

THANK YOU
FOR SUPPORTING OUR
RESEARCH INSTITUTE
AT CHOC CHILDREN'S



RECENTLY, AMAZING THINGS HAVE HAPPENED IN
THE RESEARCH INSTITUTE BECAUSE OF SUPPORT LIKE YOURS.

TOGETHER, WE...



STEPPED FORWARD AS A LEADER IN GENOME EDITING

Philanthropic support has allowed us to begin planning for the CHOC Children's Center for Advancing Rare disease Editing (CARE). The primary goal of CARE is to use genome editing to study and treat preclinical models of rare disorders. This will significantly elevate our understanding of gene editing and make it possible to one day utilize this expertise to help save the lives of children with rare diseases.



SUPPORTED NEWBORN HEALTH WITH ADVANCED DIAGNOSTIC TESTING

This year, the research institute launched Project Baby Bear at CHOC to offer rapid whole genome sequencing for critically-ill newborns. This program enables us to leverage genome testing to quickly and accurately diagnose newborns hospitalized in our intensive care unit. Treatment informed by whole genome sequencing results in reduced patient suffering, lower infant mortality rates and fewer hospital stays.



MOVED CLOSER TO FINDING A CURE

A CHOC clinical trial is analyzing samples from patients whose cancer is recurrent or resistant to treatment, providing valuable information on how different kinds of cancers respond to therapy. Currently, there are more than 420 open research studies at CHOC, each helping us create better treatments or bringing us closer to finding cures.



YOU HELP CHANGE LIVES

When Ely was diagnosed with Batten disease—just like his older brother, Titus—his mother, Bekah, feared the rare illness would take both her sons. But Dr. Raymond Wang at CHOC dedicated himself to making experimental treatment Brineura available for Ely. With his efforts, Ely was able to begin treatment in 2017. As of summer 2018, Ely had received over 40 infusions and is walking and eating independently, with his seizures under control. Because of Ely and his care team, CHOC is now the largest center in the United States, and the second largest center in the world, treating Batten disease with Brineura.

"We are so thankful," says Bekah. "Even though we don't know what the future holds, we feel that we're a part of the CHOC team as we pave the way for future children with Batten disease."