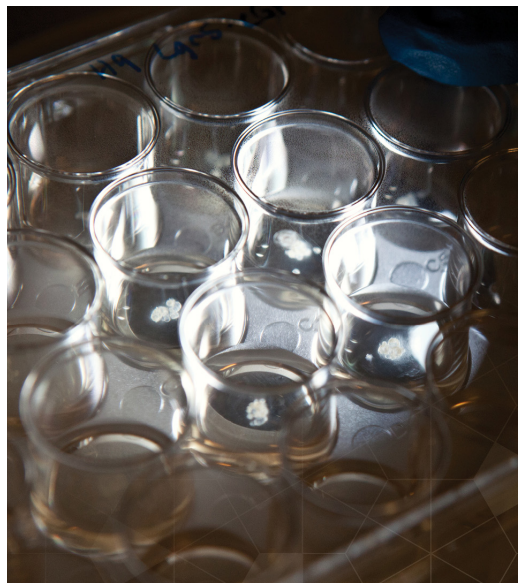


Center for Stem Cell and Organoid Medicine (CuSTOM)



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, and 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:

Phone: **513-636-4742**

Email: CuSTOM@cchmc.org

www.cincinnatichildrens.org/custom

NOVEL TECHNOLOGY

CuSTOM comprises 28 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.

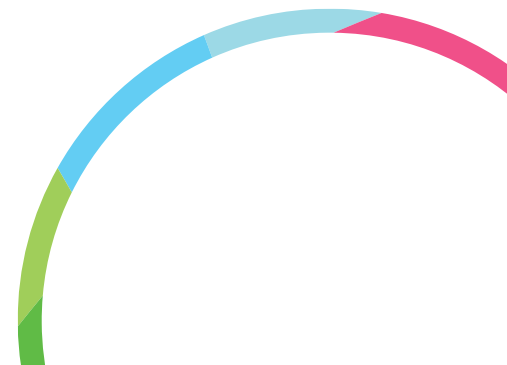
Our flagship technologies involve novel approaches using human pluripotent stem cells (hPSCs), which can be made from any patient, then using these hPSCs to bioengineer organoids. Although miniature in size, organoids function like the patient's own organ. They possess complex tissue structures, multiple cell types and perform organ functions. We are testing organoids in the laboratory to see if they can be used for transplantation-based therapies.

Organoids 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 drug development and screening, allowing for the testing of drugs in organoids prior to patient use



Cincinnati Children's is ranked third among all Honor Roll hospitals in the 2020–21 *U.S. News & World Report* listing of Best Children's Hospitals.





BY THE NUMBERS

28

Cincinnati Children's research labs participating in CuSTOM

10

Cincinnati Children's divisions represented by CuSTOM investigators

\$7.5 million+

Received in philanthropic support since 2018

\$7 million+

NIH and other foundations support in Fiscal Year 2020

80

Publications pertaining to CuSTOM research

July 2019 - June 2020

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

Three new faculty members joined the CuSTOM team last year: Drs. Ziyuan Guo from University of Pennsylvania, Perelman School of Medicine; Jason Tchieu, from Memorial Sloan Kettering Cancer Center; and Mingxia Gu, MD, PhD, from Stanford University. Their groundbreaking research uses patient-derived pluripotent stem cells to study neurodevelopmental and cardiovascular diseases with the goal of developing new therapeutic approaches.

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 25 grants of more than \$7 million from the National Institutes of Health and other foundations. Since 2018, CuSTOM received over \$7.5 million 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.

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

Takanori Takebe, MD

Director of Commercial Innovation

Divisions of Developmental Biology and Gastroenterology, Hepatology and Nutrition