

A NEWSLETTER FOR  
FRIENDS AND SUPPORTERS

# Connections

WINTER 2019



## Stopping a Fatal Blood Disease in the Womb

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### Big Dreams for Jazz

Though she battles a chronic illness, Jazz excels at school and on stage. Our expert sickle cell team keeps her healthy while leveraging the latest science to find a cure.

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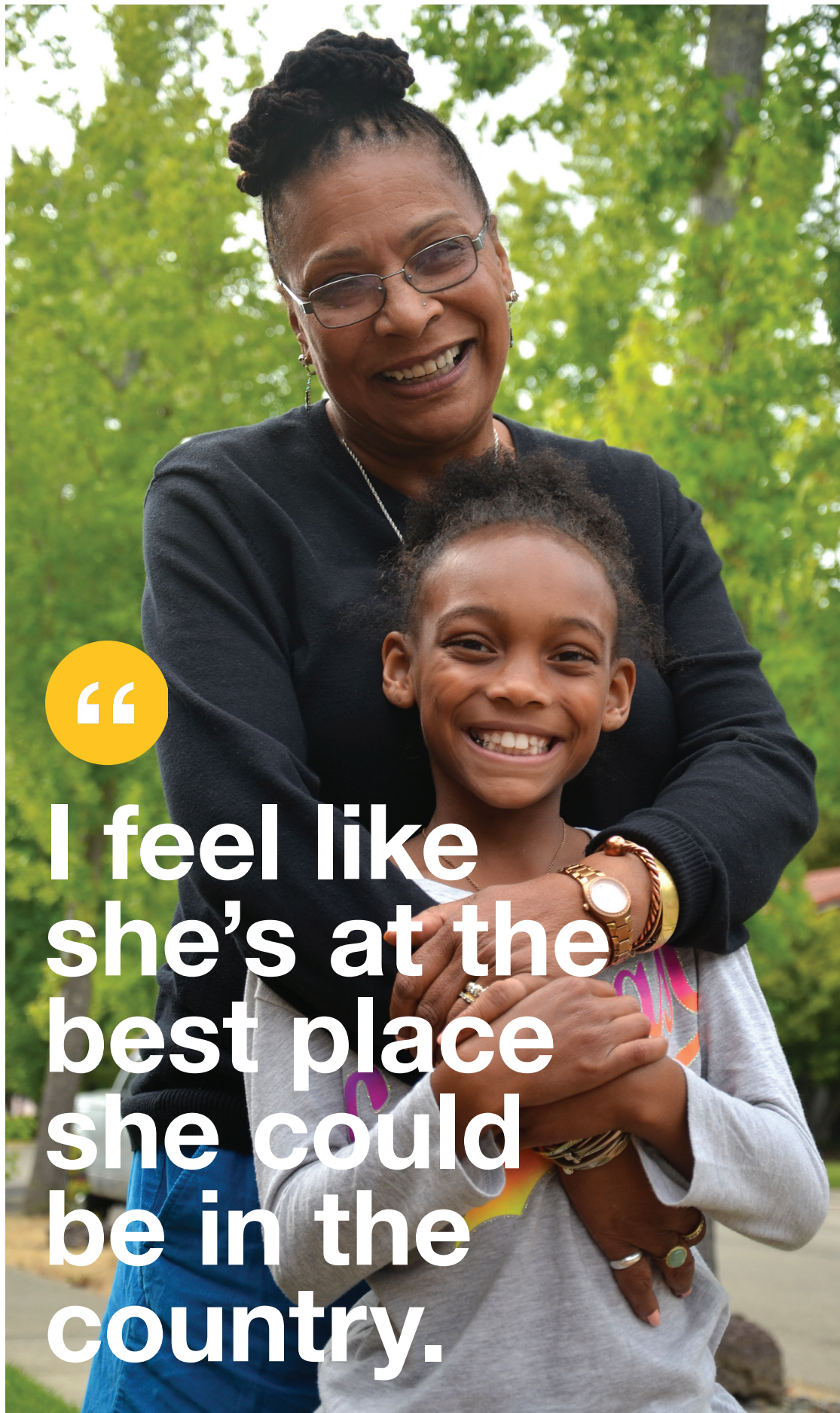
### A Champion for Kids

His career has led to clinical advancements that have improved the lives of countless children. Now, after 45 years of service to our Oakland campus, Dr. Bert Lubin reflects on a life in medicine.

