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Zydus receives USFDA Orphan Drug Designation for Desidustat for treatment of beta-thalassemia

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Desidustat is a hypoxia inducible factor (HIF)-prolyl hydroxylase inhibitor (PHI) and has the potential to increase haemoglobin and red blood cell counts



Ahmedabad-based Zydus, a leading, discovery-based, global pharmaceutical company, has announced that the USFDA has granted Orphan Drug Designation (ODD) to Desidustat, a novel oral HIF-PHI, for the treatment of beta-thalassemia.

The USFDA's Office of Orphan Drug Products grants orphan status to support development of medicines for the treatment of rare diseases that affect fewer than 200,000 people in the United States.

Speaking on the development, Dr Sharvil Patel, Managing Director, Zydus Lifesciences Limited, said, "This Orphan Drug Designation from the USFDA underlines the urgent medical need to develop Desidustat to address beta-thalassemia."

Beta thalassaemia patients have low levels of haemoglobin, which results in a lack of oxygen in many parts of the body, leading to weakness, fatigue and more serious complications. Treatment for people with beta thalassaemia often requires lifelong regimens of chronic blood transfusions for survival and treatment for iron overload due to the transfusions.

Desidustat is a hypoxia inducible factor (HIF)-prolyl hydroxylase inhibitor (PHI) and has the potential to increase haemoglobin and red blood cell counts. Research in beta-thalassaemic mice showed that desidustat treatment led to an increase in haemoglobin and red blood cell (RBC) levels. Orphan drug designation by the USFDA for Desidustat, provides eligibility for certain development incentives, including tax credits for qualified clinical testing, prescription drug user fee exemptions and a potential seven-year marketing exclusivity upon the USFDA approval.