Azafaros announces completion of 12-week Phase 2 RAINBOW study evaluating lead asset nizubaglustat in rare disease patients

- **Major milestone reached in bringing new treatment options to patients and their families.**

Leiden, The Netherlands, March 12, 2024 – Azafaros B.V. today announced the completion of its 12-week Phase 2 clinical study, RAINBOW (Phase 2 RAINBOW study NCT05758922).

The randomized, double-blind, placebo-controlled study, conducted in Brazil, involved 13 patients from the age of 12 years who are affected by GM2 gangliosidosis or Niemann-Pick disease type C (NPC). The aim of the RAINBOW study is to determine the safety, pharmacodynamics, and pharmacokinetics of two different doses of nizubaglustat in patients, in order to identify the target dosage for Azafaros' planned Phase 3 pivotal studies.

The main part of the study is now complete, and patients are entering the extension phase, in which all of them will be given the study drug. Top line results of the RAINBOW study are expected to be announced in Q2 2024 and presented to the scientific community later this year.

Prof. Dr. Roberto Giugliani, Chief of the Medical Genetics Clinical Research Group at the Hospital de Clinicas de Porto Alegre and Lead Principal Investigator for the study, said: *"This is an important milestone because nizubaglustat has a dual mode of action that represents a leap forward from other agents. This dual activity targets some of the fundamental biology of the diseases with high potency and it might offer promise in pivotal studies."*

Chris Freitag, Chief Medical Officer at Azafaros, said: *"This is a major milestone on our journey to provide patients and their families with a potentially transformative treatment. With the RAINBOW data, we will be able to identify the optimal dose for a pediatric population in our Phase 3 efficacy study, which we plan to initiate as soon as the dose is determined, and the required approvals are in place. We are looking forward to presenting the data from RAINBOW to the scientific community. Azafaros is grateful to the patients and their families who made the decision to participate in this study."*

About nizubaglustat

Nizubaglustat is a small molecule, orally available and brain penetrant azasugar with a unique dual mode of action, developed as a potential treatment for rare lysosomal storage disorders with neurological involvement, including GM1 and GM2 gangliosidoses and Niemann-Pick disease type C (NPC).

Nizubaglustat has received the following designations and support:

United States Food and Drug Administration (FDA)

Rare Pediatric Disease Designations (RPDD) for the treatment of GM1 and GM2 gangliosidoses and NPC.

Orphan Drug Designations (ODD) for GM2 gangliosidosis (Sandhoff and Tay-Sachs Diseases) and NPC.

Fast Track Designation and IND clearance for GM1 and GM2 gangliosidoses.

European Medicines Agency (EMA)

Orphan Medicinal Product Designation (OMPD) for the treatment of GM2 gangliosidosis.
UK Medicines and Healthcare Products Regulatory Agency (MHRA)
Innovation Passport for the treatment of GM1 and GM2 gangliosidoses.

About GM1 and GM2 Gangliosidoses
GM1 gangliosidosis and GM2 gangliosidosis (Tay-Sachs and Sandhoff diseases) are lysosomal storage disorders caused by the accumulation of GM1 or GM2 gangliosides respectively, in the central nervous system (CNS), resulting in progressive and severe neurological impairment and early death. These diseases mostly affect infants and children, and no disease-modifying treatments are currently available.

About Niemann-Pick Disease Type C (NPC)
Niemann-Pick disease type C (NPC) is a progressive, life-limiting neurological lysosomal storage disorder caused by mutations in the *NPC1* or *NPC2* gene and aberrant endosomal-lysosomal trafficking, leading to the accumulation of various lipids, including gangliosides in the CNS. The onset of disease can happen throughout the lifespan of an affected individual, from prenatal life through adulthood.

About Azafaros
Azafaros is a clinical-stage company founded in 2018 with a deep understanding of rare genetic disease mechanisms, a compound library from Leiden University, and led by a team of highly experienced industry experts. Azafaros aims to build a pipeline of disease-modifying therapeutics to offer new treatment options to patients and their families. By applying its knowledge, network and courage, the Azafaros team challenges traditional development pathways to rapidly bring new drugs to the rare disease patients who need them. Azafaros is supported by a syndicate of leading Dutch and Swiss investors including Forbion, BioGeneration Ventures, BioMedPartners, Asahi Kasei Pharma Ventures, and Schroders Capital.

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