

A NEWSLETTER FOR
FRIENDS AND SUPPORTERS

Connections

WINTER 2020



Defeating Disease with Help from Your Genes

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Back on His Bike

Teenager Carlos sustained devastating injuries in an accident. Our team came together to give him his life back.

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Healing with Compassion

Our social services teams offer personalized support to families in crisis, when and how they need it most.

6

Continuing the Fight

After losing their son to brain cancer, a family finds strength and purpose in funding research to deliver a cure.

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

Some of the most heartbreaking cases we see here at UCSF Benioff Children's Hospitals are those in which a diagnosis remains elusive. Parents wait and worry, children endure therapy after therapy, and physicians, despite their best efforts, remain at a loss. Advancements in genomics are now unlocking answers in patients' genes that previously remained unknown and unknowable (page 7). At UCSF, we're thrilled to be at the forefront of this emerging field.

Even when a diagnosis is obvious, the treatment plan is not always easy. Carlos came to us after being hit by a car, with significant injuries from head to toe (page 3). I'm so proud of the interdisciplinary team that came together to help him heal, and of Carlos himself for persevering with bravery.

For every family that comes to us, no matter the illness or injury, a crucial component of care is delivered by our social services team (page 5). We're thankful to the generous community that supports this work, much of which is not covered by insurance.

On behalf of myself and everyone at our hospitals, thank you for all you do for children, and best wishes for an amazing 2020.

With gratitude,



Michael Anderson, MD, MBA, FAAP, FCCM
President
UCSF Benioff Children's Hospitals

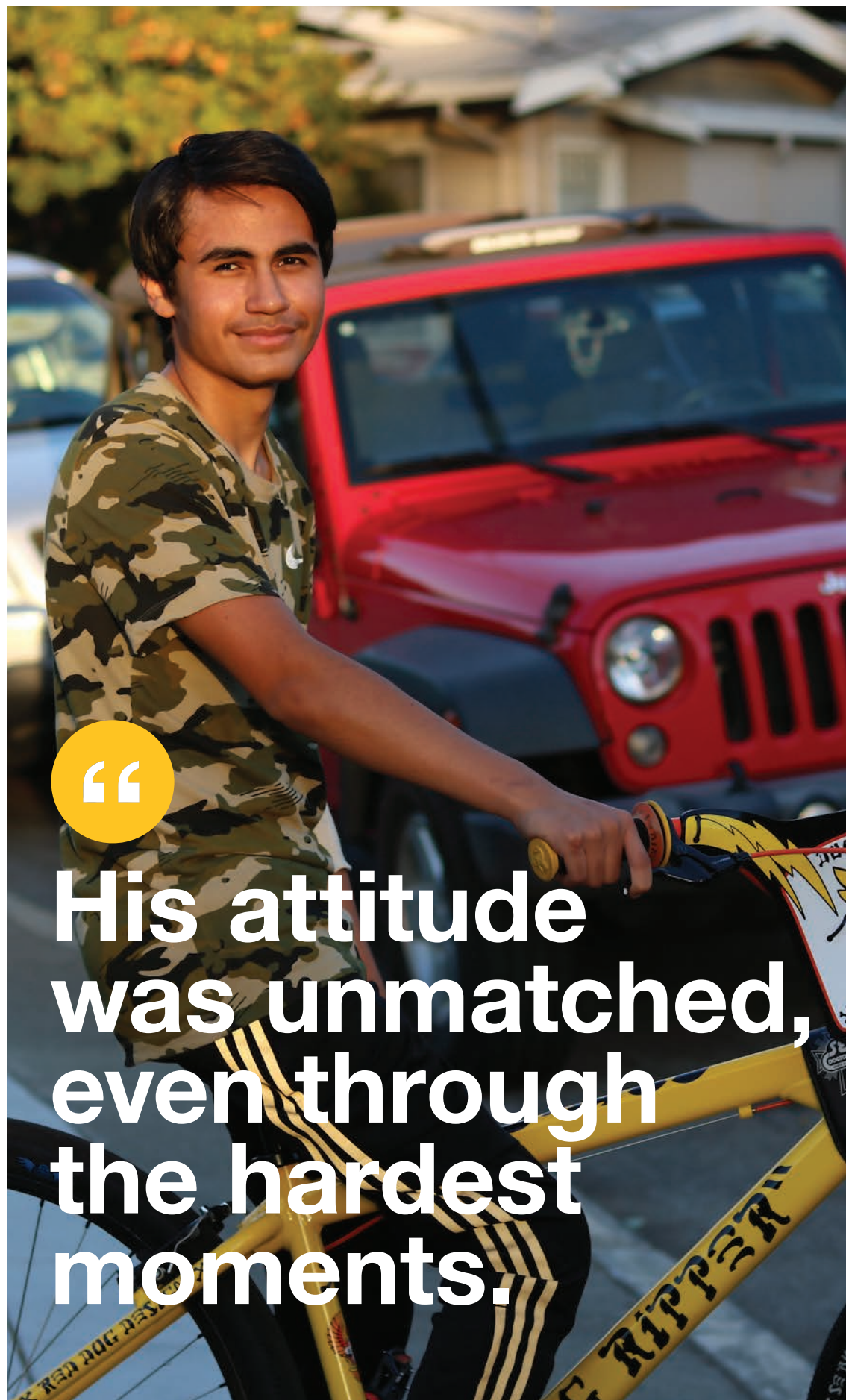
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Music therapy helps patients and families find strength and build resilience while coping with challenges like pain and stressful procedures. It's my privilege to help patients and families use music as a way to heal.

