**LEXEO Therapeutics Receives Rare Pediatric Disease Designation and Orphan Drug Designation for LX1004 for the Treatment of CLN2 Batten Disease**

*Phase 1 clinical trial of LX1004 complete; pivotal studies planned for 2022*

**NEW YORK –** July 28, 2021 (GLOBE NEWSWIRE) – LEXEO Therapeutics, a fully integrated clinical-stage gene therapy company advancing disease-modifying treatments for genetic conditions, today announced that the U.S. Food and Drug Administration (FDA) has granted Rare Pediatric Disease designation and Orphan Drug designation to LX1004 for the treatment of CLN2 Batten disease, a fatal genetic condition of early childhood caused by a mutation in the CLN2 gene, which results in cognitive impairment, blindness, seizures, and loss of motor function and, untreated, leads to death at a young age. LX1004 is an AAV-mediated gene therapy delivering CLN2 to the central nervous system.

"Both designations granted to LX1004 underscore the critical importance and urgency to advance new treatment approaches for CLN2 Batten disease, a fatal genetic disorder affecting the central nervous system (CNS)," said R. Nolan Townsend, Chief Executive Officer of LEXEO Therapeutics. "We are encouraged by the Phase I study results of LX1004 which reached clinical proof of concept, and we look forward to advancing the program through further clinical development with the hope of making it available for patients as soon as possible."

The FDA grants Rare Pediatric Disease designation for serious and life-threatening diseases that primarily affect children ages 18 years or younger and fewer than 200,000 people in the U.S. If a biologics license application (BLA) for LX1004 is approved, LEXEO may be eligible to receive a priority review voucher that may be sold or transferred.

The Orphan Drug designation is granted by the FDA to drugs or biologics intended to treat a rare disease that affects fewer than 200,000 people in the U.S. Programs with Orphan Drug status are eligible for various development incentives.

CLN2 Batten disease is an autosomal recessive lysosomal storage disease with approximately 1,000 cases worldwide. LEXEO has completed a Phase I clinical trial of LX1004 and plans to advance the program into pivotal studies in 2022.

**About LEXEO Therapeutics, Inc.**

LEXEO Therapeutics is a New York City-based fully integrated clinical-stage gene therapy company advancing disease-modifying treatments for genetic cardiovascular conditions and genetic conditions of the central nervous system (CNS). The company aims to apply cutting-edge science to target the underlying causes of both rare monogenic diseases and diseases affecting large patient populations. LEXEO's current pipeline consists of adeno-associated virus (AAV)-mediated gene therapies in rare cardiac diseases, CLN2 Batten disease, and APOE4-associated Alzheimer's disease. In addition, the company has more than 15 AAV-mediated gene therapy programs in research and development. LEXEO was founded based on well-established gene therapy research legacy at Weill Cornell Medicine's Department of Genetic Medicine by a team

of pioneering scientists, clinicians, and business leaders with deep expertise in gene therapy. For more information, please visit www.lexeotx.com or LinkedIn.

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