Source : www.thehindu.com Date : 2023-12-13

GAME-CHANGER: THE HINDU EDITORIAL ON APPROVAL FOR GENE THERAPY TO TREAT SICKLE CELL DISEASE AND BETA THALASSEMIA

Relevant for: Science & Technology | Topic: Biotechnology, Genetics & Health related developments

December 13, 2023 12:10 am | Updated 12:10 am IST

Less than a month after the U.K. drug regulator approved Casgevy, the gene therapy to treat people above 12 with sickle cell disease and beta thalassemia, the U.S. FDA has approved two gene therapies — Casgevy and Lyfgenia — to treat sickle cell disease in patients over 12. Its decision on approving Casgevy gene therapy for treating beta thalassemia is expected by March 2024. These landmark decisions mark the beginning of gene therapy using the CRISPR-Cas9 tool to treat diseases that could otherwise be cured only through bone marrow transplantation. While Lyfgenia uses a disabled lentivirus as a vector to introduce into the blood stem cells a new gene for haemoglobin that mimics the healthy version, Casgevy uses the gene-editing tool of CRISPR-Cas9 to disable a particular gene (BCL11A) that turns off foetal haemoglobin production in blood stem cells. While about 10% of adults continue to produce foetal haemoglobin, in others, the BCL11A gene prevents the production of foetal haemoglobin. By disabling the BCL11A gene, foetal haemoglobin that is produced, which does not have the abnormalities of adult haemoglobin, helps treat patients with sickle-cell disease or beta thalassaemia. In clinical trials, 28 of 29 sickle-cell disease patients who received Casgevy gene therapy were relieved of the debilitating effects of the disease for a year; for beta thalassaemia, 39 of 42 patients did not require blood transfusion for one year, and in the remaining three the need for blood transfusion reduced by more than 70%. In the case of clinical trials involving Lyfgenia, 30 of 32 sickle cell disease patients did not suffer from severe blocked blood flow caused by sickle cells, while 28 of 32 patients did not experience any blocked blood flow events six to 18 months post-infusion.

Since both gene therapies use patients' own blood cells for gene editing, the number of patients who can potentially be treated will be huge as these treatments do not rely on matching bone marrow donors. But in reality, these treatments would be exorbitantly expensive. Also, much like bone marrow transplantation, only certain hospitals will be equipped to extract a patient's blood stem cells and use the genetic editing tool to the stem cells before reinjecting them, thus limiting the number of beneficiaries. With clinical trials evaluating the therapies in a very small number of patients and for shorter duration, the compulsion to continuously monitor their safety and efficacy through real world data cannot be overemphasised: the possibility of unintended genetic modifications and their resultant side effects are real when the CRISPR–Cas9 tool is used.

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