

Presentation at Spark Plus Singapore Healthcare Day

14 March 2024 – Perth, Australia: PharmAust Limited (ASX: PAA & PAAOA) (“PharmAust” or “the Company”), a clinical-stage biotechnology company, is pleased to announce that it is presenting today at the Spark Plus Singapore Healthcare Day.

PharmAust CEO Dr Michael Thurn will present the Company’s investment proposition to an audience of funds, family offices and investors from Singapore, Hong Kong and surrounds.

A copy of the investor presentation being used for the event is attached below.

The Board authorises this announcement.

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About PharmAust Limited:

PharmAust Limited is listed on the Australian Securities Exchange (ASX Code: PAA). PAA is a clinical-stage biotechnology company developing therapeutics for human and animal health applications. The company is focused on repurposing monepantel (MPL) for human neurodegenerative diseases.

MPL is a potent and safe inhibitor of the mTOR pathway. This pathway plays a central role in cell growth and proliferation of cancer cells and degenerating neurons. The mTOR pathway regulates the cellular “cleaning process”, where toxic protein is broken down into macromolecules to be reused. This autophagic process is disrupted in most neurodegenerative diseases, including motor neurone disease (MND/ALS).

PAA’s lead MPL program is for the treatment of MND/ALS, a rare, incurable disease. The company recently announced positive top-line results for its Phase 1 study in patients with MND/ALS. PAA anticipates starting an adaptive Phase 2/3 clinical study in H2 CY 2024 that could lead to accelerated approval with the US Food and Drug Administration in 2026.

The Neurodegenerative Disease Market size is estimated at USD 55.12 billion in 2024, and is expected to reach USD 77.82 billion by 2029, growing at a CAGR of 7.14% during the forecast period (2024-2029).¹

¹ <https://www.mordorintelligence.com/industry-reports/neurodegenerative-disease-market>

PharmAust Investor Hub:

We encourage you to utilise our Investor Hub for any enquiries regarding this announcement or other aspects concerning PharmAust. This platform offers an opportunity to submit questions, share comments, and view video summaries of key announcements.

Access the investor hub by scanning the QR code or visiting:
<https://investorhub.pharmaust.com/>

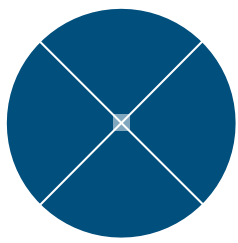


Singapore Healthcare Day Presentation

March 2024

Dr Michael Thurn





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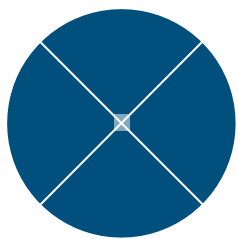
FUTURE MATTERS

This presentation contains reference to certain intentions, expectations, future plans, strategy and prospects of the Company. Those intentions, expectations, future plans, strategy and prospects may or may not be achieved. They are based on certain assumptions, which may not be met or on which views may differ and may be affected by known and unknown risks. The performance and operations of the Company may be influenced by a number of factors, many of which are outside the control of the Company. No representation or warranty, express or implied, is made by the Company, or any of its directors, officers, employees, advisers or agents that any intentions, expectations or plans will be achieved either totally or partially or that any particular rate of return will be achieved.

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Corporate Overview

Mid-stage biotechnology company targeting human neurodegenerative diseases

Share Price Performance



Board & Management

| | |
|------------------------------|---------------------------------------|
| Dr Roger Aston | Non-Exec Chairman |
| Dr Michael Thurn | Chief Executive Officer |
| Mr Neville Bassett AM | Non-Exec Director |
| Mr Robert Bishop | Non-Exec Director |
| Dr Thomas Duthy | Non-Exec Director |
| Mr Sam Wright | Non-Exec Director & Company Secretary |

Capital Structure (AUD\$)

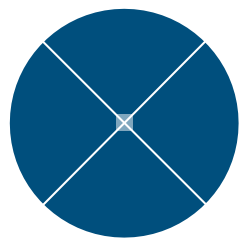
13 Mar 2024

| | |
|---|-----------------|
| Current Share Price (PAA/PAAOA) | \$0.37 / \$0.23 |
| 52 Week Low / High (PAA) | \$0.06/ \$0.53 |
| No. of Shares (PAA) | 384,965,597 |
| Listed Options (PAAOA) | 121,949,093 |
| Market Capitalisation | \$146.5m |
| Monthly Turnover | \$78.0m |
| Cash (as at 31-Dec-23) | \$5.5 m |
| Debt (as at 31-Dec-23) | Nil |
| Net Cash | \$5.5m |
| Enterprise Value | \$146.5m |
| Unlisted Options (10c/15c/17.5c) | 11.4 m |
| Enterprise Value (fully diluted) | \$153.0m |

Top Shareholders*

| | |
|---|-------|
| Hybrid Holdings Pty Ltd <Darcy Family Super Fund A/C> | 5.78% |
| Mr Gerald James Van Blommestein & Mrs Gillian Van Blommestein <Van Blommestein S/F A/C> | 4.75% |
| Dr Roger Aston | 3.91% |
| Board & Management | 7.84% |

* As at 16 Feb 2024



Product candidates for both human and animal health applications



Human and Animal Health

Mid stage biotechnology company focused on large and growing markets in human and animal health



Strong IP Position

Strong intellectual property with patent protection beyond 2030



Repurposing Monepantel

Repurposing an approved veterinary product – monepantel – anthelmintic for sheep



Pipeline Synergies

Pipeline synergies to leverage commercial infrastructure across human and animal health applications



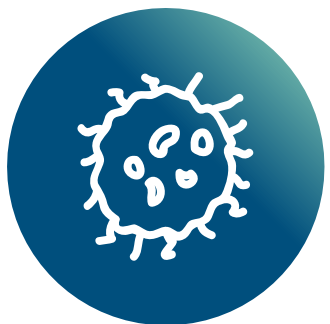
Motor Neurone Disease

Lead clinical program for the treatment of motor neurone disease (MND/ALS)



Experienced Management

Experienced management team with demonstrated execution capabilities



Canine B-Cell Lymphoma

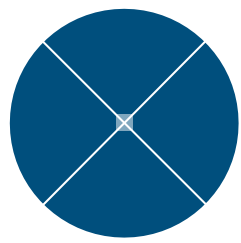
Phase 2 Veterinary program for the treatment of dogs with B-Cell Lymphoma



Broad Investor Base

Healthy mix of loyal institutional and retail investors





Meet Our Team – Chairman and Management



Dr Roger Aston Non-Executive Chairman

Roger brings more than 30 years experience in the pharmaceutical and healthcare industries in senior roles in the UK, Asia Pacific and Australia. He has had extensive experience including FDA and EU product registration, clinical trials, global licensing agreements, fundraising through private placements, and a network of contacts within the pharmaceutical, banking and stock broking sectors

Dr Michael Thurn Chief Executive Officer

Michael has over 25 years experience in technical, regulatory, commercial and management roles in research organisations and industry, including early stage, fast growing, private and publicly listed biotechnology companies. Michael has led a variety of US IND applications across a range of therapeutic areas and evaluated drugs and vaccines for registration during his engagement at the TGA.

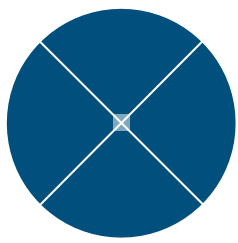
John Clark Chief Operating Officer

John has over 20 years of pharmaceutical industry experience in phase I – IV clinical trials across numerous therapeutic areas and multiple geographical regions. John has a thorough knowledge of ICH-GCP and regulatory requirements and held clinical operations leadership roles responsible for implementing global clinical programs.

