



## **Azafaros Announces Multiple Presentations at 18<sup>th</sup> Annual WORLDSymposium™**

### **- Presentations Include Initial First-in-Human Data on Lead Candidate AZ-3102 in Healthy Volunteers -**

**Leiden, The Netherlands, January 18, 2022** – Azafaros B.V. today announced its participation at the 18<sup>th</sup> Annual WORLDSymposium™, a research conference dedicated to lysosomal storage diseases, taking place from February 7 – 11, 2022, in San Diego, CA. The conference is also offering virtual participation. Azafaros will share first-in-human data on its lead candidate AZ-3102, a novel orally available azasugar molecule with a unique dual mode of action, as well as the design for a natural history study in GM1 and GM2 gangliosidosis and preclinical *in vivo* data on AZ-3102 in Niemann-Pick and Sandhoff disease mouse models.

Details on the presentations are as follows:

**Title:** *A first-in-human, randomized, double-blind, placebo-controlled, ascending single- and multiple dose study to evaluate the safety, tolerability, pharmacokinetics and pharmacodynamics of the L-ido azasugar AZ-3102 in healthy volunteers; Virtual Poster LB- 54.*

**Presenter:** Cecile Paquet Luzy

**Date & Time:** Wednesday February 9 from 3:00 – 5:00 pm Pacific Time (PT)

**Title:** *Prospective longitudinal study of neurological disease trajectory in children living with late infantile or juvenile onset of GM1 or GM2 gangliosidosis (PRONTO study); In Person Poster 98.*

**Presenter:** Roberto Giugliani

**Date & Time:** Wednesday February 9, from 3:00 – 5:00 pm PT

**Title:** *Characterization of AZ-3102, a novel brain-penetrant small molecule, in the Niemann-Pick disease type C mouse model; Contemporary Forum Platform Presentation, Poster 161.*

**Presenter:** Kyle Landskroner

**Date & Time:** Thursday, February 10 at 2:00 pm PT

**Title:** *AZ-3102, a novel brain-penetrant small molecule, significantly improves survival of Sandhoff disease mice; Late-Breaking Science Platform Presentation, Poster LB-70*

**Presenter:** Jagdeep Walia

**Date & Time:** Friday February 11 at 11:15 am PT

The posters will be available on the [Azafaros website](#) following presentation at the WORLDSymposium™ conference.

### **About Azafaros**

Founded in 2018 with a deep understanding of rare genetic disease mechanisms 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-staged program is AZ-3102, a highly differentiated, orally available, small molecule with the potential to treat GM1 and GM2 gangliosidosis and other metabolic disorders. 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.



**For further information:**

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