. aureus*. If either or both tested positive, the researchers genotyped their bacteria and then randomized both parents to one of two groups: The "intervention" group participants were instructed to put antibiotic ointment in their noses — a common site that these bacteria colonize — and to wipe their hands with antibiotic-impregnated cloths before they entered the NICU. The "control" group placed petroleum jelly in their noses and wiped their hands with soap cloths. Parents were unaware of which group they were assigned to.

When Milstone and his colleagues analyzed the data, they found that about 58% of babies who developed *S. aureus* infections caught them from their parents, evident because the genotypes of the strains matched. However, babies whose parents were in the intervention group had about half the rates of *S. aureus* infections caught from parents as those in the control group — a testament to the

effectiveness of this simple prophylactic treatment, Milstone says.

Over the years, he and his team have developed a number of other seemingly simple but powerfully protective interventions that can cut infection rates in these very young and vulnerable patients, he adds. They've published studies showing that having parents and other care providers bathe pediatric ICU babies using a special antibiotic solution, rather than regular soap, can cut blood-borne infections by a third. He and his team have further cut infections by screening all NICU babies for not only methicillin-resistant *S. aureus* (a strain commonly known as MRSA, which is resistant to a common antibiotic used to treat these infections), but also for susceptible strains of *S. aureus*, which kill three times as many pediatric patients each year. They also screen all the clinicians who treat PICU and NICU babies. Babies and providers who test positive receive nasal antibiotic ointment.

Milstone says that he and his team will continue to search for ways to incrementally bring his patients' hospital-acquired infection rates as close to zero as possible.

"For me, the most rewarding part of these strategies is not having to tell a family, 'I'm sorry, your kid got a hospital onset infection, a surgical site infection, or a blood-borne infection.' I don't want to have to apologize to families for infections that potentially are preventable." 🌟

Improving Respiratory Care Where Most Needed

By Karen Blum

ERIC MCCOLLUM'S first job after his pediatrics residency was with the Baylor Pediatric AIDS Corps, where he spent time at a government hospital in Malawi helping to initiate antiretroviral therapy for HIV-infected children. But over the course of that four-year stint he became aware of an equally pressing problem: respiratory illnesses.

"Pneumonia alone is the leading infectious cause of death in children under 5 globally," he says. "Depending on where you look, up to one million children a year die from pneumonia, and 85-90% of them live either in Sub-Saharan Africa or in South Asia."

Children living in these areas are vulnerable to respiratory illnesses for several reasons. Malnutrition is a concern and contributes to immunosuppression,

so children have less ability to fight infection. In addition, McCollum says, "There's a lack of resources and trained health care personnel, and not a lot of investment into supportive care such as oxygen. When kids come into the hospital and need more than antibiotics, often they're not going to do well." Compounding the situation, nurse-to-

patient ratios at hospitals in Malawi can be as high as 1:40, so it's more difficult for them to monitor patients.

Now McCollum, who joined the Johns Hopkins faculty in 2015 after completing a pediatric pulmonary medicine fellowship there, spends his time trying to improve respiratory care for children in the nations where children are at the highest risk of pneumonia deaths. As a pediatric pulmonologist, he also sees patients at Johns Hopkins Children's Center several weeks each year.

Through one project in Nigeria, Africa's most populated nation, McCollum and international colleagues are performing a comprehensive study of pneumonia care. This includes providing pulse oximeters — medical devices that measure oxygen levels in the blood — and related training to about 30 primary care clinics. The goal is that providers can test children coming in with possible signs of pneumonia for hypoxemia (low oxygen in the blood), one of the most important markers for severe pneumonia, and quickly triage them to a hospital for oxygen treatment.

A series of papers on the research, co-funded by pharmaceutical Glaxo Smith-Kline and the international nonprofit Save the Children, was published in January in *Pediatric Pulmonology*. Key findings discovered by the group include an urgent need to train and mentor health care providers and provide community health education for caregivers.

Based in Lesotho, Africa, with his family, McCollum also has overseen projects

“Although the research projects don’t go on forever, they leave behind improved capacity to manage children with respiratory illnesses.”

— ERIC MCCOLLUM



Eric McCollum with physicians Brittany Hunter, left, and Chiara Bertolaso at Kamuzu Central Hospital in Lilongwe, Malawi, where they volunteered for one month to provide clinical care for hospitalized Malawian children.

Opioid Overdose Survivors Not Getting Timely Treatment

in Bangladesh examining pulse oximetry in children with pneumonia and studying the effectiveness of the pneumococcal vaccine, and in Malawi investigating bubble continuous positive airway pressure in children hospitalized with severe clinical pneumonia.

"I'm following a passion, so I'm grateful for that," he says. "Although the research projects don't go on forever, they leave behind improved capacity to manage children with respiratory illnesses."

McCollum also was part of an international team studying the effects of high altitude on respiratory rate and oxygen saturation in healthy children 0-23 months in India, Guatemala, Rwanda and Peru. Results, published in March in *The Lancet, Global Health*, suggested that higher altitudes can impact these values to the point that physicians who use the World Health Organization definitions for fast breathing could potentially misdiagnose pneumonia in children.