Dr Carol Worth CMC Operations Manager

Carol brings over 30 years of industry experience and a passion for focusing on quality control and quality assurance. She recently served as Quality Manager at Epichem Pty Ltd as Chief Technical Officer at Suda Pharmaceuticals and Solbec Pharmaceuticals. Carol has also led product development programs at Thermalife International Pty Ltd/Pharmasolv Laboratories Pty Ltd



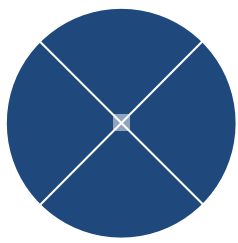


Pipeline

Multiple synergistic product opportunities in human and animal health by repurposing monepantel

| Human Health | | | | | | |
|--|-------------|---------|---------|--------------------|---|--|
| Indication | Preclinical | Phase 1 | Phase 2 | Phase 3 | Approved / Marketed | Next Major Catalysts |
| Motor Neurone Disease <small>(Amyotrophic Lateral Sclerosis)</small> | ✓ | | | | Accelerated approval possible based on Phase 2 data | <ul style="list-style-type: none"> Orphan Drug Designation HREC Approval Open IND |
| Neurodegenerative Diseases | ✓ | | | | | <ul style="list-style-type: none"> Identify next target indication Preclinical data |
| Cancers | ✓ | | | | | <ul style="list-style-type: none"> Under review Seek partnership opportunities |
| Animal Health | | | | | | |
| Indication | Preclinical | Phase 1 | Phase 2 | Field Study / TASS | Approved / Marketed | Next Major Catalysts |
| Canine B-Cell Lymphoma | ✓ | | | | Conditional approval possible | <ul style="list-style-type: none"> MUMS Waiver Open INAD Begin Field Study |

IND – Investigational New Drug
 MUMS – Minor Use Minor Species
 INAD – Investigational New Animal Drug
 TASS – Target Animal Safety Trial

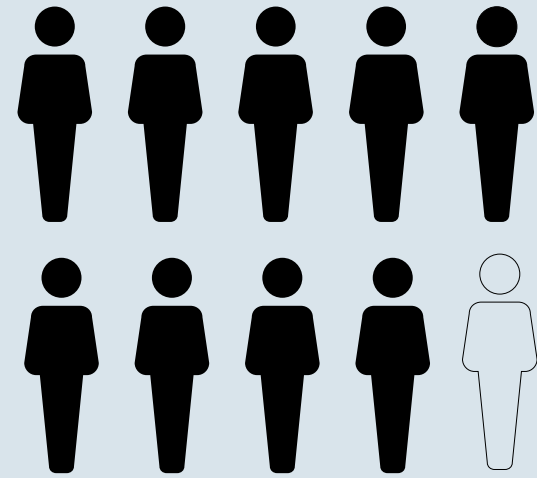


MND /ALS Statistics & Treatments

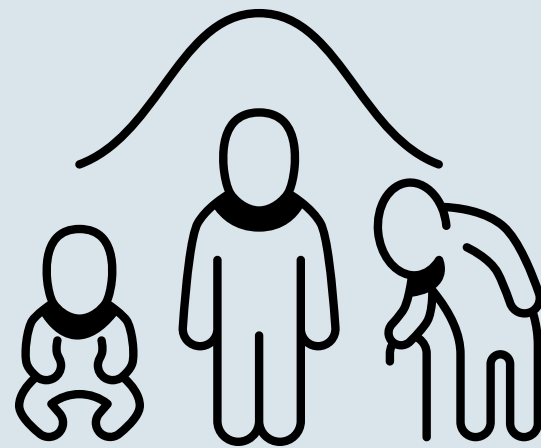
There is no cure and MND/ALS is always fatal



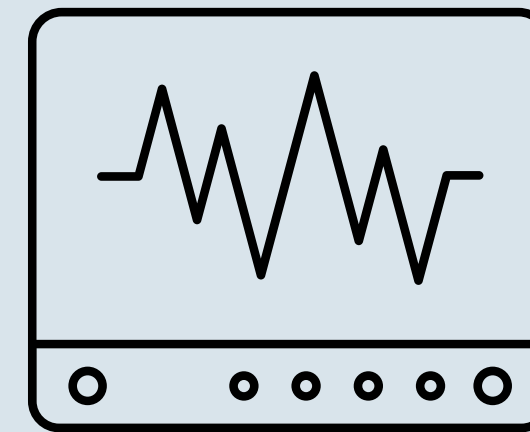
Every **90 minutes** someone is **diagnosed and dies** with MND/ALS



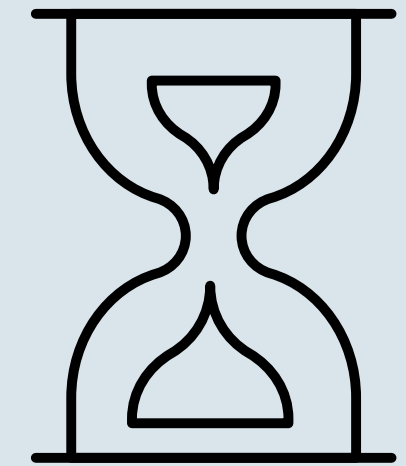
90% of cases occur **without a family history**



Onset is usually between the ages of **40 & 70 years**



Life expectancy on average is just over **2 years**



By **2040** the **incidence** of MND/ALS is expected to **increase by 70%**

Current Treatments



Qalsody (tofersen)
Developed to treat ALS associated with a mutation in the superoxide dismutase 1 (SOD1) gene. The FDA approved Qalsody to treat SOD1-ALS in 2023.



Rilutek (riluzole)
This was the first FDA-approved drug available to treat ALS — in 1995. It inhibits glutamate release and prolongs life ~3 months.

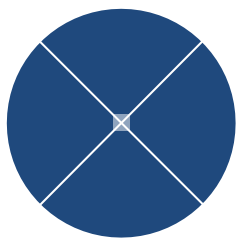


Radicava™ (edaravone)
The FDA approved Radicava™ in 2017, making it the first new treatment specifically for ALS in 22 years. Prolongs life ~6 months.



Relyvrio (AMX0035)
RELYVRIO is a combination of two drugs, sodium phenylbutyrate and taurursodiol. The FDA approved RELYVRIO for use to treat ALS in 2022. Prolongs life ~ 9 months.

These drugs provide limited relief are controversial and slow disease progression by only months



Controversial Treatments

Recent clinical trial failures by leading FDA approved drugs highlights the significant unmet need for safe and effective drugs for the treatment of ALS

Amylyx ALS drug fails crucial study, putting company's future in doubt

The company says it will pause promotion of the drug, Relyvrio, and may pull it from the market in the coming weeks.

Published March 8, 2024

Jacob Bell
Senior Reporter

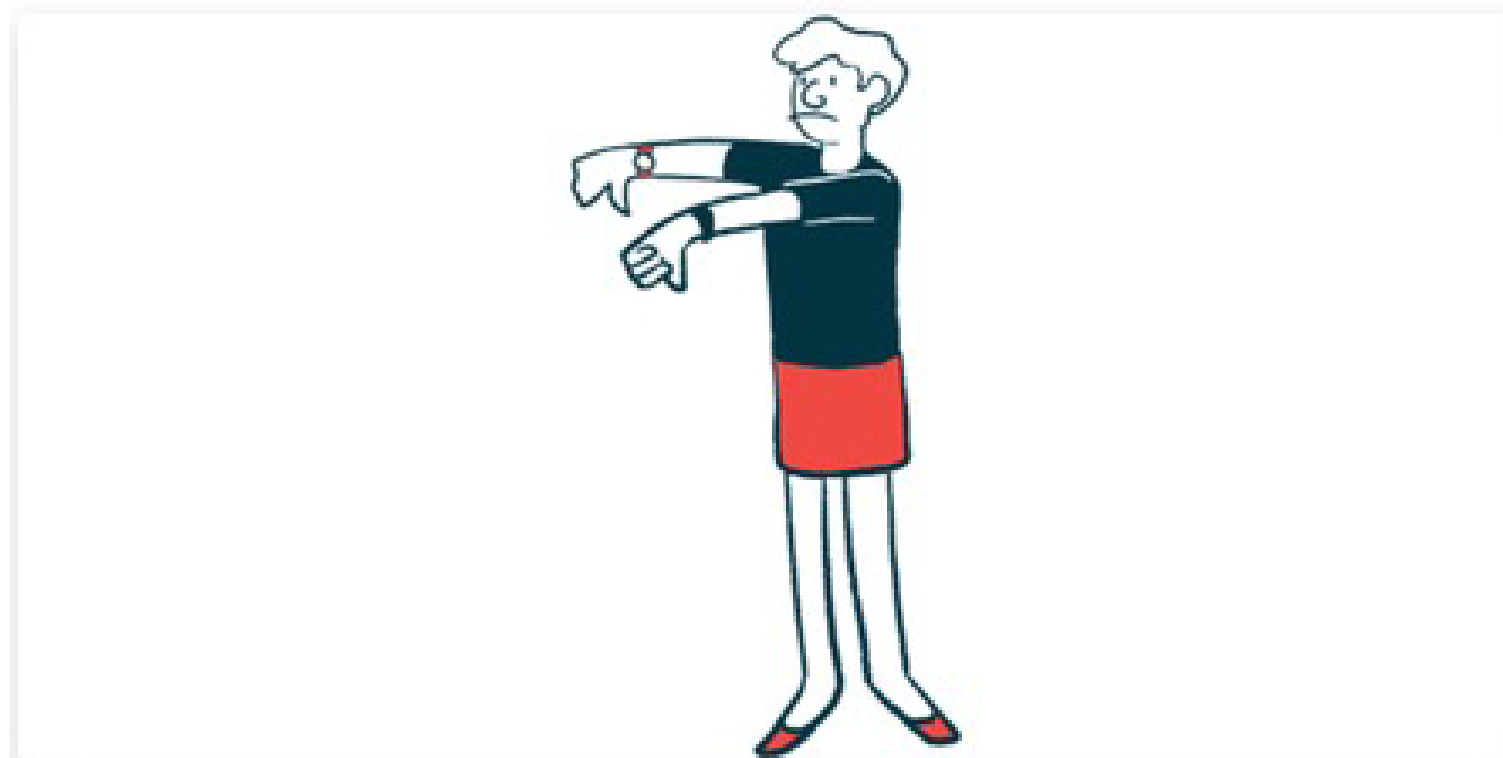


Edaravone formulation FAB122 fails to slow ALS progression

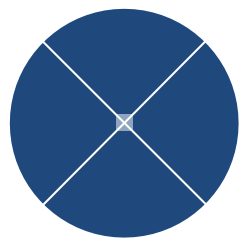
Ferrer to cancel open-label extension ADOREXT, following results



