Novadip reports 12-month results from phase 1b/2a trial of NVD003 to treat rare pediatric disease Congenital Pseudarthrosis of the Tibia

88% efficacy demonstrated across all patients (n=17) treated with NVD003 to date (nine adults with bone non-union and eight children with Congenital Pseudarthrosis of the Tibia)

100% efficacy demonstrated when mechanical stability of fracture is achieved

Company now ramping up activities for pivotal phase 3 trial

Mont Saint-Guibert, Belgium, January 28, 2025 – Novadip Biosciences, a clinical stage biotechnology company specializing in regenerative medicine, today announces the completion of the 12-month post-surgical visit of the last patient implanted with its investigational tissue regeneration product, NVD003, in the phase 1b/2a clinical trial (NCT05693558) which treated four patients with Congenital Pseudarthrosis of the Tibia (CPT), a rare pediatric bone condition. Novadip is developing NVD003, an autologous therapy derived from adipose stem cells (ASC), as a potential single treatment to save limbs and restore mobility in 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 whom had prior failed surgeries, achieved healing of their fractures with NVD003.

"I am very pleased with the results I've seen in the children I have treated with NVD003," said Prof. Pierre-Louis Docquier, MD, PhD, a specialist in pediatric surgery at the University Hospital Saint-Luc in Brussels (Belgium) and EU principal investigator for this study. "For orthopedic surgeons, CPT fractures are among the most difficult to treat due to the underlying pathophysiology of the condition."

At the end of 2022, Novadip announced positive data from a phase 1/2 clinical trial evaluating the safety and clinical activity of NVD003 in adult patients with severe Bone Non-Union (BNU) of the lower limb following trauma. In this trial, eight of the nine patients with recalcitrant BNU had durable bone healing at 24 months and beyond.

The two treatment failures (one in CPT and one in BNU) occurred in the setting of post-operative mechanical instability of the fracture.

"We are excited by these new results in CPT," said Denis Dufrane, MD, PhD, CEO of Novadip Biosciences. "We are now one step closer to being able to offer a safer and more efficacious bone graft to children who are at high risk of amputations."

"This data reinforces our confidence in NVD003 as we push forward with our pivotal phase 3 trial," said Judy Ashworth, MD, chief medical officer at Novadip Biosciences. "Pediatric surgeons desperately need more treatment options for children."

The company is selecting sites for its phase 3 pivotal trial in CPT with the aim of starting enrollment in Q2, 2025.

About CPT
Congenital Pseudarthrosis of the Tibia (CPT) is a very rare, debilitating and devastating condition. Its incidence is reported to be between 1:140,000 to 1:250,000 live births. Treatment of CPT is difficult and once a fracture occurs, subsequent fractures are likely. Children with CPT can face impaired mobility and years of corrective surgeries to try to repair and stabilize the bone. Congenital pseudarthrosis may also occur in other long bones (femur, ulna, radius). It is not uncommon for patients to ultimately undergo amputation of the affected limb.

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).

NVD003 aims to be on the market in 2027 and has a potential peak sales of $1.4bn (for large bone defects in pediatric and adult patients). (Source: Evaluate analysis)

About Novadip Biosciences
Novadip is a clinical stage biotech company aiming at advancing the standard of care for patients undergoing bone and tissue regenerative medicine.
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).
www.novadip.com

Media and analyst contact
Andrew Lloyd & Associates
Celine Gonzalez – Saffiyah Khalique
UK: +44 1273 952 481 / US: +1 203 724 5950