NEWS RELEASE

Navepegritide Designated as an Orphan Drug by Japan's Ministry of Health, Labor and Welfare

Tokyo, Jun 3, 2025: Teijin Pharma Limited announced today that the Japanese Ministry of Health, Labor and Welfare (MHLW) has granted Orphan Drug designation to navepegritide (developed as TransCon CNP) for the intended indication of achondroplasia, a rare genetic skeletal dysplasia that affects bone growth and a range of medical complications. Teijin Pharma has licensed navepegritide from Ascendis Pharma, A/S, and is developing it for use in children with achondroplasia in Japan. This drug, which is administered subcutaneously, will be the first medication for the treatment of achondroplasia in Japan that can be administered once per week thanks to the use of TransCon technology[1] that prolongs C-type natriuretic peptide (CNP) action. Navepegritide is an investigational prodrug of C-type natriuretic peptide (CNP), designed to provide continuous exposure to active CNP.

Teijin Pharma plans to initiate a domestic Phase III clinical trial of navepegritide in the first half of fiscal year 2025. Ascendis Pharma filed its new drug application (NDA) for navepegritide in the United States in March 2025, and plans to submit a Marketing Authorization application (MAA) in Europe in the third quarter of 2025.

Achondroplasia is a rare genetic condition arising from a pathogenic fibroblast growth factor receptor 3 (FGFR3) variant, which causes serious muscular, neurological, and cardiorespiratory complications in addition to the well-characterized skeletal dysplasia. It is thought to affect approximately one in every 20,000 live births. It is designated as an intractable disease by the MHLW.[2] Currently, growth hormone therapy and treatment with CNP analogue may be prescribed for patients with achondroplasia who meet certain criteria. Surgery to lengthen the legs or arms may be performed to correct short legs or arms.[3]

Navepegritide demonstrated superior annualized growth velocity compared to placebo at Week 52 in the ApproaCH Trial, a pivotal clinical trial conducted by Ascendis Pharma in children with achondroplasia aged 2 to 11 years. [4]

In Japan, drugs can be designated as orphan drugs if they are intended for use in less than 50 000 patients in Japan and for which there is a high medical need. They are designated by MHLW based on the opinion of the Pharmaceutical Affairs and Food Sanitation Council (PAFSC).

Navepegritide has orphan designation for the treatment of achondroplasia in both the United States and Europe, and measures to support the development activities will be taken in Japan.

Teijin Pharma has been contributing to the improvement of patients' quality of life through the provision of various treatments for rare diseases. The company aims to help solve the challenges faced by patients, families and communities in need of greater support. The launch of new drugs for rare diseases is a key pillar of this vision.

1. https://ascendispharma.com/technology/
2. https://www.nanbyou.or.jp/entry/4570
3. https://jspe.umin.jp/public/nankotu.html
4. TransCon™ CNP (navepegritide) ApproaCH Trial Topline Results

About the Teijin Group

Teijin (TSE: 3401) is a technology-driven global group with two core businesses: high-performance materials and healthcare solutions. Established in 1918 as Japan’s first rayon manufacturer, Teijin today comprises some 170 companies employing 20,000 people in 20 countries. Teijin is committed to its Purpose, “Pioneering solutions together for a healthy planet.” Teijin works together with employees and external partners to achieve its Long-Term Vision, “To be a company that supports the society of the future.” Teijin posted consolidated revenue of JPY 1,005.5 billion and total assets of JPY 1,061.3 billion in the fiscal year ending March 31, 2025.

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