



PRESS RELEASE

Azafaros enters clinical stage with AZ-3102, an oral small molecule being developed for rare neurogenetic disorders

- **Phase 1 clinical study investigates safety and tolerability, pharmacokinetics and pharmacodynamic effects of first-in-class azasugar AZ-3102 in healthy subjects**
- **AZ-3102 is being initially developed for the treatment of patients suffering from GM1 and GM2 gangliosidoses, two rare neurogenetic lysosomal storage disorders**
- **Company announces receipt of an additional funding of EUR 2.75 M from lead investor Forbion in an extension of the Series A financing round**

Leiden, The Netherlands, April 6, 2021 – Azafaros B.V. announced today that the first cohort of healthy subjects has been dosed in a two-part Phase 1 clinical study with clinical candidate AZ-3102, a first-in-class small molecule compound being developed for the treatment of rare neurogenetic disorders.

This double-blind, placebo-controlled, Phase 1 study is evaluating the safety and tolerability of ascending single- (SAD) and multiple-doses (MAD) of AZ-3102 administered orally to healthy subjects. The pharmacokinetic properties of AZ-3102 and its pharmacodynamic effects on specific glycosphingolipid biomarkers will also be assessed. The results of this study will support establishing the dose levels and dosage regimen suitable for administration to patients in future clinical studies.

Azafaros' initial objective with AZ-3102 is to develop a potential disease-modifying therapy for GM1 and GM2 gangliosidoses, two rare life-threatening neurogenetic lysosomal storage disorders affecting infants, adolescents, and adults, for which there is only palliative care. Both conditions lead to the harmful accumulation of GM1 or GM2 gangliosides in multiple organs, in particular in the brain causing progressive neurological deterioration. AZ-3102 is an orally available azasugar designed to reach the central nervous system and to interfere with the metabolism of glycosphingolipids through a unique and selective dual mode of action with equally potent inhibition of glucosylceramide synthase (GCS) and non-lysosomal glucosylceramidase (GbA2). With this new paradigm, AZ-3102 has the potential to reduce metabolite accumulation and to ameliorate the function of the impaired lysosome.

Beside the clinical trial initiation, Azafaros announced that it has raised an additional EUR 2.75 million from the Company's lead investor Forbion, which exercised an option to increase its investment level, thus bringing the total funds raised in Azafaros' [Series A](#) to EUR 28.75 million.

"Reaching clinical stage with AZ-3102 is a major turning point for Azafaros and is the concrete result of a tireless team effort," said **Olivier Morand, PhD, Azafaros' Chief Executive Officer**. "Azafaros has a solid financial foundation to progress the development of our clinical candidate and advance further our pipeline of disease-modifying therapies for people living with rare metabolic disorders."

"Currently, GM1 and GM2 gangliosidoses can only be treated symptomatically and carry the inevitable risk of severe neurological impairments and early death, especially for infants and children," said **Carlo Incerti, MD, Azafaros's Chairman of the Board**. "With its novel dual mode of action and ease of oral

administration, AZ-3102 holds the potential of a convenient life-long therapy which could mitigate the underlying pathophysiology of these diseases and favorably alter the patients' clinical trajectory."

About Lysosomal Storage Disorders

Lysosomal storage diseases such as GM1 and GM2 gangliosidoses, Niemann-Pick disease Type C, or Gaucher disease, are a group of rare genetic metabolic disorders caused by lysosomal functional defects. Lysosomes are cellular compartments hosting a variety of enzymes, activators, and transporters. Physiologically, lysosomal catalytic enzymes are responsible for the turn-over and degradation of proteins, polysaccharides, nucleic acids, or lipids. Failure of the aforementioned enzymes due to genetic mutations, particularly in the central nervous system, results in the pathological accumulation of metabolites, the malfunction of diverse brain cells, and neuroinflammation, which are the underlying cause of severe symptoms including neurodevelopmental delays, seizures, respiratory infections, loss of vision and hearing, and cognitive dysfunction. For more information on lysosomal storage disorders, please see [here](#).

About AZ-3102

Azafaros' proprietary clinical candidate AZ-3102 is an orally available low-molecular weight azasugar, originally based on discoveries¹ from Leiden University and Amsterdam University Medical Center.

About Azafaros

Azafaros B.V. is supported by a syndicate of leading Dutch and Swiss investors including Forbion, BioGeneration Ventures, BioMedPartners and Schroder Adveq. Azafaros was founded in 2018 by BioGeneration Ventures and Olivier Morand with the support of experienced industry professionals and scientists aspiring to address rare genetic metabolic disorders through a pipeline of oral small molecules with disease-modifying potential. Leveraging the know-how and experience of its team and partners in orphan drug development, the Company is advancing its lead program into the clinic while further expanding its product pipeline into other rare metabolic diseases through its drug discovery efforts.

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¹ [Ghisadoobe et al., 2014, J Med Chem, doi: 10.1021/jm501181z](#)