—John Mulcahy, MS, MT-BC
Music therapist
Oakland campus

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**His attitude
was unmatched,
even through
the hardest
moments.**

PATIENT STORY

FROM TRAGEDY TO TRIUMPH

Is this really happening?

When the car struck, knocking him off his bike, Carlos Prieto was overcome not by pain or fear, but disbelief. “Am I in a nightmare?” the then 14-year-old wondered, as the driver dragged him for four blocks, then fled the scene.

Pain would come later, as the result of 30 broken bones, severe internal bleeding, a detached left ear, and such significant skin loss that doctors at UCSF Children’s Hospital Oakland treated Carlos as a burn victim. During his three-month hospital stay, Carlos endured more than 10 surgeries, excruciating daily wound care, and over 100 grueling therapy sessions.

But fear never showed up.

“His attitude was unmatched, even through the hardest moments,” says surgeon Chau Tai, MD. “This kid is made of love.”

Carlos and his family credit the hospital’s staff for helping him stay positive.

“He trusted them,” says his mother, Arline. “And so did I. I had a lot of peace at Children’s because the doctors and nurses are such knowledgeable and caring people.”

One instance stands out in her mind: A surgery to rebuild Carlos’ knee, which was expected to take 8 hours, ended up taking almost 17 when surgeons faced complications. Carlos emerged from the operating room at 3 a.m.

“Nobody would have blamed the team if they said, ‘We’ve done all we can do,’ and gave

up,” Arline says through tears. “But they kept going. My son still has his leg because of that.”

Despite the hardships, Carlos has happy memories of the hospital: bonding over Legos with younger patients, partying at the hospital prom, and keeping up with his studies.

“One of my favorite nurses told me, ‘Your brain is working, your right hand is working, let’s get the teacher in here,’” Carlos recalls. A hospital teacher visited him every day, and he completed his sophomore year on schedule. In addition to daily physical and occupational therapy sessions, he also met with a psychologist three times a week to help process the emotional impact.

Arline is optimistic about her son’s future. “He’s handled something so traumatic, and this is going to make him a lot stronger,” she says. “Whatever comes his way, he’ll be ready for it.”

Carlos is grateful and proud that he’s now riding his bike again, and he’s even able to find something positive in his experience.

“I learned for every one bad person, there are thousands more good people,” Carlos says. “I think it’s a gift to find that out.”

▶ Watch Carlos’ story: bitly.com/bch-carlos

Every Step of the Way

Our social services teams are a vital part of the patient journey.



Social worker Sharon Nomburg greets a new mother and two of her quadruplets.

They don't diagnose illnesses, write pre-prescriptions, or perform surgeries. But the social services staff members at our hospitals are, without a doubt, healers.

Our dedicated social workers and chaplains deliver expert, personalized support when families need it most.

"Sometimes the things we do seem really small," says Rebecca Gates, who manages the department on our San Francisco campus. "But for a family, they might be huge."

Gates recalls a mother from Fresno bursting into tears when she offered to put the family up in a hotel following a difficult appointment. "She said, 'It's such a lovely gift because we didn't know how to pay for it,'" Gates recalls. "These kinds of gestures can transform the entire experience."

Anticipating Needs

Shabren Harvey-Smith, director on the Oakland campus, says her team excels at assessing and meeting families' needs beyond the obvious medical concerns. In some cases, this includes ensuring a family can make it to the hospital.

"Without the assistance of our department, some patients literally could not even get to their appointments because the family wouldn't have the resources to pay for transportation," Harvey-Smith says.