by Margarida Maia, PhD | January 23, 2024

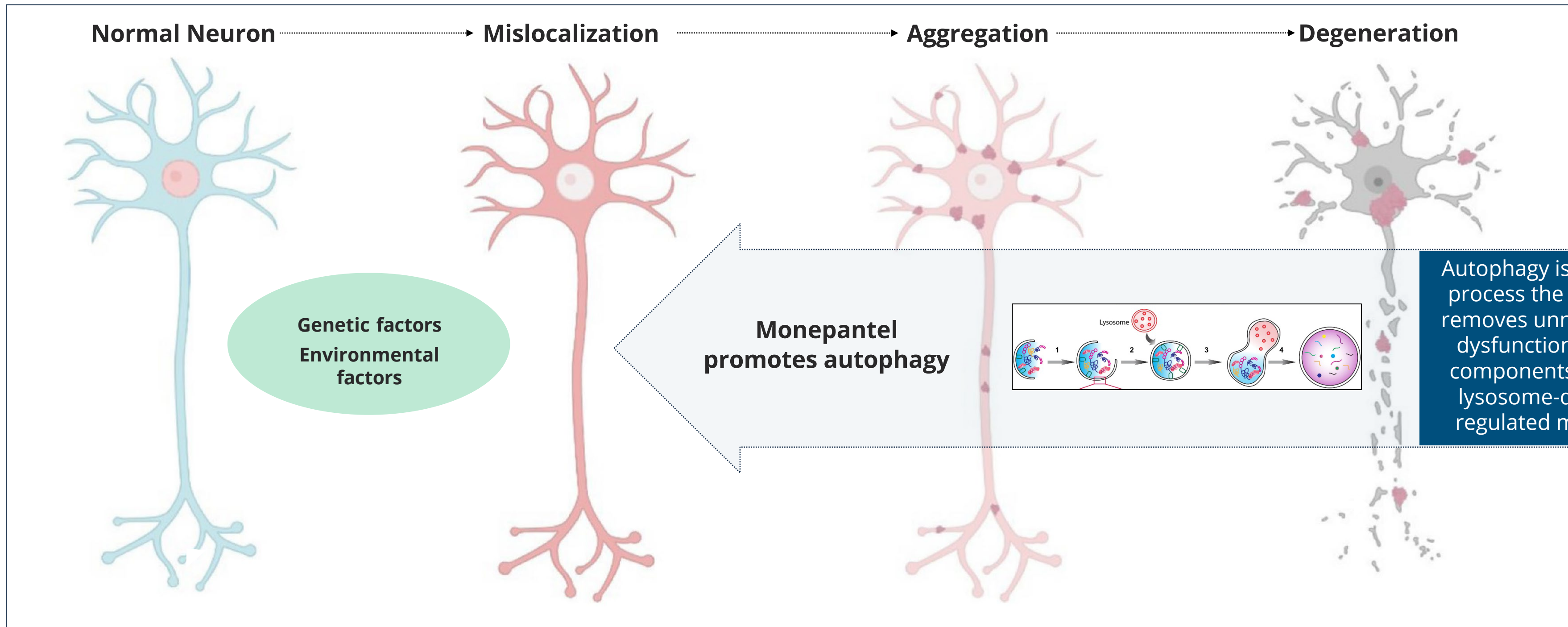


Note: This story was updated Jan 24, 2024, to correct that Radicava and Radicava Oral Suspension are available in Switzerland but not in other European countries.



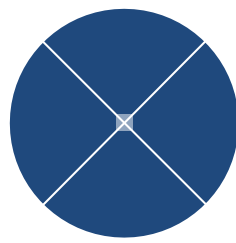
MND /ALS Pathology & Disease Progression

Characterised by progressive degeneration of nerve cells in the spinal cord and brain, MND/ALS affects the voluntary control of the arms and legs, eventually leading to trouble with breathing and death



Protein aggregation¹ is an important feature of MND/ALS pathology. Amyloid deposits from different proteins such as TDP-43, C9ORF72 dipeptide repeats, phosphorylated high molecular weight neurofilament protein, rho guanine nucleotide exchange factor, and FUS have been detected in MND/ALS motor neurons. These aberrant protein deposits become toxic to the cells, leading to neurodegeneration and are targets for therapeutic interventions.

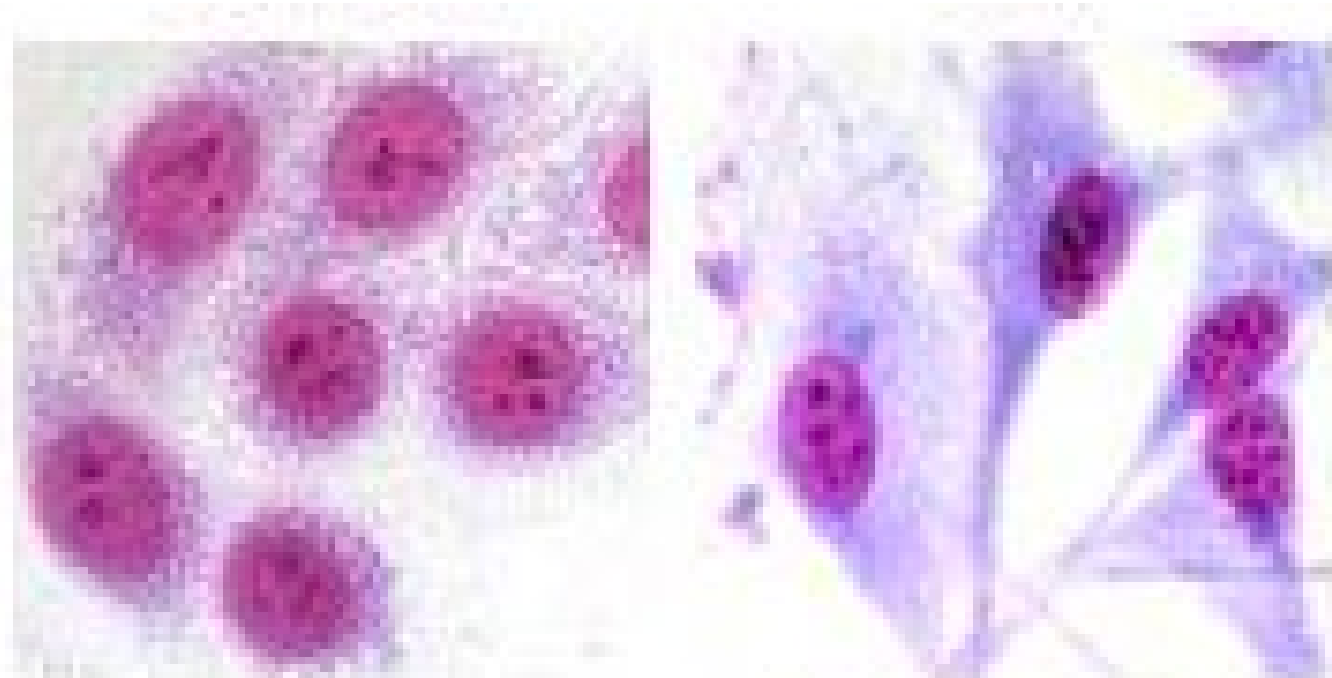
¹Suk, T.R., Rousseaux, M.W.C. The role of TDP-43 mislocalization in amyotrophic lateral sclerosis. *Mol Neurodegeneration* **15**, 45 (2020). <https://doi.org/10.1186/s13024-020-00397-1>



