Zydus Lifesciences initiates Phase II clinical trial of ZYIL1, a novel oral NLRP3 inflammasome inhibitor in patients with Amyotrophic Lateral Sclerosis (ALS)

- The Phase 2 clinical trial will assess the safety and efficacy of ZYIL1 in Amyotrophic Lateral Sclerosis (ALS) patients [ClinicalTrials.gov ID: NCT05981040]
- ALS is a rare, progressive and fatal neurodegenerative disease, with an average life expectancy of 3 to 5 years from the time of symptom onset.

Ahmedabad, India, October 25, 2023

Zydus, a leading discovery-based, global pharmaceutical company, announced today that it has received permission from CDSCO, India, to initiate the Phase II clinical study of NLRP3 inhibitor "ZYIL1" in patients with Amyotrophic Lateral Sclerosis (ALS).

Mr. Pankaj R. Patel, Chairman, Zydus Lifesciences Ltd. said, "Zydus has always aimed at improving the quality of life of patients through its life changing discoveries. This study is a positive step in this direction to address very high unmet medical needs of patients suffering with ALS. By targeting neuroinflammation and neurodegeneration with ZYIL1, we hope to open up new possibilities in treating ALS."

ALS patients experience neuroinflammation and rapid neurodegeneration leading to steady loss of the ability to move, speak, eat and eventually breathe. ALS results in loss of motor neurons in the brain and spinal cord which controls voluntary muscle movement.

ALS affects approximately 31,000 people in the U.S.A and on average 5,000 new patients are diagnosed every year with this disease in USA as per statistics from Centers 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. People living with ALS have a median survival of approximately two years from diagnosis.

The Phase II clinical trial will study safety, tolerability, pharmacokinetics and pharmacodynamics in patients with ALS. The change from baseline in the Revised Amyotrophic Lateral Sclerosis Functional Rating Scale (ALSFRS-R) score will be measured at week 4, week 8 and week 12, as the trial's primary endpoint is the placebo-controlled, randomised, double-blind Phase 2 clinical trial. The trial will also evaluate Key Secondary Endpoints including Slow Vital Capacity (SVC), a predictor of functional loss in ALS and neurofilament levels at week 4 and week 12.

PRESS RELEASE

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ZYIL1 is a novel oral small molecule NLRP3 inhibitor. Studies have demonstrated that ZYIL1 is highly potent in human whole blood assay and can suppress inflammation caused by the NLRP3 inflammasome. ZYIL1 was found distributed in the brain and CSF of various nonclinical species including mice, rats and non-human primates. The efficacy of ZYIL1 has been established in several validated pre-clinical models of neuroinflammation, Parkinson's disease, Inflammatory Bowel Disease (IBD) and Multiple Sclerosis (MS). The candidate, ZYIL1, has an acceptable ADME profile, with a good safety margin. In Phase I studies, ZYIL1 was found to be safe and well-tolerated [NCT04731324, NCT04972188].

Zydus has established the Phase 2 proof-of-concept in CAPS patients [NCT05186051] and has now published the data in Clinical Pharmacology in Drug Development. The USFDA has granted Zydus an 'Orphan Drug Designation' for ZYIL1 to treat patients with Cryopyrin Associated Periodic Syndrome (CAPS), a rare auto-inflammatory disease.

Reference:

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. 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), Clinical Pharmacology in Drug Development, 2022. DOI: 10.1002/cpdd.1162
5. 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.

About Zydus

The Zydus Group 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 nearly 25,000 people worldwide 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 path-breaking discoveries. For more details visit www.zyduslife.com

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