

## **Tackling** Sickle Cell Disease From Within

Evie Junior participated in a clinical trial funded by the California Institute for Regenerative Medicine (CIRM) to find and accelerate a potential gene therapy for sickle cell disease.







**Evie Junior was born with sickle cell disease**, a lifethreatening condition that affects around 100,000 Americans, most of them Black and Latino. People with sickle cell disease have blood cells that are shaped like a hook (or sickle) rather than smooth and round, which can create clogs causing intense pain, organ damage, and stroke.

By 18, Evie experienced severe pain crises once or twice a month. In his mid-20s, he took part in a CIRM-funded clinical trial at UCLA, where researchers collected his blood-forming stem cells and, in the lab, inserted a gene that prevented the sickling of the cells. The modified cells were returned to his body to create a new blood supply.

Since the treatment, Evie hasn't had any pain crises requiring medications or trips to the emergency room.

CIRM

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