Stanford MEDICINE | Center for Definitive and Curative Medicine

Lucile Packard Foundation for Children's Health

Curing the Incurable:

Stanford's Center for Definitive and Curative Medicine

A Moment to Innovate

"There is no cure." These words haunt millions of families whose children face a lifetime of illness. While medications and surgical interventions can improve outcomes, these therapies are quickly reaching their limits. Most afflicted children will never lead a healthy life as an adult. For all, traditional therapies may lessen symptoms, but they often come with significant risks and debilitating side effects.

But there is good news: We're entering a new era in medicine. In recent years, novel therapies that were once only dreams have begun to take shape, pointing the way toward a dramatically different future. Discoveries in cell and gene therapies take radically new approaches, using patients' own cells as living drugs and correcting broken genes. These transformative technologies create a path to not only treat but actually cure diseases.

"This is not science fiction; this is reality. We will witness so many more successes. This is just the tip of the iceberg."

—MARIA GRAZIA RONCAROLO, MD, CDCM DIRECTOR

Leading the Charge

At Stanford, the promise of progress has inspired bold action. Leaders developed a grand vision: to leverage Stanford's incredible expertise in stem cell biology, exceptional research facilities and technology, and world-class clinical program into an unparalleled effort to translate discoveries from the bench to the bedside.

In 2017, the Center for Definitive and Curative Medicine (CDCM) launched as a fully integrated research-to-clinical trials program that includes:

- Leading physician-scientists with expertise in pediatrics, stem cell biology, gene therapy, immunology, hematology, oncology, neurology, and other fields, strategically recruited over a decade
- A 23,000-square-foot Stanford-owned cell-manufacturing facility, one of only 50 academic facilities worldwide, to develop and produce cell and gene therapy products for use in clinical trials
- State-of-the-art clinical space with specially designed rooms to care for patients undergoing CDCM-developed therapies, staffed by personnel with expertise in biostatistics and clinical trial protocols
- A Stem Cell and Gene Therapy Clinical Trial Office to provide essential support for first-in-human clinical trials, with a gold-standard team of seasoned regulatory experts

Platforms for Innovation

The CDCM's scientists and doctors have developed five technology platforms to deliver cures to children with genetic diseases and cancer. They focus on rare diagnoses related to a single gene defect, then build to widespread applications in more common and complex diseases.

- Stem Cell Engineering and Transplantation
- Gene Editing for Curative Therapies
- Engineering the Immune System
- Tissue Regeneration
- In Vivo Gene Transfer

These platforms serve as an engine to unite and galvanize some of the world's leading thinkers in these fields to conceive, test, refine, and prove therapies that will change the future of children's health.

"The idea of correcting gene mutations has been around for decades, but we finally have the technology and knowledge to make it a reality.

With our in-human sickle cell trial, the first trial ever to directly correct a mutation, we can potentially deliver a cure for people all over the world. It's an incredibly exciting moment."

—MATT PORTEUS, MD, PHD, CDCM CO-DIRECTOR

Transforming Children's Lives

Patients with Genetic Diseases

Siblings Kruz and Paizlee (pictured above) came from Alabama to access the CDCM's specialized care for SIOD, a rare and terminal form of dwarfism. Kruz underwent a groundbreaking method of stem cell transplant pioneered by CDCM experts, which allowed him to receive the kidney transplant he urgently needed. The siblings' life expectancies have been extended, and CDCM experts continue their search for a cure.

Patients with Leukemia

Diagnosed with high-risk leukemia, teenager Mark benefited from a CDCM innovation that enabled him to undergo a life-saving stem cell transplant even without a donor match. The clinical trial used novel regulatory cells that strengthen immunity and prevent relapse. Three years later, he's cancer-free and excelling at school and sports, and a new clinical trial is further refining this important therapy.

Opportunity for Impact

The CDCM team has many lab-tested ideas for curative therapies—and countless young patients are eagerly awaiting these innovative solutions. Philanthropy is essential to accelerate groundbreaking research that can only be accomplished by these experts at Stanford. Investments in this vision have the highest potential impact: helping millions of children around the world live without disease.

To learn more, please contact:
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