

Viewpoint: I took the exa-cel treatment for sickle cell disease. My symptoms ‘virtually disappeared overnight’

On a picturesque fall day a few years ago, I opened the mailbox and took out an envelope as thick as a Bible that would change my life. The package was from Vertex Pharmaceuticals, and it contained a consent form to participate in a clinical trial for a new gene-editing drug to treat sickle cell disease.

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After I received exa-cel, I started to experience things I had only dreamt of: boundless energy and the ability to recover by merely sleeping. My physical symptoms—including a yellowish tint in my eyes caused by the rapid breakdown of malfunctioning red blood cells—virtually disappeared overnight. Most significantly, I gained the confidence that sickle cell disease won’t take me away from my family, and a sense of control over my own destiny.

Today, several other gene therapies to treat sickle cell disease are in the pipeline from biotech startups such as Bluebird Bio, Editas Medicine, and Beam Therapeutics as well as big pharma companies including Pfizer and Novartis—all to treat the worst-suffering among [an estimated US patient population](#) of about 100,000, most of whom are Black Americans.

[**This is an excerpt. Read the full article here**](#)