McCollum is preparing a webinar on childhood pneumonia in low-income and middle-income countries for the International Union Against Tuberculosis and Lung Disease to broadcast this summer. Additionally, he promotes his efforts through teaching. Two years ago, he started the Global Program in Pediatric Respiratory Sciences at Johns Hopkins, which brings residents to low-income and middle-income countries to help care for children and train health care providers. The program has hosted three pediatric residents from Johns Hopkins in Malawi, two of whom are now working in the hospital where McCollum started. 🍌



A STUDY of more than 4 million Medicaid claims records during a recent seven-year period concludes that less than a third of the nearly 3,800 U.S. adolescents and young adults who experienced a non-fatal opioid overdose got timely (within 30 days) follow-up addiction treatment to curb or prevent future misuse and reduce the risk of a second overdose. The analysis, led by researchers at Johns Hopkins Medicine, also found that only 1 in 54 — less than 2% — received standard-of-care counseling and medications recommended by the American Academy of Pediatrics (AAP) for treating opioid use disorder (*JAMA Pediatr.* 2020;174(3):e195183). "If 1 in 54 young people with asthma or diabetes failed to receive standard therapies for emergency situations with their diseases, we wouldn't accept it," says **Rachel Alinsky**, a pediatrician and adolescent medicine fellow at the Johns Hopkins Children's Center and lead researcher of the study. "Yet, this is where we are now with the treatment our system is able to provide to youths who have survived an opioid overdose — and we need to do better for them." — *Michael E. Newman* 🍌

Cats and Dogs and Schizophrenia



EVER SINCE humans domesticated the dog, the faithful, obedient and protective animal has provided its owner with companionship

and emotional well-being. Now Johns Hopkins researchers suggest that being around "man's best friend" from an early age may have a health benefit as well — lessening the chance of developing schizophrenia as an adult. "Serious psychiatric disorders have been associated with alterations in the immune system linked to environmental exposures in early life, and since household pets are often among the first things with which children have close contact, it was logical for us to explore the possibilities of a connection between the two," says pediatric neurovirologist and lead author **Robert Yolken**. In the study, Yolken and colleagues at Sheppard Pratt Health System in Baltimore investigated the relationship between exposure to a household pet cat or dog during the first 12 years of life and a later diagnosis of schizophrenia or bipolar disorder. For schizophrenia, the researchers were surprised to see a statistically significant decrease in the risk of a person developing the disorder if exposed to a dog early in life. Across the entire age range studied, there was no significant link between dogs and bipolar disorder, or between cats and either psychiatric disorder (*Plos One*, Dec. 2, 2019). The researchers caution that more studies are needed to confirm these findings, to search for the factors behind any strongly supported links, and to more precisely define the actual risks of developing psychiatric disorders from exposing children under age 13 to pet cats and dogs. — *MEN* 🍌

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people&philanthropy



A Special Bond Leads to a Professorship

By Karen Blum

AS A **TEENAGE PATIENT** in Johns Hopkins Hospital's Pediatric Intensive Care Unit, **Jackie Julio** was referred to pediatrician **Catherine "Cathy" DeAngelis**, someone she knew nothing about. Little could she imagine how dramatically that would change.

At the time, Julio, who has a rare neuromuscular disorder called Kugelberg Welander syndrome, had spent considerable time in and out of the hospital. Reviewing her medical records, DeAngelis noted that many of Julio's hospitalizations resulted from her becoming anxious and having difficulty breathing.

"I told her, I don't care what time of day or night it is: When you start to feel like this, call me and I will talk to you," DeAngelis recalls. So began what would become a decades-long friendship.

"When I would go to appointments at Johns Hopkins, you would hear [Cathy] coming down the hallway laughing, talking with people and smiling — she's always like that," Julio says. "I could talk to her about anything — boy stuff, worries or stressors, and she always had the time. I never felt rushed. If I had a cold, we would call her, and she would call me right back. She's amazing."

DeAngelis would go on to serve as vice dean for academic affairs and faculty at the Johns Hopkins University School of Medicine and become editor-in-chief of the *Journal of the American Medical Association* — the first woman to fill that role. Today, she is University Distinguished Service Professor Emerita, professor of pediatrics emerita at The Johns Hopkins University School of Medicine and professor of health policy and management at the Johns Hopkins Bloomberg School of Public Health.

In spite of these additional demands on her time, DeAngelis has remained concerned about Jackie Julio, who — having only minimal movement in her hands — attended college and law school, moved to California to pursue acting and then settled in Florida. Through it all, she maintained close contact with DeAngelis, who continues to offer medical advice and support.

In gratitude, Julio's family recently established the Catherine DeAngelis, M.D., and Jackie Julio Endowed Professorship.

"To have a professorship named for Jackie and me is something very special, because Jackie's very special," DeAn-

gelis says. "You know the word 'joy'? That best describes how I feel about this honor: Great joy on many levels."

The inaugural recipient of this professorship is **Barry Solomon**, professor of pediatrics and chief of the Division of General Pediatrics.

"I'm really honored to be the first recipient," says Solomon, who is also assistant dean for medical student affairs at Johns Hopkins. "I have spent much of my academic career thinking about how we can use primary care to help patients and families with their social and behavioral health needs."

