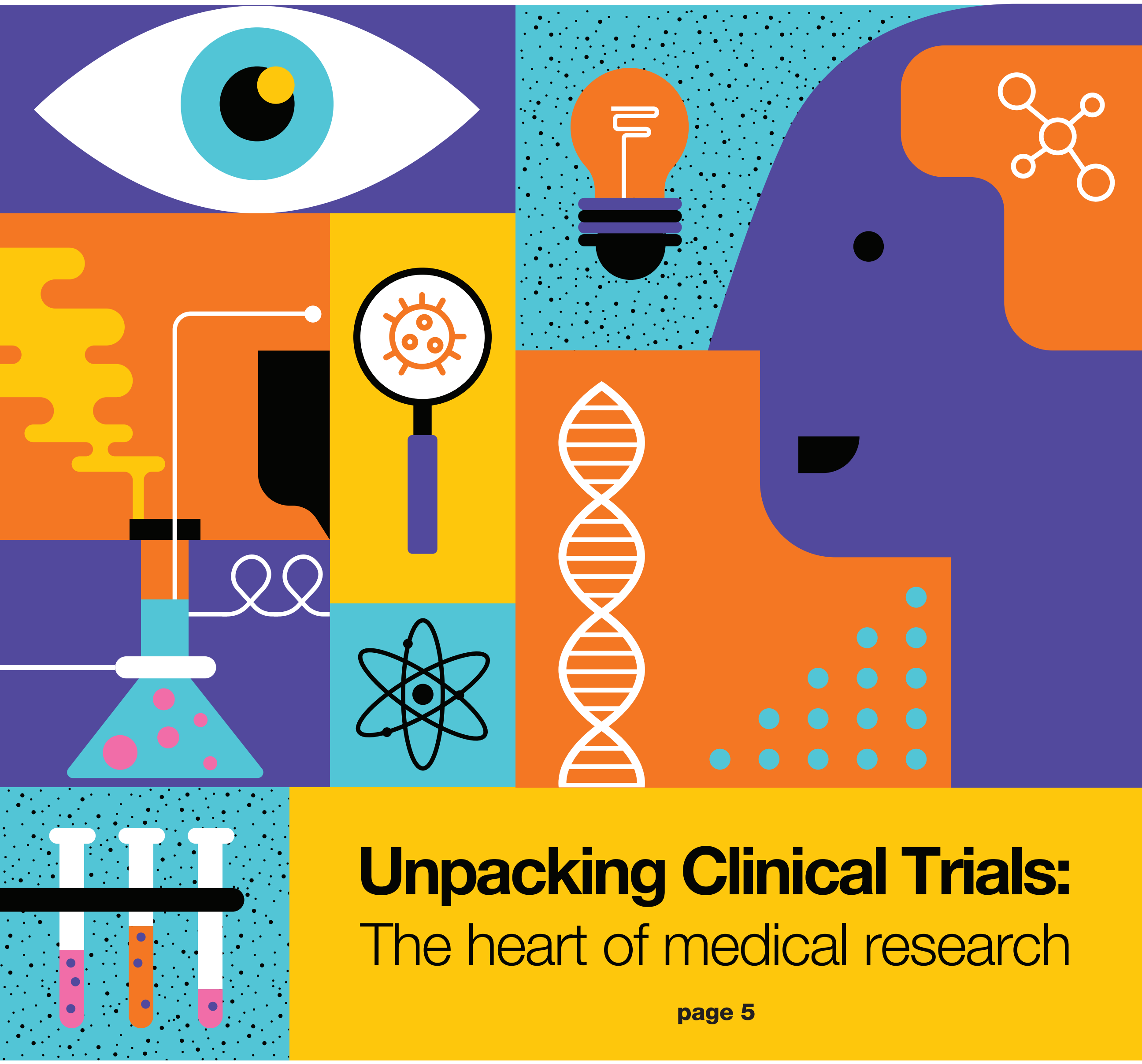
UCSF Benioff Children's Hospitals

A NEWSLETTER FOR
FRIENDS AND SUPPORTERS

Connections

WINTER 2023



Unpacking Clinical Trials:

The heart of medical research

page 5

As I write this letter, I'm just two weeks into my position as chief medical officer at UCSF Benioff Children's Hospitals. The most exciting part so far? This place is everything I expected it to be.

