Bone Therapeutics' allogeneic cell therapy product, ALLOB, meets primary endpoints in Phase IIa study in patients undergoing a lumbar spinal fusion procedure

- **Treatment with ALLOB resulted in significant clinical and radiological improvements**
- **Treatment with ALLOB was well-tolerated, consistent with previous reported results**
- **At 12 months 73.3% of patients showed successful fusion and 86.7% at 24 months**
- **Confirmation of the broad potential of ALLOB to significantly improve treatment options in orthopaedics and bone diseases**
- **Bone Therapeutics' management and Professor Bronek Boszczyk will host a conference call today at 3 pm CEST. See details below**

Conference call and presentation

Bone Therapeutics' management and Professor Bronek Boszczyk will host a conference call today at 3 pm CEST. The presentation that will be used during the call can be accessed by clicking here. To participate in the Q&A, please dial one of the numbers below ten minutes in advance, using confirmation code **7885631**. The conference call will be conducted in English.

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Gosselies, Belgium, 13 June 2019, 7 am CEST – BONE THERAPEUTICS (Euronext Brussels and Paris: BOTHE), the leading biotech company focused on the development of innovative cell and biological therapeutics to address high unmet medical needs in orthopaedics and bone diseases, today announces that its allogeneic cell therapy product, ALLOB, successfully completed the 12 months follow-up period of a Phase IIa study in patients undergoing a lumbar spinal fusion procedure.

This multi-centre, open-label proof-of-concept Phase IIa study was designed to evaluate the safety and efficacy of ALLOB administered in addition to the standard of care procedure in which an interbody cage with bioceramic granules is implanted into the spine to achieve fusion of the lumbar vertebrae. The primary endpoints of the study assessed at 12-month included radiological assessments to evaluate fusion (continuous bone bridges) and clinical assessments to evaluate improvement in patients' functional disability. The secondary endpoints included the assessment of intervertebral mobility (absence of motion at the treated lumbar level), back and leg pain reduction, as well as safety and tolerability. The study evaluated 30 patients treated with ALLOB in combination with standard of care procedure.

Radiological data collected from CT-scans over a 12-month period showed successful fusion ($p < 0.001$) of the lumbar vertebrae in 22 out of 30 patients (73.3%), while the remaining 8 patients showed evidence of bone formation. For the first 15 patients who already reached the 24-month follow-up time point, 13 out of 15 patients (86.7%) showed successful fusion. Furthermore, treatment with ALLOB resulted in a clear and statistically significant clinical improvement from the pre-treatment baseline in functional disability, with a mean score improvement of 63.0% ($p < 0.001$) on the Oswestry Disability Index[(1)].

In addition, radiological data collected from dynamic X-rays at 12 months demonstrated that treatment with ALLOB resulted in the immobilisation of the treated intervertebral segment in all patients. Finally, treatment with ALLOB resulted in a strong reduction in back and leg pain of 67.0% and 75.0% respectively.

The results from the study demonstrated that treatment with ALLOB was generally well-tolerated. As previously described in the literature covering clinical studies with allogeneic mesenchymal stem cells or their derivatives, pre-existing or treatment-emergent antibodies have been detected in 64.5% of patients, however no clinical consequences were observed.

These strong results are an improvement (60.0% to 73.3%) compared to the Phase IIa spinal fusion 12-month interim analysis reported in September 2017. Moreover, the results are in line with the Phase I/IIa study with ALLOB in patients with delayed union fractures reported in September 2018, confirming ALLOB's potential to significantly improve treatment options in orthopaedics and bone diseases.