As former medical director of the Harriet Lane Clinic, Solomon worked with Johns Hopkins colleagues, private foundations and community organizations to incorporate services such as on-site mental health care, parenting groups and safety resources. For nearly 15 years, a resource desk — now called Hopkins Community Connection and staffed primarily by Johns Hopkins undergraduates at Harriet Lane and two Johns Hopkins Bayview Medical Center clinics — continues to screen families for social needs, and connect them to local resources.

"It's very exciting to receive this professorship because not only can this help us move this work further; it can also help support trainees and junior faculty interested in reducing health disparities and promoting equity through innovations in primary care," says Solomon. He also notes that he's found himself inspired listening to DeAngelis share her life story in her audiobook, *Onward*, a reading of her memoir, *Pursuing Equity in Medicine: One Woman's Journey*. Through the professorship, says Julio, "I feel like our bond will go on. It's been a very special, loving relationship that started as doctor-patient and evolved into family." 🍷



Making Connections, Supporting the Community

By Amanda Leininger

“WOULD YOU LIKE HELP finding transportation to clinic appointments? Paying your utility bills? Feeding your family?” These might not be questions patients expect to be asked at a doctor’s visit, but thanks to Hopkins Community Connection (HCC), these questions are not only being asked, they’re a catalyst for overcoming obstacles.

Formerly known as Health Leads™, HCC works to identify and address patients’ essential social needs and connect them with the basic resources they need to be healthy. These social determinants of health — which can range from access to healthy food, safe housing and education to safe neighborhoods and freedom from discrimination — can directly impact patients’ health, putting them at greater risk for chronic illnesses like diabetes, cardiovascular disease and asthma.

“We really serve the whole health picture, not just of the patient, but of the family,” says **Kristin Topel**, Hopkins Community Connection’s program manager. “Families could have questions about their insurance, where to get food or how to get the lights back on. And, no matter who they ask, they’ll be connected to a resource to solve the problem.”

To that end, undergraduate students from The Johns Hopkins University volunteer their time to staff resource desks at the Harriet Lane Clinic, Johns Hopkins Bayview Children’s Medical Practice and Hopkins Bayview Comprehensive Care Practice. Students typically carry a caseload of about 10 families, with whom they personally follow up regularly to continually assess their needs.



“Our program is very trustworthy and it’s because of the incredible relationships our advocates are able to build with patients and their families,” says **Sarah Hill**, a program coordinator at the Harriet Lane Clinic, who joined HCC as an undergraduate volunteer.

What would happen to the 3,000+ families receiving HCC services each year if the program dissolved? That almost happened in June 2019, when a change in national funding resources threatened the future of HCC. Recognizing the potential distress to families, the team immediately sought private funding.

Enter the Straus Foundation, which supported the program at its inception nearly 14 years ago, under **Barry Solomon’s** leadership as the then-medical director of the Harriet Lane Clinic. The Straus Foundation aims to understand the barriers underserved populations face, and funds programs that help to overcome these obstacles, says Solomon, now chief of the Division of General Pediatrics. When he reached out to **Jan Rivitz**, the Foundation’s president, she knew they needed to help.

“We have this world-class institution sitting in one of the most under-resourced neighborhoods in the country, and any effort we can make to connect those two just makes sense,” Rivitz says. “We believe in taking the kind of calculated risks that others don’t. If philanthropy doesn’t, who will?”

Thanks to ongoing financial support from The Johns Hopkins Health System, with additional support from the Straus Foundation and other funders, including Rite Aid and Morgan Stanley, Hop-

In the Harriet Lane Clinic, Hopkins Community Connection staff members, from left, Ashely Skipper, Sarah Hill and Nicholas Machado, prepare packages of infant formulas and diapers for families in need.

kins Community Connection is funded through 2021 — and none too soon. While the program typically helps 400 families at a time, the needs exacerbated by the COVID-19 pandemic have skyrocketed enrollments. Currently, HCC is actively helping nearly 850 families to combat food insecurity and to provide access to basic necessities, such as infant formula and diapers.

“Our program offers a connection to those resources that families often don’t know they’re eligible for or may not know how to access,” Solomon says.

The HCC team has expanded to include community health workers and social work trainees, helping them to address more complex social needs and conduct home visits. HCC has also hired employment specialists who assist with resumes and job applications, and created a new on-site food assistance program supporting families and essential health care workers facing financial hardship.

“We really wouldn’t be operational without funders like the Straus Foundation,” says Hill. “It’s incredible to have people who recognize that this is impacting some of the most vulnerable families in Baltimore.” 🌟

To support the Hopkins Community Connection program, email hopkinschildrens@jhmi.edu.

Panda Express Rings a Bell for Patients

THE RINGING OF A BELL can symbolize many things: the passage of time, a celebration, a beginning or even an ending. At Johns Hopkins Children's Center, patients ring a bell to celebrate the completion of chemotherapy treatments. At Panda Express, associates ring a bell to celebrate every time a customer makes a donation. And when that bell rings at Panda Express, the associates cheer, remembering the patients they know they're helping.

Nationwide, Panda Express proudly supports Children's Miracle Network Hospitals — Johns Hopkins Children's Center is one — through its *Panda Cares* program. Locally, 15 locations (and counting) raise funds year-round that benefit the Children's Center through an employee giving campaign and by asking customers to donate. Children's Center leadership uses the funds raised wherever the need is greatest.

