



NEWS RELEASE

Prilenia and Ferrer to Share New Pridopidine Data and PREVAiLS Study Updates at the NEALS Consortium 2026 Annual Meeting

2026-10-28

In a new post hoc analysis of a sub-group of participants with ALS from the Phase 2 HEALEY Platform Trial and its open-label extension phase, presented as a poster, pridopidine, an investigational drug, was associated with positive results across ALSFRS-R, respiratory measures and median survival time through Week 48 when compared to PSW-matched PRO-ACT controlsⁱ

The companies also announce that the ongoing Phase 3 PREVAiLS clinical study, evaluating the efficacy and safety of pridopidine in participants with ALS early in their disease course, is now approximately 60% enrolled.

NAARDEN, Netherlands & WALTHAM, Mass. & BARCELONA, Spain--(BUSINESS WIRE)-- **Prilenia Therapeutics B.V. and Ferrerⁱⁱ** today announced the presentation of new data and PREVAiLS study updates at the upcoming Northeast Amyotrophic Lateral Sclerosis (NEALS) Consortium 2026 annual meeting in Florida.

In a new post hoc analysis of a sub-group of 36 participants with definite or probable ALS (El Escorial Criteria) who are within 18 months from first onset of disease symptoms from the Phase 2 HEALEY Platform Trial (NCT04615923) and its open-label extension phase, pridopidine, an investigational drug, was associated with positive results across ALSFRS-R, respiratory measures and median survival time through Week 48 when compared to external Pooled Resource Open-Access ALS Clinical Trials (PRO-ACT) controls using propensity-score (PSW) methodsⁱⁱⁱ.

This analysis builds on prior post hoc subgroup findings from the 24-week placebo-controlled phase of the HEALEY Platform Trial^{iv}. The findings have informed the rationale and design of the ongoing PREVAiLS registrational Phase 3 randomized, controlled study, being undertaken in a similar patient population, which is now approximately 60% enrolled. Recruitment of the approximately 500 participants for PREVAiLS is expected to be completed in the first quarter of 2027 and top line results are expected in Q1 2028.

ENDS

[This press release is intended for informational purposes only. Pridopidine is an investigational drug and is not approved for commercial use by any regulatory authority. Its safety and efficacy have not been established. Information contained herein does not constitute medical advice. Patients should consult their healthcare provider for guidance regarding diagnosis or treatment options. Local regulations may vary; this release is not intended to promote or advertise any product.]

About ALS

Amyotrophic lateral sclerosis (ALS), also known as Lou Gehrig's disease or often referred to as motor neuron disease (MND), is a progressive neurodegenerative disease, which means it is a chronic condition that gets worse over time as damage builds up in parts of the nervous system. It affects the motor neurons in the brain and spinal cord and leads to a gradual loss of muscle function that eventually results in paralysis and death^v.

ALS has a global prevalence of approximately 4.42 per 100,000 people^{vi}, and is more frequent in men than in women^{vii}. The average survival is 2 to 5 years^{viii}. The exact cause of ALS is not fully understood, but it is believed to be a combination of genetic and environmental factors.

ALS typically appears in individuals aged 40 to 70 and presents a range of symptoms, including muscle weakness and atrophy, mobility issues such as lack of coordination and balance, muscle spasms, difficulty speaking clearly (dysarthria), trouble swallowing (dysphagia), fatigue, and emotional and cognitive changes^{ix}. Symptoms worsen over time and significantly impact the quality of life of patients and their families by affecting their ability to perform daily activities independently. Current treatment options are limited.

About pridopidine

Pridopidine is an investigational highly selective, oral sigma-1 receptor (S1R) agonist. S1R has been shown to play a role in stimulating multiple neuroprotective pathways impaired in neurodegenerative diseases, such as Huntington's disease (HD) and amyotrophic lateral sclerosis (ALS)^x.

In clinical studies to date, pridopidine (taken as an oral capsule twice a day) has shown a generally favorable safety and tolerability profile, with data from more than 1,600 people (mostly from HD studies), some of whom have received active treatment for up to seven years^{xi}.

Pridopidine is currently in late-stage clinical development for HD and ALS. It has Orphan Drug Designation in HD and ALS in the US and EU, and FDA Fast Track designation in the US for the treatment of HD. These designations do not imply that pridopidine has been approved, or that safety and efficacy have been established.

About PREVAiLS (Pridopidine Phase 3 Study to Evaluate Efficacy and Safety in ALS)

PREVAiLS is a pivotal, global, 500-participant, randomized, placebo-controlled Phase 3 study designed to further evaluate the efficacy and safety of pridopidine, an investigational medicinal product, in people living with amyotrophic lateral sclerosis (ALS) early in the course of their disease.

The study seeks to enrol participants with definite or probable ALS, according to the El Escorial Criteria, who are within 18 months from the first onset of disease symptoms. Its design is informed by findings from a subgroup analysis of the Phase 2 HEALEY ALS Platform Trial in a similar early, rapidly progressing patient population. The pridopidine regimen of the HEALEY ALS Platform Trial did not meet its primary endpoint in the overall study population.

PREVAiLS comprises a 48-week placebo-controlled stage, with participants randomised to receive pridopidine or placebo on a 3:2 basis, followed by a 48-week open-label extension stage. The primary endpoint is the change from baseline in the Revised Amyotrophic Lateral Sclerosis Functional Rating Scale (ALSFRS-R), adjusted for mortality, at Week 48.

Secondary and exploratory endpoints include survival and measures of speech, respiratory function, bulbar function and quality of life, as well as patient-reported outcomes of communication and plasma biomarkers.

