

Dream Grow Live

the University
of Florida
Department of
Pediatrics



Children deserve
the best...



A chance to dream, grow and live



that's why we're here.



In a perfect world...



Children wouldn't get cancer. Tiny lungs would always be strong and full of breath. And young hearts would never break.

Our biggest worry would be fending off the imaginary monsters in our children's closets.

But unfortunately, in our world, monsters such as cancer, diabetes and heart disease are real.

When kids get sick, they deserve the best that health care can offer — the best treatments, the best doctors and the best researchers searching for new medicines and cures.

That's why so many parents bring their children to us.

With some of the country's leading specialists and researchers, the UF Department of Pediatrics is focused on bringing the latest research and most innovative treatments to patients. Families come from across the state and country for the type of care

offered here. UF&Shands, the University of Florida Academic Health Center, is home to one of only a few centers for proton beam radiation in the country, a congenital heart disease center, a Diabetes Center of Excellence and experts in glycogen storage disease, pediatric surgery and neonatology, to name a few.

U.S. News & World Report ranks our hospital, Shands Hospital for Children, in six medical specialties, including diabetes and endocrinology, nephrology, pulmonology, neurology and neurosurgery, and cardiology and heart surgery. Our diabetes and endocrinology and gastroenterology programs were ranked higher than any other program in Florida. We took another step forward in 2011 with the opening of our Pediatric Emergency Room, which was the first step in a planned renovation of the Shands Hospital for Children.

In the following pages, you will meet a few of our amazing patients and the people who make UF Pediatrics a national leader in children's health. I am happy to introduce them to you.

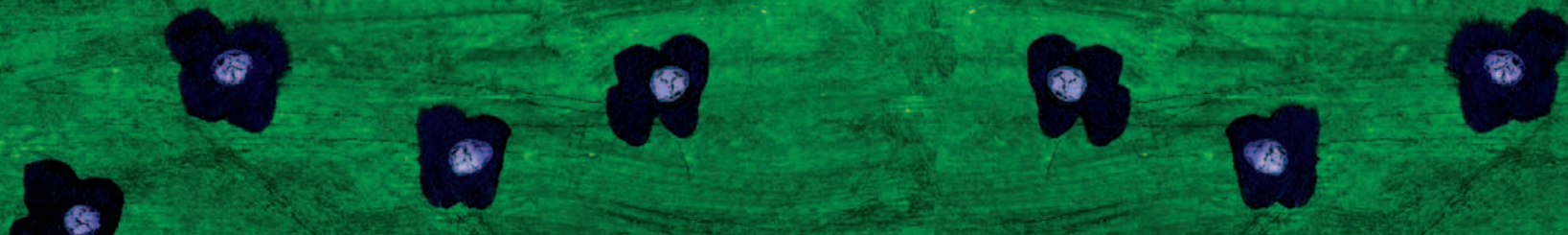
But a piece of the puzzle is missing.

UF&Shands needs funds for pediatric programs and education, new equipment and endowed professorships, which give researchers more time to focus on research or teaching, laying the foundation for advances in children's health care. And most importantly, we must continue to improve the facilities where our smallest patients go for their care, where the doctors understand their physical ailments and realize growing up is hard enough to do without worrying about a health problem.

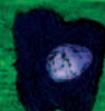
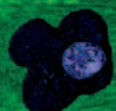
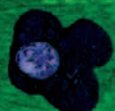
Some needs just shouldn't wait.

It's not a perfect world, but with your help, we can make it better.

Thank you,
Scott A. Rivkees, M.D.
Chairman and Professor
Physician-in-Chief, Shands Hospital for Children



Children's
Hospital





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David's last seizure



David Coron has been seizure-free since his surgery at UF

Story by Cindy Spence

At 10:15 on the evening of April 26, 2005, an epileptic seizure overpowered 8-year-old David Coron. As he came out of it, he grabbed paper and a pen.

His father, Jonathan, held him from behind to steady him. His mother, Annemieke, watched as David tried to draw the chaos in his brain. When he dropped the paper and pen, there, in black and white, was a picture of David's epileptic seizure.

"He started drawing and wouldn't stop," Annemieke Pronker-Coron says. "It was like he had to do this drawing. His language skills were not very good, so we felt like he was trying to tell us, by way of the drawing, what he experienced."

It was David's last seizure before checking in to Shands at UF the following month. There, after his neurologist pinpointed what part of his brain was causing the seizures, David underwent surgery to control his epilepsy. He has been seizure-free since then.

The surgery is considered a success when it reduces seizures by 60 percent, so David's experience has been amazing, says Dr. Paul Carney, a UF pediatric neurologist.

But his journey, Annemieke says, was arduous. David's first seizure occurred when he was just 3. Although there were long intermissions between them, the seizures were lengthy and terrifying. David took increasingly more medication as the years went by, but still the seizures came. Soon, he was having seizures every other week.

Doctors told them surgery would be David's best chance for a life without seizures, but the decision was difficult.

To find the spot responsible for disrupting the normal electrical activity of his brain, David's electrodes would be placed directly on the surface of his brain. He would have to remain hospitalized in that state, waiting for his next seizure, so the electrodes could pinpoint its origin.

"Always, with every decision that is difficult, you have to think, 'what if you don't do it?'" Annemieke says. "If we didn't, then seizures would always be a part of his life, and we didn't want that."

So the Coron family moved into Shands. Annemieke, a violinist, played music. David covered the walls — literally top to bottom — with artwork. A friend brought in a milkweed plant with butterflies on it, and David watched as they transformed from egg to caterpillar to chrysalis to butterfly.

"We created a room like no other, the three of us, and made

"One day we'll deliver therapy with a chip, like a pacemaker. We won't have to do surgery."

— Paul Carney, M.D.

the room our house," Annemieke says. "I would wave Jonathan out as he left for work in the morning, down the corridor, like I would wave him out at home."