"As a company, we think that we have to do something in the community, especially for the children," says **Cristina**

Erika Ramos with the bell local Panda Express Associates ring when a customer donates to Johns Hopkins Children's Center.



Hernandez, northeast area coach of operations. "Our practice is that we ask every customer, it's part of our training. We want to give our customers the opportunity to give and help those kids who are really in need."

And this "ask every customer" mentality has proved to be successful. Over the last five years, Panda Express' fundraising has shown unparalleled growth locally, increasing from \$1,600 raised in 2015 to an impressive \$145,000 for the Children's Center in 2019. This wouldn't be possible without the encouragement and support of Panda Express leadership and management, who lead by example through their voluntary payroll donations.

"As a company, we think that we have to do something in the community, especially for the children. Our practice is that we ask every customer, it's part of our training."

— CRISTINA HERNANDEZ, NORTHEAST AREA COACH OF OPERATIONS, PANDA EXPRESS

"Caring and loving is part of our culture. We want to support everyone and want everyone's life to be better," Hernandez says. "We took the managers to Johns Hopkins, and they were able to share these experiences with their associates to help inspire them. The money doesn't go to medical bills, but to things like toys and playrooms. It really makes a difference for the kids in the hospital."

In a crucial time of need, Panda Express also stepped up to help those on the front line during the COVID-19 pandemic, donating 60,000 single-use surgical masks to The Johns Hopkins Hospital.

"We are so grateful to Panda Express, both our local stores and corporate leadership, for their never-ending support of our patients, families and care-providers," says Cynthia Palacz, senior associate director of development at the Children's Center. "It's reassuring that even in these unknown times, we can rely on our community to step up and make a difference."

—AL 🍜

For more information on community fundraising opportunities, contact hopkinschildrens@jhmi.edu.



The “Big Reveal” of \$1,423,688.31 for Johns Hopkins Children’s Center set a new record for Children’s Miracle Network Radiothons.

The Biggest Radiothon Ever

Mix 106.5’s annual event for patients at Johns Hopkins Children’s Center sets a national record in radiothon fundraising.

IT’S A BIRD, it’s a plane, it’s ... superheroes at Mix 106.5’s 31st Annual Radiothon! From February 20–21, Mix 106.5 DJs **Reagan Warfield**, **Bethany Linderman**, **Priestly**, **Corinna Delgado**, and **Maria Dennis** skillfully shared the powerful stories of Children’s Center patients, families and their care providers — real-life superheroes — live from the lobby of The Charlotte R. Bloomberg Children’s Center building.

As each Radiothon ambassador arrived, Mix talent presented them with a Children’s Center superhero cape before interviewing them on air. They shared stories of hope, healing and even heartbreaking loss with the Maryland community, asking listeners to donate by calling the phone bank staffed by volunteers — many of whom have a personal connection to the Children’s Center.

New this year, some friends of the Children’s Center received

“It’s just an awe-inspiring event. For two days, we get to share the stories of amazing kids and how they were helped by amazing people, and our listeners respond with such generosity.”

— REAGAN AND BETHANY

invitations to a private reception held in the ground-floor lobby, just one floor below the live broadcast. A livestream allowed attendees to view the festivities occurring during the last hour of the event while socializing with patients and their families, and faculty and staff members — including co-directors **Tina Cheng** and **David Hackam**.

After 27 hours of broadcasting, the DJs, volunteers, patients, their families and staff joined the reception downstairs to reveal the amount raised over the two days. Anticipation filled the air: This year’s event raised \$1,423,688.31 for the Children’s Center — setting a new record for the most funds raised by a single radio station in one year during a Children’s Miracle Network Hospitals Radiothon in the U.S.

“It’s just an awe-inspiring event. For two days, we get to share the stories of amazing kids and how they were helped by amazing people, and our listeners respond with such generosity. From the Mix and Hopkins teams to the volunteers, to the people behind each and every penny donated, it’s truly a community effort,” said Reagan and Bethany in a joint statement. “And the fact that Baltimore now holds the record for the single biggest Radiothon in the country? It’s thrilling to think about the real and many ways that money will make kids’ lives better.” —AL 🌟



Save the date for Mix 106.5’s 32nd annual Radiothon, February 25 and 26, 2021!

'Mo' Gaba, a Champion Among Champions

Fourteen-year-old Mosilla "Mo" Gaba is battling cancer for the fourth time at Johns Hopkins Children's Center, but he has never let his illness hold him back. Mo and his mom, Sony, first shared their story during the 2012 Mix 106.5 Radiothon broadcast while Mo was an inpatient undergoing treatment for retinoblastoma. Since then, Mo and Sony have continued to tell their story, stealing the hearts of the Baltimore community. Mo regularly calls Mix 106.5 to say hi to his DJ friends and their listeners, and as an avid sports fan, he can be heard frequently on 105.7 The Fan with his predictions and analyses. Also beloved by Ravens and Orioles players for his tenacity, sports knowledge and infamous laugh, Mo has become a local celebrity — making history in March 2019 as the first person to announce an NFL draft pick that was read in Braille.