The study is planned across up to 60 leading ALS treatment centers across 13 countries, including in the US, Canada, EU, UK and Israel.

More details on PREVAiLS can be found on **ClinicalTrials.Gov (NCT07322003)** / **EU CT Number: 2025-524002-16-00** and at **www.prevailstrial.com**.

About Prilenia

Prilenia is a private biopharmaceutical company driven by an unwavering commitment to scientific excellence and accelerating progress for people affected by Huntington's disease (HD), amyotrophic lateral sclerosis (ALS) and other neurodegenerative disorders. Our mission is simple but urgent: to develop and provide sustainable access to transformative medicines for people affected by devastating neurodegenerative diseases.

The company is incorporated in the Netherlands and backed by leading life sciences investors. Prilenia and Ferrer

are partners in the co-development and commercialisation of pridopidine. For more information, please visit www.prilenia.com, and connect with us on [LinkedIn](#), [X](#) or [Instagram](#).

About Ferrer

At Ferrer, we use business to fight for social justice. We have long been a company that wants to do things differently; instead of maximizing shareholder returns, we reinvest much of our profit in initiatives that give back to society. Back where it belongs. We go beyond compliance and are guided by the highest standards of sustainability, ethics and integrity. As such, since 2022, we are a B Corp.

Founded in Barcelona in 1959, Ferrer offers transformative solutions for life-threatening diseases in more than one hundred countries. In line with our purpose, we have an increasing focus on pulmonary vascular and interstitial lung diseases and rare neurological disorders in adults and children. Our 1,800-strong team is driven by a clear conviction: our business is not an end in itself, but a way to change lives.

We are Ferrer. Ferrer for good. www.ferrer.com

ⁱ Propensity score weighting-matched (PSW) Pooled Resource Open-Access ALS Clinical Trials (PRO-ACT). Atassi, N., Berry, J., Shui, A., Zach, N., Sherman, A., Sinani, E., Walker, J., Katsovskiy, I., Schoenfeld, D., Cudkowicz, M., & Leitner, M. (2014). The PRO-ACT database: design, initial analyses, and predictive features. *Neurology*, 83(19), 1719–1725. <https://doi.org/10.1212/WNL.0000000000000951>

ⁱⁱ In April 2025, Prilenia and Ferrer signed a commercialization and co-development agreement for pridopidine. <https://news.prilenia.com/press-releases/press-release-details/2025/Prilenia-Enters-into-a-Collaboration-and-License-Agreement-with-Ferrer-for-the-Commercialization-and-Co-Development-of-Pridopidine-in-Europe-and-Other-Select-Markets/default.aspx>

ⁱⁱⁱ Comparisons with external controls are not randomized and may be affected by residual confounding and selection bias and may not confirm efficacy.

^{iv} Geva, M., Goldberg, Y. P., Leitner, M. L., Cruz-Herranz, A., Hand, R., Chen, K., ... Hayden, M. R. (2025). Pridopidine treatment in ALS: subgroup analyses from the HEALEY ALS Platform trial. *Amyotrophic Lateral Sclerosis and Frontotemporal Degeneration*, 1–13. <https://doi.org/10.1080/21678421.2025.2597935>

^v Goutman SA, Hardiman O, Al-Chalabi A, et al. Recent advances in the diagnosis and prognosis of amyotrophic lateral sclerosis. *Lancet Neurol*. 2022;21(5):480-493. doi: 10.1016/S1474-4422(21)00465-8.

^{vi} Ilieva H, Vullaganti V, Kwan C. Advances in molecular pathology, diagnosis, and treatment of amyotrophic lateral sclerosis. *BMJ*. 2023;383:e075037. doi: 10.1136/bmj-2023-075037.

^{vii} Masrori P, Van Damme P. Amyotrophic lateral sclerosis: a clinical review. *Eur J Neurol*. 2020;27(10):1918-1929. doi:10.1111/ene.14393

^{viii} Goutman SA, Hardiman O, Al-Chalabi A, et al. Recent advances in the diagnosis and prognosis of amyotrophic

lateral sclerosis. *Lancet Neurol.* 2022;21(5):480-493. doi: 10.1016/S1474-4422(21)00465-8.

^{ix} Muscular Dystrophy Association. Amyotrophic Lateral Sclerosis (ALS). Signs and Symptoms [Internet]. MDA [Accessed October 18th, 2024]. Available from: <https://www.mda.org/disease/amyotrophic-lateral-sclerosis/signs-and-symptoms>

^x Lachance V, Bélanger S-M, Hay C, Le Corvec V, Banouvong V, Lapalme M, Tarmoun K, Beaucaire G, Lussier MP, Kourrich S. Overview of Sigma-1R Subcellular Specific Biological Functions and Role in Neuroprotection. *International Journal of Molecular Sciences.* 2023; 24(3):1971. <https://doi.org/10.3390/ijms24031971>

^{xi} Goldberg YP, Navon-Perry L, Cruz-Herranz A, Chen K, Hecker-Barth G, Spiegel K, Cohen Y, Niethammer M, Tan AM, Schuring H, Geva M, Hayden MR. The Safety Profile of Pridopidine, a Novel Sigma-1 Receptor Agonist for the Treatment of Huntington's Disease. *CNS Drugs.* 2025 May;39(5):485-498. doi: 10.1007/s40263-025-01171-x. Epub 2025 Mar 7. PMID: 40055280; PMCID: PMC11982116.

Media:

Prilenia

Rob Cohen, Head of Corporate Communications

rob.cohen@prilenia.com

Ferrer

Alba Soler, Director of Communication

asolerc@ferrer.com

Source: Prilenia Therapeutics B.V.