Professor Bronek Boszczyk, Head of the Spinal Surgery Benedictus Clinic, Visiting professor at Nottingham Trent University, Director of NSpine, commented: "*These strong results demonstrate that treatment with ALLOB is well-tolerated and provides a clinically meaningful improvement supported by radiological evidence in patients undergoing a spinal fusion procedure. Degenerative spine disorders put a high burden on patients and their families and there is a high unmet medical need for safe and effective procedures to eliminate painful motion and restore stability of the spine. ALLOB, in combination with the current standard procedure, has the potential to address these needs and to become a more convenient alternative, offering a safe and significant improvement over spine fusion surgery alone.*"

Thomas Lienard, CEO of Bone Therapeutics, added: "*The positive outcome from this study further validates the potential of our unique allogeneic cell therapy platform to address high unmet medical needs in orthopaedics and bone related disorders. We will now evaluate these positive results with the regulatory agencies. Next clinical development steps that are required for ALLOB in this indication will be discussed later on. In addition, we are on track to submit the clinical trials application to start the Phase II/III program with ALLOB in patients with fractures at risk of delayed or non-union by the end of this year.*"

About Spinal Fusion

Due to ageing populations and sedentary lifestyles, the number of people suffering from degenerative spine disorders continues to increase. Today, spinal fusion procedures are performed to relieve pain and improve patient daily functioning in a broad spectrum of degenerative spine disorders. Spinal fusion consists of bridging two or more vertebrae with the use of a cage and graft material, traditionally autologous bone graft or demineralised bone matrix – placed into the intervertebral space – for fusing an unstable portion of the spine and immobilizing a painful intervertebral motion segment. Over 1,000,000 spinal fusion procedures are performed annually in the US and EU, of which half at lumbar level and the market is growing at a rate of 5% per year. Although spinal fusion surgery is routine, non-fusion, slow progression to fusion and failure to eliminate pain are still frequent with up to 35% of patients not being satisfied with their surgery.

About ALLOB and the Company's proprietary, scalable cell therapy manufacturing process

ALLOB is the Company's off-the-shelf allogeneic cell therapy platform consisting of human allogeneic bone-forming cells derived from cultured bone marrow mesenchymal stem cells (MSC) from healthy adult donors, offering numerous advantages in product quality, injectable quantity, production, logistics and cost as compared to an autologous approach. To address critical factors for the development and commercialisation of cell therapy products, Bone Therapeutics has established a proprietary, optimised production process that improves consistency, scalability, cost effectiveness and ease of use of ALLOB. This optimized production process significantly increases the production yield, generating 100,000 of doses of ALLOB per bone marrow donation. Additionally, the final ALLOB product will be cryopreserved, enabling easy shipment and the capability to be stored in a frozen form at the hospital level. The process will therefore substantially reduce overall production costs, simplify supply chain logistics, improve patient accessibility and facilitate global commercialisation. The Company will implement the optimized production process for all future clinical trials with ALLOB.

(1) The Oswestry Disability Index (ODI) is an index derived from the Oswestry Low Back Pain Questionnaire used by clinicians and researchers to measure a patient's permanent functional disability. This validated questionnaire was first published by Jeremy Fairbank et al. in *Physiotherapy* in 1980. ODI score of 0%-20%: minimal disability; 21%-40%: moderate disability; 41%-60%: severe disability; 61%-80%: crippled; 81%-100%: bed bound.

About Bone Therapeutics

Bone Therapeutics is a leading biotech company focused on the development of innovative products to address high unmet needs in orthopaedics and bone diseases. Based in Gosselies, Belgium, the Company has a broad, diversified portfolio of bone cell therapy and an innovative biological product in later-stage clinical development across a number of disease areas, which target markets with large unmet medical needs and limited innovation.

Bone Therapeutics' core technology is based on its allogeneic cell therapy platform (ALLOB) which uses a unique, proprietary approach to bone regeneration, which turns undifferentiated stem cells from healthy donors into bone-forming cells. These cells can be administered via a minimally invasive procedure, avoiding the need for invasive surgery, and are produced via a proprietary, cutting-edge manufacturing process.

The Company's ALLOB product pipeline includes a cell therapy product candidate that is expected to enter Phase II/III clinical development for the treatment of delayed-union fractures and a Phase II asset in patients undergoing a spinal fusion procedure. In addition, the Company is also developing an enhanced viscosupplement, JTA-004, which is expected to enter Phase III development for the treatment of pain in knee osteoarthritis.

Bone Therapeutics' cell therapy products are manufactured to the highest GMP (Good Manufacturing Practices) standards and are protected by a broad IP (Intellectual Property) portfolio covering ten patent families as well as knowhow. Further information is available at www.bonetherapeutics.com.

For further information, please contact:

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