In 2019, Children's Miracle Network Hospitals (CMNH) — of which Johns Hopkins Children's Center is a member hospital — selected Mo as one of 10 National Champions to represent their organization and children's hospitals for the upcoming year. Mo reflects on his experiences.

How did you find out you were a CMNH National Champion, representing Johns Hopkins Children's Center this year?

I think I was downtown with my mom when she got a phone call. She was really surprised. I didn't know how big of a deal it was, but I was excited when she told me because I love Johns Hopkins.

As a National Champion, what have you done?

I've done a lot of stuff to help people learn more about children's hospitals and why they're important. We got to travel to different states with the other National Champions. We met a lot of the Children's Miracle Network Hospitals leaders at their headquarters in Salt Lake City. My favorite was we got to go to Vegas. We spoke and shared our story

on stage with lots of people from the RE/MAX company. It was fun and a little overwhelming. The other champions are really nice. My mom didn't like the food in Vegas, though!

How does the experience make you feel?

It makes me feel really proud to be helping Johns Hopkins. If you do not give, children will not survive the diseases that they have. It makes it easier being in the hospital when people donate and help give us the stuff that we need to make it more fun. I want kids to know they should be brave, so maybe sharing my story will help other kids not be so scared.

What would you say about Johns Hopkins Children's Center?

My time at the hospital can be fun, but it can also be really hard because of the

medicines I take and the type of treatments I get. It can be really tiring. My mom is always there with me. Always. The nurses are the best. They're nice and they sometimes take time out of their day to hang out with me. That makes me feel special. This place is amazing, even though I wish I didn't have to come here anymore.

You're known for your incredible laugh. How do you stay so strong? And what would you say to people to keep them motivated?

The only thing I can say is stay positive. Don't be negative. Look on the bright side. Make sure to laugh a lot. When you laugh, others laugh too. And then everybody feels better.

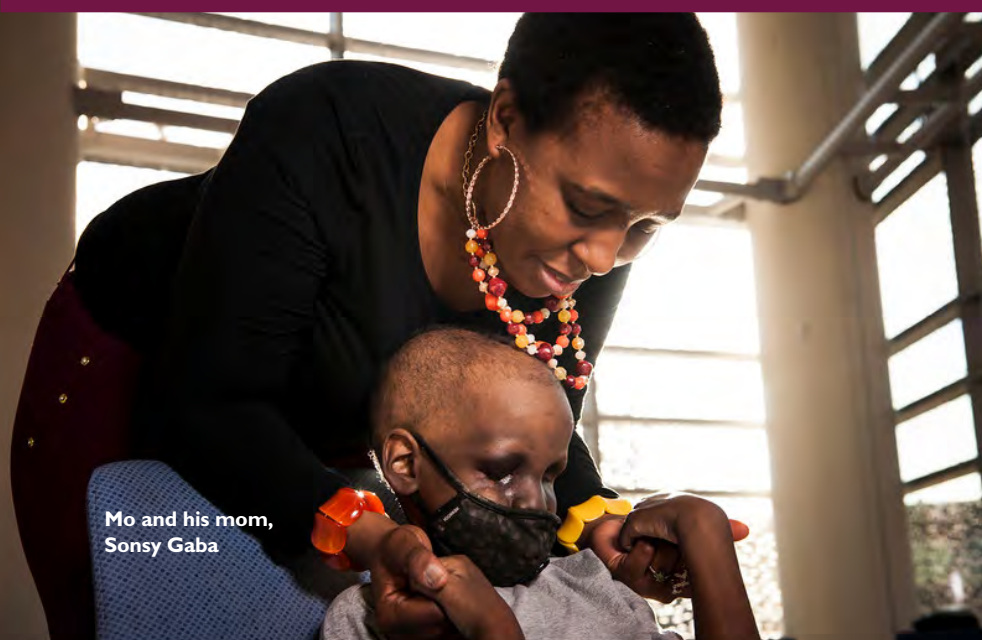
What is your favorite thing about Radiothon?

Everything! Just the whole thing. How can you choose? I really love coming back because I get to see the DJs and all the people who help take care of me, and I get to meet other kids like me. It's a big dance party, and I have the phone number to call memorized — I love saying it on air.

What do you want to do when you grow up?

I'm going to have my own sports radio station and be on the air. And of course, I'll have my own Radiothon. I mean what station wouldn't?! —AL 🌟

To read Mo's story, visit bit.ly/HopkinsKidMo.



Mo and his mom,
Sony Gaba

At the Helm: Mark Marcantano

By Gary Logan

WHEN ASKED ABOUT his own experiences in children's health care, **Mark Marcantano**, new chief administrative officer for Johns Hopkins Children's Center and administrator for the Department of Pediatrics, instead shifts the focus to his predecessor, **Ted Chambers**: "I have never had the gift of working with a prior incumbent before, particularly with someone who has so much institutional knowledge and cares about the place so much. He has been nothing but gracious, supportive, hard-working and a fabulous adviser to me in terms of understanding how things work, the key relationships critical in supporting the work of the Children's Center and how things need to be prioritized."

One immediately gets the sense that Marcantano is not only gracious himself, but also greatly values the input of others, especially when it's experiential. The Long Island native attended business school at New York University, followed by law school at Albany Law School of Union University, and began his own legal practice in environmental law.

