Prilenia and Ferrer Announce First Participant Enrolled in the "PREVAiLS" Phase 3 Study of Pridopidine in ALS

PREVAiLS is a pivotal, global, 500-participant Phase 3 study to evaluate efficacy and safety of pridopidine, an investigational drug, in slowing ALS progression (ALSFRS-R) in early, rapidly progressive participants

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The design of the potentially registrational pivotal Phase 3 study is informed by, and seeks to confirm, subgroup analysis data from the Phase 2 program in a similar rapid and progressive patient population[i]

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PREVAiLS, believed to be the only currently recruiting Phase 3 ALS study, is set to take place in up to 60 leading ALS treatment centers across 13 countries. 11 sites are already, or will imminently be, initiated, with recruitment at additional sites in the US, Canada, EU, UK and Israel expected to commence in the coming weeks and months

NAARDEN, The Netherlands and WALTHAM, Mass. / BARCELONA, Spain, 30 March 2026 --- Prilenia Therapeutics B.V. and Ferrer[ii] today announced the first enrollment in the pivotal, 500-participant, randomized, placebo-controlled Phase 3 study of pridopidine in people with rapidly progressive Amyotrophic Lateral Sclerosis (ALS) early in their disease course ("PREVAiLS", NCT07322003).

The first participant was enrolled at the at Mass General Brigham (MGB), under the supervision of **Sabrina Paganoni, MD, PhD, Co-Director of the MGH Neurological Clinical Research Institute, and PREVAiLS principal investigator**, who said: "Pridopidine is a sigma-1 receptor (S1R) agonist. The S1R has been shown to play a role in stimulating multiple neuroprotective pathways impaired in neurodegenerative diseases, such as ALS and Huntington's disease (HD)[iii]. Enrolling the first participant in this confirmatory study is a milestone in our search for potential new therapeutic options that may help to slow disease progression, preserve function, maintain speech and prolong survival – key aims of early ALS therapy."

"The ALS community urgently needs new treatment options that can delay the disease's relentless progression, and awaits the outcome of this study," said **Kuldip Dave**, Ph.D. Senior Vice President of Research, ALS Association. "The earlier we can diagnose and treat ALS, the greater the potential to preserve function and maintain quality of life for longer, which are key to making ALS livable until we can cure it".

PREVAiLS is informed by peer-reviewed and published data from a subgroup analysis of similar participants with rapidly progressive ALS early in their disease course (pridopidine: n

= 37; shared placebo: n = 35), from the randomized, double-blind, placebo-controlled Phase 2 HEALEY ALS Platform Trial. The HEALEY trial did not meet its primary or secondary endpoints in the full population; pre-specified and additional analyses showed effects in rapid progressive patients that we seek to confirm in PREVAiLS.

The PREVAiLS study is set to take place in up to 60 leading ALS treatment centers across 13 countries. 11 sites are already, or will imminently be, initiated, with recruitment at additional sites in the US, Canada, EU, UK and Israel expected to commence in the coming weeks and months.

More details on PREVAiLS can be found on ClinicalTrials.Gov (NCT07322003) / EU CT Number: 2025-524002-16-00.

[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 PREVAiLS (Pridopidine Phase 3 Study to Evaluate Efficacy and Safety in ALS)
PREVAiLS is a 48-week randomized (3:2 pridopidine:placebo), placebo-controlled study, with a 48-week open label extension phase to follow. The study seeks to enroll participants with definite or probable ALS (El Escorial Criteria) who are within 18 months from first onset of disease symptoms. The primary endpoint of PREVAiLS is the change from baseline in ALSFRS-R adjusted for mortality at 48 weeks. 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[iv].

More details on PREVAiLS can be found on ClinicalTrials.Gov (NCT07322003) / EU CT Number: 2025-524002-16-00.

About pridopidine
Pridopidine (45 mg twice daily) is an investigational selective, orally administered sigma-1 receptor (S1R) agonist. S1R has been shown to play a role in stimulating multiple neuroprotective pathways impaired in neurodegenerative diseases, such as ALS and Huntington's disease (HD)**Error! Bookmark not defined.**.

In clinical studies to date, it has demonstrated a 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[v].

In addition to ALS, Prilenia and Ferrer are planning to initiate a potentially registrational pivotal Phase 3 study in HD. The study is expected to start recruitment in the first half of 2026.

Pridopidine has Orphan Drug designation in HD and ALS in the US and EU, and FDA Fast Track designation for the treatment of HD.

Pridopidine is an investigational drug not approved by any regulatory authority. Its role in ALS and other neurological conditions is currently under clinical investigation.

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[vi]. It affects a specific type of neurons – 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[vii]. ALS affects approximately 500,000 people worldwide[viii], and is more frequent in men than in women[ix]. The average survival is 2 to 5 years[x]. 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[xi] 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[xii]. 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.

Dysfunction of the S1R has been associated with multiple forms of ALS, and maintaining S1R functionality may play a key role in protecting neuronal function.

About Prilenia

Prilenia is a private biopharmaceutical company driven by an unwavering commitment to scientific excellence and accelerating progress for people affected by HD, 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.

For more information, please visit www.prilenia.com, and connect with us on LinkedIn or X (Twitter).

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

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[i] 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

[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] 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

[iv] van den Berg L, et al. A planned Phase 3, randomized, double-blind, placebo-controlled study to evaluate the efficacy and safety of pridopidine in participants with ALS
36th International Symposium on ALS/MND, San Diego, CA, USA, December 2025

[v] 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.
[vi] https://www.mayoclinic.org/diseases-conditions/amyotrophic-lateral-sclerosis/symptoms-causes/syc-20354022#:~:text=ALS%2C%20is%20a%20nervous%20system,disease%20gets%20worse%20over%20time.
[vii] 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.
[viii] Xu L, Liu T, Liu L, et al. Global variation in prevalence and incidence of amyotrophic lateral sclerosis: a systematic review and meta-analysis. J Neurol. Apr 2020;267(4):944–953. doi:10.1007/s00415-019-09652-y
[ix] Masrori P, Van Damme P. Amyotrophic lateral sclerosis: a clinical review. Eur J Neurol. 2020;27(10):1918-1929. doi:10.1111/ene.14393
[x] 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.
[xi] https://www.als.org/understanding-als/who-gets-als
[xii] 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