FDA OKs 1st oral drug for anaemia in adults with thalassemia

The decision is a potential game changer for a lifelong genetic blood disorder that currently depends largely on repeated blood transfusions and iron chelation. The drug, however, is not yet available in India, as it has not received approval from DCGI.

New Delhi: In a landmark advance for thalassemia care, US FDA has approved Mitapivat, the first oral treatment for anaemia in adults with thalassemia, covering both transfusion-dependent and non-transfusion-dependent alpha and beta disease.

Clinicians and patient groups in India have welcomed the decision as a potential game changer for a lifelong genetic blood disorder that currently depends largely on repeated blood transfusions and iron chelation. The drug, however, is not yet available in India, as it has not received approval from DCGI.

Calling the approval a historic shift, Dr Rahul Bhargava, director and head of hematology and hemato-oncology at Fortis Institute of Blood Disorders, said Mitapivat targets the disease at its cellular core by correcting red-cell metabolism rather than only managing consequences.

From a transfusion-medicine perspective, Dr Rishiraj Sinha from AIIMS said adult patients have long borne the clinical and quality-of-life burden of lifelong transfusions and chelation. "The approval of the first oral therapy for both transfusion-dependent and non-transfusion-dependent thalassemia is a meaningful shift in disease management. Beyond improving haemoglobin, an oral option could reduce fatigue, treatment burden and long-term complications, moving care toward a more patient-centred and livable model-though access, affordability and safety monitoring will be critical," he said.Experts said the next challenge is regulatory clearance and affordability in India.

News Source:

https://pharma.economictimes.indiatimes.com/news/drug-approvals-and-launches/fda-oks-1st-oral-drug-for-anaemia-in-adults-with-thalassemia/126342685