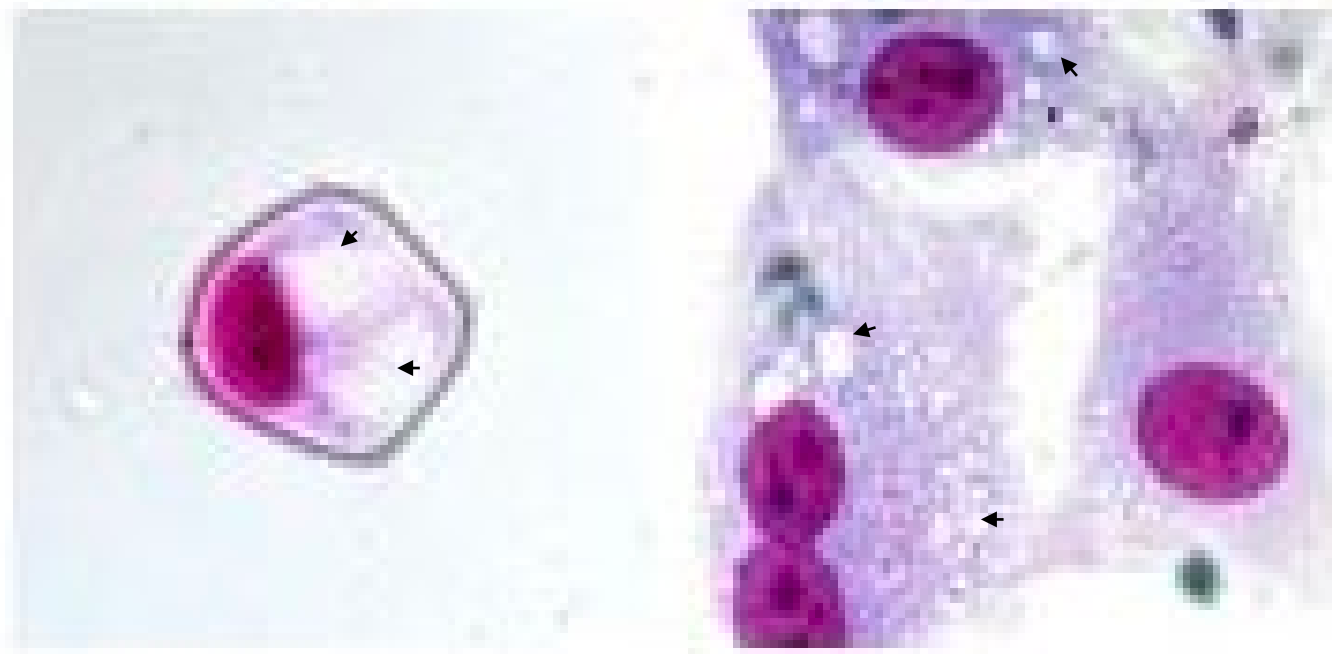
Monepantel Induces Autophagy

Accumulating evidence suggests that impaired autophagy contributes to the accumulation of intracellular inclusion bodies consisting of misfolded proteins, which is a hallmark of most neurodegenerative diseases

Control



Monepantel (25 μM)



A2780 ovarian cancer cells

U87-MG glioma cells

International Journal of Molecular Sciences

Review

Autophagy in Neurodegenerative Diseases: A Hunter for Aggregates

Hyungsun Park ^{1,2}, Ju-Hee Kang ^{2,3} and Seongju Lee ^{1,2,*}

frontiers | Frontiers in Aging Neuroscience

Targeting the autophagy-lysosomal pathway | **Huntington disease: a pharmacological perspective**

Junsheng Yang* and Chaoyue Zhang

TYPE Mini Review
PUBLISHED 25 May 2023
DOI 10.3389/fnagi.2023.1175598

REVIEW Open Access

Therapeutic potential of autophagy-enhancing agents in Parkinson's disease

Tim E. Moors^{1*}, Jeroen J. M. Hoozemans², Angela Ingrassia¹, Tommaso Beccari³, Lucilla Parnetti⁴, Marie-Christine Chartier-Harlin^{5,6} and Wilma D. J. van de Berg¹

Contents lists available at ScienceDirect

Ageing Research Reviews

journal homepage: www.elsevier.com/locate/arr

Review

Autophagy in Alzheimer's disease pathogenesis: Therapeutic potential and future perspectives

Zhigang Zhang^{a,b}, Xifei Yang^d, You-Qiang Song^{b,c,*}, Jie Tu^{a,**}

Clinical and Experimental Immunology, 2022, 209, 140–150
<https://doi.org/10.1093/cei/uxac017>
Advance access publication 16 February 2022

Review

Autophagy modulation in multiple sclerosis and experimental autoimmune encephalomyelitis

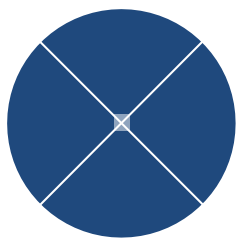
Donghui Shen^{1,*}, Kang Liu², Hongyan Wang¹ and Haifeng Wang^{1*}

REVIEW Open Access

Is amyotrophic lateral sclerosis/ frontotemporal dementia an autophagy disease?

Zhiqiang Deng^{1,2,3}, Patricia Sheehan³, Shi Chen^{1,2*} and Zhenyu Yue^{3*}

* Arrows depict autophagolysosomes (small lysosomal sacs or vacuoles that breaks down the cellular junk in our cells during the process of autophagy)



Monepantel Significant Global Opportunity

The Neurodegenerative Disease Market is estimated to be USD 55.12 billion in 2024, and is expected to reach USD 77.82 billion by 2029, growing at a CAGR of 7.14%¹

Motor Neuron Disease

- ALS, most common type of MND
- > 268,000 ALS patients globally
- no cure, always fatal

ALS sales > \$1B globally by 2029²

Parkinson's Disease

- 2nd most common neurodegenerative disorder
- > 8.5m PD patients globally
- only symptomatic treatments

