Azafaros to present data from PRONTO study in patients with GM1 and GM2 gangliosidoses at the annual WORLD*Symposium*™

- Four posters with baseline data from the natural history (PRONTO) study in patients with GM1 and GM2 gangliosidoses to be presented
- Total of eight posters from Azafaros, including study design for the Phase 2 RAINBOW study investigating the company's lead product, nizubaglustat, in patients with GM2 gangliosidosis and Niemann-Pick disease type C (NPC) selected for presentation
- Separately, the results of the Phase 1 first-in-human study, testing the safety of nizubaglustat in healthy volunteers, was recently published in the leading peer-reviewed journal, *Molecular Genetics and Metabolism*

Leiden, The Netherlands, 23 January 2024 – Azafaros B.V. announced today it will present data from its PRONTO study in patients with GM1 and GM2 gangliosidosis at the annual WORLD*Symposium*™ in San Diego, a major international research conference on lysosomal disorders. PRONTO is a landmark study and the largest prospective natural history investigation in these patients.

Out of a total of eight posters, four will focus on data from the natural history study designed to gain more insight into these rare Lysosomal Storage Disorders. Azafaros is in the clinical stage of developing its lead asset, nizubaglustat, as a potential treatment option for high unmet medical needs in GM1, GM2 and NPC diseases. Nizubaglustat has been shown to have an excellent tolerability profile and target engagement, systemically, and crucially, in the central nervous system.

As well as data from PRONTO, Azafaros will present details of its Phase 2 RAINBOW study, aimed at investigating the safety, tolerability, pharmacokinetics, and pharmacodynamics of nizubaglustat in patients with GM2 gangliosidosis or NPC.

Details of the eight poster presentations at the WORLD*Symposium*™ are as follows:

→ Title: The use of wearable sensor technology to identify digital biomarkers for monitoring gait parameters in children and adolescents with GM1 and GM1 gangliosidoses.
→ Title: A natural history study of late-infantile and juvenile GM1 and GM2 gangliosidoses (PRONTO) - patients' and caregivers' assessments.
→ Title: A natural history study of late-infantile and juvenile GM1 and GM2 gangliosidoses (PRONTO)- baseline clinical data.
→ Title: A natural history study of late-infantile and juvenile GM1 and GM2 gangliosidoses (PRONTO) - evaluation of different assessments.
→ Title: A Phase 2 clinical study in Niemann-Pick Type C and GM2 patients (RAINBOW).
→ Title: Sleep disturbance is an important feature of neurological lysosomal disease: for patients and families.
→ Title: Swallowing disturbance is an important feature of neurological lysosomal disease - for patients and families.
→ Title: Nizubaglustat regulates GM1 ganglioside neuronal health in a human brain organoid model of GM1 gangliosidosis.

Additionally, Azafaros is pleased to announce that the results of the first-in-human Phase 1 study investigating the safety of nizubaglustat in healthy volunteers were also recently published in the esteemed academic journal, *Molecular Genetics and Metabolism*.

"We are very pleased to have eight posters selected for presentation at the WORLD*Symposium*™, the leading congress for lysosomal diseases presenting the latest advances in basic science, translational research and clinical trials in this area," said Dr Christian Freitag, Chief Medical Officer at Azafaros.

"We are also thrilled that results from our first-in-human study with nizubaglustat in healthy volunteers have been published in the prestigious journal, *Molecular Genetics and Metabolism.* These results showed the excellent tolerability profile of our lead compound coupled with target engagement, systemically, and most importantly, in the central nervous system.

"The results from PRONTO, a landmark study, have been intrinsic in informing our development plans for nizubaglustat - including the ongoing Phase 2 RAINBOW study - and represent a significant step forward in our quest for novel treatment options for those patients with GM1 and GM2 gangliosidoses and NPC diseases, where there is a high unmet medical need and currently very limited treatment options."

About nizubaglustat
Nizubaglustat is a small molecule, orally available and brain penetrant with a unique dual mode of action, with the potential to treat multiple 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 (BGV), BioMed Partners, Asahi Kasei Pharma Ventures, and Schroders Capital.

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