

## **Bone Therapeutics completes patient recruitment for pivotal interim analysis of Phase III Osteonecrosis trial with PREOB®**

**Positive results could lead to further validation of Company's bone-cell therapy platform**

**Results of interim data analysis expected Q3 2018**

**Gosselies, Belgium, 8 June 2017, 7am CEST – BONE THERAPEUTICS** (Euronext Brussels and Paris: BOTHE), the bone cell therapy company addressing high unmet medical needs in orthopaedics and bone diseases, today announces that it has completed the recruitment of the 44 treated patients required for the planned interim analysis of the Phase III trial for the treatment of osteonecrosis of the hip with its autologous bone cell therapy product, PREOB®. If results from this interim analysis, based on a 12-month follow up of patients document a strong efficacy signal for PREOB®, the study recruitment could be terminated prematurely. Results from the 12-month follow up are expected in Q3 2018.

The interim analysis will be based on an updated clinically relevant endpoint, endorsed by the European Medicines Agency, which reflects the value added to patients and uses a composite responder analysis combining the original co-primary variables, without impacting the design and data collection of the study. This more stringent primary endpoint criterion in the revised analysis has enabled a reduction of the number of patients to be enrolled, to 118 from 130. Also, as stated in the protocol approved by the Competent Authorities in the countries where the study is being conducted, a strong efficacy signal at the interim analysis could enable the early termination of the study. The study is underway at centres in Belgium, France, Germany, the Netherlands and the United Kingdom.

The Phase III study is a randomized, double-blind, placebo-controlled pivotal trial that aims to confirm the safety and efficacy of PREOB® in early-stage osteonecrosis of the femoral head over a 24-month period. The patients received either a single administration of PREOB®, or a placebo into the necrotic lesion using a core decompression procedure. Results from an earlier exploratory Phase IIB osteonecrosis study, presented at the Annual European Congress for Rheumatology (EULAR) in June 2016, demonstrated a strong and prolonged improvement in pain and function as well as a reduction in fracture risk after treatment with PREOB® compared to standard of care.

Osteonecrosis is a rare but severe and painful orthopaedic condition characterised by the death of bone tissue, eventually causing the bone to fracture. The most common form of osteonecrosis occurs in the hip where the disease causes femoral head collapse and joint destruction. If left untreated, the condition ultimately requires a total hip replacement but, due to the age of people affected (typically between 30 and 50) hip replacement is not an appropriate long-term solution due to the limited lifespan of prostheses.

**Thomas Lienard, CEO of Bone Therapeutics, commented:** *“PREOB® has provided critical proof of concept for Bone Therapeutics in the development of its unique bone cell therapy platform. The completion of recruitment for the interim analysis in the PREOB® Phase III osteonecrosis trial is an important step in the validation of our platform, which is now in advanced clinical development for our off-the-shelf, allogeneic product ALLOB®. We look forward to announcing the outcome of the interim study in Q3 2018, following the completion of the twelve-month follow-up period and the potential to bring a new treatment option to patients suffering from this debilitating orphan disease.”*

*“We are proud to have treated the 44 patients needed for the interim analysis of the PREOB® Phase III trial in osteonecrosis,” commented Miguel Forte, Chief Medical Officer of Bone Therapeutics.* *“The new, robust optimised analysis we have adopted is more relevant from a patient outcome and clinical benefit perspective, compared with the initial approach and could enable us to reach an outcome with fewer patients, accelerating the progress of the study.”*

## ● **About Bone Therapeutics**

*Bone Therapeutics is a leading cell therapy company addressing high unmet needs in orthopaedics and bone diseases. Based in Gosselies, Belgium, the Company has a broad, diversified portfolio of bone cell therapy products in clinical development across a number of disease areas targeting markets with large unmet medical needs and limited innovation.*

*Our technology is based on a unique, proprietary approach to bone regeneration which turns undifferentiated stem cells into “osteoblastic”, or bone-forming cells. These cells can be administered via a minimally invasive procedure, avoiding the need for invasive surgery.*

*Our primary clinical focus is ALLOB<sup>®</sup>, an allogeneic “off-the-shelf” cell therapy product derived from stem cells of healthy donors, which is in Phase II studies for the treatment of delayed-union fractures and spinal fusion. The Company also has an autologous bone cell therapy product, PREOB<sup>®</sup>, obtained from patient’s own bone marrow and currently in Phase III development for osteonecrosis and non-union fractures.*

*Bone Therapeutics’ cell therapy products are manufactured to the highest GMP standards and are protected by a rich IP estate covering nine patent families. Further information is available at: [www.bonetherapeutics.com](http://www.bonetherapeutics.com).*

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