Azafaros Strengthens Medical and Scientific Management Team Reflecting Clinical Progress

Leiden, The Netherlands, January 9, 2023 – Azafaros B.V. today announced two key appointments, further strengthening its senior management team. Dr. Christian Freitag joins the company as Chief Medical Officer and Dr. Kyle Landskroner has been promoted to Chief Scientific Officer. With their comprehensive expertise in clinical and early-stage drug development, they will support the transition of Azafaros' lead program, AZ-3102, into late-stage clinical evaluation and the expansion of Azafaros' pipeline.

Christian Freitag, a medical doctor, brings over 20 years of experience in the pharmaceutical industry with positions in companies including Hoffmann La Roche, Shire, and BTG, where he led global clinical development projects. Prior to joining Azafaros, Dr. Freitag was Chief Medical Officer at Dynacure, where he was responsible for medical and regulatory strategy, including clinical development of the lead compound in Myotubular and Centronuclear Myopathies (CNM). Dr. Freitag replaces Dr. Ruben Giorgino, who is embarking on a new career path.

Christian Freitag, Chief Medical Officer of Azafaros, commented: "Joining Azafaros at this time is very exciting. The company has important projects, including the collection of highly relevant data on the Natural History of GM1 and GM2 gangliosidosis and the execution of the AZ-3102 Phase 2 study for the treatment of GM2 and Niemann-Pick disease type C. We are also highly encouraged to move ahead in our planning for the Phase 3 trial, in light of the promising data from pre-clinical and Phase 1 studies."

To further strengthen the executive team as the company heads into the next stage of clinical development, **Dr. Kyle Landskroner, Chief Scientific Officer,** has been promoted from his previous position as Head of Pre-Clinical Drug Development. Dr. Landskroner will be responsible for advancing Azafaros' products into clinical development.

Stefano Portolano, Chief Executive Officer of Azafaros, said: "I am very pleased to announce the strengthening of the management team with both new and established competencies. With the recent IND clearance and Fast Track designation from the FDA, we are poised to move our lead product AZ-3102 into Phase 2. Both Christian and Kyle will bring valuable insight and experience as Azafaros advances into the next level of clinical development on our mission to bring disease-modifying treatment options to patients in need. I would like to take this opportunity to thank Ruben for the significant contribution he has made in bringing the company to the stage it is today."

Further information on Dr. Christian Freitag and Dr. Kyle Landskroner can be found here.

About AZ-3102

AZ-3102 is a therapeutic candidate developed for people affected by Lysosomal Storage Disorders (LSDs) with neurological involvement. AZ-3102 is an orally available, brain penetrant azasugar, engineered to have a unique dual mode of action by inhibiting two key enzymes which modulate the metabolism of glycosphingolipids.

In 2022, the compound received Fast Track Designation for GM1 and GM2 gangliosidoses as well as Niemann-Pick disease Type C (NP-C), and Orphan Drug Designations (ODD) for GM2 gangliosidosis (Sandhoff and Tay-Sachs Diseases) and NP-C from the FDA.

About Lysosomal Storage Disorders

Lysosomal storage disorders are a group of over 70 diseases that are characterized by lysosomal dysfunction, most of which are inherited as autosomal recessive traits. These disorders are individually rare but collectively affect 1 in 5,000 live births. They typically present in infancy and childhood, although adult-onset forms also occur.

They are caused by genetic mutations affecting the function of specific enzymes, transporters, receptors, or hormones involved in metabolizing and transporting the body's building blocks such as sugars, proteins, and lipids.

These malfunctions can impair either the assembly of crucial metabolic end-products which are needed for the normal function of the body or lead to harmful accumulations of intermediate metabolites.

GM1 gangliosidosis and GM2 gangliosidosis (Tay-Sachs and Sandhoff diseases), Niemann-Pick, Krabbe, Farber, Fabry and Gaucher diseases are examples of lysosomal lipid storage disorders.

GM1 gangliosidosis and GM2 gangliosidosis (Tay-Sachs and Sandhoff diseases) are 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.

Niemann Pick disease type C (NP-C) is a progressive, life-limiting neurological condition caused by mutations in the NPC1 or NPC2 genes and aberrant endosomal-lysosomal trafficking, leading to the accumulation of various lipids, including gangliosides in the CNS. The onset of disease happens throughout the lifespan, from prenatal life through adulthood. The mainstay of therapy is symptom management.

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 patients and their families new treatment options. The company's lead clinical-stage program is AZ-3102, a small molecule azasugar, orally available and brain penetrant, with the potential to treat GM1 gangliosidosis and GM2 gangliosidosis (Tay-Sachs and Sandhoff diseases) and Niemann Pick disease type C (NP-C). By applying its know-how, 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 and Schroder Capital.

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