A decade later, feeling a bit unfulfilled, he switched his mindset to health care. How and why?

The way he explains it, sometimes opportunities and epiphanies arise in life when you least expect them. "You never know who you're going to meet and who's watching your work," he says.

The person watching his work regarding a legal matter would go on to become the chairman of the board of Albany Medical Center. And, when he did, he called Marcantano and asked him to join his senior leadership team. The conversation, says Marcantano, was short: "I said thanks but no thanks. I didn't know anything about health care and medicine was never on my radar."

But the idea gave Marcantano pause. He was going through an introspective period, one in which he felt like he wanted to do something more fulfilling than making people financially successful. This opportunity, he realized, had fallen into his lap at exactly the right moment; he had to explore it. What he found resonated deeply.

"There was no better mission than supporting world-class health care delivery, training the next generation of the best and the brightest, and advancing discovery and innovation," says Marcantano. "So I jumped on it with a thirst and a hunger that was not easily rivaled."

“The complex challenges we face, the team dynamics, to be around brilliant high-achieving people who want to change the game for patients and advance medicine — that is all very energizing for me.”

Indeed, after departing Albany Medical Center as senior executive vice president and chief operating officer, he would go on to serve as vice president of ambulatory and network services at Boston Children's Hospital. Then he accepted the position of president and chief operating officer of Women & Infants Hospital in Providence, Rhode Island.

In so doing, he amassed a 23-year career as a senior executive health care leader during which he's seen it all — in both centralized and decentralized environments. Now he finds himself at Johns Hopkins, challenged, in his own words, by the "brilliant complexity" of the institution, which he sees as vital to providing the very best care.

"It's a good thing I like big, beautiful and messy, which is how a good academic medical center works," says Marcantano. "The complex challenges we face, the team dynamics, to be around brilliant high-achieving people who want to change the game for patients and advance medicine — that is all very energizing for me." 🍌



An Officer and a Doctor

WHEN PEDIATRIC cardiologist **Joel Brenner** began caring for 8-month-old **Christian Kurowski** in 1993, little did he know he would be facing him a quarter century later on the deck of the USS Constellation in Baltimore's Inner Harbor wearing a Navy uniform similar to the one he himself once wore as a naval medical officer. Kurowski had invited Brenner to the ceremony for his promotion to full lieutenant in gratitude for diagnosing his condition, steering him toward potentially life-saving surgery as an infant, and following up as his cardiologist over two decades.

"It's truly an honor to be here," Kurowski said. "I had open heart surgery when I was a baby and there was a strong chance I wasn't going to make it until Dr. Brenner got involved. So it's very appropriate for me to have Dr. Brenner here, who himself was a lieutenant in the Navy."

"I could never imagine this — it's the highlight of my career," Brenner said. "I have been to patients' weddings and funerals, it comes with the territory. But this is really special."

Partial anomalous pulmonary venous return, the condition Kurowski was born with, requires lifelong monitoring of any changes by a cardiologist. — GL 🌟



Phoebe Bacon, with a patient, says she feels lucky to have her job because not every children's hospital has a library.

Nurse Turned Hospital Librarian Takes Patients on a Literary Journey

By Allison Smith

STRESSFUL, SCARY AND ISOLATED are some of the ways a hospital stay can feel for a child. A hospital library, however, can provide comfort and a break from all the medical talk.

"Books take you to another world or to another idea or to another way of thinking about things," says **Phoebe Bacon**, the librarian at the Grace Rea Garrett Children's Library and Mr. & Mrs. G. Lloyd Bunting Sr. Family Resource Center at Johns Hopkins Children's Center.

Bacon began her career as a nurse, but often pondered what else she might want to do. A book lover, Bacon noticed patients glued to their screens, and this was almost 30 years ago. She thought, "There will be a day when no one wants to read."

This experience motivated her to leave the hospital and become an advocate for reading. Then, after a decade of working as a school librarian, she decided to combine her library degree and nostalgia for the medical setting. When the new Charlotte R. Bloomberg Children's Center building opened in 2012 complete with a library and librarian position, she snatched it up. "It feels like I've come full circle," says Bacon.

The attraction? She saw the need for

someone to encourage kids to enjoy reading even more while in a hospital. She believes books have the potential to reassure and add normalcy for a child recovering from a serious illness or injury — and to distract them from a long hospital stay. Whether she creates voices for characters or the "blipity-blopity-bloop" sound effect of a turkey rolling down a hill, Bacon is a storyteller who can transport any audience into any story she is reading.

"You have to be a little theatrical to be a librarian," Bacon notes.

Most of her hands-on work happens on the units, where Bacon rolls a cart of books for patients to check out during their stay. Also, she spends about an hour each week with patients from the psychiatry unit when they venture down to the library.

When children first walk in, they are welcomed by Bacon's engaging smile. They sit in a circle, introduce themselves and share what they want to explore that day. As the kids browse the shelves, Bacon glides around the room and helps each child find a book or activity that matches their interests. Her heart is particularly warmed when a patient has left the hospital and the book comes back with a note: "I loved this book." 🌟

Healing Arts

An artist and a musician help children in the hospital feel more like themselves.

