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Exjade gets FDA nod for iron overload treatment

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FDA approved Exjade for chronic iron overload in thalassemia

Singapore: The US FDA has approved Exjade (deferasirox) for the treatment of chronic iron overload in patients 10 years of age and older with non-transfusion-dependent thalassemia (NTDT) syndromes and with a liver iron concentration of at least 5 mg of iron per gram dry weight and a serum ferritin measurement greater than 300 micrograms per liter. Exjade is the first treatment indicated for patients with these types of thalassemia in the US.

"Patients with NTDT can suffer severe and life-changing complications from chronic iron overload," said Dr Elliott Vichinsky, medical director, hematology and oncology, Children's Hospital and Research Center, Oakland, US. "In these thalassemia patients, excess iron starts to accumulate at birth yet is often undetected until serious symptoms appear in early adulthood. With this approval of Exjade, physicians will be able to offer NTDT patients a treatment option, helping fulfill a critical unmet need."

Thalassemia refers to a diverse group of genetic disorders that affect red blood cell production, causing anemia. Unlike patients with other types of thalassemia, those with NTDT syndromes don't require regular transfusions, a significant cause of chronic iron overload. However, even without transfusions, NTDT patients still accumulate excess iron through intestinal absorption, leading to debilitating health complications like liver fibrosis and cirrhosis, blood clots, bone disease, pulmonary hypertension, and vascular and endocrine diseases.

"For years, Exjade has effectively treated chronic iron overload in transfused thalassemia patients," said Dr Alessandro Riva, global head, oncology development and medical affairs, Novartis Oncology. "Now, for the first time, thalassemia patients who do not receive regular transfusions but suffer the same debilitating effects from chronic iron overload, have an approved treatment option."