UCSF Benioff Children's Hospitals
Oakland | San Francisco

A NEWSLETTER FOR
FRIENDS AND SUPPORTERS

Connections

FALL 2019

Found in Translation: Leaping Over Language Barriers to Care

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Born with No Immune System

Although she seemed healthy, baby Riley had a rare and potentially fatal condition. With our expert care, she's now a thriving first-grader.

5

Advancing Diabetes Treatment

Diabetes affects more than 200,000 kids and teens in the United States, and the number is rising. Our experts are making an impact.

7

Finding Answers in Frog Brains

Using gene-editing technology, a UCSF researcher alters frog brains to improve understanding of how autism develops.

Message from **Dr. Michael Anderson, President**

We hold our mission sacred: to serve every child in need. It's a steadfast commitment that's clear in so many ways throughout our hospitals – and in the pages that follow.

From immune disorders (page 3) to diabetes (page 5) to autism (page 7), we deliver comprehensive, patient-centered care for children today, and we advance new research and therapies to move medicine forward for future generations.

Given our diverse community and our dedication to promoting health equity, we're also committed to ensuring that families that speak languages other than English feel empowered and engaged throughout their hospital experience. Every day, our hard-working, highly trained interpreters partner with clinical staff to help give families a voice in their child's care (page 9).

And we're proud to say that this care is world-class. In the most recent U.S. News & World Report survey, we again ranked among the nation's preeminent children's hospitals in all 10 pediatric specialties.

This achievement would not be possible without the generous support of community members like you. Time and again, I'm inspired by the champions for children who come together to help us deliver on our mission.

With gratitude,

Michael Anderson, MD, MBA, FAAP, FCCM
President, UCSF Benioff Children's Hospitals
Healthy Children … Healthy Communities … Healthy World

NOAH BERGER

Game On

Daniela's leg prosthetic doesn't hold her back. Thanks to UCSF's expert care, the 12-year-old can run, swim, ride a scooter, and play basketball, as she did at a special camp organized by the UCSF Amputee Comprehensive Training Program. "When I meet other people like me, it inspires me," Daniela says.

PATIENT STORY

Making Her Mark

Riley's life was saved by our experts.
Now her mom is giving back through photography.

THE MARK MAKERS

Alissa McDonald was settling into her hectic new reality as a mom of two when she got the phone call that changed her life. Baby Riley's routine newborn screening had come back positive for Severe Combined Immunodeficiency (SCID), often called "Bubble Boy Disease."

Though she seemed fine, the 3-week-old lacked a functional immune system, making her highly vulnerable to infections. "She looked perfectly healthy," Alissa recalls. "We had no idea."

Luckily for Riley, a national leader in SCID research and treatment was close to home: UCSF Benioff Children's Hospital San Francisco. UCSF's Dr. Jennifer Puck created the screening test that caught Riley's SCID, and Dr. Morton Cowan is leading the development of a novel gene-therapy cure.

These experts had to move quickly to save Riley's life. She would first undergo grueling chemotherapy followed by a bone marrow transplant when she was just 3 months old. Then she would remain in isolation as her body created an immune system.

> **It was amazing to see how invested the team was, not just in the modern medicine that they've advanced but also in watching it work for kids.**
>
> – Alissa,
> Riley's mother

"It was amazing to see how invested the team was, not just in the modern medicine that they've advanced but also in watching it work for kids," Alissa says.

For months, the hospital was Riley's home, and the staff – from nurse Francis "the Ninja," who could start a blood transfusion without waking anyone, to Dr. Chris "the Cheerleader" Dvorak, whose contagious optimism gave them reason to hope – became like family.

"I don't even know how it's possible to have that many amazing humans in one place," Alissa says.

When Riley was 6 months old, the family got fantastic news: Her new stem cells, donated by Alissa, had done their job. Riley had a working immune system. "It was just incredible," Alissa says. "She could finally live like a normal kid."

After overcoming some life-threatening setbacks – all expertly dealt with by her UCSF team – Riley, now 6, has grown into a wise-beyond-her-years social butterfly with a special appreciation for getting to play with friends and scarf down burgers like any other first grader. And with Riley's health stable, Alissa is giving back in a way that honors her family's hospital experience.