I can't remember a time in my medical career when I wasn't aware of these hospitals and the top-notch, life-changing work that they do for this community.

I started my new role amid an outbreak of RSV, a common respiratory virus that can be serious in small children. I am amazed by how hard our people are working to tackle this crisis and how everyone consistently goes above and beyond for each patient and their loved ones.

While this situation unfolded, I reflected on our mission of caring for all children with equity and excellence. You see it in action every day: Whether it's in the emergency rooms, our main hospitals, or our Federally Qualified Health Center in Oakland, our team is so attentive to their patients and every single detail of their care.

As chief medical officer, I hope to enable these incredible people – from the nurses, physicians, and researchers to the respiratory therapists, environmental service workers, child life therapists, and cafeteria staff – to do their best work on behalf of our families every day.

I hope we can be partners in this effort. Your impact is visible everywhere in our hospitals, from the moment you walk through the front doors. Without you, it's simply not possible to care for children equitably at this remarkable level.

Thank you for your continued support. I look forward to working with and for you in the years to come.

Sincerely,

Joan Zoltanski
Chief Medical Officer
UCSF Benioff Children's Hospitals

REVOLUTIONARY CARE: AN OAKLAND STORY

Oakland has been a nexus for social change, including health care. Our podcast, "Revolutionary Care: An Oakland Story," explores the frontiers of science, medicine, and health equity, told through the journey of a community and an Oakland hospital to cure sickle cell disease – all in the voices of our patients, providers, and partners.

One of those partners was the Black Panther Party, which provided free, community-based care for children with sickle cell disease in the 1970s when funding for sickle cell research and care was virtually nonexistent. In Episode 2, Norma Stoker-Mtume, Black Panther and clinic worker, reflects on that transformative time:

> **"We learned to draw blood. We learned how to give injections. We learned how to operate the lab. And we developed a partnership with Children's to come out and do pediatric clinics on Thursdays. Oakland Children's has always been at the forefront of making sure that folks get what they need in the community. They were really exceptional working with us."**

Check out "Revolutionary Care: An Oakland Story" by scanning the QR code or visiting ucsfbenioffchildrens.org/sickle-cell.

Plastic Surgery, 3D Printing and the Magic of Innovation

AN INTERVIEW WITH ALEXANDER LIN, MD

In 2022, Dr. Alexander Lin joined UCSF Benioff Children's Hospitals as co-director of the Craniofacial Center and director of surgical innovations for the UCSF Division of Plastic and Reconstructive Surgery. He also co-directs the UCSF Center for Advanced 3D+ Technologies and is developing UCSF's first site for in-house, sterilizable 3D-printed models on our Oakland campus. We spoke with Dr. Lin about why he loves his work and how our hospitals are prioritizing innovation and technology in patient care.

What led you to specialize in pediatric plastic surgery?

In medical school, you rotate through all sorts of different specialties, and I enjoyed learning about all the different ways we can help people. But when I rotated onto the plastic surgery service, I saw such incredible ways of healing people. Surgeries that looked like magic.

I remember this young girl whose nose had been completely bitten off by a dog. There was nothing left. But the plastic surgeons were able to carve cartilage to simulate the nose architecture, then carefully cut and turn over tissues from her forehead onto the carved cartilage and in a few weeks it had healed into something that looked like a new nose.

It was amazing to me that our bodies can be transformed into new shapes to restore people's humanity. I had to learn it.

Why is innovation so important to your work?

