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Which Therapy Could Trump Thalassemia?

01 May 2023 | Views | By Dr Manbeena Chawla

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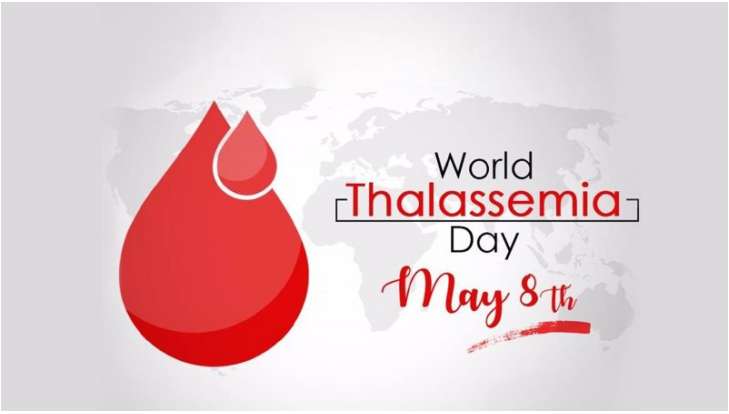

Every year, May 8 is observed as International Thalassemia Day to increase awareness about this genetic blood disorder that affects millions of people across the globe. Although people with thalassemia usually have mild to severe anaemia and associated symptoms such as fatigue, pale or yellowish skin, dark urine, and an enlarged spleen, afflicted children might experience stunted growth.

Thalassemia affects approximately 4.4 out of every 10,000 live births throughout the world, caused by mutations in the DNA of cells that make haemoglobin molecules that are made of alpha and beta chains. While, alpha-thalassemia is particularly common among certain populations of Southeast Asian descent, beta-thalassemia is the most common form of thalassemia among populations of Mediterranean, African, and South Asian ancestry.

As per the laws of genetics, for a child to inherit thalassemia, both parents must be carriers. The severity of thalassemia one has, depends on the number of gene mutations the child inherits from the parents.

Studies have revealed that India has the largest number of children suffering from thalassemia, with more than 10,000 children born with the disease every year. Four million Indians are thalassemia carriers, while more than 100,000 are actual patients. The beta-thalassemias are prevalent in India, with an estimated 7,500-12,000 new births each year. The prevalence of beta trait in Central India ranges between 1.4 and 3.4 per cent, while in South India it ranges between 8.5 and 37.9 per cent.

But the real challenge is the lack of awareness of this disease in India, because both or either parent of a child could be a thalassemia gene carrier without showing any symptoms or with just mild anaemia, that can go undetected throughout their lives. Adding on, the treatment involves blood transfusion, bone marrow transplant and gene therapy, making it highly cost intensive for parents of children born with thalassemia.

Over the past few years, there has been a great deal of global progress in the development of novel treatments for thalassemia, including gene therapy and gene editing. For instance, last year the US Food and Drug Administration (FDA) approved the first cell-based gene therapy to treat adult and paediatric patients with beta-thalassemia who require regular blood transfusions. bluebird bio, Inc.'s Zynteglo is a one-time gene therapy product administered as a single dose. Although a potential risk of blood cancer has been associated with this treatment, no cases have been seen in studies of Zynteglo.

Another US-based firm Vertex Therapeutics is nearing a historic US FDA submission of the first CRISPR-based gene editing therapy for treating beta-thalassemia. If approved, exa-cel will be the first gene editing therapy based on CRISPR technology, likely to compete with bluebird bio's Zynteglo. While bluebird is pricing Zynteglo at $2.8 million, Vertex aims to ensure that broad patient access and reimbursement deals are in place when exa-cel is approved.

On the other hand, US-based Bristol Myers Squibb (BMS) has announced the commercialisation of its first-in-class therapy, particularly in Egypt, indicated for the treatment of adult patients with transfusion-dependent anaemia associated with beta thalassemia.

In the Middle East region, Burjeel Medical City will be assessing the effectiveness and safety of Mitapivat, a drug that has shown promise as a treatment for thalassaemia. Abu Dhabi will take part in the phase 3 trials, called Energise and Energise-T, which will test Mitapivat's effectiveness on adults with thalassemia, in 2023.

While novel drug/therapy development is still miles away for Indian pharmaceutical companies, multiple initiatives are being taken in the country to create networks of healthcare professionals who can work together to provide the best possible care for patients with beta-thalassemia. In the end, a comprehensive multidisciplinary approach of medical, psychological and social support is the need of the hour.

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