LEXEO Therapeutics Receives Orphan Drug Designation for LX1004 from European Commission

– LX1004 is an adeno-associated virus (AAV) based gene therapy for the treatment of CLN2 Batten disease –

– In a completed Phase 1/2 clinical trial, LX1004 demonstrated a favorable safety profile and showed statistically significant reduction in rate of decline compared to natural history studies –

NEW YORK – October 18, 2022 (GLOBE NEWSWIRE) – LEXEO Therapeutics (LEXEO), a clinical-stage biotech company advancing a pipeline of adeno-associated virus (AAV)-based gene therapy candidates for genetically defined cardiovascular and central nervous system (CNS) diseases, today announced that the European Commission has granted Orphan Drug Designation to LX1004 for the treatment of CLN2 Batten disease. LX1004 is an AAV-mediated gene therapy designed to deliver a fully functional CLN2 gene to the CNS via intracisternal injection to restore TPP1, the secreted protein that is deficient in patients with CLN2 Batten disease.

CLN2 Batten disease is a fatal autosomal recessive lysosomal storage disease of early childhood caused by a mutation in the CLN2 gene, which results in cognitive impairment, blindness, seizures, and loss of motor function and leads to death at a young age.

An End of Phase 1/2 Meeting with the FDA will be held by the end of 2022. LEXEO has previously been granted Rare Pediatric Disease and Orphan Drug designations by the FDA.

About LEXEO Therapeutics
LEXEO Therapeutics is a New York City-based, clinical-stage gene therapy company focused on addressing some of the most devastating genetically defined cardiovascular and central nervous system diseases affecting both larger-rare and prevalent patient populations. LEXEO's foundational science stems from partnerships and exclusive licenses with leading academic laboratories at Weill Cornell Medical College and the University of California, San Diego. LEXEO is advancing a deep and diverse pipeline of AAV-based gene therapy candidates in rare cardiovascular diseases, *APOE4*-associated Alzheimer's disease, and CLN2 Batten disease, and is led by pioneers and experts with decades of collective experience in genetic medicines, rare disease drug development, manufacturing, and commercialization. For more information, please visit www.lexeotx.com or LinkedIn.

Media Contact:
Evan Feeley
Evoke Canale for LEXEO
(619) 849-5392
evan.feeley@evokegroup.com