Nurse practitioner Donna Lilly credits the family's creativity with easing David's fears. But David faced another complication. Patients normally have to wait, electrodes in place, for 10 days to three weeks. As the six-week mark approached, the medical team worried about how long David had been waiting with the electrodes on the surface of his brain.

"We talked about removing the electrodes, not doing the surgery," Carney says. "The next day he had a seizure."

Carney mapped the location of the seizures and a neurosurgeon removed a piece of David's brain.

Seizures are only one of the problems children with epilepsy face, Carney says. Often, children struggle with learning and behavioral problems too. Relieving the seizures allows the family to focus more on those issues, he says.


"We need to get the word out that there really is hope for these children," Carney says. "We are developing new techniques for prevention, control and diagnosis. One day we'll deliver therapy with a chip, like a pacemaker. We won't have to do surgery."

Needles figure prominently in David's memories of the hospital, but he feels better these days.

"They stuck me a little bit and put something on my head," says David, now 10. "Then they took me to Dr. Carney. Then I was well again. I could sleep. I could eat."

The Corons believe the surgery removed a roadblock from David's life.

"The likelihood is we're drawing a winning card here," Annemieke says. "Now it's up to us, and David."



More than 2 million people in the United States have epilepsy, a disease that usually strikes first in childhood and lasts a lifetime. As part of one of the South's largest pediatric epilepsy programs, UF pediatric neurologists are expert in epilepsy surgery, diagnosis, nerve stimulation and other therapies. Researchers are studying potential cures, such as gene therapy, and neuroprosthetic devices to control seizures — a UF-developed pacemaker already stops seizures before they happen. Along every step, UF faculty members partner with families to ensure treatments make as much sense in the real world as they do in the lab. The division is also home to North Florida's only learning disorders clinic, and to clinical and research programs for sleep disorders, neuromuscular diseases, cerebral palsy, movement disorders and other neurological conditions.

Neurology



Pediatrics People



Nicole Paradise Black

Overseeing the education of 50 individuals is a hefty but rewarding task, says Dr. Nicole Paradise Black, residency program director for the UF Department of Pediatrics. "It's not just about training the next batch of top-notch pediatricians, it's about making sure their well-being is taken care of," Black says. Residents in the pediatrics residency program come from across the nation and world, and Black is in charge of their education, keeping the program up-to-date, innovative and exciting. As a pediatric hospitalist, she teaches residents in the hospital and is heavily involved with education at the national level, as well. Black recently collaborated with other educational leaders across the country and crafted an award-winning national pediatric nighttime curriculum for residency training. However, her first priority is always her residents. "It's what I love doing," Black says. "We're training pediatricians of the future."

General Pediatrics



UF's primary care pediatricians ensure children stay healthy year to year by providing medical homes that oversee their care. Six nurse practitioners and 21 physicians make up UF's general pediatrics division, one of the leading divisions of its kind in the country. The division offers a variety of services that keep children and families happy and healthy, including adolescent medicine, a newborn nursery, development and behavior pediatrics, and the PED-I-CARE program, which helps families manage the complexities of caring for a child with special needs. The division also is home to the UF Child Protection Team, which provides medical evaluation in cases of abuse and neglect. In addition, the division's researchers are working to understand children's medical conditions and ways to improve children's health.



Sanjeev Tuli

For Dr. Sanjeev Tuli, it's all about the children. As chief of the division of general pediatrics, Tuli is well known for his interest and expertise in pediatric dermatology, allergy, asthma and creating and managing a medical home for children with special health care needs. He also mentors and educates medical students and pediatric residents. "We're making sure they get the best education possible to become the best pediatricians they can be," Tuli says. An advocate for children, Tuli serves on the American Academy of Pediatrics' National Committee on Coding and Nomenclature. He also serves on the AAP's Coding Publications Editorial Advisory Board and as the AAP "CPT Alternate Advisor" to the American Medical Association. In this role, he helps review other national organizations' code change proposals for potential impacts to pediatric medicine and shepherds academy-sponsored proposals through the code development process.







tiny. survivor



Bella Pannett is 4, thanks to UF doctors

Story by April Frawley Birdwell

Something was wrong with her baby. Lisa Pannett knew it.

Sixteen weeks pregnant with her second child, Pannett lay still while the ultrasound technician studied the swirling black and white image on the screen. The same tech had performed her ultrasound with her first daughter just two years earlier. They had laughed and smiled together as the technician pointed to the throbbing little heartbeat. Now, the technician was quiet.

"It was what she wasn't saying," remembers Pannett, who lives in St. Louis. "I knew something was wrong."

Pannett's doctor confirmed her fears. The baby, a girl, had a rare defect known as congenital diaphragmatic hernia — a hole in her diaphragm allowing her abdominal organs to move into her chest and

blocking her lungs from developing properly. The doctor said Pannett and her husband had two options: deliver a baby that would not survive or abort.

"I probably cried for like 10 minutes," Pannett says. "And then I just started asking him questions. His answer was that she had a zero percent chance of living."

But Pannett couldn't accept that. Determined to try to save her daughter, she scoured the Internet. There, on a Web site started by parents who came to UF for treatment, she found Dr. David Kays, a UF pediatric surgeon who specializes in treating children born with CDH.

"We have a fairly severe patient population," Kays says. "People tend to come here after being told their child will die. There is a huge number of these children that are terminated. I have had families call as they were driving to their (scheduled) termination."

Because of the gentler methods Kays and his team use to treat CDH, babies with the condition were thriving at Shands at UF. Babies with CDH born at Shands at UF had a 92 percent survival rate, compared to about 50 percent at other centers.

The difference at UF is the overall approach to caring for the babies, Kays says. UF doctors coax air into babies' lungs after birth using gentle ventilation techniques. Surgery to correct the diaphragm defect is often delayed while their tiny lungs gain strength. Often, the children are so ill they need a form of heart-lung bypass geared specifically for babies, too.

And because Kays sees about 25 CDH cases per year, a large number for a defect so rare, the doctors and nurses involved have become adept at caring for children with the disease, he says.

