VectorY Therapeutics Announces First Participant Dosed in Phase 1/2 PIONEER-ALS Clinical Trial of VTx-002 in People with Amyotrophic Lateral Sclerosis (ALS)

VTx-002 is a first-in-class vectorized antibody to holistically target TDP-43 pathology in ALS
recently granted FDA Fast Track Designation for expedited review

Amsterdam, The Netherlands, and Boston, MA — February 9, 2026 — VectorY Therapeutics, a leader in vectorized antibody therapies for neurodegenerative diseases, announced today that the first participant has been dosed in its Phase 1/2 PIONEER-ALS trial evaluating VTx-002, a first-in-class vectorized antibody targeting TDP-43 pathology in people with amyotrophic lateral sclerosis (ALS). The participant was treated at the lead clinical trial site, the Sean M. Healey & AMG Center for ALS at Mass General Brigham, a premier treatment center for neurodegenerative diseases and pioneering research.

"ALS is a fatal neurodegenerative disease with no cure and only limited symptomatic treatment options available. The initiation of dosing in the PIONEER-ALS trial is a significant milestone for VectorY as we strive to transform the neurodegenerative disease landscape with novel disease-modifying approaches," said Olga Uspenskaya-Cadoz M.D., Ph.D. and chief medical officer of VectorY. "This trial marks the first ever clinical evaluation of a therapy designed to holistically target TDP-43 pathology in ALS, and thereby reduce TDP-43 aggregation, correct mis-splicing abnormalities, and restore normal nuclear function, and we are excited to advance this novel potential therapeutic strategy to a community of patients who are actively looking for hope."

PIONEER-ALS is a multicenter, open label, dose-escalation Phase 1/2 clinical study that will evaluate 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.

"There remains a critical need for disease-modifying therapies in ALS. VTx-002 is engineered to enable sustained, localized delivery of an antibody targeting pathological TDP-43, a hallmark of ALS, with the goal of addressing a central driver of disease progression," said Global Coordinating Investigator James Berry, MD, MPH, chief of the

Division of Motor Neuron Diseases and director of the Neurological Clinical Research Institute (NCRI) at Mass General Brigham. "The initiation of this study reflects an innovative approach aimed at one of the most important pathological features of the disease. I look forward to leading this exploration of VTx-002 and its potential to impact people living with ALS."

VectorY Therapeutics expects to continue enrolling participants in the PIONEER-ALS trial across multiple clinical sites.

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. More information about the PIONEER-ALS clinical trial can be found at: clinicatrials.gov: Study is to Assess the Safety and Tolerability of VTx-002 in Participants With ALS | ClinicalTrials.gov.

About ALS

ALS is a universally fatal neurodegenerative disease with no cure and only limited symptomatic treatment options. In the United States, more than 5,000 new cases of ALS are diagnosed each year, and more than 30,000 people are currently living with the disease. Median survival is 2–3 years after a diagnosis, and someone is either diagnosed with or dies from ALS every 90 minutes. The disease has an incidence comparable to major indications such as multiple sclerosis, yet far lower prevalence reflecting a persistent disease burden and urgent unmet medical need.

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

Instinctif Partners (Media)
Julian Walker / Tim Pearson
E-mail: VectorY@instinctif.com
Tel: +44 207 802 2657