MEG PEROTTI

As an infant, Riley was diagnosed with a life-threatening disease (above). Now she's a thriving 6-year-old (opposite).

When Riley was in the intensive care unit, a photographer friend of Alissa's stopped by and did an impromptu photo shoot. "My memories of that time are so fuzzy, but those photos show me that there actually were beautiful moments," Alissa says.

A professional photographer herself, Alissa has made it her mission to capture those same moments for other parents. Through her nonprofit organization The Mark Makers, she photographs families in the hospital at no cost to them. In the past two years, she's seen it all, from parents getting the glorious news that their child is cured to an infant drawing his last breaths.

"It's heartbreaking, but to give somebody that – to let them see their love – is a gift. I know that will be a tool for their healing," she says. "And in turn, that heals me."

She also finds another source of strength: Riley. "This girl can handle anything because she's been doing this literally her whole life," Alissa says. "Any resilience I have comes from her."

ASK THE EXPERTS

More than 200,000 kids in the U.S. live with diabetes.

From studying the cells that drive disease to the eating habits that put kids at risk, how are our experts advancing the field?

Q. & answers

Maya Lodish, MD, MHSc

Chief, Division of Pediatric Endocrinology

Technology advances provide young people with the flexibility to manage their blood sugar without multiple injections or finger sticks, giving them the freedom to just be kids. Yet low-income African American and Latino youth are less likely to have access to this technology.

I am committed to addressing these kinds of health-equity issues. We're working to provide support and interventions for our most vulnerable patients. We believe that improving type 1 patients' access to insulin pumps and glucose sensors, along with community outreach, will lead to improved health outcomes and reduced hospital admissions and emergency visits. We're preparing to launch a study to test that hypothesis.

Stephen Gitelman, MD

Director, Pediatric Diabetes Program

Type 1 diabetes is one of the most common chronic diseases of childhood, and very difficult to manage clinically. After decades of research, we finally discovered a way to predict who will develop the disease and to delay its onset. Our multi-center clinical trial found that a two-week course of an experimental immunotherapy developed by UCSF's Jeff Bluestone dramatically reduced diagnosis rates in children and adults at high risk for the disease.

Median time to diagnosis was delayed by two years, and some study participants have been followed for over 7 years and have still not developed disease. Moving forward, I'll be leading several clinical trials to test new combinations of drugs that may be even more effective.

June Tester, MD, MPH

Pediatrician

My research focuses on improving nutrition in low-income children at risk of chronic diseases that are affected by diet. As part of my "Food as Medicine" research, I have studied the effects of providing "food prescriptions" to families of children at risk for diabetes. These children and their families received weekly boxes of fresh produce and whole grains, with access to healthy recipes and cooking classes.

Parents in the study who had diabetes improved their blood sugar, and children improved what they ate, particularly when it came to whole grains. It's one thing for a doctor to say "You should eat more vegetables and whole grains – it's good for you." But it's another thing to actually prescribe healthy foods in the same way you would medicine. It makes a difference.

Suneil K. Koliwad, MD, PhD

Associate Professor, Diabetes Center

We recently discovered a molecular signature that defines insulin-producing pancreatic beta cells during infancy. As babies grow, their beta cells transform and start functioning like those of mature adults. We found in mice that this transformation is naturally triggered by weaning from mother's milk. Remarkably, we learned that beta cells of individuals with type 2 diabetes revert back to the immature, infantile state.

This is problematic, as immature beta cells make too much insulin when we are not eating but respond sluggishly to meals. That leads to an unhealthy rise in blood sugar, particularly after eating. Our next step is to figure out exactly what flips this switch and leverage that to find new therapies for diabetes.

Finding Answers in Frog Brains

A New Approach to Decoding Autism


HELEN WILLSEY

Colorful imaging helps Willsey assess the results of her work. This tadpole's brain and neurons are shown in green, muscle in red, and DNA in blue.