Pannett and her husband flew to Gainesville to meet with Kays. While touring the hospital, the couple met one of Kays' patients, a baby with CDH. Pannett knew then coming to UF was her daughter's best chance.

"I just knew it was a done deal," she says. "It's hard to put

into words how I felt. Everybody had told me these kids don't live."

Pannett made arrangements to give birth at Shands, which would allow doctors to care for the baby immediately afterward.

Several weeks after Pannett moved into a temporary apartment in Gainesville, her daughter, Bella, was born. With only 5 percent of her diaphragm intact, she was placed on a ventilator.

"They didn't want us to touch her because her heart rate would escalate," Pannett says.

Kays operated two weeks later, placing an artificial abdominal patch in Bella to keep her organs in place — her stomach and liver were where her lungs should have been. After that, she progressed gradually. She had to be fed through a feeding tube for a while and she had up and down days, but after two months in the hospital, Bella went home to St. Louis.

Now 4, Bella is like most other little girls her age. She wears her blonde hair in pigtails, plays pirates with her older sister Alex and recites her ABCs. Children with CDH are often susceptible to learning disabilities, but Pannett has seen none of that in Bella.

"I really feel like (coming to UF) was God-sent," Pannett says. "Now it seems surreal, like somebody else went through that."

"Everybody had told me
these kids don't live."

— Lisa Pannett

Surgery

The first of only two programs in the state certified to train pediatric surgeons, UF's division of pediatric surgery is staffed by surgeons who are regional and national leaders. One of the nation's foremost experts on congenital diaphragmatic hernia heads the division. UF pediatric surgeons have wide-ranging expertise in many areas, including fetal anomalies, gastric and intestinal dysmotility, inflammatory bowel disease, neonatal intestinal inflammation, pediatric trauma and minimally invasive surgery. Our pediatric surgeons emphasize patient care first and foremost, but also take seriously the task of advancing their specialty for the benefit of their patients. And by training new surgeons in the UF mold, they are ensuring that future patients here and across the country can benefit from the same expertise, advances in research and caring manner.



Pediatrics People



Jay Fricker

Dr. Jay Fricker is about getting results — life-changing, long-lasting results. “We have a saying at the Congenital Heart Center,” he says. “Our success grows up.” Fricker has led pediatric cardiology at the University of Florida since his arrival in 1995. His dream was to develop a Congenital Heart Center at Shands at UF to provide the most advanced cardiovascular care to children and adults with congenital heart disease. Fricker serves as medical director of the UF Congenital Heart Center and specializes in the nonsurgical aspects of pediatric heart and lung transplantation and the treatment of pulmonary hypertension. His research has focused on immune-suppression strategies after heart transplantation in children. “We always have something to offer children with congenital heart disease,” he said. “With some, you can repair their hearts and offer them long-term survival with a great quality of life.”

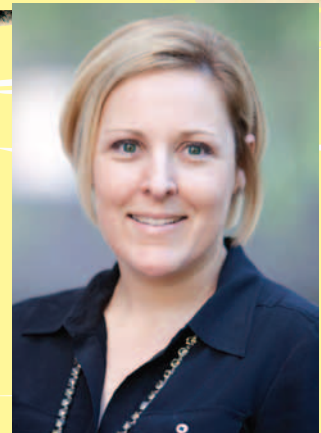
Critical care

In the Pediatric Intensive Care Unit, doctors, nurses and therapists care for some of the hospital's most fragile patients. The needs of children who are critically ill or injured are many and complex, and it is the job of the pediatric critical care team to ensure these needs are met. The PICU provides 24-hour care by specialists trained in pediatric critical care medicine in the setting of a 24-bed multidisciplinary medical/surgical ICU. Critical care physicians direct and coordinate care for each patient. The PICU admits over 1,300 patients per year and accepts referrals from all over the state of Florida and southern Georgia. The PICU provides highly specialized care to patients with organ failure, respiratory failure, congenital heart disease and shock, as well as those who have received heart, kidney, lung or liver transplants. Advanced care, such as dialysis and ECMO, are also offered in the PICU. The pediatric critical care team of doctors, nurses and therapists is here around-the-clock, ready to support children and their families with compassion and clinical excellence.



Shelley Wells Collins

After spending a few years as a private practice pediatrician, Dr. Shelley Wells Collins returned to the place she loved most as a medical student and resident — the hospital. “I get to see my patients get better,” says Collins, who worked as a hospitalist at Children’s National Medical Center in Washington, D.C., before coming to UF in 2006. “They come in to the hospital sick, and we help them get better. It is very rewarding.” In 2009, after working as a hospitalist in the UF Pediatric Intensive Care Unit and the Congenital Heart Center, Collins helped establish the Division of Pediatric Hospital Medicine. As chief, she leads a team of physicians dedicated to providing coordinated and consistent inpatient care. She has been honored for three years in a row as a College of Medicine Exemplary Teacher and works with Dr. Barry Byrne on discoveries and clinical trials for patients with Pompe disease.





the sun will COME OUT ...



Addylen Reeder loves bubbles, singing and is fighting Pompe disease

Story by April Frawley Birdwell

The words were a whisper at first, but she was singing.

It's a moment etched in Jeff Reeder's mind. Watching the movie "Annie" in her hospital room, his then 17-month-old little girl, Addylen, sang for the first time, echoing the words to the song "Tomorrow." It was a few days after doctors had performed a tracheostomy, cutting a small hole in her neck so that she could be placed on a ventilator to help her breathe.

As a baby, Addylen was small, always weighing in at the bottom of the growth chart. But she met most developmental milestones until she was about 5 months old. She had trouble swallowing and grew increasingly lethargic, so Jeff and his wife, Kendra, took Addylen to a children's hospital near their home in Hebron, Ky. There, a genetic test uncovered the problem. At just 6 months old, Addylen was diagnosed with an extremely rare form of muscular dystrophy called Pompe disease. Left untreated, babies born with this inherited condition often die before they reach age 2.

"It was devastating because our daughter is our life," Jeff says. "We knew the severity of the disease. It was probably the worst feeling anyone could possibly have."