By Helen Grafton

FORMAL MUSIC THERAPY programs for adults in the hospital have been shown to lower their blood pressure, relieve pain and reduce stress. Similarly, at Johns Hopkins Children's Center, musician-in-residence **Wendy Lanxner** and artist-in-residence **Aubrey Bodt** take an informal approach designed to normalize the hospital experience for children, and they achieve therapeutic effects as well. Both are part of a robust healing arts program that is offered by the Child Life department and began at the Children's Center in 2012.

On Fridays, Lanxner can be seen and heard wheeling her music cart — complete with a drum set, guitar, piano and two waterproof ukuleles — up and down the halls of the hospital. She travels to multiple units with her entourage of instruments to play music and sing with patients. She offers color-coded sheet music but also hopes to teach patients and their families how to read notation.

"If all goes well, hopefully they'll learn something, too," she says.

They do. She recalls recently finding a teenager alone in her room and offering her a private ukulele lesson.

"I taught her three chords, used in the song 'Just the Way You Are,'" says Lanxner. "She knew the song and was able to play the chords and strum with direction, using a visual aid of a large-font simple lead sheet with chord diagrams."

Whether it is singing acappella or learning a few chords on the ukulele, her goals are to give patients and families a distraction, a reason to be active, and most importantly, an opportunity to have some fun. Her repertoire includes classics such as "Twinkle, Twinkle, Little Star" and popular songs like "Old Town Road."

"Luckily, a lot of pop songs are based on the same four repeating chord patterns," she adds.

Patients of all ages seem to benefit from Lanxner's tunes. She caught the attention of a crying 6-month-old when she began to strum "Twinkle, Twinkle, Little Star" on the guitar in the middle of a hallway. She walked the family back to their room where she continued to play until the girl stopped crying.

"I learned that the father played guitar at home, so it was a familiar sound," Lanxner says.



Ukulele in hand, Wendy Lanxner recruits patients to take a musical break from their hospital stay.

Similarly, Aubrey Bodt uses art to reach patients in ways that typical therapy doesn't. Her goal? Help children feel more human during their hospital stay.

A typical day for Bodt starts with referrals from Child Life specialists — a list of patients they believe could benefit from her practice. From 9 a.m. to 4 p.m. on Thursdays, she travels across the Children's Center, creating watercolor valentines or "thankful" mobiles to hang from the ceilings of patient rooms. When she first meets patients, she asks what types of activities they enjoy at home. The patient gets to choose the art — there is a different project in each room. Bodt also allows the patient to drive the interaction. She's just there to facilitate.

"The main goal is to make sure that they have a choice in what they do," Bodt says. "I might be the only person they get to say 'no' to today."

Having experienced the value of the healing arts program, both Lanxner and Bodt would like to see it expand and represent a broad range of artists-in-residence.

"It has so much potential to heal and grow," she says. "We all have different roles to meet and serve." 🌱

The healing arts program exists thanks to the generosity of donors. For more information on how you can support Child Life services at Johns Hopkins Children's Center, please email hopkinschildrens@jhmi.edu.

Honors & Awards



Pediatric nephrologist **Alicia Neu**, has been appointed Chair for Pediatrics at Johns Hopkins Bayview Medical Center. Among Neu's achievements, she established the only outpatient hemodialysis unit exclusively for children in the state of Maryland, expanded pediatric nephrology clinical services to include outreach clinics in Annapolis, Frederick, Bethesda and Towson. Under her guidance, the outcomes for children receiving dialysis and/or kidney transplant at Johns Hopkins are now among the best in the nation. She will remain Division Director of Pediatric Nephrology in the Department of Pediatrics.



Barry Solomon has been appointed the new chief for the Division of General Pediatrics. For over a decade, as Medical Director of the Harriet Lane Clinic, Solomon developed numerous innovative programs to address the social determinants of health, including integrated behavioral health services for children, adolescents and mothers. He also conducts research on injury prevention and mental health integration in pediatric primary care. He has been the Principal Investigator on numerous NIH and foundation funded studies.



Pediatrician **Hoover Adger** has received the Arnold P. Gold Humanism in Medicine Award from the Association of American Medical Colleges (AAMC). The AAMC noted that Adger, who has focused his research, medical practice, and educational efforts on adolescent health and substance use disorders, "Employs a patient-centered, family-first approach that has transformed the lives of his patients while modeling humanistic care for his students."



Assistant nurse manager **Philomena Costabile** received the Elsie Peyton Jarvis Start Nurse Award at the Johns Hopkins School of Nursing's annual Evening with the Stars event Nov. 2, 2019. The night honored excellence in nursing care and innovation, and the profession's commitment to health and well-being for all people.



Pediatrician **Megan Tschudy** received an AcademyHealth Nemours Child Health Services Research Award from AcademyHealth. The national award recognizes an early-career investigator in the field of child health services, particularly someone doing research on quality improvement of pediatric health services. Tschudy's research interests include community integrated health care redesign, improving the quality of the family-centered medical home, home visitation and implementing and evaluating innovative medical education curricula.



Pediatrician **Sarah Polk** has been selected as one of 35 fellows for the Robert Wood Johnson Foundation Clinical Scholars Program. Clinical scholars fellows are recognized for their leadership skills and collaborate on a project to address complex health problems, allowing them to gain new perspectives and expertise. Polk, whose clinical expertise includes pediatrics and adolescent medicine with a focus on mental health and sexually transmitted infections in adolescents, was recognized for her contribution on a project involving immigrant Latina mothers and their risk for depression and unmet social needs.



