SwanBio Therapeutics Announces FDA Investigational New Drug Clearance for First AAV-Based Gene Therapy for the Treatment of Adrenomyeloneuropathy

Initiation of the Phase 1/2 Trial of SBT101 in Patients with AMN Expected in H2 2022

PHILADELPHIA, January 25, 2022 – SwanBio Therapeutics, a gene therapy company advancing AAV-based therapies for the treatment of devastating, genetically defined neurological conditions, today announced that its Investigational New Drug (IND) application for its lead candidate, SBT101, for the treatment of adrenomyeloneuropathy (AMN), was cleared by the U.S. Food and Drug Administration (FDA).

SBT101 is the first AAV-based gene therapy in development specifically designed for people living with AMN, an adult-onset degenerative spinal cord disease caused by mutations in the ABCD1 gene. SwanBio plans to initiate a randomized, placebo-controlled Phase 1/2 clinical trial designed to assess the safety and explore the efficacy of SBT101 in patients with AMN in the second half of 2022.

“Today’s IND clearance is a formative milestone for SwanBio, enabling us to evolve from a preclinical company to a truly integrated research and development organization, underscoring the expertise of our team and potential of our technology platform,” said Tom Anderson, chief executive officer and director of SwanBio Therapeutics. “SBT101 has the potential to become the first disease-modifying treatment for patients with AMN, a devastating and progressive disease with no approved treatments. We look forward to initiating clinical development of SBT101 later this year, bringing us closer to our ultimate goal of delivering life-changing treatments to patients.”

The clinical program for SBT101 builds on SwanBio’s unique understanding of AMN, including new insights being gathered in an ongoing natural history study. SwanBio is deeply committed to the AMN community and has worked closely with patients, family members, and expert physicians – including SwanBio Co-Founder Dr. Florian Eichler – to ensure that its clinical programs are designed to meet their needs. SwanBio is supported by long-term investment partners Syncona Ltd. and Mass General Brigham Ventures, which both have proven track records in gene therapy, particularly in AAV-focused therapies.

About SBT101
SBT101 is the first AAV-based gene therapy in development designed to compensate for the disease-causing ABCD1 mutation, to increase ABCD1 expression, and reduce very long chain fatty acid (VLCFA) levels specifically for people living with adrenomyeloneuropathy (AMN). In preclinical studies, treatment with SBT101 demonstrated dose-dependent improvement of AMN disease markers in mouse models and was shown to be well-tolerated in non-human primates at six months post-treatment.

About Adrenomyeloneuropathy
Adrenomyeloneuropathy (AMN) is the adult-onset degenerative spinal cord disease that affects people living with adrenoleukodystrophy (ALD), a category of rare, genetic, and metabolic conditions. AMN is characterized by progressive loss of mobility, incontinence, and debilitating pain. It affects adults with mutations in the ABCD1 gene, which encodes a protein essential to the processing and breakdown of very long chain fatty acids (VLCFA). Without a functioning version of this protein there is an accumulation of VLCFA to toxic levels that leads to progressive dysfunction of the central nervous system. Between 8,000-10,000 men in the U.S. and E.U. are living with AMN. There are no approved therapies for the treatment of the disease; current standard of care is limited to symptom control.

About SwanBio Therapeutics
SwanBio Therapeutics is a gene therapy company that aims to bring life-changing treatments to people with devastating, genetically defined neurological conditions. SwanBio is advancing a pipeline of gene therapies, designed to be delivered intrathecally, that can address targets within both the central and peripheral nervous systems. This approach has the potential to be applied broadly across three disease classifications – spastic paraplegias, monogenic neuropathies and polygenic neuropathies. SwanBio’s lead program is being advanced toward clinical development for the treatment of adrenomyeloneuropathy (AMN). SwanBio is supported by long-term, committed investment partners, including its primary investors Syncona, Ltd. (lead investor and majority shareholder) and Mass General Brigham Ventures. For more information, visit SwanBioTx.com.

Media Contact:
Lara Furst
703-946-0183
media@swanbiotx.com

Investor Contact:
Chelcie Lister
910-777-3049
investors@swanbiotx.com