Bone Therapeutics SA : Reports strong interim results from ALLOB® Phase IIA spinal fusion study

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Bone Therapeutics reports strong interim results from ALLOB® Phase IIA spinal fusion study

Significant clinical and radiological improvements compared to baseline

Results pave the way for future development in the growing and significant lumbar spinal fusion market

Gosselies, Belgium, 14 September 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 reports strong interim efficacy and safety results for the Phase IIA lumbar spinal fusion trial with its allogeneic cell therapy product, ALLOB®.

Results for the first 15 patients in the study show evidence of successful fusion and important clinical improvements in function and pain after the 12-month follow-up period.

The Phase IIA trial in lumbar spinal fusion is designed to evaluate the safety and efficacy of the addition of ALLOB® to the standard of care procedure in which an interbody cage with bioceramic granules is implanted to achieve fusion of the lumbar vertebrae. Endpoints of the study include radiological assessments, with the evaluation of fusion (CT-scan) and intervertebral mobility (dynamic X-rays); clinical assessments, with the improvement in functional disability and reduction in pain; and safety assessments. 16 patients were eligible but 15 were treated as one patient was withdrawn due to a last-minute change in surgical procedure, which was unrelated to the study.

From a radiological perspective, dynamic X-rays reveal absence of motion at the treated level in all of the 15 patients at 12 months. Continuous bone bridges (or fusion) were observed by CT-scans in 9 out of 15 patients 12 months after the surgery, while the remaining 6 patients showed evidence of bone formation without continuous bony bridging. Clinically, a clear and statistically significant improvement in functional disability from the pre-treatment baseline was observed at 12 months using the Oswestry Disability Index, with a mean score improvement of 55%. In addition, back and leg pain was strongly reduced, by 59% and 90% respectively.

From a safety perspective, treatment with ALLOB® was well tolerated in all patients. As previously described in the literature covering clinical studies with allogeneic mesenchymal stem cells or their derivatives, it was observed that blood samples of about half of the patients contained donor-specific antibodies, either pre-existing or developed after administration, however no clinical consequences were observed.

Recruitment will continue in the study to allow the Company to perform a final analysis on 32 patients with completion of recruitment expected by the end of 2017 or early 2018.

Thomas Lienard, Chief Executive Officer of Bone Therapeutics commented: "*These positive data add to a growing body of evidence on the safety and efficacy of ALLOB®, giving us confidence in our progress towards commercialization. Degenerative spinal conditions are extremely debilitating, represent a large and growing market and current treatments can be sub-optimal. Therefore, the*

potential for ALLOB® to enhance the standard of care is an important step forward in the treatment of these conditions."

Miguel Forte, Chief Medical Officer of Bone Therapeutics commented: "*The clinically significant improvement in functional disability together with a marked reduction in back and leg pain are supportive of the feasibility of Bone Therapeutics' allogenic cell therapy product in spinal fusion. These initial clinical and radiological results and the tolerability of the treatment, make the Company confident in continuing this open-label, Phase IIA study and bringing this innovative treatment approach to patients.*"

The Company intends to capitalize on these strong interim Phase IIA results for lumbar spinal fusion and will aim at delivering a body of well controlled data on the application of ALLOB® in this major indication. As a consequence, the Company will halt the recruitment for its exploratory study in rescue treatments for failed spinal fusion and close the study upon completion of the follow-up of existing patients.

Bone Therapeutics also announced today that Miguel Forte, Chief Medical Officer, has informed the Board of his intention to pursue a new opportunity outside the Company. Miguel will leave his position at the end of October to take up a new role as Chief Executive Officer of a non-competing biotechnology company. Bone Therapeutics has already begun the search for a replacement and will make an announcement in due course. Guy Heynen, Chief Clinical and Regulatory Officer will act as Interim Chief Medical Officer until the transition is complete.

About Spinal Fusion

Spinal fusion is considered as the gold standard surgery for treating a broad spectrum of degenerative spine disorders, including degenerative disc disease, spondylolisthesis, scoliosis and stenosis, to relieve pain and improve function. Spinal fusion consists of bridging two or more vertebrae with the use of a cage and graft material, traditionally autologous bone graft, - placed into the intervertebral space - for fusing an unstable portion of the spine or immobilizing a painful vertebral motion segment. Although spinal fusion surgery is routine, non-union and failure to relieve lower back pain are still frequent and up to 35% of spinal fusion patients are not completely satisfied with their surgery (Owens et al. 2016 and Aghion et al. 2012).

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®, 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®, 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.

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