People with Pompe disease are born with a faulty gene that prevents their bodies from producing an enzyme called acid alpha-glucosidase, which is necessary for processing glucose. Without this enzyme, stored sugar builds up in the muscles, causing them to degenerate. As the disease wears on, patients often develop an enlarged heart and liver and their breathing worsens, requiring the use of a ventilator.

After meeting other patients and families online, Addylen's family found Dr. Barry Byrne, director of the Powell Gene Therapy Center at UF and a clinician-scientist who specializes in treating patients with Pompe disease.

"He was the first practitioner we came across that she was not just another patient to him," Jeff says. "Dr. Byrne does what he is doing because he truly cares. He wants to do the best in his ability to save the lives of children. He has made himself open and available to ensure Addylen's needs are met."

Until enzyme therapy was approved for use in 2006, there was no effective treatment for Pompe disease. Enzyme therapy, which Addylen takes, slows the progression of the disease, but other treatments are needed to help patients recover lost function. In addition, enzyme therapy does not

help with aspects of the disease that affect the brain and spinal cord, which play a role in a patient's need to be on a ventilator, Byrne says.

In addition to her enzyme therapy, Addylen will soon begin a gene

therapy trial at UF, led by Byrne and a dedicated research team. The trial involves injecting a corrective gene to help restore respiratory function in patients with Pompe disease. The hope is that this will allow patients like Addylen to come off the ventilator.

"None of the patients who have ever begun assisted ventilation have ever returned to fully independent breathing," Byrne says. "It is a lifelong commitment."

Because of her condition, Addylen's mobility is limited. She cannot walk or crawl and can only sit up by herself for 20 to 30 minutes at a time, but she can talk and sing and is into everything going on around her, Jeff says. She loves bubbles, the Backyardigans and has already earned the nickname "Addy-tude."

"She likes everything for five minutes," her dad says with a laugh. "Addy is very busy. If you are not grabbing what she wants to play with in time, she lets you know about it. She is a typical 2-and-a-half-year-old. She's full of love."

"Dr. Byrne does what he is doing because he truly cares. He wants to do the best in his ability to save the lives of children." — Jeff Reeder

Not every disease is easy to understand. Some genetic conditions stump even the savviest doctors and specialists. These are the cases geneticists try to solve. Unlike other pediatric specialists, UF's pediatric geneticists don't see the same types of patients each day. Their work is more akin to deciphering a genetic puzzle to discover what is plaguing a child, and it often takes hours of research before clues are uncovered. UF's pediatric geneticists and genetic counselors see patients from across Florida — either in Gainesville, in rural clinics or during telemedicine clinics, which allow patients in other areas to meet with doctors without traveling. They also assist doctors across the state who have unusual cases and aren't sure how to handle them. Genetics faculty members also help patients with rare metabolic diseases, such as phenylketonuria and oversee a UF molecular chromosome lab.



Genetics

Pediatrics People



Arun Srivastava

Scientists used to joke that AAV stood for “almost a virus,” remembers Arun Srivastava, Ph.D. Adeno-associated virus is generally harmless, so many researchers didn't see a reason to study it. UF researchers saw it differently, says Srivastava, who worked in UF researcher Kenneth Berns' lab as a postdoctoral associate in the 1980s. They were right. Because the virus is common and harmless, researchers began using it as a vector for gene therapy. Now, it's considered one of the safest ways to transport corrective genes that could cure diseases into people. UF researchers are studying possible cures for hemophilia and for a form of blindness, both delivered by AAV. After studying AAV his entire career, Srivastava came back to UF in 2004 to lead the department of pediatrics' cellular and molecular therapy division. Here, he and other researchers are using 10 different forms of AAV in their efforts to develop gene therapies for a variety of diseases. “Now, the new name for AAV is ‘an awesome vector,’” Srivastava says.

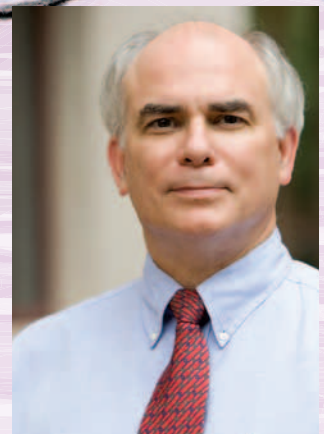
Cellular and molecular therapy

One day, curing hemophilia or rare diseases such as glycogen storage disease could be as simple as getting a shot in the doctor's office. By injecting a corrective gene into patients, doctors can cure diseases and prevent new ones from beginning. UF is one of the country's leading institutions for the study of gene therapy using a harmless virus called adeno-associated virus, which acts as a shuttle in the body to take the gene where it needs to go. Researchers in the division of cellular and molecular therapy are focused on examining possible delivery vehicles for gene therapy, including AAV, and how the body responds to them. The division's scientists are using what they learn to develop potential gene-therapy-based treatments for pediatric liver cancer, hemophilia, brain tumors and certain forms of blindness. But gene therapy isn't the only weapon these UF researchers have for taking on disease. They're also looking at gum and mouth spray as possible ways to administer a hormone that curbs human appetite and might help fight obesity.



Daniel Driscoll

Padlocked refrigerators. Insatiable hunger. Morbid obesity. Welcome to Prader-Willi syndrome. As the director of one of the largest Prader-Willi syndrome research and treatment programs in the country, UF medical geneticist Dr. Daniel Driscoll has spent his career studying this condition. He developed the most widely used genetic test to accurately diagnose the disorder, and his research has led to breakthroughs in scientists' understanding of both Prader-Willi and Angelman syndromes, which both involve a genetic glitch on chromosome 15. Aside from helping children and adults with these conditions, Driscoll and his team of researchers also are using their studies to gain a better understanding of obesity in general.





Cancer warrior kid

Tyler Kleine is on the road to recovery from T-cell lymphoma

Story by April Frawley Birdwell

Tyler was breathing funny. His mom, Angela Kleine, noticed it. It was April 15, 2010. Then 8, Tyler had been playing with some other kids at church the night before when someone accidentally hit him in the throat with a football. Worried something might be wrong with Tyler's airway, Kleine brought her son to the doctor.

