

Business Standard

Meet Kiran Musunuru, Indian-origin doctor behind gene-editing therapy

Indian-origin cardiologist Kiran Musunuru leads breakthrough CRISPR gene editing therapy for rare genetic disorder focusing on heart disease genetics and new treatments

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Kiran Musunuru is an American cardiologist who graduated from Harvard College in 1997 with a degree in Biochemical Sciences | Photo: Kiranmusunuru.com

A nine-month-old baby in the US, suffering from a rare genetic disorder, was successfully treated with personalised CRISPR gene editing therapy.

According to a report from the *Children's Hospital of Philadelphia*, the treatment for KJ, the nine-month-old infant, was designed by a team of doctors at the Children's Hospital of Philadelphia (CHOP) and Penn Medicine. [The infant was suffering from severe carbamoyl phosphate synthetase 1 \(CPS1\) deficiency](#), a rare metabolic disease that prevents the body from safely processing ammonia.

He was treated by Rebecca Ahrens-Nicklas, director of the Gene Therapy for Inherited Metabolic Disorders Frontier Program (GTIMD), and Kiran Musunuru, MD, PhD. The

two doctors designed, manufactured and tested the personalised treatment for KJ within six months.

Who is Kiran Musunuru?

Early life, education and career

Kiran Musunuru is an American cardiologist who graduated from Harvard College in 1997 with a degree in Biochemical Sciences. He later obtained a PhD in Biomedical Sciences from Rockefeller University in 2003 and an MD from Weill Cornell Medical College in 2004.

He also completed a Master of Public Health (MPH) in Epidemiology from the Johns Hopkins Bloomberg School of Public Health in 2009. In 2019, he earned an M.L. in Law from the University of Pennsylvania Law School.

Since the early stages of his medical career, Musunuru has focused on heart disease, beginning his training in Internal Medicine at Brigham and Women's Hospital, followed by Cardiovascular Medicine at Johns Hopkins Hospital. He also completed postdoctoral work at Massachusetts General Hospital and the Broad Institute.

Research focus

According to his profile on kiranmusunuru.com, his research focuses on the genetics of heart disease, identifying genetic factors that protect against disease and leveraging them to develop new therapies. He is a leading expert in genome-editing techniques, particularly CRISPR-Cas9.

Musunuru's laboratory was the first to develop a technique that could genetically alter human pluripotent stem cells and differentiate them to model disease. He also discovered two new genes involved in coronary artery disease — SORT1 and ANGPTL3. His pioneering work has been central to using genome-editing tools as therapies for heart disease.

In 2019, Musunuru was part of a research team at the University of Pennsylvania that developed a stem-cell-based test to evaluate the effects of genetic variants on heart muscle cells.

Awards and recognition

Presidential Early Career Award for Scientists and Engineers (2013) – presented by the White House

Fannie Cox Prize for Excellence in Science Teaching (2014) – awarded by Harvard University

Award of Meritorious Achievement (2016) – from the American Heart Association

Judson Daland Prize for Outstanding Clinical Investigation (2018) – awarded by the American Philosophical Society

Musunuru is a member of the American Society for Clinical Investigation and the Association of American Physicians. He also serves on the NIH National Heart, Lung, and Blood Institute Advisory Council.

Additionally, he is on the Board of Directors at the American Society of Human Genetics and recently served as editor-in-chief of Circulation: Genomic and Precision Medicine, a leading scientific journal.

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