For immediate release

PRESS RELEASE

BOOST Pharma announces positive top-line results from Phase I/II BOOSTB4 study, demonstrating over 75% reduction of fracture rates in children born with rare bone disease Osteogenesis Imperfecta (OI)

- Top-line clinical data show that treatment is safe and well tolerated
- Over 75% reduction in bone fracture rate observed
- Latest clinical results and development program to be discussed with relevant regulatory authorities

Copenhagen, September 17, 2024. BOOST Pharma, a clinical-stage biopharmaceutical company focused on the development of a novel therapy for rare genetic disease Osteogenesis Imperfecta (OI), announces today positive top-line clinical data from Phase I/II BOOSTB4 study. The study demonstrated that the therapy is both safe and well tolerated following postnatal and prenatal administration. Importantly, it also showed a significant reduction by over 75% in bone fracture rate, a critical and relevant clinical endpoint.

In total 17 patients from seven European countries, and diagnosed with Type 3 & severe Type 4 OI, were treated at the Karolinska University Hospital over a period of twelve months. The study's primary objective was reached i.e. no treatment-related serious side effects were seen and the treatment was well tolerated. The key secondary endpoint regarding treatment efficacy showed a consistently meaningful over 75% reduction in bone fracture twelve months after the last dose was administered.

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 especially 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 U.S. and EU.

"It is very encouraging to see these latest results of a therapy that would give neonates and young children a first treatment option directly after the diagnosis of this severe and disabling disease." Prof. Dr Oliver Semler, Division of Pediatric Endocrinology, Metabolic Diseases and Osteology, Children's Hospital University of Cologne, Germany.

"*These latest results are very encouraging and support our mission to improve the quality of life for children born with OI and for whom there are currently no treatment options*" said Evert Kueppers, Chief Executive Office of BOOST Pharma.

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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