The football had not done any damage, but the CT scan revealed something much more menacing — a mass in his throat. The Ocala family was rushed to Shands Hospital for Children, where additional scans showed a mass stretching from his throat to his diaphragm, 10 inches across his small chest.

The next day Tyler was diagnosed with T-cell lymphoma, a form of blood cancer, and started chemotherapy immediately.

"Life as we knew it on April 16, 2010 ended then," Kleine says. "Everything changed. Everything stopped and centered on getting Tyler the treatment he needed and getting him to his appointments."

Tyler often had to be in the hospital four days out of the week at the beginning of his treatment. With these new responsibilities and two other children at home, Angela quit her job and took on a new role as a "cancer warrior mom."

They enlisted the help of Dr. William Slayton, chief of hematology and oncology in the UF Department of Pediatrics and an expert on pediatric blood cancers who oversees Tyler's care.

The past two years have been fraught with roadblocks, namely the 16 days Tyler spent in the hospital in December 2010 through Christmas and New Year's Day, when he developed a blood clot in his leg and was battling sepsis. He also lost 30 pounds in the first months of his treatment.

But every roadblock has had its counterpoint. During that stay in the hospital in 2010, the Kleines met another family whose daughter was diagnosed with the same disease as Tyler on Christmas Eve. The two families are close now. Also, Tyler began working with a UF physical therapist to help him regain his strength, and he has been able to attend camps, go on a Make-A-Wish Foundation trip with his family and meet many UF athletes.

"It's been hard, but fun, too," Tyler says.

Angela credits the staff who work at UF&Shands for her son's positive attitude.

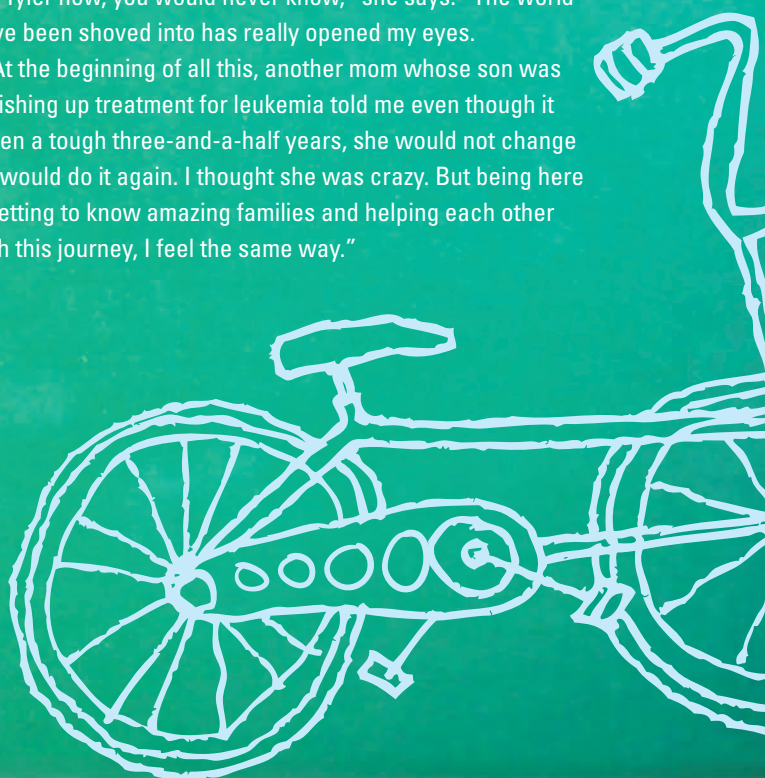
"Never once has he complained to come to chemo," she says. "He looks forward to coming here, because everyone is so good to him. As his mom, that means the world to me."

Two years after he was diagnosed with cancer, Tyler is still undergoing long-term chemotherapy. He takes a chemo pill every day and comes to the hospital once a month for an infusion.

An active kid, Tyler is back to playing basketball and riding his bike, but he still gets winded because of his chemo. In August, his chemo will end and he will be able to delve back into sports. Life will be more normal than it has been in a long time. But Angela knows it will never be the same, and she is OK with that. In fact, she wants to continue doing what she can to raise awareness about childhood cancer and help other families who are walking the same journey.

"People are so unaware of childhood cancer. Even if you look at Tyler now, you would never know," she says. "The world we have been shoved into has really opened my eyes."

"At the beginning of all this, another mom whose son was just finishing up treatment for leukemia told me even though it had been a tough three-and-a-half years, she would not change it. She would do it again. I thought she was crazy. But being here now, getting to know amazing families and helping each other through this journey, I feel the same way."



Hematology and oncology



Cancer. It's not a word most parents want to hear, but more than 10,000 children are diagnosed with the disease each year, according to the American Cancer Society. As the largest pediatric hematology and oncology program in the state, the division of hematology and oncology has specialists who are skilled in caring for all forms of cancer — even those that have resisted treatment — and blood diseases, such as sickle cell anemia. The division has burgeoning clinical and research programs for blood cancers, bone tumors and stem cell transplantation and an early phase clinical trials program to provide cutting-edge treatments for children with cancer in the state. Patients also have access to the latest innovations at UF. At the UF Proton Therapy Institute in Jacksonville, patients can undergo a targeted form of radiation that blasts the tumor without damaging as many nearby healthy cells. This is particularly beneficial for children, who are more susceptible to radiation. UF experts are also focused on taking care of the emotional needs of children dealing with cancer and the longterm health needs of those who survive it.

