



VectorY Expands PIONEER-ALS Phase 1/2 Clinical Trial Size

- *Study increased from 12 to 36 participants to broaden evaluation of VTx-002, a first and potentially best-in-class vectorized antibody targeting TDP-43 pathology in people living with amyotrophic lateral sclerosis (ALS)*
- *Tripling of study size occurs prior to receipt of data pertinent to any patient efficacy readouts, and materially enhances the opportunity for emergence of evidence of disease modifying properties, as measured through secondary and exploratory endpoints evaluating the progression of ALS*
- *Study expansion facilitated by rapid study enrollment coupled with prompt global site activation*

Amsterdam, The Netherlands, and Boston, MA - September 29, 2026 — VectorY Therapeutics (“VectorY”), a clinical-stage biotechnology company pioneering vectorized antibody therapies for neurodegenerative diseases, today announced the expansion of its ongoing, global, Phase 1/2 PIONEER-ALS® clinical trial of VTx-002 from 12 to 36 participants. The PIONEER-ALS trial is currently evaluating the safety, tolerability, pharmacokinetics and pharmacodynamic effects of VTx-002 in people living with ALS.

The Company announced the first participants dosed in the PIONEER-ALS trial in the United States in February 2026 at the Sean M. Healey & AMG Center for ALS at Mass General, and Europe in September at University Medical Center Utrecht. The expansion is supported by rapid study enrollment and prompt global site activation and will enable VectorY to generate a broader and more robust set of clinical data on VTx-002 while helping to advance understanding of how VTx-002 may address the underlying pathology of ALS.

“Expanding PIONEER-ALS from 12 to 36 participants is a direct reflection of the significant interest from people living with ALS, and the rapid pace of global site activation and study enrollment we’ve seen thus far,” said **Jim Scibetta, chief executive officer, VectorY**.

“There remains a critical need for disease-modifying therapies in ALS. With VTx-002, we directly target TDP-43, which is the pathological driver in the vast majority of ALS cases. Tripling the study size significantly increases our opportunity to utilize its secondary and exploratory endpoints to obtain a better preliminary understanding whether VTx-002 is impacting the underlying biology of ALS in a way that may ultimately translate to clinical benefit and disease modification.”



VectorY's lead program, 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% 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 and modify the disease. VTx-002 is an investigational therapy and has not been approved by any regulatory authority.

VectorY is currently enrolling participants in the PIONEER-ALS trial across multiple clinical sites. More information about the trial can be found at: clinicaltrials.gov: Study is to Assess the Safety and Tolerability of VTx-002 in Participants With ALS | ClinicalTrials.gov.

About PIONEER-ALS

PIONEER-ALS is a global, multicenter, open label, dose-escalation Phase 1/2 clinical study that is evaluating two dose levels of VTx-002 enrolling adults with ALS 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 ALS

ALS is a universally fatal neurodegenerative disease with no cure and 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 and manufacturing, VectorY is building a pipeline that includes VTx-002 targeting TDP-43 in ALS, VTx-003 targeting mutant HTT and TDP-43 in Huntington's disease, VTx-004 targeting TDP-43 in frontotemporal dementia (FTD), and VTx-005 targeting phosphorylated tau in Alzheimer's disease.

To learn more about the company, please visit our corporate website at VectorYtx.com and follow us on [LinkedIn](#).

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