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### A Hidden Disease

In many families, eating disorders go unnoticed. But early detection is crucial to avoiding major health setbacks. Our nationally recognized clinic has answers.



“

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## PATIENT STORY

# A LIFE IN THE SPOTLIGHT



Being in the spotlight comes naturally to Jazz Hardrict. Just ask her grandmother, Jan Hunter. At just 18 months old, Jazz visited Jan at Skyline High School, where she worked as drama director. Without prompting, Jazz toddled up to the stage and into the spotlight, twirled around, and took a bow.

Drama has been a huge part of Jazz's life ever since. At age 8, her stage credits already include *Rent*, *Cinderella*, *The Lion King*, and *Dreamgirls*.

Another major part of Jazz's life is sickle cell disease, a blood disorder that afflicts approximately 100,000 people in the US.

But with expert care at UCSF Benioff Children's Hospital Oakland – one of the nation's leading centers for sickle cell treatment and research – this chronic illness doesn't hold Jazz back.

Jazz has been treated at UCSF Benioff Oakland since she was 3, and Jan says the quality of care has been incredible.

"I think love is a great healer, and it makes a difference when they treat the children with the kindness they do at Children's," she says. "When a child is looking at something that can be deadly, their fears can come out. But because of the care Jazz has received, she's not afraid."

It's not just the expert medical care that sets UCSF Benioff Oakland apart: Comprehensive wraparound services take all of a family's needs into account. For example, social workers helped Jan navigate complex issues like filing for guardianship of Jazz and communicating her medical needs to a difficult teacher.

Jazz's symptoms are well-managed with a combination of daily medications and a healthy diet. Still, she sometimes battles complications, including sickle cell's trademark symptom: severe pain crises.

"When she goes into crisis, our whole world stops," Jan says. "It's so hard to see a little one suffering."

But Jan feels better knowing that Jazz is being treated by leaders in the field: "I feel like she's at the best place she could be in the country."

Jan also acknowledges the important role that donors play in ensuring that all children can benefit from the same level of care, regardless of their family's financial circumstances.

"The doctors do lifesaving work, but philanthropists are also doing lifesaving work by allowing children in need to get help," she says. "They improve children's lives every day."

Jazz, who excels at school while pursuing her passion for acting, has lofty goals for her future: to become an artist and a doctor. Thanks to UCSF Benioff Oakland, there's no reason she shouldn't dream big.

Watch Jazz dream big while battling sickle cell: [bit.ly/jazzvideo](https://bit.ly/jazzvideo).



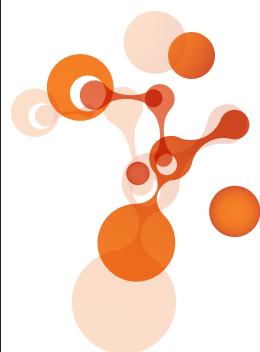
# Stopping a Fatal Blood Disease in the Womb

Pushing the Limits of Fetal Therapy as We Know It



BARBARA RIES

**Dr. Tippi MacKenzie** visits with newborn Elianna who received a stem cell transplant before birth.



Tippi MacKenzie, MD, is stopping disease before it starts – in the womb.

In a first-of-its-kind clinical trial, she has safely transplanted stem cells from an expectant mother into her growing fetus to halt alpha thalassemia major (ATM), a potentially lethal disorder that can lead to progressive anemia and heart failure before birth.

The first patient enrolled in the clinical trial was born in February 2018. Elianna, who had been critically ill during the second trimester of

pregnancy, was delivered at 37 weeks weighing just under 5 pounds.

