12-Year-Old Starts New Sickle Cell Treatment

One young boy started on Lyfgenia to cure symptoms of sickle cell disease.

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Last week, at Children's National Hospital in Washington, DC, a 12-year-old boy with sickle cell disease became the first patient to start a new treatment approved in December by the Food and Drug Administration (FDA).

Physicians collected stem cells from Kendric Cromer in preparation for the first commercial infusion of a gene therapy called Lyfgenia, developed by biotechnology company Bluebird Bio.

Sickle cell disease (SCD) is a genetic blood condition in which red blood cells become hard and sticky due to hemoglobin abnormalities, restricting blood flow and causing pain and other serious complications.

SCD affects an estimated 100,000 people in the United States, most of whom are Black, according to the Centers for Disease Control and Prevention (CDC). In fact, about one in every 365 Black newborns has SCD in the United States.

Cromer, who is Black, experiences serious symptoms of SCD, including a painful condition known as bone death in his hips, shoulders and back.

The new genetic treatment modifies a patient's own stem cells and in clinical trials cured common symptoms of SCD in 88% of participants, according to BioPharma Dive. The treatment modifies the genetic coding of a patient's stem cells, allowing researchers to reengineer cells and ship them back to hospitals to be infused into patients. From start to finish, the process takes about between 70 and 105 days, according to Bluebird estimates.

Lyfgenia is now one of two approved genetic therapies that may lead researchers toward a cure.

The other therapy Casgevy, developed by Vertex and also approved in December, had five patients begin stem cell collection for treatment.

Previously, the only options for people with SCD were treatments to manage symptoms or donor stem cell transplants, which can carry serious risks, such as infection and immune system problems.

Promising though they may be, these drugs are prohibitively expensive. At $3.1 million and $2.2 million, respectively, Lyfgenia and Casgevy are among the pricies drugs on the market, raising concerns about patients' access to care.

In addition, medical centers have limited capacity for gene therapy patients, as each person requires expert and intensive care, according to the The New York Times. Children's National, for example, can accept only about 10 gene therapy patients per year.

To learn more, click #Sickle Cell. There, you'll find headlines such as "Research Reveals Opportunities to Better Address Blood Disorders Early in Life," FDA Approves First CRISPR Gene Therapy for Sickle Cell Disease" and "For People With Sickle Cell Disease, ERs Can Mean Life-Threatening Waits."