Innovation is all about finding new and better ways to achieve one's mission, and my mission is to help children and their families. It's not necessarily about coming up with the newest widget or device, although it certainly can be. Innovation can also mean taking something that already exists and modifying it to apply it in a more useful way – in this case, to help patients. In research, we call that translation – translating from theory to patient care.

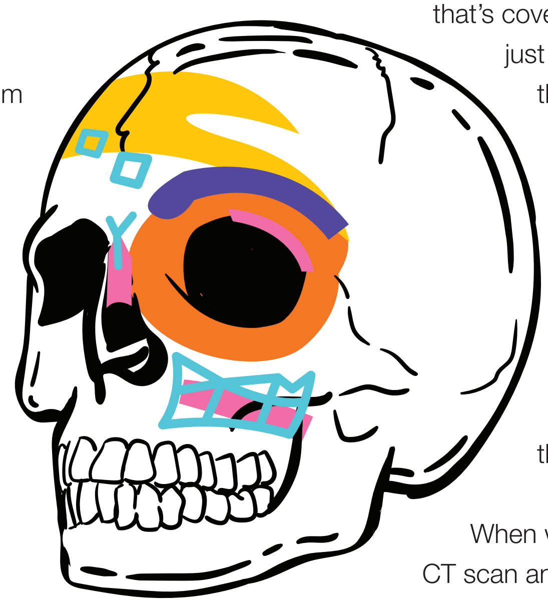
Can you give an example?

3D printing has been around for a long time, but it has taken a while for people to learn how to apply it. Even when people started using 3D printing technology to visualize complicated surgeries, it was really just about looking at the printed model to better understand the patient's unique anatomical problem. It took more innovative steps to figure out how to translate that technology into better patient care and apply it to highly complicated medical problems.

How do you apply 3D printing in the surgical process?

There are multiple ways. First, let's say a patient has a face fracture. We can use a model of the fracture to pre-bend plates and screws the day before surgery, so that these are already bent perfectly for the patient's anatomy, even before we operate.

We can also create custom splints for use during surgery, which means placing the sterile guide against the craniofacial bones to show us where to cut or shape without injuring nerves or blood vessels. And we can print patient-specific implants that we literally put inside the child's body.



A illustration of a 3D-printed model showing the bone grafts that Dr. Lin used to reconstruct a patient's skull.

Before surgery, we can use these models to see things better, to really clinch a diagnosis or understand the feasibility of a procedure. For example, we used 3D printing when we had an adult donor for an infant in need of a liver transplant. We used the model to see whether the liver would fit inside the baby.

What makes this so useful in children's health?

Kids are much smaller than adults, so you have less margin for error. If we understand the surgery better, we can make more precise incisions with smaller scars and improve the accuracy of the reconstructions.

> **It was amazing to me that our bodies can be transformed into new shapes to restore people's humanity. I had to learn it.**

We just had an 11-year-old patient who was run over by a vehicle. All the bones in his face were broken. That's like fixing a broken egg that's covered by a blanket. I can't just open the face to see the egg and fix it. I have to make little hidden incisions under the eyelids, under the mouth, and the lips – I'm basically making holes in the blanket and then trying to fix the egg through the holes.

When we 3D print the patient's CT scan and that sterile model is next to the patient's face while we are doing the surgery, it's like I have X-ray vision. The greater accuracy increases the speed of surgery, which lowers potential blood loss. Everything is better.

What sets UCSF apart when it comes to innovation in care?

I have just been amazed by the people here. This place is full of people with brains and heart. And you need both. You can tell people here really care about each other. They care about kindness. And they care about progress and innovation. That's a unique trifecta of mind, heart, and forward thinking.

Unpacking Clinical Trials

Clinical trials epitomize the heart of medical research. They're a crucial pathway for connecting our patients with potentially lifesaving therapies and technologies – even before they're available to the general public.

At UCSF Benioff Children's Hospitals, we are conducting thousands of clinical trials at any given time that have the potential to improve the health of millions of young people around the world.