Helen Willsey, PhD, is unlocking a new understanding of autism. And she's doing it with frog brains.

First, Willsey gives frogs the human pregnancy hormone known as human chorionic gonadotropin, which causes them to produce thousands of embryos at once. Then she takes each frog embryo and, at the stage when it's only two cells, mutates genes in one of those cells. The mutated cell develops into one half of the frog, and the unchanged cell develops into the other.

In this way, Willsey says, "you can alter the genes of half of the frog's brain, leaving the other side untouched."

This helps scientists understand how gene mutations affect the brain during development. "By comparing the altered half of the brain to the other, fully functional half, you can pick up subtle differences," Willsey explains.

Specifically, she wants to know how certain mutations might cause symptoms of autism. Many of these mutations were discovered by Matthew State, MD, PhD, chair of the UCSF Department of Psychiatry and the Oberndorf Family Distinguished Professor, whose work inspired Willsey to join his lab in 2016 as a postdoctoral fellow.

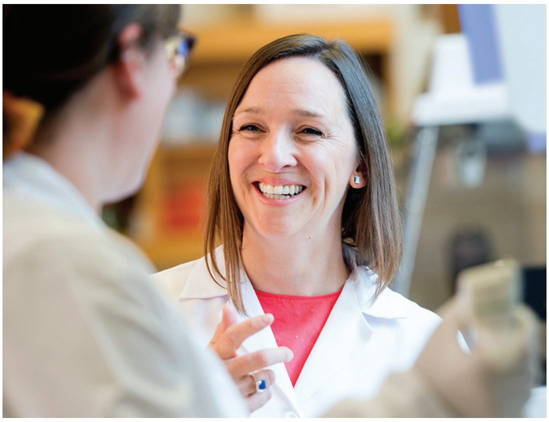
To mutate her frogs' genes, Willsey uses the revolutionary CRISPR-Cas9 DNA-editing tool. "This is powerful technology," she says. "It enables us to study many genes at once, whereas historically, you could only study one at a time."

Willsey has found that the gene mutations she imposes on her frogs take effect quickly. "Six days into development – the equivalent of about 25 weeks for a human – we see differences in the size of the frogs' forebrains," she says. The forebrain is responsible for social intelligence and higher-order thinking and learning – areas where people with autism tend to struggle.

The ultimate goal of this work is to illuminate how the mutations that State has helped identify lead to autism. "This is the essential step required to really understand what's going on in autism and eventually find drugs to reverse it," Willsey says.

Why You Should Rethink Your Gut Instinct

Tiny organisms inside us have a big impact


BARBARA RIES

Could the trillions of microbes that live in and on our bodies be the piece of the biomedicine puzzle we've been missing?

The role of the human microbiome – the vast ecosystem of bacteria, viruses, fungi, and other infinitesimally small organisms that live on our skin and throughout our bodies – is one of the great, unexplored frontiers of human biology.

Paving the Way

Scientists still have much to learn, but there is increasing focus on how an individual's microbiome can be altered to improve health. Susan Lynch, PhD, head of the UCSF Benioff Center for Microbiome Medicine, is at the forefront of this emerging field.

"We thought for a long time that microbes in the gut simply help us digest foods," Lynch says. "We're now beginning to understand how microbial activities in the gut and elsewhere influence cell types and ultimately the health of the host."

The organisms and genes present or absent in any one human microbial community are influenced by a multitude of factors, including the way we were birthed, where we live, the pets we own, the air we breathe, and the food we eat.

"Babies are born with an extremely simple microbiome," explains Lynch. "But by age 3, healthy children have cultivated a community that is as diverse as that of an adult."

Preventing Disease

Lynch posits that altering the microbiome of certain at-risk subjects early in life might prevent disease further down the road. Her lab has already found a connection between the neonatal gut microbiome and a child's propensity to develop allergies and asthma.

"This finding paves the way for early-life gut microbiome interventions to prevent these diseases from developing," Lynch says.

The holy grail in this field will lie in figuring out how to manipulate the microbiome and create new molecular environments that promote health. The UCSF Benioff Center for Microbiome Medicine, made possible by a $25 million gift from Marc and Lynne Benioff, aims to drive these and other microbiome-based innovations with Lynch at the helm.