Pediatrics People



David Weinstein

Only about one in 100,000 children has glycogen storage disease, a condition that occurs when children are born with a faulty gene that prevents their bodies from releasing glucose from the liver between meals. As a result, fasting causes severe hypoglycemia, which can lead to brain damage, seizures and even death when not properly treated. Dr. David Weinstein oversees the largest program in the world for the liver forms of glycogen storage disease. In 2005, Dr. David Weinstein moved his entire clinical practice and research program to the UF Department of Pediatrics. Under Weinstein's direction, UF's program has sparked new discoveries and treatments. In 2010, he and colleagues reported success using gene therapy to reverse the condition in a dog naturally born with the disease, and a new treatment for the condition was introduced in 2012 from work performed at UF. Patients come from 48 states and 31 countries for Weinstein's expertise and to participate in research studies that could lead to a cure.

Immunology, rheumatology and infectious disease



Shands at UF is one of the few places where children can undergo bone marrow transplant to treat primary immunodeficiencies. But that's not the only thing that makes the division of immunology, rheumatology and infectious diseases so unique to UF. With specialists who are expert in all facets of immunology and rheumatology, a pediatric HIV clinic and faculty who hold outreach clinics across the state, the division is able to offer the most innovative treatments to help children with complex diseases. For example, UF pediatric rheumatologists use chemotherapy to treat autoimmune diseases, such as juvenile arthritis, lupus and vasculitis. "These are complex cases," says Dr. Melissa Elder, chief of the division. "It's not just joint pain and infections. It's everything ... Who else would take care of these kids?"

William Slayton

Leukemia is one of the most curable childhood cancers, but sometimes even a cure comes with a price tag. The chemical assassins doctors use to target hard-to-kill leukemia cells destroy healthy tissue too, says Dr. William Slayton, a UF pediatric oncologist and chief of the division of hematology and oncology. "Kids have strokes, hip replacements," Slayton says. "With better treatments, these horrible side effects could be eliminated." The answer could be how leukemia forms new blood vessels — ones that point straight to cancer cells, he says. In his lab, Slayton studies how blood-producing stem cells spur blood vessel growth and how some leukemia cells mimic this. He also studies and treats hard-to-cure leukemias, such as infant leukemia, which is linked to a cellular glitch that increases production of a blood vessel-causing protein. "If you target the ability to make blood vessels, you might be able to improve the killing of leukemia cells," Slayton says.





Fixing a broken heart



William Chavez is thriving since surgery to repair a congenital heart defect

Story by Cindy Spence

Like many mothers with uncanny intuition, Karen Thurston Chavez had a gut feeling something was terribly wrong with her baby boy.

William had been treated for stridor and pneumonia, asthma and a heart murmur, and one respiratory infection after another. He had been tested for cystic fibrosis. She had been told to take him out of day care. But Chavez sensed the cause of William's problems had not been identified and knew he would not get well until it was.

"He was sick all the time," says Chavez, who had become an Internet research junkie, subscribing to medical journals in search of information.

In 2005, still seeking answers for their 15-month-old boy, the Chavez family traveled from Tallahassee to Gainesville to see UF cardiologists.

"Within 24 hours of getting to Gainesville, we knew more about William's health and his heart and his lungs than in the previous year and a half,"

Chavez says. "It was scary, but we were comforted that the doctors had a name for it and had seen it before and treated it before.

"It was the first time that we felt like William was going to be better."

The name for William's condition: partial anomalous pulmonary venous return, or PAPVR. In PAPVR patients, one or more of the pulmonary veins is not connected to the heart in the right place. The blood flowing into the heart is supposed to go into the left atrium. William had two misplaced veins, sending half his blood into the right atrium of his heart.

The condition had slowed William's growth. Because he was so small, the Chavez family prepared to wait until William was at least 4 before surgeons would repair his heart.

That fall, Dr. Mark Bleiweis, a pediatric cardiovascular surgeon, became the director of the Congenital Heart Center at UF. When William's case came up for review the following January, Chavez remembers getting a Saturday morning call. The doctors were ready; was the family?

"I researched Dr. Bleiweis, and I thought, 'This guy looks so young. What does he know?'" Chavez says. "But we were so reassured and comforted when we met with him. And more research showed he was way ahead of the curve in congenital heart surgery and treatment. There was no question he would do the surgery."

Bleiweis trained with one of the world's most renowned congenital heart surgeons and came to UF with vast experience operating on smaller babies. His philosophy, he says, is to repair the heart as soon as possible to give the child a better start in life.

"If you leave the abnormality in the heart, that continues to have other effects on heart development, lung development and brain development," Bleiweis says. "If you operate on smaller babies earlier in the course of their disease, you can prevent problems they might experience later. We can do this sooner, and it's better for the child."

"At Shands, there is compassion and professionalism, not just for the child, but for the family... the experience there is better than you ever think a hospital experience could be."

- Karen Thurston Chavez

William had the operation in June 2006 at the age of 2. Within months he started preschool and gained almost 4 pounds, weighing in at a "whopping 25 ¾ pounds."

"I can tell a difference," Chavez says. "Before, there was a subtle, intangible level of constant agitation. Since the surgery, there's a sort of calm about him. He's more comfortable, not struggling to catch his breath. He can play all day."

Bleiweis says he is glad he could reassure the Chavez family about the surgery.

"I tried to go through the procedure carefully with them, describe the problem, describe the options and answer their questions," Bleiweis says. "I wasn't going to operate and walk away."

Although the drive from Tallahassee to Gainesville is long, Chavez says she'll do it every week if need be.

"At Shands, there is compassion and professionalism, not just for the child, but for the family," she says. "From admissions to surgery to discharge, the clinics, the ICU, the experience there is better than you ever think a hospital experience could be."



Congenital Heart Center

About one in 100 babies are born with a congenital heart defect, making it the most common birth defect in the United States. The UF Congenital Heart Center is a nationally and internationally recognized center of excellence for providing the most advanced care to pediatric and adult patients with congenital heart disease. U.S. News & World Report has ranked the Congenital Heart Center among the Top 50 pediatric cardiology and heart surgery centers in the country. As one of the largest pediatric transplant programs in the nation, the center exceeds expected and national transplant survival rates, according to a recent report from the Scientific Registry of Transplant Recipients. In 2006, the center was the first in Florida to use the Berlin Heart, a ventricular-assist device for children. The center provides a multidisciplinary approach to care, bringing together the expertise of pediatric cardiology and cardiovascular surgery, including interventional and diagnostic cardiac catheterization, invasive electrophysiology, echocardiography, valve repair/replacement and heart transplantation.