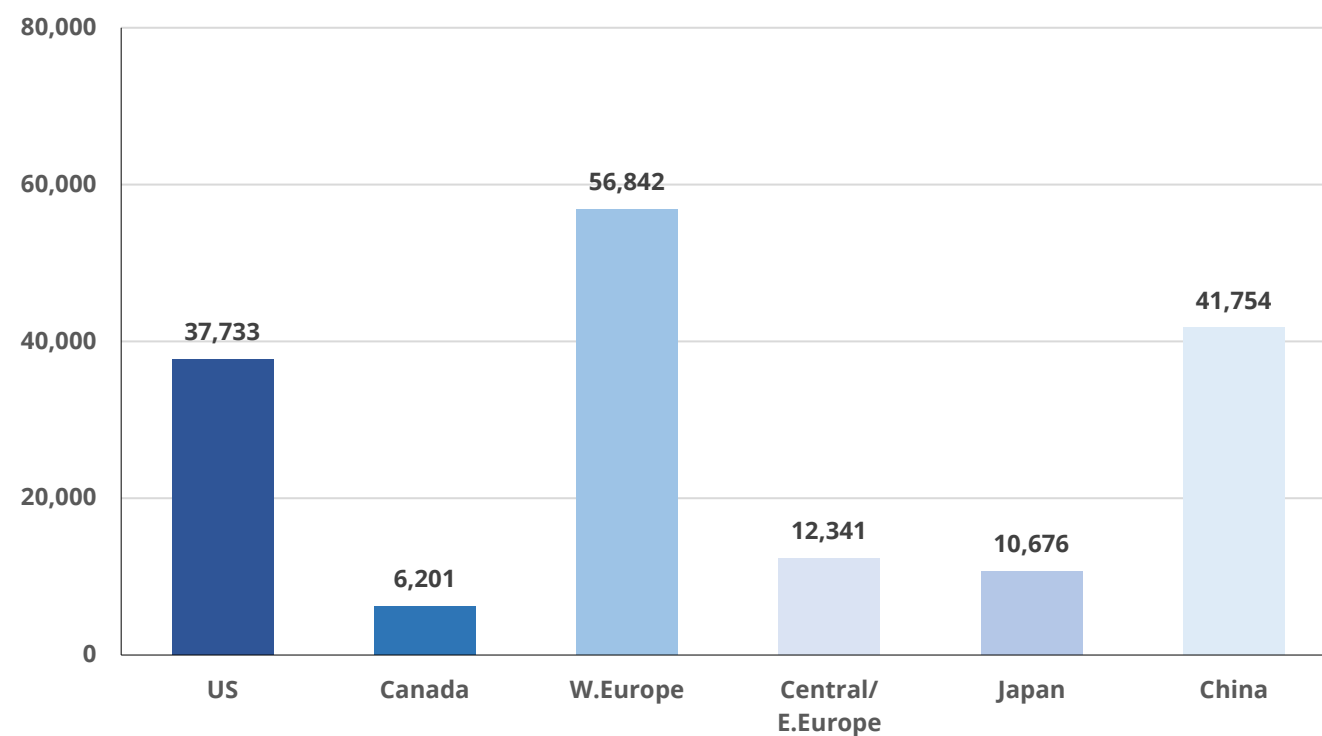
PD sales > \$6B globally by 2029³

Dementias

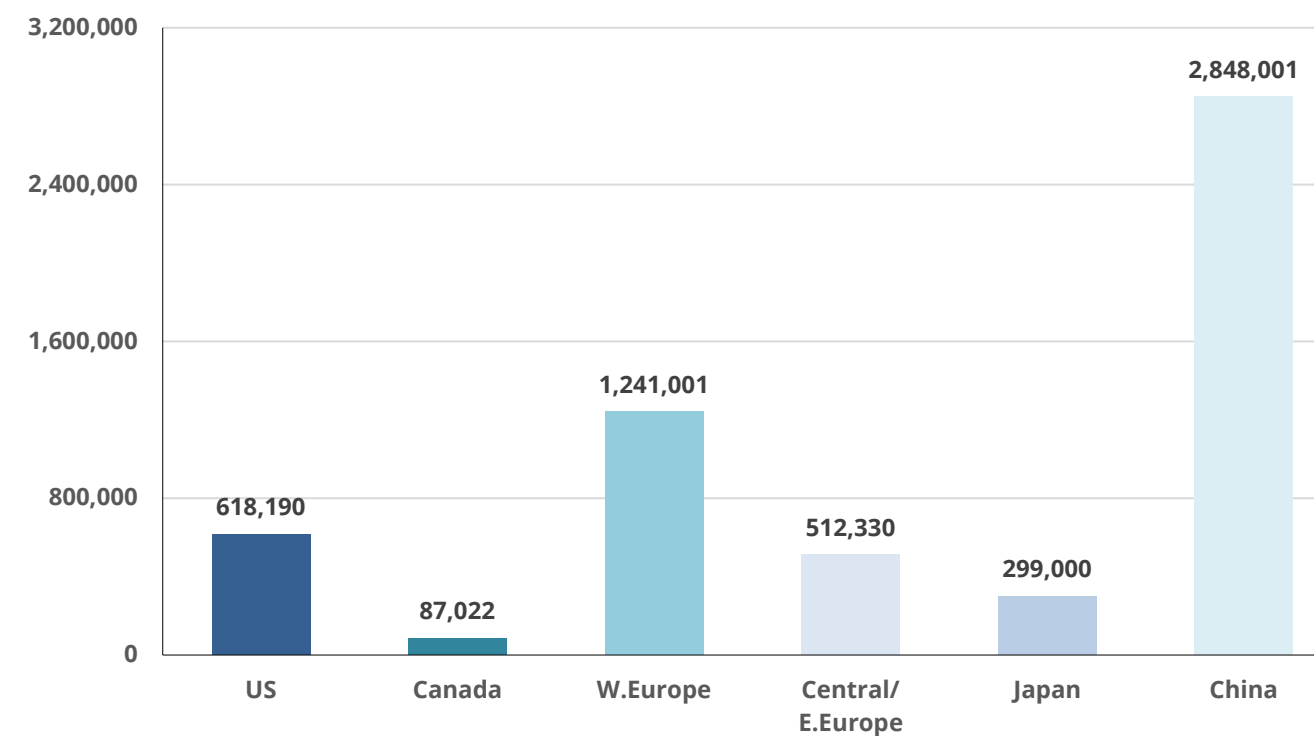
- Alzheimer's disease - most common dementia & neurodegenerative disorder
- > 57m dementia patients globally
- very limited treatment options

AD sales > \$9B globally by 2029⁴

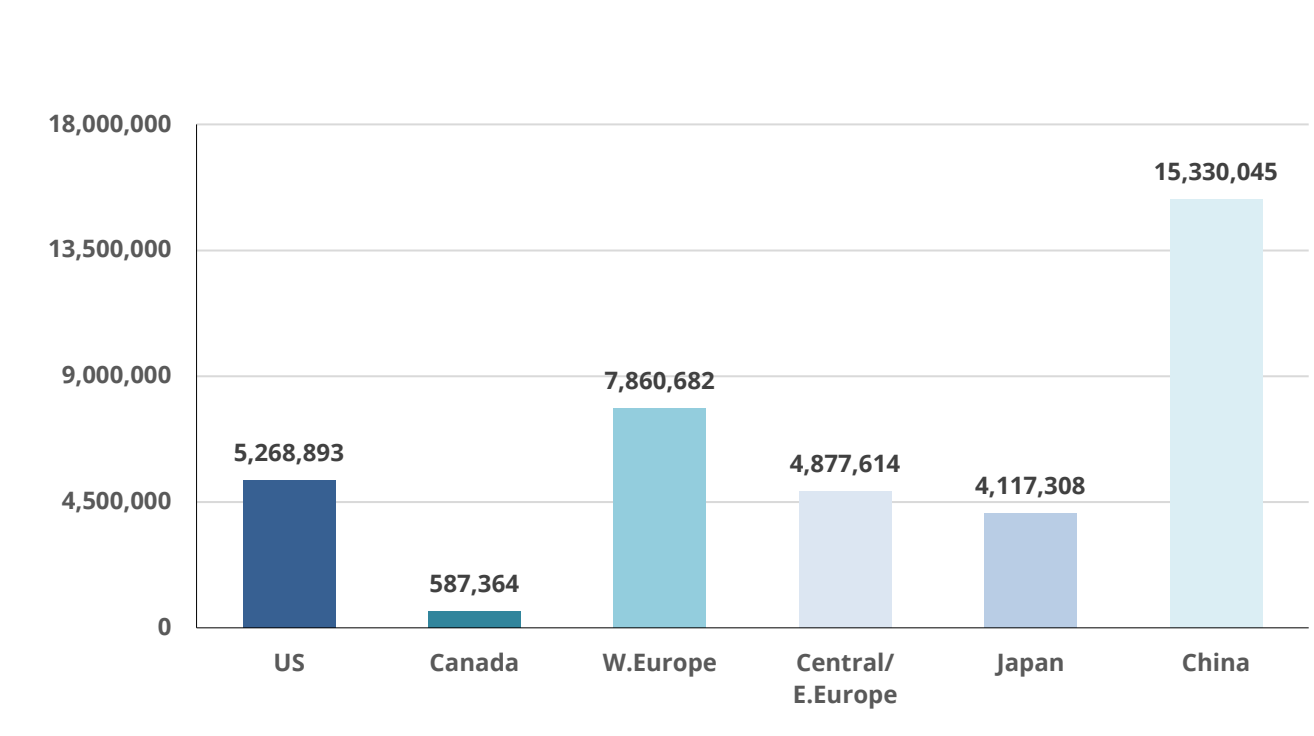
Est. Diagnosed MND Patients by Region⁵



Est. Diagnosed PD Patients by Region⁶



Est. Diagnosed Dementias Patients by Region⁷



¹ <https://www.mordorintelligence.com/industry-reports/neurodegenerative-disease-market>

² Clarivate, DRG, ALS 2020

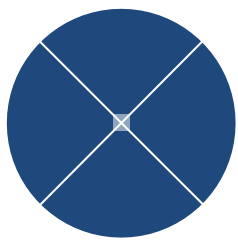
³ Parkinson's Disease Drugs Market Size & Share Analysis - Growth Trends & Forecasts (2024 - 2029)

⁴ Mordor Intelligence Alzheimer's Disease Diagnostics and Therapeutics Market Size & Share Analysis - Growth Trends & Forecasts (2024 - 2029)

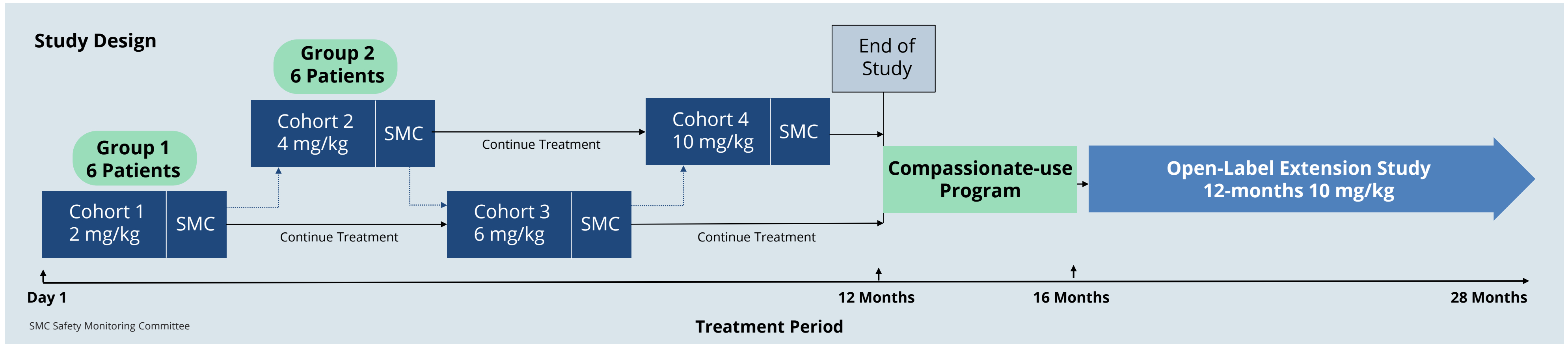
⁵ Frontiers of Neurology April 2022 Vol 13 article 864439

