$3 million barrier to sickle cell gene therapy: How prohibitive costs could limit practical benefits of newly-approved drugs

In a much-anticipated move, US regulators have approved two new gene therapies for sickle cell disease. Casgevy, a CRISPR-based therapy from Vertex Pharmaceuticals and CRISPR Therapeutics (co-founded by Emmanuelle Charpentier) and to Lyfgenia, a lentiviral-based gene therapy from bluebird bio that does not use gene editing.

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The FDA’s Peter Marks has put the number of people in the US who would be eligible for either therapy at around 20,000.

Even patients eager to undergo the treatments will find that barriers to access are considerable. First and foremost, they are eye-poppingly expensive. Casgevy will cost $2.2 million, and Bluebird priced Lyfgenia even higher, at $3.1 million (one of several mis-steps that sent the company’s stock price sliding). Those prices are just for the gene editing product itself — they do not include hospital stays or other aspects of the protocol.

Access is therefore likely to be very limited in the US and around the world. How will Medicaid, which funds treatment for around half of adults with sickle cell disease in the US, afford it? And what about patients living in states that have not expanded access to their Medicaid programs?

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