"What really excites me about doing this now at UCSF is that we have all the pieces in place," Lynch says. "We have the computational capacity and expertise to work with big data, the immunological and microbiological skills to profile human immunity, and the technology to study the microbiome at very high resolution. The marriage of these three pieces will accelerate breakthrough discoveries and improve human health."

COMMUNITY

Found in Translation

Our interpreters help give families a voice

THE MARK MAKERS

Interpreter Jose Luis Caicedo shares a smile with a patient at the day clinic while helping her mother communicate with a nurse.

Medical terminology can be difficult to understand – baffling gene mutations, complicated treatment options, the winding road of follow-up care. And that's in your native tongue.

For families that speak languages other than English, the fear of misunderstanding is added to the countless stresses of caring for a sick child. That's why special teams at our hospitals are devoted to ensuring that every family can fully understand their medical care.

"Language access is a family's right," explains Hilda Diaz, manager of the program on our Oakland campus. "Every family deserves to have a complete understanding of what their child will encounter."

Interpretation is offered in 72 languages, from Albanian to Vietnamese, via a team of 13 full-time and four on-call interpreters, with additional video and phone service. This highly trained team is frequently described as "unsung heroes."

And for good reason: Their work dramatically impacts families' experiences throughout the hospital. Requests average between 150 to 200 per day, with the biggest demand for Spanish; more than 25% of our families identify as native Spanish speakers.

At a recent neurology appointment, for example, an interpreter was able to save the day when friction between a new teen patient and his parents boiled to the surface.

> **My work is to make sure families feel comfortable and respected, to come from a place of compassion and try to add a little joy.**
>
> – Adriana Arriaga, interpreter

Adriana Arriaga has been an interpreter at the hospital for 20 years.

THE MARK MAKERS

“The interpreter helped me lead a group therapy session, which turned out to be a wonderful interaction,” says Dr. Marisa Gardner. “We discuss many difficult topics during visits, and interpreters help create an emotional connection with families that would be impossible without them.”

Mam, an indigenous Mayan language spoken in Guatemala, was recently added to the roster. In this and many other dialects, interpreters face a unique challenge: Many Western medical terms simply don’t exist. Interpreters find themselves doing verbal gymnastics to convey, for example, a complex diagnosis in basic, but accurate, vocabulary.

Cultural nuances are also at play. Patients often feel more at ease divulging sensitive personal information, like abuse or mental health issues, with someone who shares their culture and language.

“Interpreters connect two worlds, and the value of that work is underestimated,” Diaz says. “We can laugh and play during a physical therapy appointment, then go straight into relaying terrible news. You become a chameleon, adapting to whatever is thrown at you.”

Diaz shares one of her favorite memories from her eight years of front-line service. After having to interpret the worst words a parent can imagine – “Your child isn’t going to make it” – the mother accepted the heartbreaking news, then asked to have her infant daughter baptized. Diaz interpreted the ceremony right in the middle of the intensive care unit.

“We had our hospital chaplain, doctors, nurses, social workers, and so many more – everyone came together,” Diaz recalls. “There was a true sense of community, and it didn’t feel sad despite the circumstances. It was a celebration. To be able to support the family in that way was special.”

This experience gets to the heart of the team’s role: delivering compassion and comfort. That’s what keeps Myra Guzman-Teare passionate about her work after more than 10 years of interpreting.

“We can’t change the outcome,” she says. “But at least we can be there to make sure parents understand and help them cope.”

To learn more about interpreter services, contact Hilda Diaz at (510) 428-3063.

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By inspiring friends and family to sponsor her 50 kilometer run, **Emilie Shoebottom** (right) raised more than $8,000 to help our experts fight childhood cancer.

Together
We Can Change Lives

It's never been easier to rally your community for children in need! Create a fundraising campaign to celebrate a birthday, honor a loved one, or host a party with a purpose. The options are endless.

Visit **together.ucsf.edu/benioffchildrens** to get started.