⁶ Frontiers in Public Health Dec 2021 Vol 9 article 776847

⁷ Lancet Public Health 2022; 7: e105-25



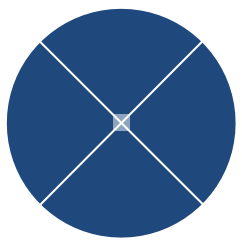
The Phase 1 MEND Study is an open label, multicentre study involving 12 patients with MND/ALS with the goal of determining the recommended Phase 2/3 dose based on safety and preliminary efficacy



Study Update



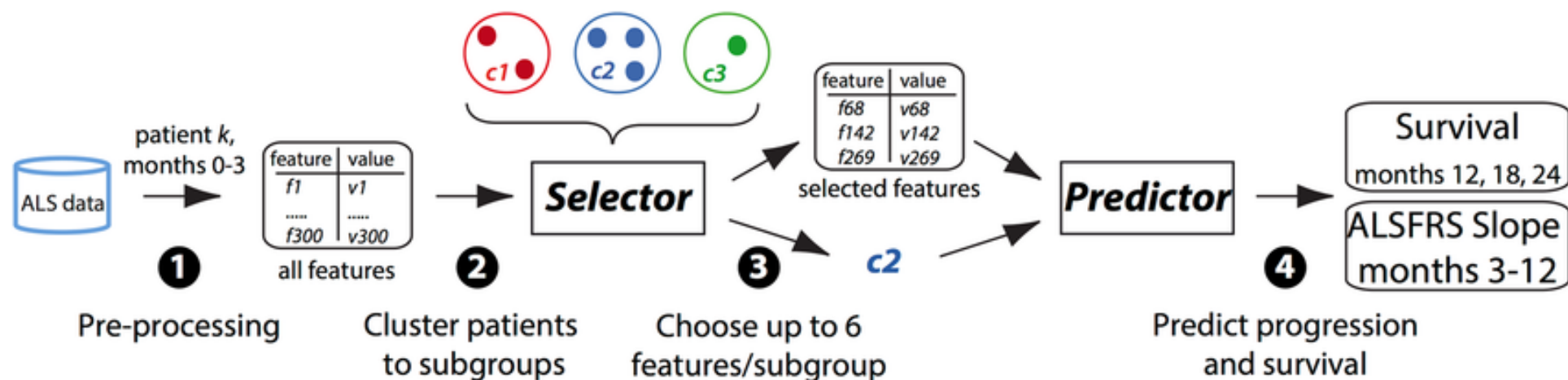
- Positive top-line data released in Q1 CY24
- Patients have continued treatment with monepantel under a compassionate-use program
- 8 of the 12 patients have rolled-over into 12-month Open-Label Extension Study
- Treatment continues to be very well-tolerated
- First Group of 6 patients entering their 17 month of continuous treatment with monepantel
- Data has be used to support the Orphan Drug Designation request and to begin a global registration adaptive Phase 2/3 Study in H1 CY24



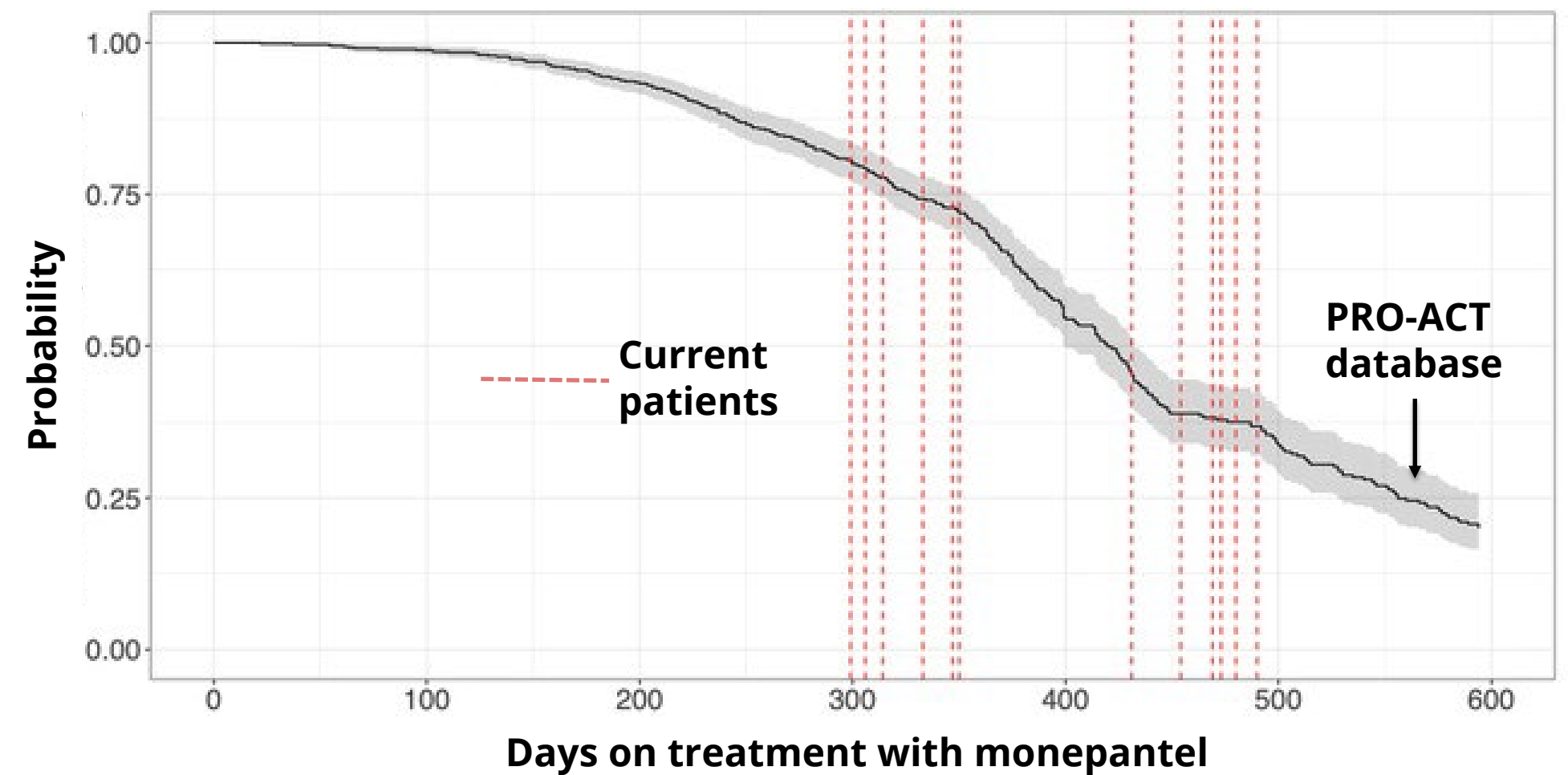
Statistical survival estimations based on comparisons to the PRO-ACT¹ historical ALS database, points to the probability that all 12 patients treated with monepantel being alive today being less than 1 in 1,000

Berry Consultants Statistical Analysis

- Berry's analysis involved **comparing patients in the PRO-ACT database** with **similar characteristics** to those in PharmAust's **Phase 1 MEND Study** adjusting for differing diagnosis durations
- **One-year study survival** rate estimate of **67.7%** with a 95% Confidence Interval
- Considering differential diagnosis durations, the **probability estimates of all 12 Phase 1 MEND patients surviving today** without treatment are **less than 0.1%** (less than 1 in 1,000)

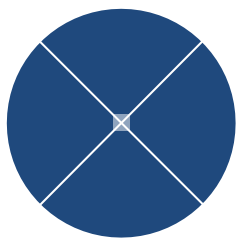


Overall Survival Probability



The PRO-ACT database is the largest publicly available repository of merged ALS clinical study data. Data were pooled from 16 completed Phase 2/3 ALS clinical studies and one observational study. Over 8 million de-identified longitudinally collected data points from more than 8,600 persons with ALS, including demographics, family histories, and longitudinal clinical and laboratory data.

