

ForeBatten Foundation Announces Pre-IND Meeting with FDA under Individualized ASO Products Guidance

ForeBatten Foundation announces a productive Pre-Investigational New Drug (IND) meeting on February 9, 2024, with the Food and Drug Administration evaluating the development plan for FBF-001 under the N of 1 guidance for individualized Antisense Oligonucleotide (ASO) drug products. The meeting provided direction and support for the ongoing development plan required to gain IND clearance for CLN3 c.569dupG mutation, expected in May 2024.

This **major milestone** was made possible by ForeBatten’s financial contributions funding basic scientific research on the common mutation found in ~85% of CLN3 Batten Disease and the unique c.569dupG mutation. “While our previous fundraising efforts have advanced us to this point and demonstrate the power of public support for research, there is an urgent need for additional funding to continue,” said David Kahn, ForeBatten Foundation.

A collaborative team from Rosalind Franklin University of Medicine and Science, Ionis Pharmaceuticals and other partners have achieved a groundbreaking advancement using a synthetic nucleotide sequence akin to RNA, known as ASO. **Their research has shown partial restoration of a defective gene linked to CLN3 Batten disease** – an enigmatic and fatal pediatric neurodegenerative disorder. The ASO was designed specifically for the unique c.569dupG mutation. Such ASO is being prepared for an IND application through a cooperative effort of scientists, clinicians, and an experienced drug development team. **We are enthusiastic about the trail we are blazing, which unlocks new possibilities for personalized RNA-based treatment solutions.** Specifically, this ASO was engineered to correct a particularly rare mutation tied to the disease. Considerable research is being conducted in parallel to refine an ASO approach for the common mutation, with current findings giving hope for an ASO-based therapy soon.

Guided by the ForeBatten Scientific Advisory Board, a passionate and dedicated Drug Development Team was assembled including members from Michelle Hastings’ Lab, Ionis Pharmaceuticals, Vanguard Clinical, BioDev Consulting, Keane Consulting, SciLucent, and University of North Carolina, School of Medicine – Neurology. The Team has been diligently and methodically working towards crafting **an N of 2 ASO clinical trial** specific to the CLN3 c.569dupG mutation of which there are only two known patients. **“This Team delivered an encouraging Pre-IND meeting on a best-in-class timeline.** Thanks to the support of our loyal donors over the past seven years, the foundation’s mission to explore treatment options is within reach,” said Carol Schwimmer, ForeBatten Foundation.

The drug manufacturing along with in vivo pilot studies have been completed with great success. The final manufacturing and toxicology studies are expected to proceed in supporting treatment of the patients in Q2 2024. “The Development Team has been excellent in adapting to the rare advantages offered by the regulatory guidance and the power of ASOs. We expect our collaborative interactions with the FDA to continue throughout the IND process. The guidance strikes a great balance between the urgent need for the treatment and best practice drug development. **The Team is truly mission-driven not only in developing this treatment but also in establishing a road map for future individualized medicine,**” said Gavin Malenfant, ForeBatten Foundation Scientific Advisor.

“We believe that FBF-001 is paving a path towards a better future for newly diagnosed children and will be part of the evolving story of personalized medicine. **We hope this effort also provides proof of concept to develop an ASO treatment for the common CLN3 mutation,**” said Karen Kahn, ForeBatten Foundation.

About CLN3

CLN3 disease is an inherited disorder that primarily affects the nervous system. After 4 to 6 years of normal development, children with this condition develop vision impairment, intellectual disability, movement problems, speech difficulties, and seizures, which worsen over time. There is no current treatment for CLN3.

About ForeBatten Foundation

Founded in 2017, Forebatten Foundation has invested over \$5M in research toward understanding Batten disease and the development of CLN3 therapies. Our mission is to provide funding, support, and hope for the Batten community. ForeBatten Foundation is a 501(c)(3) tax-exemption organization, contributions to which are tax deductible to the full extent of the law.

The ForeBatten Foundation seeks funding and partnership opportunities in support of the development of both FBF-001 and a treatment for the common mutation. For further information, please contact Carol Schwimmer, ForeBatten Foundation, carol@forebatten.org