For immediate release

PRESS RELEASE

BOOST Pharma receives U.S. FDA Orphan Drug Designation for its innovative therapy to treat Osteogenesis Imperfecta

- FDA Orphan Drug Designation (ODD) underscores strength of BOOST Pharma's innovative therapy, as well as, high need for effective treatments for Osteogenesis Imperfecta (OI), a disease resulting in severe physical disability
- ODD allows BOOST Pharma to benefit from various incentives to develop BOOST cell therapy for OI, and may result in seven-year market exclusivity on approval for this indication
- The Company already obtained an ODD designation in Europe for the product from the European Medicines Agency (EMA)
- BOOST cell therapy is currently the only therapy in clinical development that treats underlying cause, and not the symptoms; it could potentially be used directly upon diagnosis of OI

Copenhagen, May 16, 2022. BOOST Pharma, a clinical-stage biopharmaceutical company focused on the development of novel cell therapy treatments, announces today that its BOOST cell therapy has been granted Orphan Drug Designation (ODD) by the U.S. Food and Drug Administration (FDA) for treatment of Osteogenesis Imperfecta (OI).

The FDA grants ODD to support the development and evaluation of new treatments for rare diseases. To give drug companies certain financial benefits for developing orphan drugs that are safe and effective, ODD confers numerous incentives including tax credits for qualified clinical trials, exemption from user fees and potential seven years of market exclusivity in the U.S. on marketing authorization approval.

An ongoing Phase I/II trial, called BOOSTB4, is evaluating safety and efficacy of postnatal, or prenatal and postnatal administration, of allogeneic expanded mesenchymal stem cells for the treatment of severe Osteogenesis Imperfecta (OI) compared with a combination of historical and untreated prospective controls. To date, all patients have been recruited, whilst DSMB safety review is positive. The aim is to develop a first-in-class cell therapy to decrease the severity of inherited OI in unborn and young children.

NOTES FOR EDITORS

About BOOST Pharma

Supported by the BioInnovation Institute (BII) in Copenhagen, Denmark, 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 between 1 in 15,000 people globally.

About BOOSTB4 trial

“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 mesenchymal 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 decrease the severity of inherited OI in un-born 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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