Sepul Bio, a business unit of Laboratoires Théa, Doses First Participant in Phase 2b Clinical Trial of ultevursen for USH2A-associated retinitis pigmentosa.

Clermont-Ferrand, France, December 12th, 2024 - Sepul Bio, an innovative business unit of Laboratoires Théa ("Théa")dedicated to the advancement of RNA therapies for inherited retinal diseases, has today announced the first clinical participant has been dosed in LUNA clinical study, a Phase 2b clinical study for ultevursen, in individuals with retinitis pigmentosa (RP) or non-syndromic RP due to variants in exon 13 of the Usher Syndrome Type 2a gene.

"Usher Syndrome type 2a mutations represent the most common cause of autosomal recessive RP and syndromic RP and there is currently no treatment for these patients," said Dr Mark Pennesi, Director of Ophthalmic Genetics, Retina Foundation of the Southwest. "We are excited to take part and dose the first participant into this phase 2b LUNA study. The LUNA clinical study is assessing the safety and efficacy of ultevursen, an investigational RNA therapy, in individuals with RP due to mutations in exon 13 of the USH2A gene."

"The initiation of enrollment in the LUNA study is an exciting milestone for the Usher syndrome community," said Krista Vasi, MPA, Executive Director of the Usher Syndrome Coalition. "We have been eagerly waiting for the re-initiation of the ultevursen program, and today's announcement reflects renewed hope for the community. The Coalition hosts the USH Trust, the largest international contact database of individuals with Usher syndrome from 76 countries. Our reach further extends through our corps of 80 USH Ambassadors worldwide, who serve as the point of contact for the Usher community in their state or country. These valuable resources strategically position the Coalition as a key community partner of Sepul Bio. We look forward to working alongside the Sepul Bio team as they advance RNA therapies to potentially help children and adults who are living with specific mutations in the USH2A gene."

Retinitis pigmentosa (RP) is a clinically and genetically heterogeneous condition and mutations in over 100 genes have been implicated. Mutations in exon 13 of the USH2A gene result in both syndromic and non-syndromic (NSRP) forms of Retinitis pigmentosa. In NSRP, RP is not associated with other signs and symptoms as part of a genetic syndrome. Usher syndrome type 2A is an inherited disease that affects the retina and the inner ear and is the leading cause of combined deafness and blindness. RP initially manifests as decreased night vision in the first to second decade of life, due to degeneration of rod photoreceptors, progressive restriction of the VF when photoreceptor degeneration progresses, leading to a residual central island of vision, which ultimately progresses to complete blindness. Exon 13 mutations in the USH2A gene targeted by ultevursen cause vision loss in approximately 16,000 individuals in the Western world. There are currently no approved therapies for the treatment of RP due to mutations in exon 13 of the USH2A gene, and disease management is supportive; therefore, a significant unmet medical need exists in this sight-threatening condition.

About the Phase 2b LUNA study

LUNA, or SB-421a-006, is a two-year double-masked, randomized, sham-controlled study of ultevursen that will enroll 81 adults and children (over eight years of age) who have retinitis pigmentosa due to variants in exon 13 of the USH2a gene.

About Sepul Bio

As a dedicated business unit of Théa, Sepul Bio is at the forefront of advancing transformative RNA therapies for inherited retinal diseases, with a particular emphasis on the further development of two cutting-edge ophthalmic products—ultevursen and sepofarsen.

Ultevursen is an antisense oligonucleotide product is designed to target mutations in exon 13 of the *USH2A* gene, which encodes the usherin protein with the aim of potentially preventing or slowing down the progression of photoreceptor degradation and as such, preserving visual function in these patients.

Sepofarsen targets Leber congenital amaurosis 10 (LCA10), a rare genetic ailment causing severe visual impairment.

More information on www.sepulbio.com

About Théa

Théa is the leading independent European pharmaceutical company specialized in the research, development, and commercialization of eye care products. Based in Clermont-Ferrand, France, this family-owned and run company comprises about 2000 collaborators and has expanded by opening more than 35 affiliates and offices in Europe, North Africa, North and South America, and the Middle East. Its products are available in 75 countries.

More information on https://www.laboratoires-thea.com/en

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