“We are encouraged by how well she and her mother have tolerated this complex treatment,” says MacKenzie, a professor in the UCSF Department of Surgery. “Her birth suggests that fetal therapy, including fetal transfusions, is a viable option to offer to families with this diagnosis.”

Elianna has been discharged from the hospital and has

returned with her family to their home in Hawaii but will require further blood transfusions or another stem cell transplant to remain healthy.

Currently, children with ATM who survive past birth face a lifetime of blood transfusions. For some newborns, a stem cell transplant with a matched donor can be a cure, but the procedure comes with a risk of rejection and serious side effects from immunosuppressive drugs.

A transfusion in utero, on the other hand, takes advantage of the fetus’ nascent immune system. Because the mother’s cells flow freely through her fetus’ bloodstream, a fetus will not reject its mother’s stem cells, MacKenzie explains. With a mother’s healthy cells, a fetus will have all it needs to produce blood cells of its own and avoid immunosuppressive drugs.

## Unique Expertise at UCSF

During the pregnancy, MacKenzie’s team at the UCSF Fetal Treatment Center treated the fetus with regular in utero blood transfusions, after a medical center near the family’s hometown detected life-threatening swelling, or hydrops, during an ultrasound.

The swelling was the result of the body’s response to



**[Elianna’s] birth suggests that fetal therapy, including fetal transfusions, is a viable option to offer to families with this diagnosis.**

– Dr. Tippi MacKenzie

severe anemia and lack of oxygen, which is the hallmark of alpha thalassemia major, the most dangerous variant of thalassemia.

“Intrauterine blood transfusions were needed to treat the hydrops before the stem cell transplant could be performed,” says Juan Gonzalez Velez, MD, PhD, an obstetrician-gynecologist in the UCSF Division of Maternal-Fetal Medicine, who performed the transfusions.

“During the last 30 years, this has been the treatment of choice for patients developing severe fetal anemia, and we believe that fetuses with alpha thalassemia also can benefit from these transfusions,” he adds.

The fetus’ poor prognosis improved slowly with each

transfusion during the pregnancy, Gonzalez Velez said. The stem cell transplant took place during one of those transfusions.

The clinical trial brings together the unique expertise of both UCSF Benioff Children’s Hospital campuses. The Fetal Treatment Center at UCSF Benioff Children’s Hospital San Francisco is the birthplace of fetal surgery and continues to offer lifesaving interventions for fetuses with multiple birth defects. UCSF Benioff Children’s Hospital Oakland established its thalassemia program in 1991 to provide multidisciplinary care to the growing number of thalassemia patients. It is an international leader in stem cell and gene therapy for beta thalassemia.

## Moving the Field Forward

“Once universally fatal, thalassemia can now be managed as a chronic disease,” says Elliott Vichinsky, MD, who is overseeing the baby’s treatment and is the founder of the Northern California Comprehensive Thalassemia Center at UCSF Benioff Oakland.

“In utero stem-cell transplantation may take it one step further: as a disease that can be successfully treated before birth.”





## DONOR SPOTLIGHT

# A Champion for Those in Need

Bert Lubin, MD, Reflects on a Life in Medicine

After growing up selling fruit at his father's grocery store in a small town outside Pittsburgh, Dr. Bertram Lubin became the first in his family to attend high school, then college. Next, he stunned everyone by going to medical school. Lubin came to our Oakland campus in 1973 as chief of hematology and oncology, then was named director of medical research and eventually, president and CEO. After 45 years of service to our Oakland campus, Lubin recently retired from his role as associate dean for children's health, but will continue

to focus on advocacy for the health and well-being of kids.

## What professional accomplishment gives you the most pride?

I'm extremely proud of our sickle cell program. Dr. Elliott Vichinsky and I started a newborn screening program that has now been implemented nationally and is saving countless children's lives. We've also been a longtime leader in addressing health disparities and educating others about sickle cell disease.

