NERVOSAVE
THERAPEUTICS

NIKA BIOLOGY
INNOVATIVE THERAPIES

Executive Summary – April 2023

Nervosave is an early-stage biotech company incorporated in Montpellier, France & New York, USA, with a focus on the prevention and cure of myelin-related disorders.

Founded by Nicolas Tricaud, PhD, an internationally renowned and European Research Council laureate neuroscientist from France, the company has attracted highly seasoned directors and scientific advisors across the EU and US. The company plans to build a highly talented team, starting with a **high-profile US-based CEO**, and has just commenced their accelerator program at the Westchester Country Biosciences Accelerator (WCBA).

Nervosave's lead program, **NVO-101**, is a late-stage preclinical gene therapy program aimed at providing treatment for **Charcot-Marie-Tooth disease type 1A** ("CMT1A"). CMT1A is an inherited neurological disorder that affects the peripheral nerves, leading to muscle wasting at limb extremities. The disease is severely debilitating, and often causes severe pain, deformities of the hands and feet, and poor quality of life.

CMT1A is one of the most common inherited neurological disorder, representing 30% of all hereditary peripheral neuropathies, or over 68,000 patients in the United States and Europe. There is no currently approved pharmacological treatment option.

The disease is caused by the duplication of the Peripheral Myelin Protein 22 (PMP22) gene, resulting in dysfunctional myelin. Nervosave's fully patented gene therapy product is an **AAV9** viral vector expressing an interfering RNA (**shRNA**) that lowers the production of PMP22 mRNA. It is currently the most advanced disease-modifying candidate for this disease.

The treatment is **locally injected** into peripheral nerves through a well-controlled, rapid, and safe procedure, which offers greater opportunity for efficacy and safety. The injection is anticipated to produce a **multi-year long** therapeutic effect.

Preclinical work has been completed, establishing efficacy and safety of NVO-101 in rodent models of the disease and in non-diseased monkeys. Successful pre-IND meeting with the FDA occurred in March 2022, with the aim of an Accelerated Approval for commercialization as early as 2026.

The company intends to perform a Phase I/II proof-of-concept (PoC) trial in North America within two years, after which the program will be out-licensed to a pharma partner determined at that time to be best positioned to bring the program forward to successful launch. Nervosave's expertise in gene therapy and myelin-related disorders serves as the basis for a highly valuable pipeline including platform gene therapy candidate targeting all CMT diseases (NVO-301) and peptide-based products (NVX-401) targeting more common myelin-related diseases, such as diabetic peripheral neuropathy and Guillain-Barré syndrome.

The company is seeking investment for a **$35M Series A** round. This investment will bring NVO-101 into the clinic with top-line data by **early 2025** and allow the company to achieve preclinical PoC for NVO-301 and NVX-401.

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