So how does it work? Clinical trials use a rigorous testing process to evaluate the safety and efficacy of experimental treatments in volunteers, and in some cases, turn scientific curiosity into remarkable discoveries. Here's how.

PHASE I

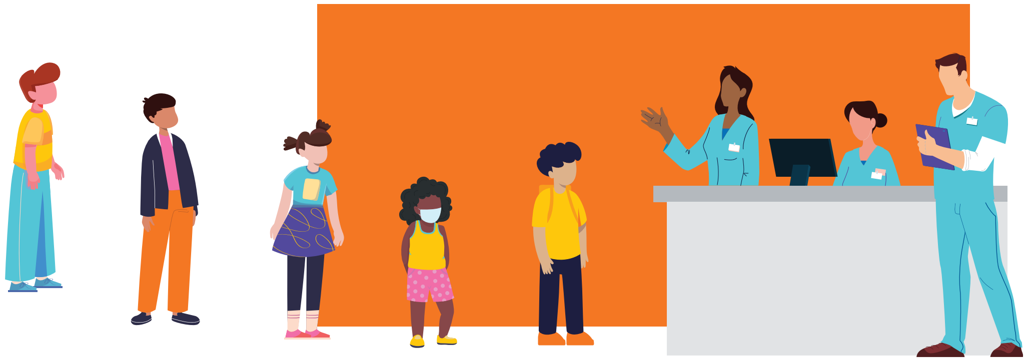
Is it safe?

In phase I, researchers test a new treatment for the first time on 15-30 patients. These participants can either be healthy or have the condition that the treatment is intended to help. The goal is to assess the treatment's safety.

PHASE II

Does it work?

Once researchers determine that a treatment is safe, they test it in a larger group of people – say, 100 patients – to make sure it is also effective.



PHASE III

Is it better than what we have now?

In phase III, researchers compare the new treatment with an existing treatment (or treatments) to see which is safer and works better in hundreds or thousands of participants across multiple institutions. If the study shows that a treatment is safe and effective, the US Food and Drug Administration (FDA) reviews it for approval.

PHASE IV

What else can we learn?

After a treatment is approved by the FDA and available to the general public, researchers may do a phase IV study in newly diagnosed patients to examine whether the treatment is useful for other conditions or leads to problems in the long run.

WHAT'S THE IMPACT?

Just ask Hataalii "HT" Begay, the first child to receive a new stem cell treatment for Artemis-SCID, a severe immunodeficiency otherwise known as bubble baby disease. Children with Artemis-SCID are highly susceptible to infections that can be fatal in the first year of life. But thanks to a UCSF clinical trial, HT is the first child to receive a brand-new gene therapy. Today, he's 4 years old, running around in cowboy boots, and dreaming of becoming a pilot. That's the power of clinical trials.

◀ Bella at 16

> “I realized that I needed to see the cup as half full rather than half empty.

RESEARCH

A Determined Mom Takes on Neuroblastoma

In 2016, Joy Lavigne received unimaginable news.

Her daughter, Bella, had just turned 10. She was experiencing symptoms – fevers, stomachaches, and loss of appetite – that baffled doctors. As Bella’s discomfort worsened, Joy demanded more tests. Eventually they found the cause: a 14-centimeter neuroblastoma tumor – a rare form of cancer – sitting above Bella’s kidney.

Bella was admitted to a local hospital for chemotherapy. “I remember the hospital room had this amazing view of San Francisco Bay, and I thought, this has got to be so bad for them to put us in this beautiful room,” Joy says. “That’s when I broke down.”

A nurse talked Joy through the emotional journey that lay ahead, and during Bella’s hospitalization, Joy met families that had spent years supporting their children through cancer treatment. “I realized that I needed to see the cup as half full rather than half empty,” Joy says. “Bella was going to feed off of my energy, and I had to believe that she would survive.”

