Novadip receives RMAT designation for NVD003, its regenerative treatment for congenital pseudarthosis of the tibia

Novadip is developing NVD003, an autologous therapy derived from adipose stem cells, as a potential single treatment to save limbs and restore mobility in patients with CPT

The RMAT designation is part of the IND that Novadip obtained for NVD003, with plans to reach the US market in 2027

Mont Saint-Guibert, Belgium, June 23, 2025 – Novadip Biosciences, a late-stage clinical biotechnology company specializing in regenerative medicine, today announces that the FDA has granted the Regenerative Medicine Advanced Therapy (RMAT) designation to its tissue regeneration product, NVD003, for the treatment of congenital pseudarthosis of the tibia (CPT).

The RMAT designation, issued to cell therapies, therapeutic tissue engineering products, and human cell and tissue products, demonstrates the FDA's confidence in Novadip's preliminary clinical evidence that NVD003 has the potential to address unmet medical needs in CPT, a rare pediatric bone condition. This designation can only be granted within the context of an IND, which Novadip received in 2021.

Supporting results for the FDA's RMAT decision came from a phase 1b/2a trial (NCT05693558) which treated four patients with CPT. Combining the 12-month results from this trial together with those from four other children previously treated with NVD003 in two compassionate use programs in Belgium, 88% of patients, most of whose prior surgeries had failed, achieved healing of their fractures with NVD003.

"The RMAT designation is a major achievement for Novadip on our path towards getting NVD003 on the market in 2027, especially in the US," said Denis Dufrane, MD, PhD, CEO of Novadip. "NVD003 perfectly matches the FDA requirements for this designation, as it is a tissue regeneration product intended as a treatment for CPT, a serious, rare and debilitating condition affecting children."

"Given the inherent novelty of regenerative therapies, there's often no regulatory precedence for what evidence is necessary to demonstrate efficacy in rare disease indications," said Judy Ashworth, MD, chief medical officer at Novadip. "Receiving RMAT designation for NVD003 to treat children with CPT gives us confidence that the FDA finds our approach and preliminary results compelling and that we are on the right track as we kick off our pivotal phase 3 trial this month."

In 2020, Novadip received Orphan Drug Designation and Rare Pediatric Designation from the FDA for NVD003 for the treatment of CPT, followed by Fast Track Designation in 2023.

Impacting less than 3.5 in 150,000 live births, CPT is a rare condition for which treatment is difficult. Once a fracture occurs, subsequent fractures are likely. While children with CPT can face impaired mobility and years of corrective surgeries to try to repair and stabilize the bone, it is not uncommon for patients to ultimately undergo amputation of the limb.

Before the end of June 2025, the first clinical site will open in a pivotal phase 3 trial in CPT with recruitment in the US and Europe. Enrollment of patients will start soon after.

NVD003 represents potential peak sales of $1.4bn for large bone defects in pediatric and adult patients. (Source: Evaluate analysis)

About NVD003

NVD003 is a three-dimensional (3D) osteogenic graft derived from autologous adipose derived mesenchymal stem cells (ASCs) combined with hydroxyapatite/beta-tricalcium phosphate (HA/TCP) particles. NVD003 was specifically developed to improve bone healing in severe pathophysiological conditions (e.g. hypoxia, lack of mineralized callus formation, bone resorption and low osteogenicity) as found in congenital pseudarthosis, bone tumors (after an extensive surgical resection), osteolytic syndromes like Gorham-Stout disease, genetic bone resorption syndromes with osteoporosis as found in Hajdu-Cheney syndrome and following severe trauma (casualties of war).

About Novadip Biosciences

Novadip is a late-stage clinical biotech company aiming at advancing the standard of care for patients undergoing bone and tissue regenerative treatment.

Based on the scientific discoveries of founder Prof. Dr. Denis Dufrane, MD, PhD, and research from UCLouvain and St. Luc University Hospital, the company is developing its unique 3M³ tissue regeneration technology platform, designed to create a new class of regenerative tissue products that accelerate the healing of large bone defects, bone non-union and spine fusion in a single treatment, for patients with limited or no treatment options.

Novadip's pipeline includes two lead products: NVD003, an autologous cell-based therapy currently in phase 1b/2a clinical trials in adults with bone non-union, and pediatric congenital pseudarthrosis of the tibia; and NVDX3, an allogenic bone grafting material currently in phase 1b/2a trials in trauma surgery and lumbar intervertebral spine fusion. Novadip is ready to start two phase 3 trials for NVD003 in the US and EU, and the FDA has granted approval to start a phase 2b/3 IND (Investigational New Drug) trial with NVDX3 in level two cervical spine fusion.

Founded in 2013 in Belgium, Novadip employs 45 staff. Since inception, it has raised €88 million in equity and non-dilutive funding. The company targets a total addressable market of $13.5 billion (€13.06bn).

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