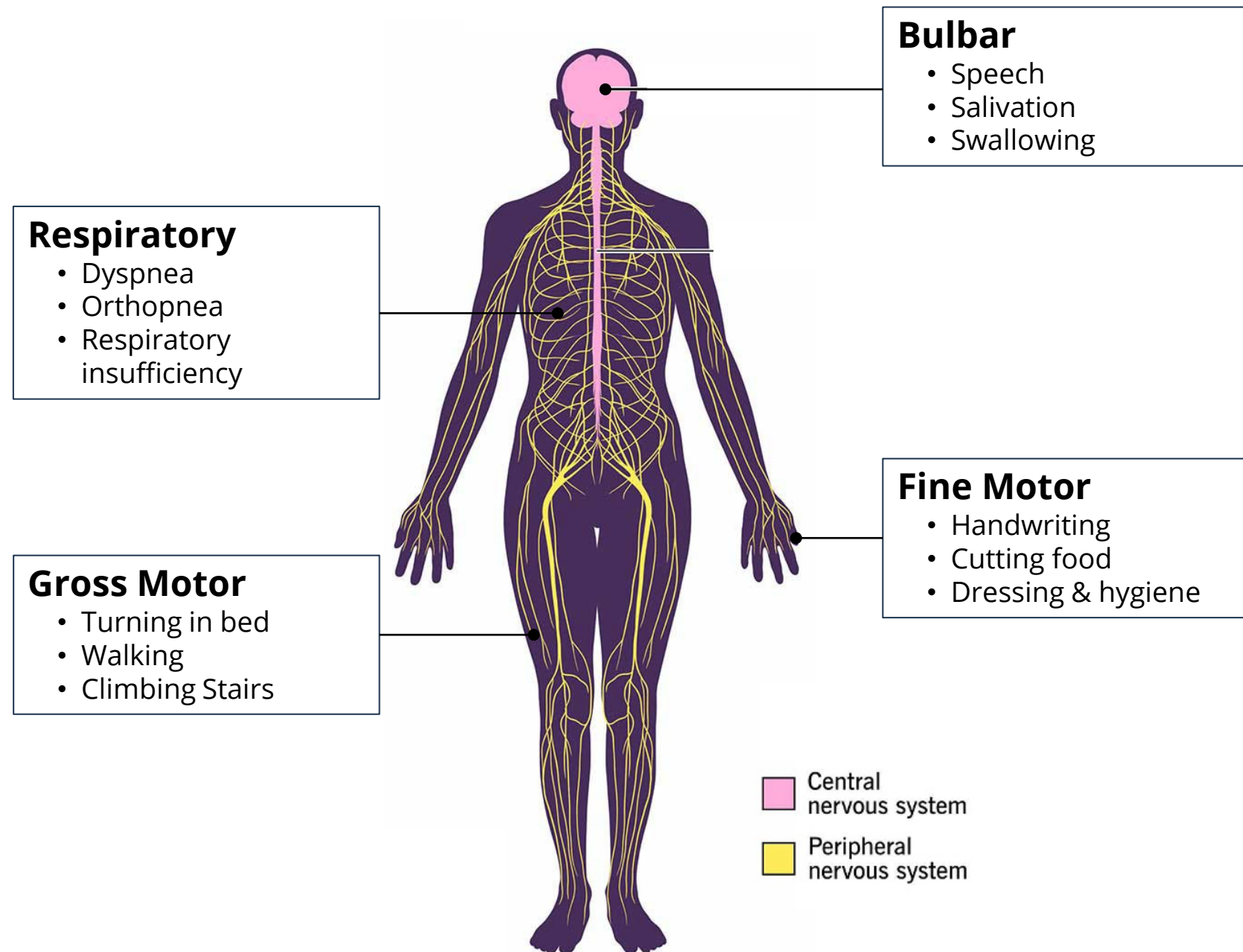
¹Atassi N, Berry J, Shui A, Zach N, Sherman A, Sinani E, Walker J, Katsovskiy I, Schoenfeld D, Cudkowicz M, Leitner M. The PRO-ACT database: design, initial analyses, and predictive features. Neurology. 2014 Nov 4;83(19):1719-25. doi: 10.1212/WNL.0000000000000951. Epub 2014 Oct 8. PMID: 25298304; PMCID: PMC4239834.



Phase 1 Amyotrophic Lateral Sclerosis Function Rating Score – Revised (ALSFRS-R)

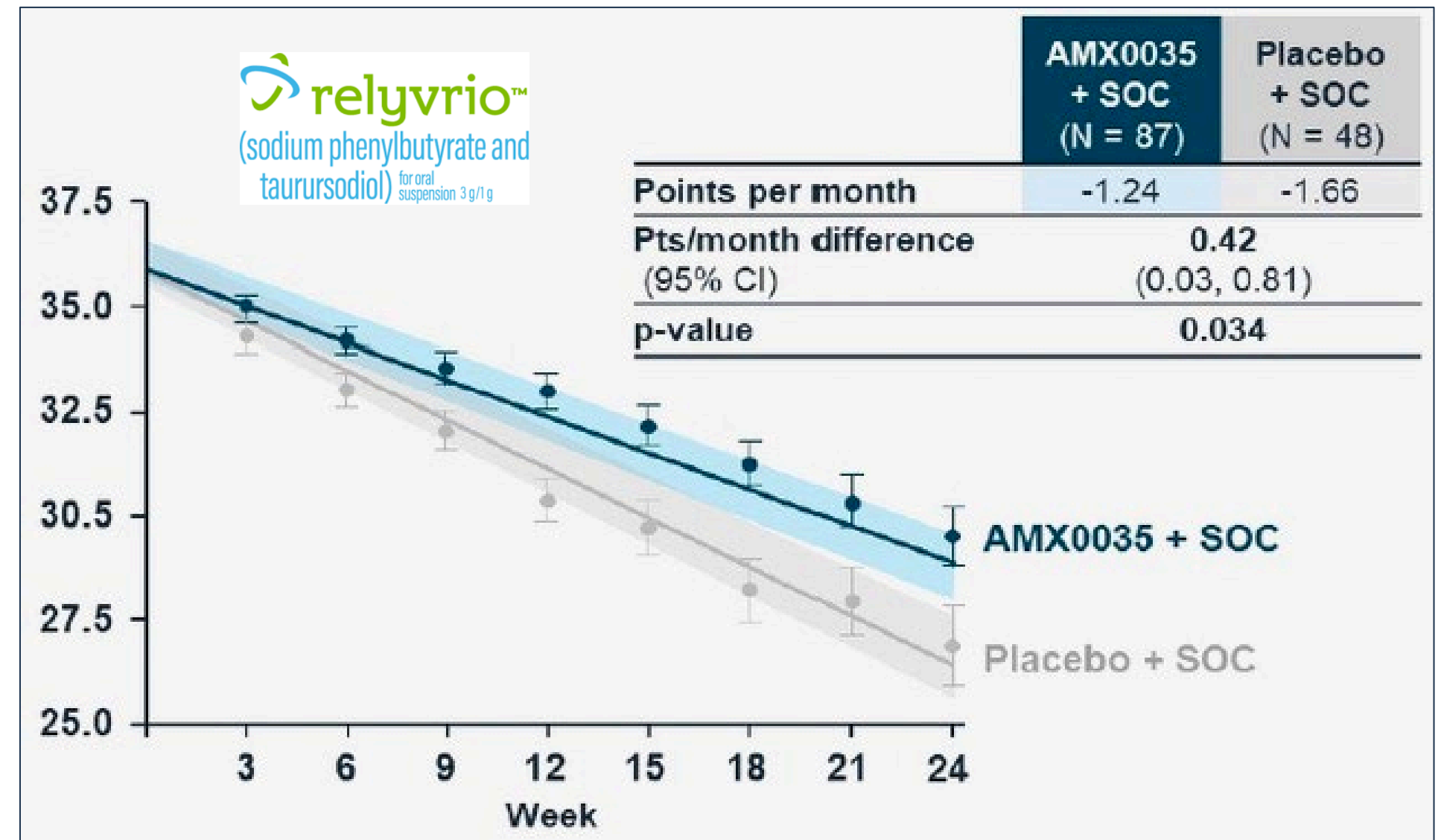
ALSFRS-R is a predictor of survival time in ALS patients.¹ The speed at which ALS progresses, measured by the rate of decline in a patient's ALSFRS-R score over time, can be used to confidently predict disease prognosis.

ALSFRS-R Domains Assessed



Each task is rated on a five-point scale from 0 = can't do, to 4 = normal ability. Individual item scores are summed to produce a reported score of between 0=worst and 48=best.

