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Aro Biotherapeutics to Present New Preclinical Data Highlighting the Potential of Centyrin-siRNA Conjugates for the Treatment of Pompe Disease

Results of recent studies demonstrate that Aro's muscle-targeted Centyrin-siRNA conjugate robustly reduces toxic accumulation of muscle glycogen in a murine model of Pompe disease

Presentation and posters illustrate first proof-of-concept data for lead Centyrin therapy candidate, ABX1100 for the treatment of Pompe disease.

Aro anticipates commencing a first-in-human study for ABX1100 in mid-2023

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PHILADELPHIA--(BUSINESS WIRE)--Aro Biotherapeutics, a biotechnology company pioneering the development of tissue-targeted genetic medicines, today announced upcoming data presentations highlighting the therapeutic potential of its proprietary Centyrin-siRNA conjugate therapies for the treatment of Pompe disease. During the TIDES USA 2022 meeting taking place in Boston from May 9 to 12, Aro Biotherapeutics Co-Founder and CSO Karyn O'Neil, PhD, will deliver an oral presentation alongside two poster presentations by Aro Biotherapeutics' scientists.

Pompe disease is a rare, genetic disease characterized by debilitating muscle weakness that progresses over time and arises from a mutation in the enzyme responsible for breakdown of glycogen in the muscle, acid alpha-glucosidase (GAA). As a result of this mutation, patients with Pompe disease have elevated levels of glycogen which drive disease progression. Patients are currently treated with enzyme replacement therapy (ERT), with recombinant GAA administered intravenously. However, the inability to target and efficiently deliver ERT to skeletal muscle has limited the efficacy and safety profile of these therapies, and new medicines with novel mechanisms are needed to further improve patient outcomes.

Mittie Doyle, MD, FACR, Chief Medical Officer, Aro Biotherapeutics, noted, "Aro's preclinical data for ABX1100 are very encouraging, and we are excited to advance this first-in-class therapeutic with a novel mechanism of action into clinical trials next year. While treatment options exist in Pompe disease, they are limited and there is still great unmet need for the individual facing this disease."

Glycogen synthase 1 (GYS1) is an enzyme that is responsible for glycogen synthesis, and inhibition of GYS1 has been shown to reduce glycogen levels. Aro Biotherapeutics uses its Centyrin platform to achieve efficient and selective delivery of an siRNA targeting GYS1 to the primary site of disease, skeletal muscle, addressing the challenge associated with muscle-specific delivery of therapeutics. Across multiple in vivo studies evaluating Aro's Centyrin-GYS1 siRNA conjugate,

potent and muscle-specific reductions in GYS1 mRNA and protein have been observed in the Pompe mouse model. Additionally, skeletal muscle glycogen levels in the Pompe mouse treated with Aro's Centyrin-GYS1 siRNA conjugate were reduced to levels observed in age-matched normal mice.

"These data represent a significant milestone for Aro," said Susan Dillon, PhD, Co-Founder and CEO of Aro Biotherapeutics. "We have made considerable progress in advancing our Centyrin-oligonucleotide platform and in building our pipeline of novel therapies. These robust data in Pompe disease give us further confidence that our platform, which enables tissue-specific delivery of oligonucleotides and other genetic drugs, has potential to address a broad array of diseases."

Following are details on the Aro Biotherapeutics presentation and posters to be highlighted at the TIDES USA 2022 meeting:

Title: Centyrin-targeted siRNA conjugates demonstrate potential new therapeutic approach for reduction of skeletal muscle glycogen in Pompe disease
Presenter: Karyn O'Neil, PhD, Co-founder and CSO
Date of Presentation: May 11, 2022

Presentation Highlights:

- Aro's unique Centyrin-siRNA conjugate approach demonstrates durable and tissue-specific activity in muscle
- Centyrin-siRNA conjugates demonstrate lack of immunogenicity potential in human T cell screens, and are well tolerated in preclinical in vivo models
- Compelling preclinical data demonstrates efficient GYS1 mRNA and protein knockdown, leading to robust glycogen reduction in skeletal muscle in Pompe disease mouse model

Title: Design and synthesis of Centyrin-targeted siRNA conjugate as a potential treatment for Pompe Disease
Presenter: Swapnil Kulkarni, PhD, Associate Director, Chemistry
Date of Presentation: TIDES USA 2022 Poster Session

Title: Centyrins as novel platform for extrahepatic oligonucleotide delivery
Presenter: Yao Xin, PhD, Principal Investigator, Protein Engineering
Date of Presentation: TIDES USA 2022 Poster Session

About Aro Biotherapeutics

Based in Philadelphia, Aro Biotherapeutics is a biotechnology company pioneering the development of tissue-targeted genetic medicines with a platform based on a proprietary protein technology called Centyrins. The company is developing a wholly-owned pipeline of Centyrin-based therapeutic candidates and is working with industry partners to leverage Centyrins for tissue-specific targeting of therapeutics for a diverse set of diseases. For more information, visit www.arobiotx.com.

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