## You've generously donated to the hospital. What inspires your philanthropy?

My wife and I see this hospital as an important part of the health of our community, and we want to

## To honor

Dr. Bert Lubin's profound commitment to Bay Area children, his friends and colleagues have established the **Dr. Bertram Lubin Health Career and STEM Scholars Fund**. Initiated with seed funding from Kaiser Permanente, the fund will support the aspirations of hundreds of underserved East Bay youths to pursue careers in research and clinical care.

support it any way we can. It's who I am – it's my home and my family. I feel fortunate to be able to do what I've done for the hospital, especially coming from a family without substantial resources.

## What inspires you most about our staff?

They are hardworking, and they care about kids. Whatever their jobs are, our staff members want to make our hospital as good as they can for our families. We also care about each other. I can walk down the hall and have 10 people stop to wish me well. It's lovely.

## What are some of the most important life lessons you've learned here?

Love your children, and do your best by them. Respect

them, listen to them, and support them. Be a champion for those in need. And everyone has a skill, but we all don't need to have every skill.

## What are some of the greatest health advances that you've witnessed?

Innovations like gene therapy and immunotherapy would never have been imaginable before. We could not be in a better place to advance this field, with partners like UCSF and UC Berkeley nearby.

## What message do you have for our donors?

If you want to do good for society, it all starts with children. The future of our society is with the health and well-being of our children. If you recognize that and want to be part of that mission, our hospitals are the place to invest.

## What message do you have for our patients?

Never give up. There is always hope.

**Dr. Bert Lubin with his wife Vivian Scharlach** at our Notes & Words benefit event.





# Join Us At Our Next Event



## St. Baldrick's Brave the Shave

**March 16, 2019 | 9:00 a.m. - 2:00 p.m.**  
**UCSF Benioff Children's Hospital Oakland**

Be a hero for kids with cancer. Whether you shave your head, volunteer, or donate, we hope you'll be part of the excitement.

03/16/19



## Snuggly Soirée

**March 21, 2019 | 6:30 p.m.**  
**Getty Residence, San Francisco**

Enjoy an evening fete for all ages benefiting the Glogau Teddy Bear Rescue Fund, providing support for patient families.

03/21/19



## Credit Union SACTOWN Run

**April 7, 2019 | 6:30 a.m.**  
**State Capitol, Sacramento**

Hit the ground running in support of our patients at this annual event featuring 10-mile and 5k races, as well as a Miracle Mile fun run.

04/07/19



## Notes & Words

**April 27, 2019 | 8:00 p.m.**  
**Fox Theater, Oakland**

You won't want to miss this epic, one-night-only event when music megastars and luminary authors come together to support world-class medical care.

04/27/19

05/04/19

## Ambassadors' Derby Party

**May 4, 2019 | 1:00 p.m.**  
**Private Residence in Marin**

Don your best hat and head off to the races at this popular event supporting babies being treated in our Intensive Care Nursery.



05/08/19

## Rowan Branch Shopping Spree

**May 8, 2019 | 10:00 a.m.**  
**Round Hill Country Club**

You don't want to miss this popular fundraiser featuring wares from numerous local vendors, lunch, and a fashion show.



05/11/19

## Children's Health Guild Spring Gala & Auction

**May 11, 2019 | 6:00 p.m.**  
**Round Hill Country Club, Alamo**

Join the Guild for a festive evening of gourmet fare, enticing auction items, and uplifting stories of hope and healing.



05/15/19

## Acoustic-4-A-Cure

**May 15, 2019**  
**The Fillmore, San Francisco**

Bay Area music legends Sammy Hagar and Bob Weir are once again throwing this must-see concert in support of pediatric cancer.



# For Further Information

Please visit [give.ucsfbenioffchildrens.org](http://give.ucsfbenioffchildrens.org) or contact Jeanne Marquis at (510) 428-3869 or [jmarquis@mail.cho.org](mailto:jmarquis@mail.cho.org).

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# Eating Disorders Are Easy to Overlook

## Learn What's at Stake

Sara Buckelew, MD, often hears the same shocked reactions from patients' parents: "This was my easy kid. I've never had to worry before."