Pediatrics People

Michael Haller



Dr. Michael Haller and the entire UF diabetes research team are dedicated to improving the lives of children with Type 1 diabetes. Studies performed at the UF Diabetes Center of Excellence recently showed that a combination of two FDA-approved drugs can reverse Type 1 diabetes in more than 85 percent of mice. Dr. Haller and his team are now performing a clinical trial to determine if these drugs (Neulasta and Thymoglobulin) can preserve the capacity to produce insulin in patients who have been diagnosed with Type 1 diabetes for as long as two years. "Preliminary data from this combination therapy approach are very encouraging," he says. "We are excited to complete enrollment in this trial and have plans to soon expand combination therapy approaches to patients with recently diagnosed and long-standing Type 1 diabetes." An associate professor of pediatrics, Dr. Haller received the JDRF Mary Tyler Moore and S. Robert Levine Award for Excellence in Clinical Research in 2008 and the International Society for Pediatric and Adolescent Diabetes Young Investigator Award in 2011.

Some books weigh more than the typical patient in the Donald V. Eitzman Regional Neonatal Intensive Care Unit in Shands Hospital for Children. UF neonatologists care for the hospital's smallest and most medically complex patients. Caring for premature babies requires advanced tools, such as neonatal ventilators geared to supply the tiniest breaths to babies and equipment for mechanical heart and lung support. With experts in neonatal brain injury, nutrition, resuscitation, exposure to drugs and epidemiology, UF neonatology faculty not only offer specialized care, they are leaders in research. Faculty members in the division are studying the effects of cooling therapy on babies born with brain injuries, the benefits of breastmilk for premature infants and the causes of and prevention strategies for necrotizing enterocolitis, one of the most challenging complications that premature babies face. In addition, faculty members in the division have extended their expertise outside the hospital walls through two unique programs. The Florida Neonatal Neurologic Network was established to help babies throughout Florida born with brain damage, and the Center for Breastfeeding and Newborns assists new mothers with successful breastfeeding.

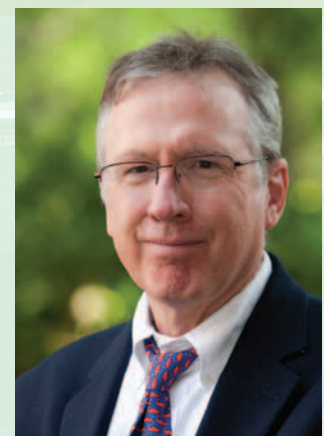


Neonatology



David Burchfield

More than 4 million health care providers in the United States have been trained in neonatal resuscitation, a fact that has likely saved the lives of countless newborn babies. In 1988, Dr. David Burchfield was a member of the first national American Academy of Pediatrics steering committee focused on neonatal resuscitation, making recommendations and devising how to train people to do it. In the years since then, Burchfield and other neonatologists and researchers have built upon those early recommendations and have studied the best ways to resuscitate the tiniest patients. "It really educated physicians and nurses in evidence-based practice, and it has been fantastic to watch that evolution, going from best opinion to best evidence," says Burchfield. Now chief of the neonatology division, Burchfield is proud of his faculty's accomplishments and the fellows his division has trained. He also serves on the AAP Perinatal Section and was recently elected to chair the group.







Lights, camera, action

Sydney Thomas loves acting, playing sports and her UF doctors

Story by April Frawley Birdwell

There are two types of toddlers: The mysterious calm ones who like to sit quietly ... and the 99.9 percent who don't stop moving until every toy, twig and piece of fuzz on the ground has been explored. At 18 months old, Sydney Thomas definitely fell in the latter category.

So her grandmother and mother knew something was not right when the little girl spent the day lying on the couch. There were other symptoms, too. She had been really thirsty, and her diapers were unusually wet.

"I realized how serious it was when the doctor cleared the waiting room," says Nicole Thomas, Sydney's mother. "They sent us to a pediatric E.R., and the doctor in charge took one look at her and knew what was wrong. Her eyes were beginning to roll up in her head."

Sydney was diagnosed with Type 1 diabetes, an autoimmune condition that prevents the pancreas from producing enough insulin. Nearly 15,000 children are diagnosed with the disease in the United States each year, according to the National Institute of Diabetes and Digestive and Kidney Diseases.

Not even sure what questions to ask doctors, Thomas immediately revved into research mode, devouring information about the disease.

"At this stage of the game, I still thought I could conquer diabetes," Thomas says. "At some point, I realized if we can manage it and hit her (blood sugar) target most of the time, it will be OK."

Six months after Sydney's diagnosis, she attended a conference held by JDRF, a national foundation devoted to Type 1 diabetes. There she met a researcher who referred her to Dr.

Desmond Schatz, medical director of the UF Diabetes Center of Excellence. She liked the thought of Sydney coming to a place focused not only on treating patients, but also on uncovering more about the disease and developing and testing the latest treatments. Thomas began making the two-hour drive north from Orlando every few months so Sydney could get her care at UF.

The Diabetes Center of Excellence gives parents a crash course on taking care of a child with diabetes, and doctors

keep close tabs on their patients between appointments. Thomas spoke to Schatz every week through email and sent him color-coded charts to show how they were managing Sydney's blood sugar levels. She also switched from insulin injections to an insulin pump, a device that looks a lot like a pager and shoots a

steady stream of insulin to the body through a narrow tube.

