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Vico Therapeutics Announces Patient Dosing in Twice-Annual Regimen of VO659 in Phase 1/2 Trial in Huntington's Disease, Spinocerebellar Ataxia Type 3 and Type 1

February 24, 2026

- *FDA clearance of IND application for VO659 with plans to initiate US-based clinical trial later this year*
- *New data highlight distinctive mechanism of action of exon 1–directed CAG repeat–targeting ASO presented at CHDI's 21st Annual HD Therapeutics Conference*
- *VO659 is the only clinical-stage therapeutic specifically targeting the pathogenic CAG repeat expansion underlying these disorders, engineered to preferentially reduce mutant HTT while preserving wild-type HTT expression*

LEIDEN, Netherlands, February 24, 2026 /BusinessWire/ – Vico Therapeutics B.V., a biotechnology company developing first-in-class RNA-targeted therapies for severe neurological diseases, today announced that patient dosing has commenced in an expanded cohort of participants using a twice annual dosing regimen. The Phase 1/2a clinical study is being conducted in multiple European countries to evaluate VO659 for the treatment of Huntington's disease (HD), spinocerebellar ataxia types 3 (SCA3) and 1 (SCA1).

VO659 is an antisense oligonucleotide (ASO) investigational therapy designed to target the CAG repeat expansion that causes all nine known polyglutamine diseases including HD, SCA3 and SCA1. Vico previously announced a 38% reduction of CSF mutant HTT (mHTT) and a 2.5% reduction of CSF Nf-L in HD patients dosed with VO659 at four months.

The every 6 month dosing regimens of the Phase 1/2 study are being assessed over 12-months for safety, tolerability, pharmacokinetics and pharmacodynamic effects of VO659 dosed intrathecally (IT). To date, several participants have been dosed and VO659 has been safe and well-tolerated with no serious adverse events.

“I am very encouraged by our earlier results showing significant reduction of mHTT directly in the central nervous system with a favorable CSF Nf-L profile early in the time course,” said Micah Mackison, CEO of Vico Therapeutics. “The twice annual patient-friendly dosing regimens will enable us to assess longer-term safety and pharmacodynamic effects, taking advantage of VO659’s long half-life.”

The trial is being conducted under an approved Clinical Trial Application (CTA) in Europe and is registered on [ClinicalTrials.gov](https://clinicaltrials.gov) (Identifier: NCT05822908).

Investigational New Drug Application Cleared

In addition, Vico has received Investigational New Drug (IND) clearance from the U.S. Food and Drug Administration (FDA) and plans to initiate U.S.-based clinical trials for VO659 later this year, further expanding its global development program.

CHDI’s 21st Annual HD Therapeutics Conference Poster Presentation

Vico’s Chief Scientific Officer, Nicole Datson, PhD, will present new data this week in a poster at CHDI’s 21st Annual HD Therapeutics Conference on the distinctive mechanism of action of VO659 and its ability to inhibit multiple drivers of CAG repeat–mediated toxicity. Vico’s Chief Medical Officer, Scott Schobel, MD, will also present a Phase 1/2 clinical trial update of VO659.

Poster Presentation Details:

Poster 43: *Distinctive mechanism of action of CAG repeat-targeting ASO VO659: Inhibition of multiple drivers of CAG repeat-mediated toxicity*

Presented by Nicole Datson, PhD, Chief Scientific Officer

Wednesday, February 25th from 1:00-2:30 pm PST

Poster 145: *Clinical update from a first-in-human, phase 1/2a trial of VO659, an allele-preferential ASO for CNS neurodegenerative diseases caused by CAG repeat expansion*

Presented by Scott Schobel, MD, Chief Medical Officer
Wednesday, February 25th from 2:30-4:00 pm PST

About Vico Therapeutics B.V.

Vico Therapeutics is a clinical-stage genetic medicines company developing RNA-targeted therapies for severe neurological diseases caused by defined genetic mutations. The company is pioneering a precision antisense oligonucleotide (ASO) platform designed to selectively target disease-causing RNA while preserving normal gene function. Vico's lead program, VO659, is being evaluated in Huntington's disease and spinocerebellar ataxias, with additional pipeline programs advancing in other CNS disorders. Headquartered in Leiden, the Netherlands, Vico is committed to translating deep genetic and molecular insights into disease-modifying therapies for patients with limited or no treatment options.

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