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FDA approves landmark CRISPR therapy for sickle cell disease

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The Buzz This Week

Last Friday, the U.S. Food and Drug Administration (FDA) followed in the United Kingdom's footsteps and approved the first-ever medication to use clustered regularly interspaced short palindromic repeats (CRISPR) technology. The tool works by editing genes to alter DNA. The cell-based gene therapy, Casgevy, is approved to treat sickle cell anemia in patients aged 12 years and older. Sickle cell disease affects more than 100,000 people in the United States and more than 20 million worldwide. The disease is more prevalent among Black and Hispanic individuals, with approximately 8% of Black Americans estimated to be carriers of the sickle cell trait.

Sickle cell disease is an inherited red blood cell disorder that causes cells to be C-shaped and sticky. This can result in early death of the cells (which leads to anemia) and cells clogging blood vessels (which leads to infection and stroke). Sickle cell disease is also known to cause severe pain and be disruptive to quality of life. Hospital admissions and emergency room visits are common for patients with sickle cell disease. A 5-year study using Florida Medicaid claims data showed an average of nearly 4 hospitalizations per person, with an average length of stay of 6.5 days per admission.

CRISPR technology was invented in 2009 by Nobel Prize winners Drs. Jennifer Doudna and Emmanuelle Charpentier. For use in sickle cell disease therapy, CRISPR works by correcting stem cells so they no longer produce red blood cells with a sickle shape. For the therapy to be effective, patients must first undergo chemotherapy to eliminate current stem cells. And treatment comes at a steep cost of \$2.2 million per patient (though average medical expenses for someone with sickle cell disease are nearly \$1 million by age 45). While an arduous and expensive process to get Casgevy treatment, the clinical trial results are impressive—all but 1 of the 30 enrolled participants reported being free of severe pain for at least 18 months, with the average reporting 22 months.

Why It Matters

This is a landmark development in the treatment of sickle cell disease. While there are currently medications to help alleviate pain symptoms and raise hemoglobin levels, the only available curative treatment is a stem cell transplant, which requires a matching donor—something that is rarely found. Previous research and clinical trials have also seen roadblocks. A 2015 analysis of sickle cell clinical trials found 30% were terminated or withdrawn, half of those for insufficient enrollment.

While there is much to celebrate and great potential for Casgevy, there is also skepticism about how accessible the treatment may be due to the cost, how the therapy will be reimbursed, the difficulty of treatment procedure, and the daunting logistics of treatment. Additionally, many Black patients feel excitement for the possibility of pain relief mixed with ongoing distrust of the medical community due to historical injustices. The clinical trials, while promising, were conducted on a relatively narrow set of patients. Vertex Pharmaceuticals Inc, the company that developed Casgevy, plans to follow patients for 15 years to better understand long-term benefits and complications, including whether there is any “off-target” or unintended changes to patients’ cells. But those potential inadvertent consequences are still unknowns at this point.

Sickle cell disease is the first to see an approved CRISPR technology therapy option, but there are likely to be many more in the coming years, with cancer and Alzheimer disease noted as long-term areas for development. While other treatments will face similar obstacles as Casgevy related to cost, reimbursement, and complex treatment plans, the ability to target the root cause of disease and potentially cure patients could change medicine forever.

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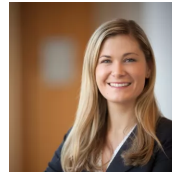
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