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Press release

New advanced therapy to repair cartilage defects in the knee

Spherox recommended for marketing authorisation

The European Medicines Agency (EMA) has recommended granting a marketing authorisation in the European Union (EU) for a new advanced therapy medicinal product (ATMP) to treat adult patients who have symptomatic articular cartilage defects in the femoral condyle (the ball-shaped end of the thigh bone in the knee) and the patella (knee cap), where the size of the affected area is no larger than 10 cm².

Damage to the articular cartilage of the knee is a common orthopaedic problem which often occurs in young, active people. It can result from direct trauma, repetitive injuries, fractures, or degenerative and inflammatory conditions. People with this type of damage experience recurrent pain and swelling of the joint, locking of the knee and may be impaired in their ability to walk or participate in sports. To restore functionality of their knee, patients often opt for surgery which aims to fill the cartilage loss.

Spherox is an ATMP composed of spheroids, i.e. spherical aggregates of chondrocytes (cells that are found in healthy cartilage). In this therapy, a small piece of cartilage is excised from the healthy cartilage and chrondrocyte spheroids are produced in a laboratory. These spheroids are then inserted arthroscopically in the patient knee where they form new tissue to heal the defect.

The effects of Spherox were studied in two clinical trials, with patients between 18 and 50 years of age. The first study, a phase II trial, included 75 patients with cartilage defect sizes from 4 to 10 cm² and, the second, a phase III trial, involved 102 patients with defect sizes from 1 to 4 cm². The effect of the treatment was assessed using the Knee injury and Osteoarthritis Outcome Score (KOOS) questionnaire which evaluates patients' views on their knee-associated problems, such as pain, impact on daily living, sport and recreational activities, and quality of life. Statistically significant improvement of the score was seen in both studies.

The most important adverse events reported in the studies were side effects that are often seen after surgery of the knee such as delayed wound healing, joint lock, joint effusion, and joint swelling, as well as very limited or single cases of venous thrombosis and pulmonary embolism.

To obtain more information on the longer-term effects of Spherox, the applicant committed to following up patients for another 60 months in the ongoing clinical studies.



Spherox was assessed by the Committee for Advanced Therapies (CAT), EMA's specialised scientific committee for ATMPs, such as gene or cell therapies. At its May 2017 meeting, the CAT recommended a positive opinion for Spherox to the Committee for Medicinal Products for Human Use (CHMP). The CHMP agreed with the CAT's recommendation and adopted a positive opinion for the authorisation of Spherox across the EU at its 15-18 May 2017 meeting.

The applicant received scientific advice from the Agency during the development of Spherox.

The opinion adopted by the CHMP at its May 2017 meeting is an intermediary step on Spherox's path to patient access. The CHMP opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once a marketing authorisation has been granted, decisions about price and reimbursement will take place at the level of each Member State, taking into account the potential role/use of this medicine in the context of the national health system of that country.

Notes

- 1. This press release, together with all related documents, is available on the Agency's website.
- 2. The applicant for Spherox is CO.DON AG.
- 3. ATMPs are innovative medicines that are derived from gene therapy, cell therapy or tissue engineering.
- 4. More information on the work of the European Medicines Agency can be found on its website: <u>www.ema.europa.eu</u>

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