Sepul Bio Update December 2024

Sepul Bio doses first participant in Phase 2b clinical trial of ultevursen for USH2A-associated retinitis pigmentosa

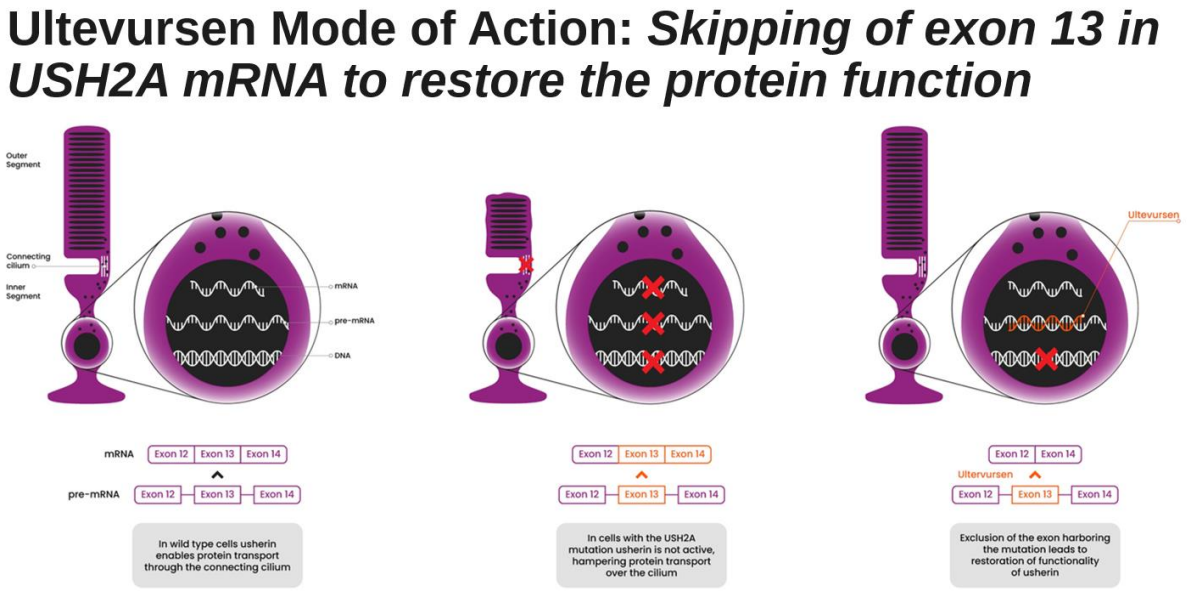
Sepul Bio, an innovative business unit of Laboratories Théa ("Théa") is dedicated to the advancement of mRNA therapies for inherited retinal diseases. Today announced the first clinical participant has been dosed in the LUNA clinical study, a Phase 2b clinical study for ultevursen, in individuals with retinitis pigmentosa (RP) or non-syndromic RP due to variants in exon 13 of the Usher Syndrome Type 2a gene.

LUNA

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. The first clinical site activated is in the United States, but over the coming months the Sepul Bio team will be opening sites around the world and will share these details as they are confirmed.

Ultevursen

The Sepul Bio messenger RNA or 'mRNA' therapies belong to a class of compounds known as antisense oligonucleotides (AONs) or ASO treatments. These mRNA therapies utilize short, synthetically designed RNA sequences that are chemically modified to enhance their stability and cellular uptake. mRNA is a type of single-stranded RNA involved in protein creation. mRNA is made from a DNA template during the process of transcription.



By precisely targeting specific RNA sequences associated with disease-causing genes, AONs can modulate gene expression and correct aberrant protein production. This approach has demonstrated efficacy in treating various genetic disorders, offering a promising avenue for personalized medicine and targeted therapeutics.

Several mRNA therapies utilizing AONs have already received regulatory approval and are being used to treat patients, underscoring the growing significance of this innovative therapeutic approach in modern medicine.

Sepul Bio

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

USH2A-associated retinitis pigmentosa is a devastating condition, and as a team, we are excited to advance ultevursen into our Phase 2b LUNA clinical study with the hopes of making a difference for the Usher community. The Sepul Bio team was established in December 2023 and has worked hard to re-start the ultevursen program. The clinical team look forward to quickly enrolling the study with the goal of advancing ultevursen's development and bringing this potentially novel therapy to the USH2A patient community as expeditiously as possible.

For individual, family, or community questions, please share the contact details: contact@sepulbio.com. Members of the IRD community will receive a direct response from the Sepul Bio team.

For more information on Sepul Bio, please visit our dedicated website (www.sepulbio.com) or follow us on LinkedIn: Sepul Bio: Overview | LinkedIn.