The Impact of Giving

Much of the budget for this support, which is not covered by insurance, comes from philanthropy. But generous gifts from the community don't cover the needs of every family, with as many as two-thirds relying on government insurance.

"Philanthropy is absolutely essential to all that we do," Harvey-Smith says. "When it comes to meeting the needs of our families, I want us to always be able to say, 'Yes' and 'How?' instead of 'No' and 'I'm sorry.'"

Both Gates and Harvey-Smith take tremendous pride in leading passionate teams – more than 100 staff members dedicated to lightening the load for families in need.

"You can't fix whatever's going on, but you can offer information and support to help them get through it," Gates says.



Mithil, age 11

DONOR SPOTLIGHT

IN MITHIL'S MEMORY

A foodie, Lego enthusiast, Star Wars fanatic, and starting pitcher on his baseball team, 12-year-old Mithil was both wise beyond his years and a goofball who could make anyone laugh.

When Mithil began walking strangely during a trip to visit family in India, his parents assumed he was jetlagged. But after a visit to a doctor, a terrifying diagnosis was confirmed: diffuse intrinsic pontine glioma (DIPG), an aggressive brain tumor.

In the year and a half that followed, Mithil fought hard against the disease.

"If you came to our house during that time, you wouldn't know there was a sick person there," says Sarva, his mother.

The experts at UCSF Benioff Children's Hospitals, recognized as best in the Bay Area for cancer treatment, battled right alongside him.

"It was a terrible time and terrible disease, but we feel very fortunate because of the great team we had at UCSF," Sarva says.

"Mithil used to say, 'You should do more for kids like me,' says his father, Prasad. "He was smart enough to figure that out."

This spirit inspired his family to continue Mithil's fight after he passed away in November 2016. In their first year of fundraising, the Mithil Prasad Foundation donated more than \$100,000 to DIPG research and patient family support.

Mithil's sister Rachna, a senior at UCLA, is carrying the torch in a unique way. For the past three summers, she has worked in the lab of UCSF's Sabine Mueller, MD, PhD, MAS, one of the world's leading DIPG researchers.

"Everyone I've met is so passionate and dedicated," Rachna says. "Knowing there are people like that working on this disease gives me a lot of hope."

A Precision Medicine Revolution

For children with rare diseases, genetic testing offers new hope.

By Ophir Klein, MD, PhD
Charles J. Epstein Professor
of Human Genetics; Chief,
Division of Medical Genetics;
Medical Director,
Craniofacial Center

Doctors were baffled. Over the course of a year, young adult patient “Sophie” went from perfectly healthy to dangerously ill, becoming wheelchair-bound, then falling into a coma. But no one knew why.

Too many patients face a similarly tragic fate. An estimated 15 million children in the United States – including tens of thousands in the Bay Area – suffer from rare diseases, nearly all with genetic roots.

In the past, most children with rare diseases had little chance of receiving an accurate diagnosis. In best-case scenarios, these children and their families endured long diagnostic odysseys that ended with no conclusive or effective treatment. Even worse, many received an incorrect diagnosis and ineffective care.

A Transformational Moment

The field of genomics — an interdisciplinary science focused on understanding the structure, function, mapping, and editing of our genetic makeup



STEVE BABULJAK

— has triggered a revolution in precision medicine. We’re at a transformational moment, entering an era of explosive growth.

Faster and more affordable gene sequencing, gene editing tools, and more computational power are transforming experts’ ability to diagnose illness and deliver the correct treatment, preventing months, years, or even decades of searching and suffering. We can now help families like Casen’s (see opposite page), who are often desperate for answers.

Delivering Cures

At UCSF Benioff Children’s Hospitals, we envision a future in which every child has access to comprehensive genetic screening, diagnostics, and effective therapies specifically designed to eliminate the consequences of genetic diseases. We already have the largest and most sophisticated team of genetic providers in the Bay Area.



We envision a future in which every child has access to comprehensive genetic screening.

This team is changing lives, including Sophie’s. With her condition growing dire, her doctors consulted UCSF genetic experts who evaluated the possibility of thousands of disorders by studying her genes. They quickly identified her rare condition, delivered the correct treatment, and restored her to good health.

We’re just at the beginning of an enormously exciting journey, and we know the road ahead will contain both known and unforeseen obstacles. But we’re ready and able to take on the challenge.

BENCH TO BEDSIDE

Genetic testing has given Casen’s family the gift of time.

Casen is a whiz at puzzles, cracks jokes constantly, and loves to run on the playground.

