Final 4/23/26

FOR IMMEDIATE RELEASE

VectorY Therapeutics Receives U.K. MHRA Authorization and EMA Approval to Initiate Phase 1/2 PIONEER-ALS Clinical Trial of VTx-002 in People Living with Amyotrophic Lateral Sclerosis (ALS)

The authorizations represent an expansion of the PIONEER-ALS clinical trial, currently enrolling participants at clinical trial sites in the U.S.

Amsterdam, The Netherlands, and Boston, MA — April 28, 2026. VectorY Therapeutics, a leader in vectorized antibody therapies for neurodegenerative diseases, today announced the U.K. Medicines and Healthcare Products Regulatory Agency (MHRA) and, the European Medicines Agency (EMA) have both granted the company clearance to initiate a first-in-human clinical trial of its investigational first-in-class vectorized antibody targeting TDP-43 pathology in people living with amyotrophic lateral sclerosis (ALS).

In the EU, the approval covers clinical trial sites in Belgium and The Netherlands, which will serve as primary European centers for the global study. The company recently announced that it has treated its first participant at its lead clinical trial site in the United States.

"Authorization from the MHRA and the EMA to initiate our Phase 1/2 clinical trial enables us to expand the clinical evaluation of our therapy globally and collaborate with leading investigators across numerous regions as we advance this potential new treatment for people living with ALS," **said Jim Scibetta, chief executive officer of VectorY.** "These milestones build on the progress we've made in the U.S. where the study is already underway, and we're excited to be able to offer clinical trial participation to people living with ALS in the UK and the EU."

ALS is a rapidly progressive and fatal neurodegenerative disorder that affects motor neurons in the brain and spinal cord, leading to loss of muscle control, paralysis, and respiratory failure. Median survival is 3 to 5 years after a diagnosis. Despite advances in understanding the disease, there remains a significant unmet medical need for treatments that can meaningfully slow or halt its progression.

PIONEER-ALS is a multicenter, open label, dose-escalation Phase 1/2 clinical study that evaluates two dose levels of VTx-002 and is expected to enroll 12 adults with ALS across sites in the U.S., Europe and the U.K. The primary objective of this study is to evaluate VTx-002 safety, tolerability, pharmacokinetics, and exploratory efficacy in people living with ALS. To optimize the path to pivotal clinical development, secondary and exploratory endpoints include assessment of the post-treatment Neurofilament light chain (NfL) and novel TDP-43 pathway-related biomarker trajectories, as well as clinical endpoints, including ALSFRS-R, slow-vital capacity, hand-held dynamometry and survival.

About VTx-002

VTx-002 is a first-in-class vectorized antibody therapeutic designed to target pathological TDP-43, a protein that aggregates abnormally in up to 97 percent of ALS patients. By leveraging a vector-based delivery platform, VTx-002 is designed to enable sustained antibody expression within the central nervous system, with the potential to address the underlying biology of ALS.

About VectorY Therapeutics

VectorY Therapeutics is dedicated to changing the trajectory of neurodegenerative diseases by developing precision-targeted, durable therapies that have the potential to arrest and ultimately reverse disease progression. With deep expertise in neurobiology, antibody engineering, and AAV-based delivery, VectorY is building a pipeline that includes VTx-002 targeting TDP-43 in ALS, VTx-001 targeting oxidized phospholipids in ALS, VTx-003 targeting mutant HTT and TDP-43 in Huntington's disease, VTx-005 targeting phosphorylated tau in Alzheimer's disease, and VTx-004 targeting TDP-43 in frontotemporal dementia (FTD). To learn more about the company, please visit our corporate website at Vectorytx.com and follow us on LinkedIn.

Contacts

VectorY Therapeutics B.V.
E-mail: info@VectorYtx.com
Tel: +31 20 226 8020

Vigo Consulting (Media)
Melanie Toyne-Sewell / Rozi Morris
E-mail: VectorY@vigoconsulting.com
Tel: +44 207 390 0237 / +44 20 7390 0231