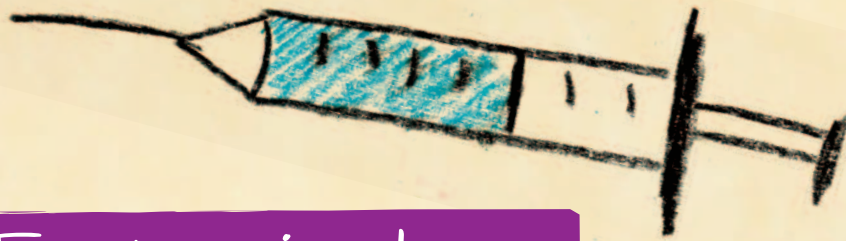
Now 8, Sydney works to keep her diabetes under control, with the help of her mom and Schatz. The responsibility for managing the disease is shifting to the second-grader, too, says Thomas.

"During our last appointment, Dr. Schatz talked to Sydney about her responsibility and 90 percent of the appointment was between him and her," she says. "I liked that. He, as a physician, understands this is the child's responsibility, too. I love that about him."

Sydney can give herself insulin, check her own blood sugar and count her carbs, but other than that, she is a regular kid. She loves acting in plays — and has been in several community theater productions — learning the piano, singing and playing sports. And like other kids, she angles for her share of sugary sweets.

"Our current battle is Fun Dip," Thomas says. "It's a give and take. One thing I remember Dr. Schatz saying is 'This is a kid first, diabetes comes second.' It has always stuck with me."

"One thing I remember Dr. Schatz saying is 'This is a kid first, diabetes comes second.' It has always stuck with me." — Nicole Thomas



Endocrinology



With internationally acclaimed programs in diabetes, growth, glycogen storage disease and Prader-Willi syndrome, the division of pediatric endocrinology attracts patients to Gainesville from 49 states and 32 countries. These programs not only attract patients for clinical care, but also are world leaders in research and innovative technology. Researchers in UF's Diabetes Center of Excellence have created tests to identify patients at risk for diabetes to try and prevent it, and trials are underway for reversing new-onset diabetes. UF's pediatric endocrinology experts have also devised new treatments for children with glycogen storage disease and Prader-Willi syndrome. The work does not stop in the clinic or the lab, either. The division has been active in creating opportunities to improve quality of life for children with chronic disease by helping to establish camps for children with diabetes and telemedicine clinics for people who lack immediate access to health care.

Nephrology

Kidneys are the silent workhorses of the body. They filter out waste products and regulate the chemistries of the body, hormones and blood pressure, either alone or in concert with other organs. When a child's kidneys go awry, UF pediatric nephrologists step in to find a solution. Our pediatric nephrology team specializes in caring for a variety of kidney diseases, offering the latest advances and technology to diagnose and treat kidney ailments. Pediatric nephrologists care for children with kidney infections, high blood pressure and renal stones as well as children whose kidneys no longer work and require dialysis or a kidney transplant. Faculty members also conduct research to improve kidney transplant outcomes, study growth and development in children whose kidneys have failed and have developed tools to be able to accurately diagnose specific types of kidney diseases.



Pediatric Residency Program

They're preparing to take care of the children of tomorrow. The University of Florida Pediatric Residency Program includes 50 bright, energetic individuals from across the nation and world, working to take excellent care of patients, says Dr. Nicole Paradise Black, the residency program director. "They are team players, have a very can-do attitude and also experience a lot of camaraderie and fun outside of the hospital," Black says. The Pediatric Residency Program is an academically rigorous program, with a comprehensive education curriculum that allows residents to learn about general pediatrics as well as many rare diseases. About half of the residents who graduate become general pediatricians, while the other half train to become pediatric sub-specialists.



Hospital Medicine



In the hospital, sick children need a constant presence — physicians who understand the complexities of caring for acutely ill children and can coordinate all the specialists and providers involved in their care. This is what the Division of Hospital Medicine provides to patients at Shands Hospital for Children. Pediatric hospitalists work closely with pediatric sub-specialists and patients' primary providers to bring coordinated, comprehensive care to patients in the hospital. Because pediatric hospitalists practice only in the hospital, they are available to reevaluate a patient's condition often and ensure timely testing, treatment and discharge. "Because our outpatient counterparts are so good at what they do, we don't see that many mildly ill patients anymore. The children who come here are pretty sick," says Dr. Shelley Wells Collins, chief of the division. "It makes sense to have a team of doctors dedicated to inpatient care." Physicians in the division are dedicated to providing the safest and highest quality of care to patients by staying current on medical innovations and serving as physician-leaders in the hospital and educators for medical students and residents.



Gastroenterology

Sometimes children won't eat, tummies ache for unexplainable reasons and livers don't function properly. That's when pediatricians seek the expertise of UF pediatric gastroenterologists. With one of the country's leading experts on pediatric hepatitis B and C, a feeding aversion clinic and faculty who are expert in a range of gastroenterological conditions including inflammatory bowel disease, UF's pediatric gastroenterology program is one of the most experienced in the state. The division is the highest ranked pediatric gastroenterology program in the state, according to U.S. News & World Report. UF is also one of only two centers for childhood liver transplantation in Florida. Like most pediatric specialties, UF pediatric gastroenterologists and hepatologists see a wide range of patients, from those with general stomach problems to children needing more intensive care. Several members of the team are also involved in research, such as clinical trials on hepatitis B and C treatment and inflammatory bowel disease treatment. With one of three pediatric gastroenterology fellowships in the state, UF is also training the next generation of gastroenterologists.

For kids with lung disease, playing tag or even sleeping soundly can be impossible tasks. Lung disorders and breathing problems are the leading causes of death in infants under 1, and chronic pulmonary conditions such as asthma, cystic fibrosis, interstitial lung disease and sleep disorders disrupt the lives of many children. UF's pediatric pulmonology division is nationally recognized as a leader in patient and family care, education and clinical research for children with lung disease. And it's the only center in Florida to offer lung transplants for children. Because so many patients with breathing problems have other health issues too, collaboration is key for UF pulmonologists. Working with specialists and general pediatricians in other divisions, UF pulmonologists find solutions for patients with complex conditions. And to help the families of children with lung diseases, the division has a family advisory board to offer emotional support. Researchers in the division are also focused on defining better treatments for diseases such as asthma and cystic fibrosis through clinical research.



Pulmonology







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