Eating disorders afflict as many as 5 percent of US teens. But they can be difficult to recognize, says Buckelew, medical director of UCSF's Eating Disorders Program, a nationally recognized specialty clinic for adolescents and young adults battling disorders like anorexia nervosa and bulimia.

"Our patients typically do well in school and don't cause trouble, so their parents kind of have them on autopilot," Buckelew says. "And a lot of families don't eat dinner together these days, so these problems can sneak under the radar."

But early detection and treatment are key, says Charles



Irwin Jr., MD, director of UCSF's Division of Adolescent and Young Adult Medicine.

"We worry about impacts on brain development, bone health, and stunted growth," Irwin says. "The earlier you get treatment and restore caloric needs, the better the outcomes."

At our clinic, which treats about 200 patients each year and is a national leader in developing treatment

protocols, a multi-disciplinary team addresses each patient's individual needs. In addition to a doctor, families typically consult with a psychologist, nutritionist, and social worker during weekly sessions. And parents are included as a crucial part of the care team.

"We know that parents can be their kids' best advocates," Buckelew says. "It's important that we empower them to feel confident about helping their children."

# The Ultimate Explorer

## Pushing the Limits of What's Possible

As a new dental graduate, Pamela Den Besten, DDS, MS, appreciated the idea of pursuing scientific research alongside patient care. "Being a scientist is like being the ultimate explorer," she says. "I loved that I might learn and discover something nobody else in the world knew."

Becoming a pediatric dentist may have been the perfect career choice for her. She was attracted to the profession in part because she admired people who worked with children. "They always seemed a little childlike themselves, open to doing things differently," says Den Besten, who admits that she has always sought to implement change, not just follow.

"I started out in dentistry with a few basic questions," she says. "What is a tooth? How is it connected to the rest of the body? How does it relate to oral health, and how does oral health relate to our general health?" As director of the UCSF Center for Children's Oral Health Research, she bridges the gap between clinical care and research and works to unravel the mysteries of tooth development and disease.

### Tackling Complex Challenges

Perhaps the most ambitious research area Den Besten champions is gaining a better understanding of the biological mechanisms that lead to tooth formation. She hopes to discover how to re-engineer enamel because



ELENA GRAHAM

poorly formed enamel increases the susceptibility of teeth to cavities, cracks, and erosion.

Understanding the process by which enamel and dentin – the bony tissue under the enamel that forms most of the tooth – is created could help other top researchers in UCSF's Craniofacial Center accomplish their bold challenge to engineer stem cells to regenerate teeth.

### Reducing Oral Complications

Den Besten also studies the special dental-care needs of children undergoing treatment for cancer and autoimmune diseases such as type 1 diabetes. In collaboration with UCSF's Bone Marrow Transplant Unit, she is helping manage a clinical trial that is testing a tooth gel designed to prevent infections in the mouths of immune-compromised children being treated for blood cancer. Infections caused by oral bacteria can spread to the bloodstream and cause serious complications.

By helping severely ill kids keep their mouths healthy, Den Besten expects to learn information that will serve all children.

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# Why Do You Give Back?

FROM MISSION TO MAGIC, OUR DONORS SHARE WHAT DRIVES THEM TO GIVE BACK



## Magic

"I'm part of a team that inspires healing. I never believed in magic until I worked here. I've seen so many miracles happen."

– Malik Ali, hospital staff



## Mission

"I feel very connected to the hospitals' mission to treat all children regardless of the family's financial circumstances."

– Melissa Williams,  
board member



## Inspiration

"The patients inspire us with their immense strength, will, and resiliency. We have a tremendous responsibility to help them."

– Christian De La Cruz,  
corporate partner



## Gratitude

"I would not be here today without my treatment at UCSF, but the staff made sure I was not only surviving but also thriving."

– Marissa Hayes,  
community fundraiser

## Thank you!

With partners like you,  
there is no limit to what  
we can achieve for children.  
To learn more about the  
impact of your support, visit  
**[give.ucsfbenioffchildrens.org](https://give.ucsfbenioffchildrens.org)**.