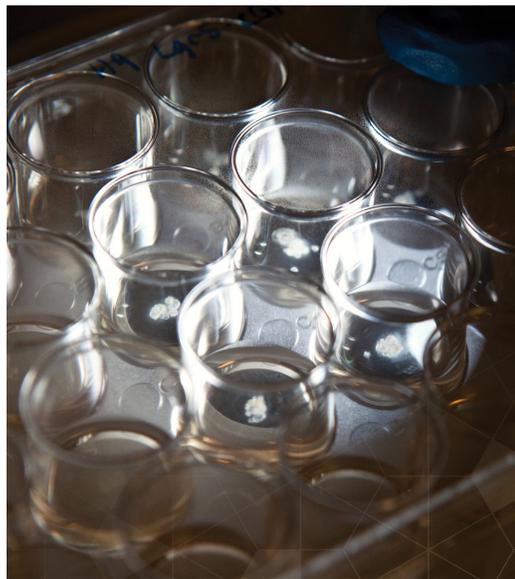


# Center for Stem Cell and Organoid Medicine (CuSTOM)



The Center for Stem Cell and Organoid Medicine (CuSTOM) at Cincinnati Children's is developing new personalized, regenerative therapies using advances in developmental biology, innovative stem cell technologies and miniature human organs, known as organoids. Our ultimate aim is to engineer patient-specific, organoid-derived tissues for therapeutic transplantation.

CuSTOM is on a rapid trajectory making significant breakthroughs that will revolutionize medicine. Partnerships with other researchers, entrepreneurial leaders and philanthropic organizations are essential in order to translate our research into effective therapies for children.

## CONTACT US

To learn more about CuSTOM, please contact program specialist:

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[www.cincinnatichildrens.org/custom](http://www.cincinnatichildrens.org/custom)

## NOVEL TECHNOLOGY

CuSTOM comprises 36 research labs at Cincinnati Children's, where investigators collaborate with clinical teams, industry partners and other research institutions worldwide with the goal of transforming the care of end-stage organ disease.

Our flagship technologies involve novel approaches using human pluripotent stem cells (hPSCs), which can be made from any patient, then using these hPSCs to bioengineer organoids. Although miniature in size, organoids function like the patient's own organ. They possess complex tissue structures, multiple cell types and perform organ functions. With a \$10 million aspirational "CURE" award from Cincinnati Children's, a CuSTOM team of scientists and clinicians, led by Michael Helmuth, MD, are pioneering approaches to transplant organoid tissue to cure intestinal failure.

Organoids also provide researchers with an unprecedented opportunity to:

- Study organ development and physiology
- Determine the causes of congenital birth defects, and in the future, grow genetically corrected tissue for surgical reconstruction
- Identify human disease processes in real time
- Diagnose patients, discover unrecognized pathologies and even predict diseases before they happen
- Provide a novel platform for personalized drug development and screening, allowing for the testing of drugs in organoids prior to patient use



Cincinnati Children's is ranked #3 in the nation among Honor Roll hospitals.



## BY THE NUMBERS

# 36

Cincinnati Children's research labs participating in CuSTOM

# 12

Cincinnati Children's divisions represented by CuSTOM investigators

# \$10 million

Received in philanthropic support since 2018

# \$9 million+

NIH and other foundations support in Fiscal Year 2021

# 62

Publications pertaining to CuSTOM research, including 14 collaborative publications

# 2

International Symposia since 2018

July 2021—June 2022

## BREAKTHROUGH DISCOVERIES

Cincinnati Children's has been investing in organoid research for more than a decade. Using cutting-edge molecular genetics, single cell genomics, live imaging and gene editing, our basic researchers pioneered efforts to understand how organs normally form during embryonic development. Our world-leading scientists, clinicians and entrepreneurs leverage our understanding of organ formation and breakthroughs in pluripotent stem cell technologies to create innovative human organoids as revolutionary platforms to understand human biology and disease, to develop in-a-dish tools for drug development for revolutionary personalized medicine, and to ultimately establish novel therapeutics for organ replacement.

Today, our researchers are creating increasingly sophisticated organoids of the small intestine, colon, stomach, esophagus, liver, brain, kidney and heart from stem cells that can be made from any patient. Exciting breakthroughs and dynamic collaborations are bringing us closer to the day when we can repair and/or replace damaged and diseased organs.

## EXPANDED EXPERTISE, EXTENSIVE RESOURCES, STRONG PARTNERSHIPS

With generous philanthropic support from the Farmer Family Foundation, Cincinnati Children's established the CuSTOM Accelerator Lab, a state-of-the-art facility with advanced robotics for high throughput manufacturing and analysis of human organoids from patient stem cells. The CuSTOM Accelerator Lab is dedicated to translating our groundbreaking discoveries into next-generation therapeutics and regenerative medicine to revolutionize child health.

Cincinnati Children's provides cutting-edge infrastructure to support CuSTOM's research. One example is the Pluripotent Stem Cell Facility and Organoid Research Core, which facilitates the development of state-of-the-art stem cell and organoid technologies.

Our research initiatives are supported by over 28 grants of more than \$20 million from the National Institutes of Health and other foundations. Since 2018, CuSTOM received over \$10 million in philanthropy to advance its pioneering research, including a \$5 million challenge grant from the Farmer Family Foundation to help us accelerate this transformational technology to the clinic. We continue building strong partnerships with biotech and pharmaceutical leaders who share our passion for innovation.

Following on the success of 2018 CuSTOM Symposium in Cincinnati, CuSTOM hosted an international Frontiers in Stem Cell & Organoid Medicine Symposium on March 24–25, 2022, co-sponsored by RIKEN-BDR from Kobe, Japan, as well as a number of leading biotechnology companies.

## LEADERSHIP TEAM

**Aaron Zorn, PhD**  
*Director*  
*Division of Developmental Biology*

**James Wells, PhD**  
*Chief Scientific Officer*  
*Divisions of Developmental Biology and Endocrinology*

**Michael Helmrath, MD**  
*Director of Clinical Translation*  
*Division of General and Thoracic Surgery*

**Magdalena Kasendra, PhD**  
*Director of Research and Development*  
*Department of Surgery, Division of General and Thoracic Surgery*

**Takanori Takebe, MD, PhD**  
*Director of Commercial Innovation*  
*Divisions of Developmental Biology and Gastroenterology, Hepatology and Nutrition*