He also has severe epilepsy caused by a rare disorder. As Casen’s brain declines, this genetic defect will eventually rob him of his ability to walk, talk, eat, and breathe on his own.

While it’s heartbreaking for his family to know the challenges that lie in wait, they are grateful for this time with their spirited little boy. Without the expert care at UCSF Benioff Children’s Hospitals, they wouldn’t even have that.

Since birth, Casen constantly got sick – really sick. “He’d catch a simple cold and end up with pneumonia,” says his mother Katie. “Nobody could figure out why.”

By age 2, Casen had lost his hearing and couldn’t walk on his own. Desperate for answers, the family flew from their Idaho home to consult with experts at UCSF Benioff Children’s Hospitals. The team diagnosed Casen with epilepsy, which they deftly treated.

A year later, when Casen entered an almost vegetative state, the nearest pediatric neurologist recommended that the family simply accept that his condition was unlikely to improve. Unwilling to give up, Katie brought Casen back to UCSF, where the team offered a new technology: whole exome sequencing to unlock information in his genes.

“It was amazing,” Katie says. “It answered questions that other tests couldn’t.” Unfortunately, the results were devastating. Casen didn’t just have epilepsy – he had a fatal genetic defect.

Under UCSF’s care, Casen has regained his hearing, motor, and cognitive functions. Now 6, he attends a normal elementary school.

“For a kid who has every reason in the world to be angry, Casen isn’t,” Katie says. “He’s so happy all the time.”

Katie remains profoundly thankful for the team that keeps him that way, despite knowing what his future holds.

“UCSF has given us this time with him, and you can’t put a measure to that,” Katie says. “We’re very aware that all of UCSF’s efforts with Casen will have an impact on other children they treat after Casen is no longer here. That knowledge alone adds value to his life.”

Katie holds Casen at our hospital, where new technology finally revealed his diagnosis.



JOHN HAWKINS

YOU'RE INVITED!

Join us at our next event.



Lombardy Branch Shopping Event

March 5
Woodhall, Orinda

Give back while you shop! Proceeds from this glamorous event help ensure all kids have access to care.

03/05/20

St. Baldrick's Brave the Shave

March 14 | 9 am
UCSF Benioff Children's Hospital
Oakland

Show your support for kids battling cancer. Volunteer, donate, or brave the shave yourself at this festive event.

03/14/20

Credit Union SACTOWN Run

April 5 | 8 am
State Capitol, Sacramento

Lace up your sneakers for our kids by running a 10-mile or 5k race, or a family-friendly fun run.

04/05/20

Notes & Words

April 25 | 8 pm
Fox Theater, Oakland

Don't miss this star-studded evening uniting writers and musicians in support of world-class medicine for every child.

04/25/20

05/02/20

Ambassadors' Derby Party

May 2
Details to come

Grab a fabulous hat and get ready for fun at this popular event supporting pet therapy and special events for patient families.

05/05/20

Rowan Shopping Spree & Luncheon

May 5
Blackhawk Country Club, Danville

Give back while you shop at this afternoon event. Vendors donate a portion of all sales to our Oakland hospital.

05/16/20

Children's Health Guild Spring Gala & Auction

May 16 | 5 pm
Diablo Country Club, Alamo

Put on a glitzy gown or dapper jacket for an elegant evening of dinner, dancing and music benefiting our kids.

06/01/20

NorCal Golf 4 Charity

June 1 | 9 am
Chardonnay Golf Club & Vineyards,
American Canyon

Enjoy a day of golf with the beauty of Napa Valley as the backdrop while making a difference for families in need.

Questions?

Visit give.ucsfbenioffchildrens.org to learn more or contact Jeanne Marquis at (510) 428-3869 or Jeanne.Marquis@ucsf.edu.



THIS YEAR I WANT TO...

“STRESS LESS AND EXECUTE MORE.”

—Charles Cole III, EdD, MPA, board member

**“ENGAGE IN MORE SELF-CARE,
SUCH AS TRAVELING.”**

—Felicie Standley, MSW, pediatric oncology social worker

**“BE MORE LIKE MY SON:
LIVE IN THE MOMENT AND DO
MY BEST WITH WHATEVER I FACE.”**

—Katie Hawkins, grateful parent

**“SPEND MORE TIME FOCUSING
ON MY PHOTOGRAPHY.”**

—Holden Hilal, patient and community fundraiser

**“KEEP UP WITH MY KIDS IN
BASKETBALL AND SWIMMING.”**

—Jennifer Michlitsch, MD