Joy threw herself into learning about her child’s diagnosis and treatment options. Given the high recurrence rate for neuroblastoma, Bella would undergo chemotherapy to reduce the size of the tumor, surgery to remove it, a stem cell transplant at UCSF, and finally, radiation and immunotherapy.

While Bella went through treatment, Joy continued to research. She learned about a clinical trial for difluoromethylornithine (DFMO), a medication for preventing relapse in neuroblastoma patients in remission. The study, which is conducted through a North Carolina-based consortium, is also available locally at UCSF Benioff Children’s Hospital Oakland – the only center in the region offering this trial.

Although Bella had already undergone so much treatment, her chance of survival remained just 60%, so in 2018, Joy enrolled her child in the clinical trial.

The experience was positive. Thanks to a close relationship between Bella’s primary oncologist and Anu Agrawal, MD, a pediatric hematologist and oncologist at UCSF, the referral process was seamless. As a study participant, Bella received DFMO free of charge. And after enduring 18 months of debilitating side effects from her previous treatments, Bella got a break – the side effects from DFMO were minimal, and hospital clinic visits were few. And the best part: The treatment has so far been successful, and Bella has not relapsed.

“It was the easiest part of treatment,” Joy says. “Everything was so well executed. I’m just so thankful this was available to us.”

Bella is now 16. While recurrence remains a concern, and she still gets tired easily, Bella is ready to live her life. She loves catching blockbuster movies with her friends, playing video games, and going to Friday night football games. After losing her hair, her curly locks have grown back and now nearly reach her waist.

Joy is cherishing every moment and milestone. “I am so grateful every day,” she says.

HEALTH EQUITY

A TURNING POINT IN COMPLEX CARE

BY ALISON REED, MD

Sometime last winter, I realized that it had been more than a year since I'd seen one of my patients – I'll call her Tonya for confidentiality – and I was worried. The last time I'd seen her, Tonya was 18, living in her car, struggling with food insecurity, and had lost her smartphone.

Those things would be hard for anyone, but Tonya was also coping with depression, anxiety, post-traumatic stress disorder, and type 1 diabetes that had landed her in the hospital with diabetic ketoacidosis 12 times in 2020 alone. Every episode of ketoacidosis caused a metabolic injury that increased her risk for coma and even death.

It was heartbreaking because these episodes could have been prevented if Tonya had not been missing her insulin.

I knew that Tonya was smart and resilient. She was using a blood glucose meter and taking insulin by injection. I knew that she was doing the best that she could.

And I knew that our medical system was doing the best that it could for her. She had a primary care provider, an endocrinologist, a public health nurse, and a social worker. Yet despite all our support, Tonya still was hospitalized 12 times in 12 months. It felt like we were failing.

Fortunately, we had a solution for Tonya, and her name is Gabrielle. Gabrielle is an interventionist in our brand-new Novel Interventions in Children's Healthcare (NICH) Program. NICH, which is 100% funded through philanthropy, was created to provide proactive support for young people with complex medical conditions who also experience significant life challenges that lead to poor health and acute complications.

Changing Young Lives

NICH interventionists provide three major kinds of support:

- Skills training and coaching for patients and their families
- Care coordination between medical providers, patients, and their family
- Intensive case management that helps patients and families access resources like housing and transportation

Right now, we have four interventionists who are available by phone or text 24/7. They conduct weekly visits to the patient's home, community, school, and medical settings. Their constant presence helps build the daily routines and medical regimens that are so crucial for patients with diabetes, like Tonya, and supports these young people with appointment reminders, skill building, and problem solving. This is where we see real change happening.

NICH was started in 2011 at Oregon Health and Science University. Their data shows that in the year before NICH began, 83% of participants had an admission for diabetic ketoacidosis, but while they were working with the NICH interventionists, that number dropped by half. The NICH program kept them out of the hospital and helped them avoid those metabolic injuries.

