Zydus announces USFDA Orphan Drug Designation to Usnoflast for the treatment of Amyotrophic Lateral Sclerosis (ALS)

- *Orphan drug designation by USFDA for Usnoflast, 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 FDA approval.*

Ahmedabad, India, January 22, 2025

Zydus, a leading, discovery-based, global pharmaceutical company today announced that the USFDA has granted Orphan Drug Designation (ODD) to Usnoflast, a novel oral NLRP3 inhibitor, for the treatment of amyotrophic lateral sclerosis (ALS). 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, Chairman of Zydus Lifesciences Limited, Pankaj Patel, said, "This Orphan Drug Designation from the USFDA underlines the urgent need to develop Usnoflast to address Amyotrophic Lateral Sclerosis (ALS), which is a fatal neurodegenerative disease. Zydus is committed to unlocking new frontiers in neuroscience and develop Usnoflast for patients with ALS."

People living with ALS have an average survival of approximately two to five years from diagnosis, with most ALS patients dying from respiratory failure. ALS patients experience neuroinflammation and rapid neurodegeneration. Axonal neurodegeneration leads to formation of neurofilaments which first accumulate in CSF of ALS patients, and then slowly these neurofilaments enter blood circulation. Owing to rapid neurodegeneration, steady loss of the ability to move, speak, eat, eventually breathe, paralysis and death have been reported in ALS patients. ALS affects approximately 32,000 people in the U.S.A and on an average 5,000 new patients are diagnosed every year with this disease in USA as per statistics from Centre for Disease Control and Prevention (CDC). More than 30,000 people are estimated to be living with ALS in Europe (European Union and United Kingdom), while India has an estimated 75,000 people living with ALS.

Usnoflast (ZYIL1) is a novel, oral small molecule NLRP3 inhibitor. Usnoflast has been studied in several pre-clinical models of neuroinflammation, Parkinson's disease, Inflammatory Bowel Disease (IBD) and Multiple Sclerosis (MS). The USFDA has earlier granted Zydus an 'Orphan Drug Designation' for

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Usnoflast to treat patients with Cryopyrin Associated Periodic Syndrome (CAPS), a rare auto-inflammatory disease. Zydus has previously completed a Phase 2(a) randomized, double-blind, placebo controlled clinical trial in 24 ALS patients across 7 clinical trial sites in India. [ClinicalTrials.gov Identifier: NCT05981040]. It is planned to present this Phase 2(a) trial data in upcoming medical conference and publish in medical journal. Zydus has recently received approval from USFDA to initiate a randomised, double blind, placebo- controlled Phase 2(b) clinical trial for Usnoflast in patients with Amyotrophic Lateral Sclerosis (ALS).

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 27,000 people worldwide, including 1,400 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 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. ClinicalTrials.gov Identifier: NCT04972188 A Phase I, Prospective, Open Label, Multiple Dose Study of ZYIL1 Administered Via Oral Route to Investigate the Safety, Tolerability, Pharmacokinetics and Pharmacodynamics In Healthy Adult Subjects
2. ClinicalTrials.gov Identifier: NCT04731324 A Phase 1, Prospective Open Label, Single Dose, Single Arm Study of ZYIL1 Administered Via Oral Route to Investigate the Safety, Tolerability, Pharmacokinetics and Pharmacodynamics in Healthy Adult Human Subjects
3. ClinicalTrials.gov Identifier: NCT05186051 A Phase 2a, Prospective, Open-Label Study to Evaluate the Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of ZYIL1 in Subjects with Cryopyrin Associated Periodic Syndromes (CAPS)
4. ClinicalTrials.gov ID NCT06398808 A Study to Evaluate the Efficacy and Safety of ZYIL1 Oral Capsules for the Treatment of Patients with Mild to Moderately Active Ulcerative Colitis Resistant or Intolerant to Oral Aminosalicylates
5. ClinicalTrials.gov ID NCT05981040 A Phase 2, Proof-of-concept, Placebo Controlled, Randomized, Multi-centre, Double Blind Study of ZYIL1 to Evaluate the Efficacy, Safety, Tolerability, Pharmacokinetics and Pharmacodynamics in Patients with Amyotrophic Lateral Sclerosis (ALS)
6. Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of the Oral NLRP3 Inflammasome Inhibitor ZYIL1: First-in-human Phase 1 studies (Single Ascending Dose and Multiple Ascending Dose),

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7. Safety, Tolerability, Pharmacokinetics and Pharmacodynamics of ZYIL1 in Three Patients with Cryopyrin-Associated Periodic Syndromes, ***Clinical Pharmacology in Drug Development,*** 2023, 0(0) 1–8. DOI: 10.1002/cpdd.1318.
8. A novel selective NLRP3 inhibitor shows disease-modifying potential in animal models of Parkinson's disease. ***Brain Res.*** 2024 Jul 27;1842:149129. DOI: 10.1016/j.brainres.2024.149129.

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