Pediatric surgeon **Alejandro Garcia** has been named a Jay Grosfeld, M.D., scholar by the American Pediatric Surgical Association Foundation. Garcia will receive \$25,000 for his research titled "Improving Reperfusion Injury in Pediatric Cardiac Arrest Using a Porcine Model of ECPR."



Pediatric residency program manager **Kathryn "Kathy" Mainhart** is the 2020 recipient of the Association of Pediatric Program Directors (APPD) Carol Berkowitz Award in recognition of her extraordinary and innovative contributions of advocacy and leadership in pediatric medical education. In receiving the award, Mainhart said, "What an incredible privilege it has been to work with so many Harriet Lane residents, chief residents and alumni over the past 25 years. You are the ones who give meaning to my work every day and have filled my life with so much joy."

Teens Grow through Giving Back

SIX YEARS AGO, we launched the Teen and Children's Council (TACC), an advisory board comprising patients or siblings of patients who have been treated at Johns Hopkins Children's Center. What began as something small has continued to grow and flourish. Somewhere along the way, we realized we were not only helping these teens find a voice; we were also fostering their talents as young leaders. How?

First off, our teens have a passion for giving back. They participate in the planning and execution of two large events — Baltimore Boogie and Prom. Baltimore Boogie, the first dance marathon for patients by patients, has raised more than \$100,000 over a three-year-period to support impactful programs. And, each spring the teens help to create a magical event for our current and past patients: the Children's Center Prom.

Why do our teens participate in the council? And what have they learned

about themselves in the process?

"TACC has helped me share my voice and story and speak out when I wanted change," says member **Kiara Burke**.

"Through TACC, I have learned how to tell my medical story. I am proud of who I am and who I have become, despite having a long medical history," adds **Julia Mead**. "I love being an advocate for pediatric patients because most of the time, adults don't understand what it feels like."

"My participation has led me to think deeper about my own experience," says **Thomas Saacks**. "To offer advice to other patients, I needed to better understand how my own experience shaped me."

"Participation in TACC has made me branch out more and become less shy," notes **Maci Janiski**. "When I taught the dance for Baltimore Boogie, I was so scared! But our council leaders helped me become less shy."



by Sue Mead,
Parent Advisor

"I enjoy working on the council because I have been in and out of this hospital countless times throughout my life," says **Trey Price**. "The doctors and nurses here saved my life over and over during the past 20 years. This is how I can give back."


We're only just beginning to appreciate the value of TACC, and it makes us proud to see these teenagers move forward with their lives. One of the teens recently ended his participation because he's heading to college in the fall. A note we received from his grateful father, **Phil Whitworth**, offers a testament to the council's impact: *As a father, I am so thankful that you all asked him to be part of the group. He has been unusually diligent in making the very long trek up there and back for the monthly meetings and events. This has been invaluable in helping him understand the need to uphold commitments, even if it seems at times inconvenient.*

Adds another TACC parent, **Ronda Janiski**, "Listening to others has influenced my daughter to be more compassionate towards others. Because of this experience, she hopes to pursue a career in health care once she graduates from high school." 🌟

TACC members, from left to right:
Sloane Huey, Victoria Everett, Kiara Burke, Jared Daniels, Maci Janiski, Julia Mead, PJ Whitworth



If you know a teen who would like to be a part of TACC, please email us at TACC@jhmi.edu.



For their future, and yours.

There are many ways to support Johns Hopkins Children's Center, options that benefit you and your family, too. A **charitable gift annuity** provides you or a loved one with guaranteed income and tax benefits — and helps us continue to transform young lives through the best pediatric treatment and care.

Melissa, with her great-granddaughters, Elle and Emme, patients at Johns Hopkins Children's Center

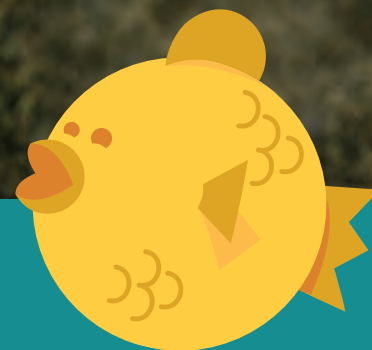
Read their story at bit.ly/JHCCEmmeElle

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800-548-1268

Email: giftplanning@jhu.edu

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JOHNS HOPKINS
CHILDREN'S CENTER

Hopkins Children's
Office of Communications & Public Affairs
901 S. Bond Street / Suite 550
Baltimore, MD 21231

The Charlotte R. Bloomberg Children's Center building

- Opened in 2012
- 205 private rooms with sleeping accommodations for parents
- Acoustical ceiling tiles and rubber flooring help create a quiet healing environment
- Supersized sculptures and literary themed art distract from the hospital experience
- Family amenities like on-demand meals, family lounges and multiple play rooms
- Expanded, easy-to-access Pediatric Emergency Department
- Dedicated pediatric trauma bays
- 40-bed Pediatric ICU
- 45-bed Neonatal ICU
- 10 state-of-the-art pediatric surgical suites

