

## Novartis' thalassemia drug Exjade get EMA nod

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**Singapore:** The Committee for Medicinal Products for Human Use (CHMP) of the European Medicines Agency (EMA) has adopted a positive opinion for Novartis' Exjade (deferasirox). The drug is used for the treatment of chronic iron overload requiring chelation therapy when deferoxamine therapy is contraindicated or inadequate in patients aged 10 years and older with non-transfusion-dependent thalassemia (NTDT) syndromes.

Novartis' Exjade would be the first oral treatment in the European Union (EU) specifically indicated for the treatment of iron overload in patients with these types of thalassemia.

Results from the first prospective placebo-controlled study of iron chelation in NTDT patients, THALASSA, showed a significant dose-dependent decrease in iron burden compared to placebo. In this pivotal study, Exjade was well tolerated, with an overall adverse event rate similar to the placebo arm.

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 can live without regular transfusions, a significant cause of 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.

"Patients with NTDT have suffered the effects of iron overload without accurate diagnosis, clear treatment guidelines or specifically approved oral therapies," said Dr Hervé Hoppenot, president, Novartis Oncology. "The CHMP recommendation

is an important step toward improving the outcomes of patients with this type of thalassemia."