

Zydus receives USFDA Orphan Drug Designation (ODD) for Desidustat for the treatment of beta-thalassemia

- Desidustat is a hypoxia inducible factor (HIF)-prolyl hydroxylase inhibitor (PHI) and has the potential to increase haemoglobin and red blood cell counts
- Orphan drug designation by the USFDA for Desidustat, provides eligibility for a potential seven-year marketing exclusivity subject to the USFDA approval

Ahmedabad, 6 November, 2025

Zydus, a leading, discovery-based, global pharmaceutical company today 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) levels¹. 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.

About Zydus

Zydus Lifesciences Ltd. with an overarching purpose of empowering people with freedom to live healthier and more fulfilled lives, is an innovative, global lifesciences company that discovers, develops, manufactures, and markets a broad range of healthcare therapies. The group employs over 29,000 people worldwide, including 1,500 scientists engaged in R & D, and is driven by its mission to unlock new possibilities in lifesciences through quality healthcare solutions that impact lives. The group aspires to transform lives through pathbreaking discoveries. Over the last decade, Zydus has introduced several



**PRESS
RELEASE**

For further information please contact :
The Corporate Communications Department

Zydus Lifesciences Limited

Regd. Office : 'Zydus Corporate Park',
Scheme No. 63, Survey No. 536, Khoraj (Gandhinagar),
Nr. Vaishnodevi Circle, S. G. Highway, Ahmedabad 382
481, Gujarat, India. | Phone : +91-79-71800000,
+91-79-48040000 | website : www.zyduslife.com
CIN : L24230GJ1995PLCQ25878

innovative, first-in class products in the market for treating unmet healthcare needs with vaccines, therapeutics, biologicals and New Chemical Entities. For more details visit www.zyduslife.com

References

1. HIF-Prolyl Hydroxylase Inhibitor Desidustat Increases Pyruvate Kinase Activity and Reduces Oxidative Stress in Red Blood Cells, Causes Erythrocytosis in Thalassaemic Mice, and Reduces Sickling in Sickle Cell Patient's Blood. *Drug Dev Res.* 2025 Apr;86(2):e70086. doi: 10.1002/ddr.70086. PMID: 40192567.
2. Desidustat in Anemia due to Non- Dialysis-Dependent Chronic Kidney Disease: A Phase 3 Study (DREAM-ND). *Am J Nephrol.* 2022. DOI: 10.1159/000523961
3. Desidustat in Anemia due to Dialysis-Dependent Chronic Kidney Disease: A Phase 3 Study (DREAM-D). *Am J Nephrol.* 2022. DOI: 10.1159/000523949
4. Prolyl hydroxylase inhibitor desidustat improves anemia in erythropoietin hyporesponsive state. *Current Research in Pharmacology and Drug Discovery.* 2022; 100102. <https://doi.org/10.1016/j.crphar.2022.100102>.
5. Outcomes of Desidustat Treatment in People with Anemia and Chronic Kidney Disease: A Phase 2 Study. *Am J Nephrol.* 2019;49:470–478.
6. Phase I Clinical Study of ZYAN1, A Novel Prolyl-Hydroxylase (PHD) Inhibitor to Evaluate the Safety, Tolerability, and Pharmacokinetics Following Oral Administration in Healthy Volunteers. *Clin Pharmacokinet.* 2018 Jan; 57(1):87-102.
7. Pharmacological Characterization of ZYAN1, a Novel Prolyl Hydroxylase Inhibitor for the Treatment of Anemia. *Drug Res (Stuttg).* 2016 Feb; 66(2):107-12.
8. Influence of acute and chronic kidney failure in rats on the disposition and pharmacokinetics of ZYAN1, a novel prolyl hydroxylase inhibitor, for the treatment of chronic kidney disease-induced anemia. *Xenobiotica.* 2018 Jan; 48(1):37-44.
9. A sensitive assay for ZYAN1 in human whole blood and urine utilizing positive LC-MS/MS electrospray ionization. *Bioanalysis.* 2017 May; 9(9):719-732.
10. Pharmacological inhibition of prolyl hydroxylase protects against inflammation-induced anemia via efficient erythropoiesis and hepcidin downregulation. *Eur J Pharmacol.* 2019 Jan 15; 843:113-120.
11. Prolyl Hydroxylase Inhibitors: A Breakthrough in the Therapy of Anemia Associated with Chronic Diseases. *J Med Chem.* 2018 Aug 23; 61(16):6964-6982.
12. Prolyl hydroxylase inhibitor desidustat protects against acute and chronic kidney injury by reducing inflammatory cytokines and oxidative stress. *Drug Dev Res.* 2021; 1–9.
13. Nonclinical Pharmacokinetic Evaluation of Desidustat: a Novel Prolyl Hydroxylase Inhibitor for the Treatment of Anemia, *European Journal of Drug Metabolism and Pharmacokinetics.* 2022 Jul 26. doi: 10.1007/s13318-022-00788-3
14. A Food-Effect Study to Evaluate the Oral Bioavailability of Desidustat. *Clin Pharmacol Drug Dev.* 2023 Apr;12(4):356-362. doi: 10.1002/cpdd.1206. Epub 2022 Dec 2. PMID: 36458679
15. HIF-PHD inhibitor desidustat ameliorates iron deficiency anemia. *Toxicol Appl Pharmacol.* 2024 Feb;483:116832. doi: 10.1016/j.taap.2024.116832. Epub 2024 Jan 22. PMID: 38266872.
16. HIF Stabilizer Desidustat Protects against Complement-Mediated Diseases. *Drug Res (Stuttg).* 2024 Jul 11. doi: 10.1055/a-2347-9919.



**PRESS
RELEASE**

For further information please contact :
The Corporate Communications Department

Zydus Lifesciences Limited

Regd. Office : 'Zydus Corporate Park',
Scheme No. 63, Survey No. 536, Khoraj (Gandhinagar),
Nr. Vaishnodevi Circle, S. G. Highway, Ahmedabad 382
481, Gujarat, India. | Phone : +91-79-71800000,
+91-79-48040000 | website : www.zyduslife.com
CIN : L24230GJ1995PLC025878