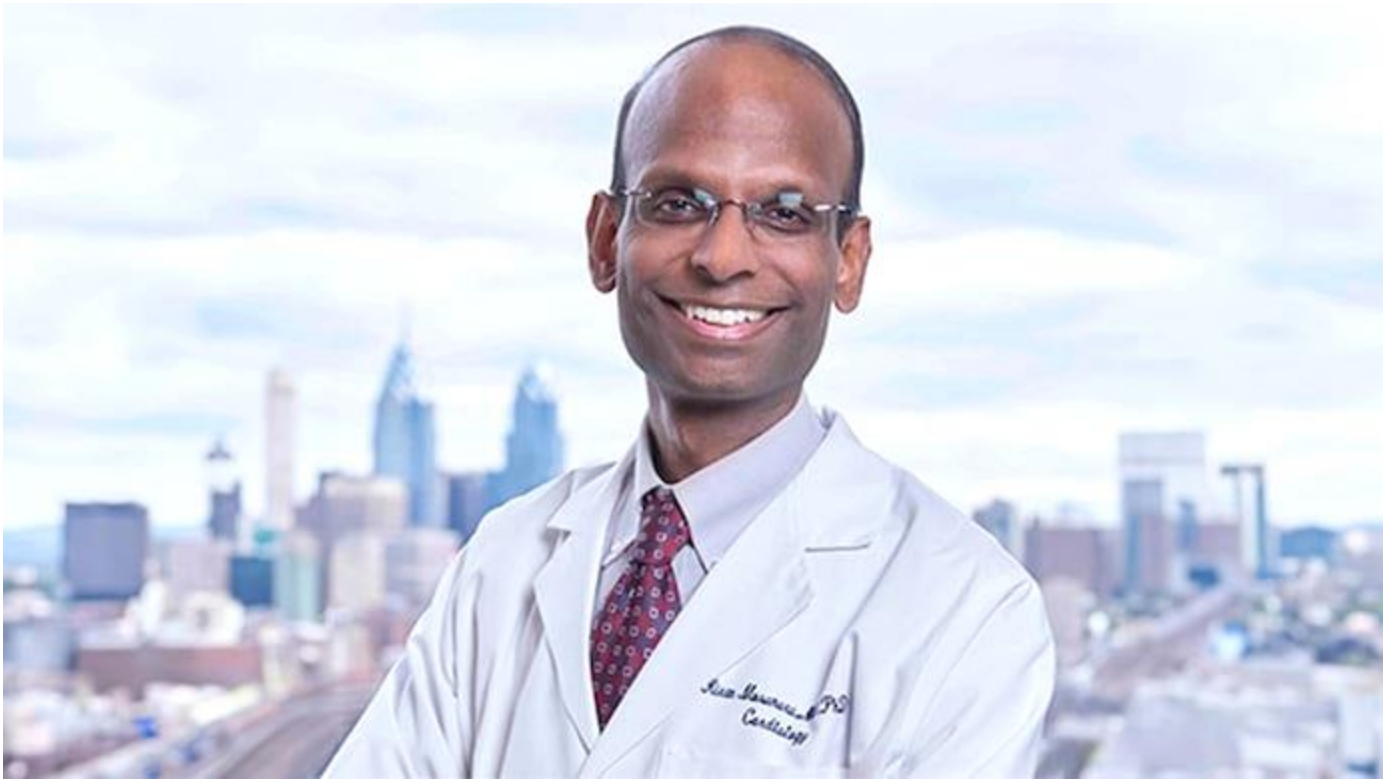
INDIA TODAY

Baby healed in world's first gene-editing therapy; Indian-origin doctor plays key role

An Indian-origin cardiologist, Kiran Musunuru, played a key role in a groundbreaking medical milestone, helping treat a nine-month-old baby boy with a rare genetic disorder using customized gene-editing therapy. Musunuru was born to Telugu parents who migrated to the US from India.



Dr Kiran Musunuru, an American citizen, is of Indian heritage, with ancestral roots in Andhra Pradesh. (Photo: National Library of Medicine, US)

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A nine-month-old baby boy, who was born with a rare and life-threatening genetic disease, **was successfully treated with an customized gene-editing treatment** made just for him. Indian-origin cardiologist Kiran Musunuru was in the team of doctors who became the first to treat the baby using the customized gene-editing therapy. The baby was diagnosed with a severe genetic disorder that typically proves fatal for about half of affected infants in early life.

The nine-month-old baby, identified as KJ, was born with severe CPS1 deficiency -- a condition that affects only one in 1.3 million people -- was treated by Rebecca Ahrens-Nicklas, a senior physician, and Doctor Kiran Musunuru.

The doctors at the Children's Hospital of Philadelphia and the University of Pennsylvania began work immediately after the boy's diagnosis, completing the complex design, manufacturing, and safety testing of the personalized therapy within six months.

The baby was just seven months old when he received the experimental treatment in February 2025.

He was born with a severe condition called carbamoyl phosphate synthetase 1 (CPS1) deficiency, a disorder so rare it affects only one in a million births. The disease is caused by a faulty gene in the liver, leading to dangerous build-ups of ammonia in the blood, which can cause brain damage, coma, or even death if not managed properly.

Dr Kiran Musunuru used the CRISPR base editing technique, which meant he carefully changed one tiny part of the baby's DNA without cutting it, to fix the gene causing the disease.

WHO IS DOCTOR KIRAN MUSUNURU?

Kiran Musunuru is a heart disease expert and Associate Professor of Cardiovascular Medicine and Genetics in the Perelman School of Medicine at the University of Pennsylvania. He is a principal expert in genetic research and medicine.

He was born to Indian immigrant parents who settled in the US. His father, Dr Rao Musunuru, is also a renowned cardiologist who moved from Andhra Pradesh and built a distinguished medical career in the United States.

Dr Kiran graduated in Biochemical Sciences from Harvard College in 1997.

Later, he completed a PhD in Biomedical Sciences at Rockefeller University in 2003, followed by a medical degree from Weill Cornell Medical College in 2004.

In addition to his medical and scientific training, the 48-year-old doctor has pursued extensive interdisciplinary education to support his work at the intersection of science, public health, and policy.

He earned an MPH in Epidemiology from the Johns Hopkins Bloomberg School of Public Health in 2009, followed by an ML in Law from the University of Pennsylvania Law School in 2019.

Most recently, in 2024, he completed an MRA in Regulatory Affairs from the Perelman School of Medicine at the University of Pennsylvania.

His research focusses on the genetics of heart disease and seeks to identify genetic factors that protect against disease and use them to develop therapies to protect the entire population, according to Dr Kiran's website.

In his recent work, he has been using gene editing to create a one-shot "vaccination" against heart attacks.

HONOURS AND AWARDS FOR GROUNDBREAKING WORK

Kiran Musunuru has received numerous prestigious honours in recognition of his groundbreaking contributions to science and medicine.

Among them is the Presidential Early Career Award for Scientists and Engineers, presented to him at the White House by former US President Barack Obama—one of the highest honours given by the US government to early-career researchers.

His accolades also include the American Heart Association's Award of Meritorious Achievement, the American Philosophical Society's Judson Daland Prize for Outstanding Achievement in Clinical Investigation, the American Federation for Medical Research's Outstanding Investigator Award, and Harvard University's Fannie Cox Prize for Excellence in Science Teaching.

In addition to his research and teaching roles, Musunuru recently served as Editor-in-Chief of Circulation: Genomic and Precision Medicine, a leading peer-reviewed journal in the field, reflecting his leadership in advancing precision medicine and cardiovascular genetics.

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