Other aspects of their health improved as well. On average, kids enrolled in NICH had a 1% lower hemoglobin A1c. While 1% may not sound like much, for every 1% decrease in hemoglobin A1c, the risk for diabetes complications drops by up to 40%.

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> **Tonya still has challenges, but when I see how she's improving with the support of NICH, I feel proud of us, and I feel proud of her.**

Patients aren't the only beneficiaries. Medicaid claims data shows a cost savings of $30,000 per patient per year. In addition, there's reduced provider burnout and improved provider satisfaction. For doctors like me, seeing tangible improvements in our patients is what really keeps us going.

One Patient's Progress

We launched NICH at UCSF in 2021, and Tonya enrolled in February. In just six months working with Gabrielle, Tonya found a home, connected with community food resources, and established care with a new therapist.

I know all this because thanks to NICH, Tonya attended her first clinic visit with me in a year. It truly made my day. She also attended her next two clinic visits and is using a continuous glucose monitor that shares data with our health care team.

In the six months since enrolling in NICH, she has been admitted to the hospital four times in diabetic ketoacidosis. That's still a lot, but it's a big improvement over the previous year. And I'm happy to say that her hemoglobin A1c has come down 1.1%.

This summer, Tonya graduated from high school and enrolled in community college. She joined the diabetes camp as a counselor over the summer and aspires to become a therapist. She still has challenges, but when I see how she's improving with the support of NICH, I feel proud of us, and I feel proud of her.

My clinical work depends on my ability to accurately diagnose diseases and prescribe effective treatment plans. I know that the systemic problems undermining those plans are profound, but now with NICH, I truly believe that we can provide lifesaving care.

Dr. Alison Reed is a clinical associate professor in the UCSF Division of Pediatric Endocrinology. NICH – which provides around-the-clock support for young people facing complex medical and personal challenges – is funded entirely by philanthropy.

UCSF Benioff Children's
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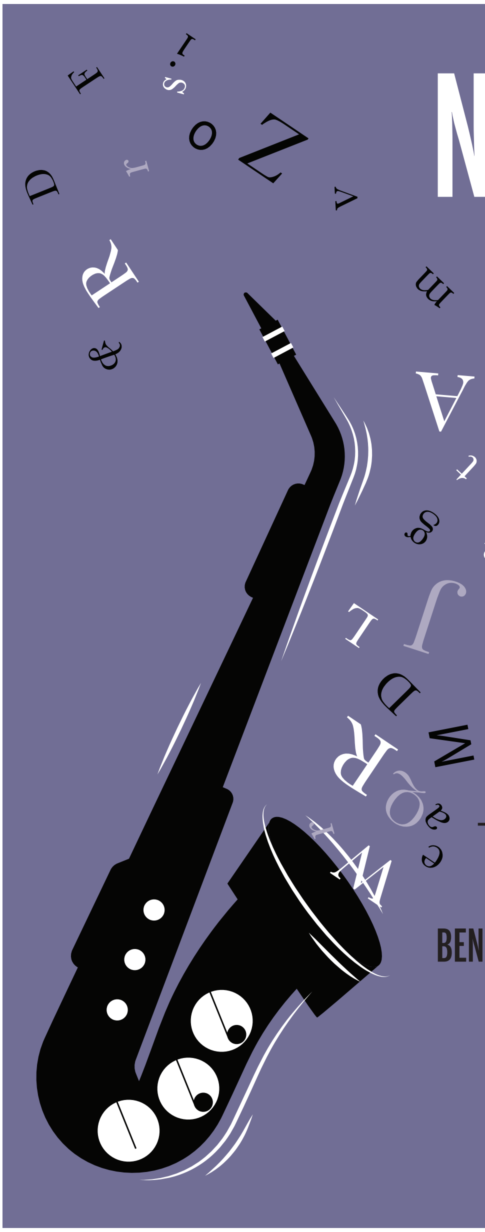

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