For immediate release

PRESS RELEASE

BOOST Pharma announces successful completion of FDA pre-IND meeting for its allogeneic cell therapy to treat children with rare bone disease Osteogenesis Imperfecta (OI)

- Full acceptance of proposed clinical development plan
- Preparations started for pivotal Phase III study to be executed in US and Europe
- Successful completion will trigger second milestone payment from investors

Copenhagen, November 5, 2024. BOOST Pharma, a clinical-stage biopharmaceutical company focused on the development of a novel therapy for rare genetic disease Osteogenesis Imperfecta (OI), announced today the receipt of the official meeting minutes from a recent pre-IND / End-of-Phase II meeting for its allogeneic cell therapy with the US Food and Drug Administration (FDA). This positive FDA feedback will trigger a second milestone payment to the Company from its existing investors.

The primary objective of the FDA meeting was to present the BOOSTB4 Phase I/II clinical results and to seek FDA concurrence on the development plans and the design of the Phase III study, to be qualified as a registration trial. The FDA provided clear and constructive feedback in the form of written responses to the pre-IND package and questions submitted by BOOST Pharma. Based on the positive feedback from the FDA, BOOST Pharma will now start preparing the IND to be submitted to the FDA, which will include a full study protocol for Phase III.

BOOST Pharma is developing a first-in-class and potentially groundbreaking cell-based treatment for the congenital disease Osteogenesis Imperfecta (OI), also known as Brittle Bone Disease, a condition characterized by fragile bones, constant fractures and bone deformity. The treatment is based on a novel cell therapy using human stem cells with high bone-forming capabilities. The treatment is designed to be administered directly upon diagnosis, either before or right after birth, providing a potential advantage in the early years of life, when most bone fractures occur. The OI therapy in development is the first treatment to target the underlying causes of the disease instead of the symptoms. The therapy has received Rare Pediatric Disease designation in the U.S. and Orphan Drug Designation in both the US and EU.

NOTES FOR EDITORS

About BOOST Pharma ApS

BOOST Pharma is a clinical-stage biopharmaceutical company focused on the development of novel cell therapy treatments. The company is currently developing a first-in-class therapy to treat Osteogenesis Imperfecta, a serious, inherited rare genetic disease leading to severe physical disability.

About Osteogenesis Imperfecta

Osteogenesis Imperfecta (OI), also know as Brittle Bone Disease, is a rare and devastating genetic disease, with currently no approved therapies. OI is characterized by fragile bones and reduced bone mass resulting in bones that break easily, loose joints, and weakened teeth. In severe cases, those with OI may experience hundreds of fractures in a lifetime. In addition, people with OI often suffer muscle weakness, early hearing loss, fatigue, curved bones, scoliosis, respiratory problems, and short stature, leading to significant effects on overall health and quality of life. Current treatment of OI is only supportive, focusing on minimizing fractures and maximizing mobility, but to date, there are no FDA or EU approved treatments. OI is estimated to affect 1 in 15,000 people globally.

About BOOSTB4 Clinical Study

“Boost Brittle Bones Before Birth” (BOOSTB4) is an exploratory, open label, multiple dose, multicenter Phase I/II trial evaluating safety and efficacy of postnatal, or prenatal and postnatal administration, of allogeneic expanded human stem cells for the treatment of severe Osteogenesis Imperfecta (OI) compared with a combination of historical and untreated prospective controls. The aim is to develop a first-in-class cell therapy to reduce the severity of inherited OI in unborn and young children. The study received funding from the European Union’s Horizon 2020 Research and Innovation Program (681045) and from the Swedish Research Council (E0720901) with Karolinska Institute as study sponsor.

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