AMX0035 – 25% Slower Decline in ALSFRS-R



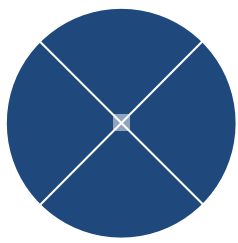
Slowing the decline in ALSFRS-R by 16.5%

=

4-5 months median survival²

¹Beghi E, Mennini T, Bendotti C, et al. The heterogeneity of amyotrophic lateral sclerosis: a possible explanation of treatment failure. *Curr Med Chem.* 2007;14(30):3185-3200

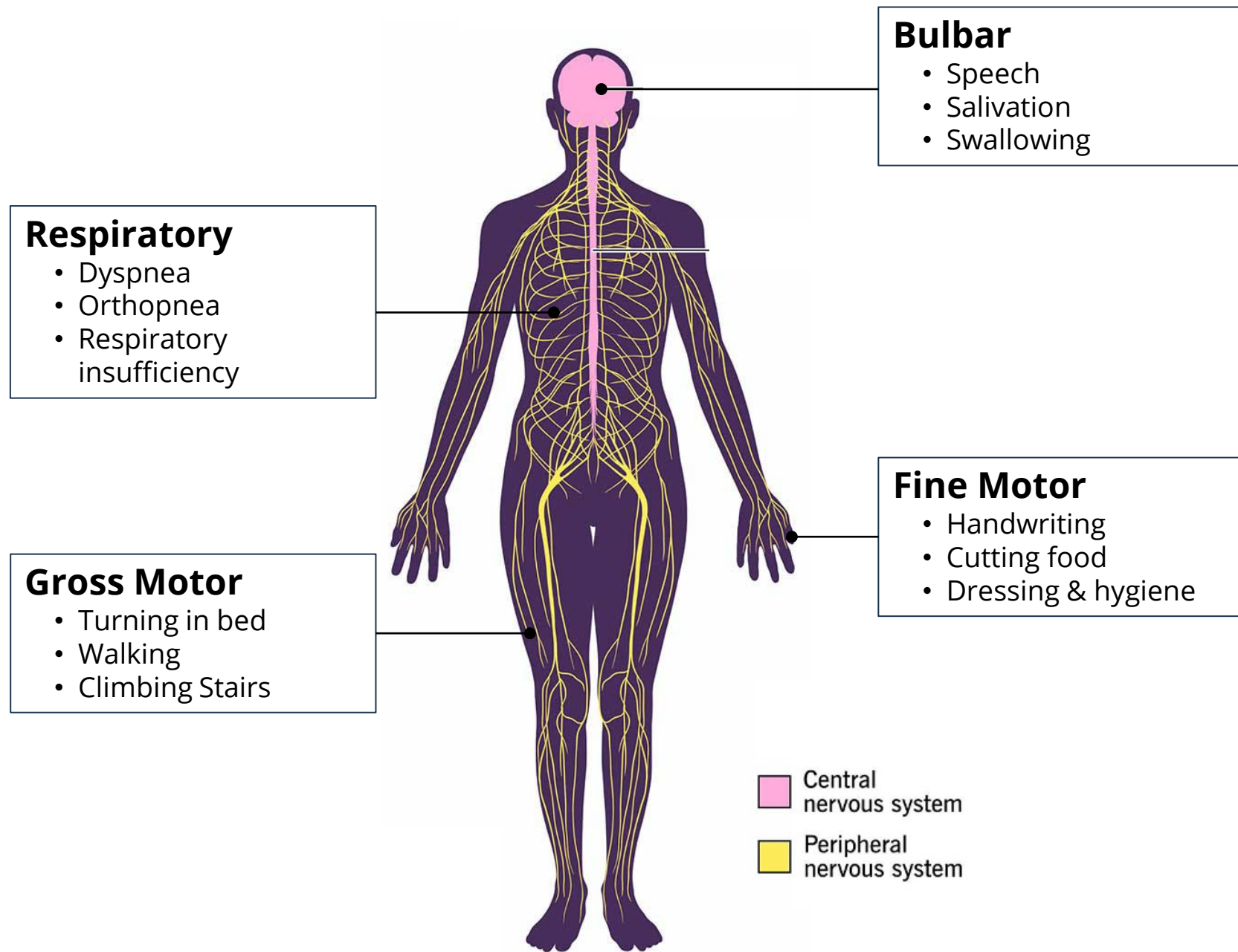
²Leigh PN, Swash M, Iwasaki Y, et al. Amyotrophic lateral sclerosis: a consensus viewpoint on designing and implementing a clinical trial. *Amyotroph Lateral Scler Other Motor Neuron Disord.* 2004;5(2):84-98



Phase 1 Preliminary Efficacy Amyotrophic Lateral Sclerosis Function Rating Scale – Revised (ALSFRS-R)

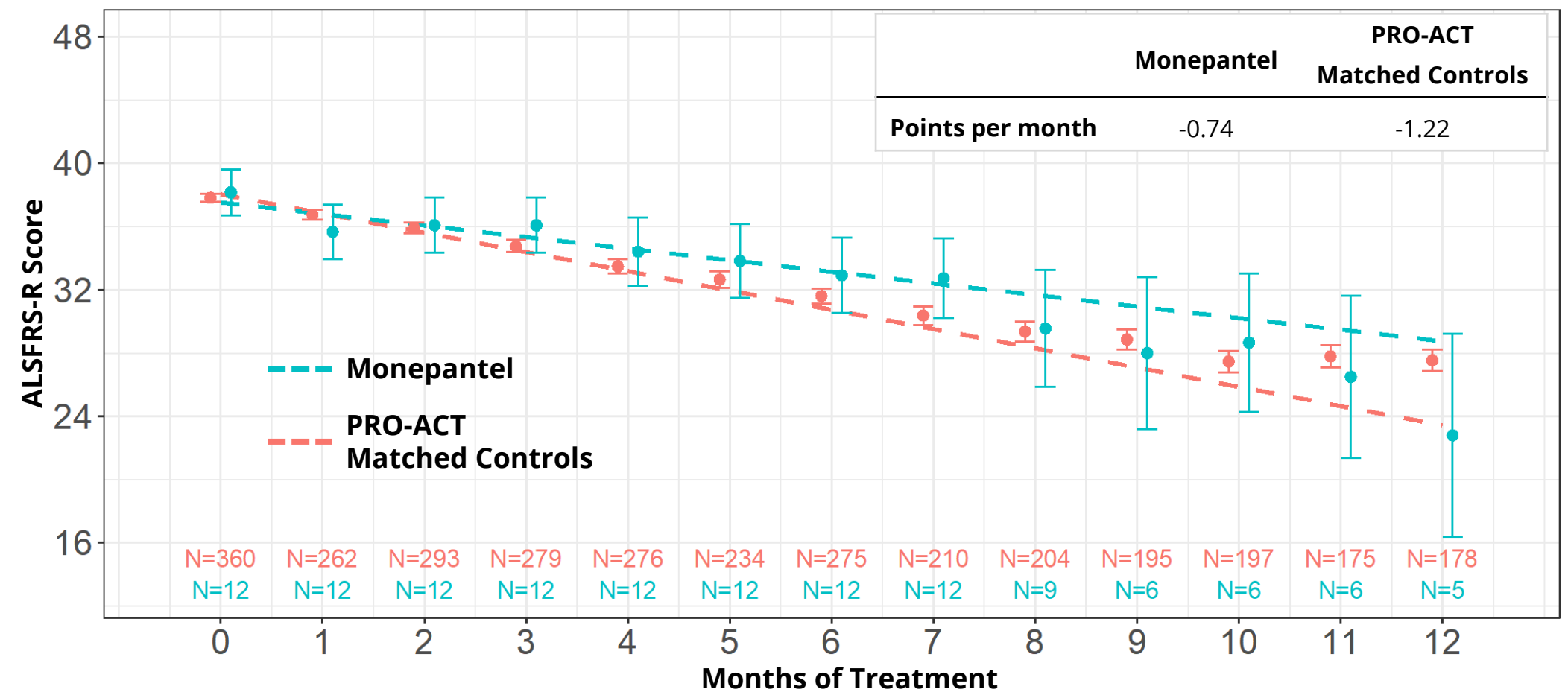
Treatment with monepantel for up to 12 months slowed the progression of MND/ALS in all 12 patients by 39% when compared to matched controls from the PRO-ACT database¹

ALSFRS-R Domains Assessed



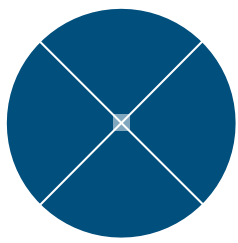
Each task is rated on a five-point scale from 0 = can't do, to 4 = normal ability. Individual item scores are summed to produce a reported score of between 0=worst and 48=best.

MPL – 39% Slower Decline in ALSFRS-R



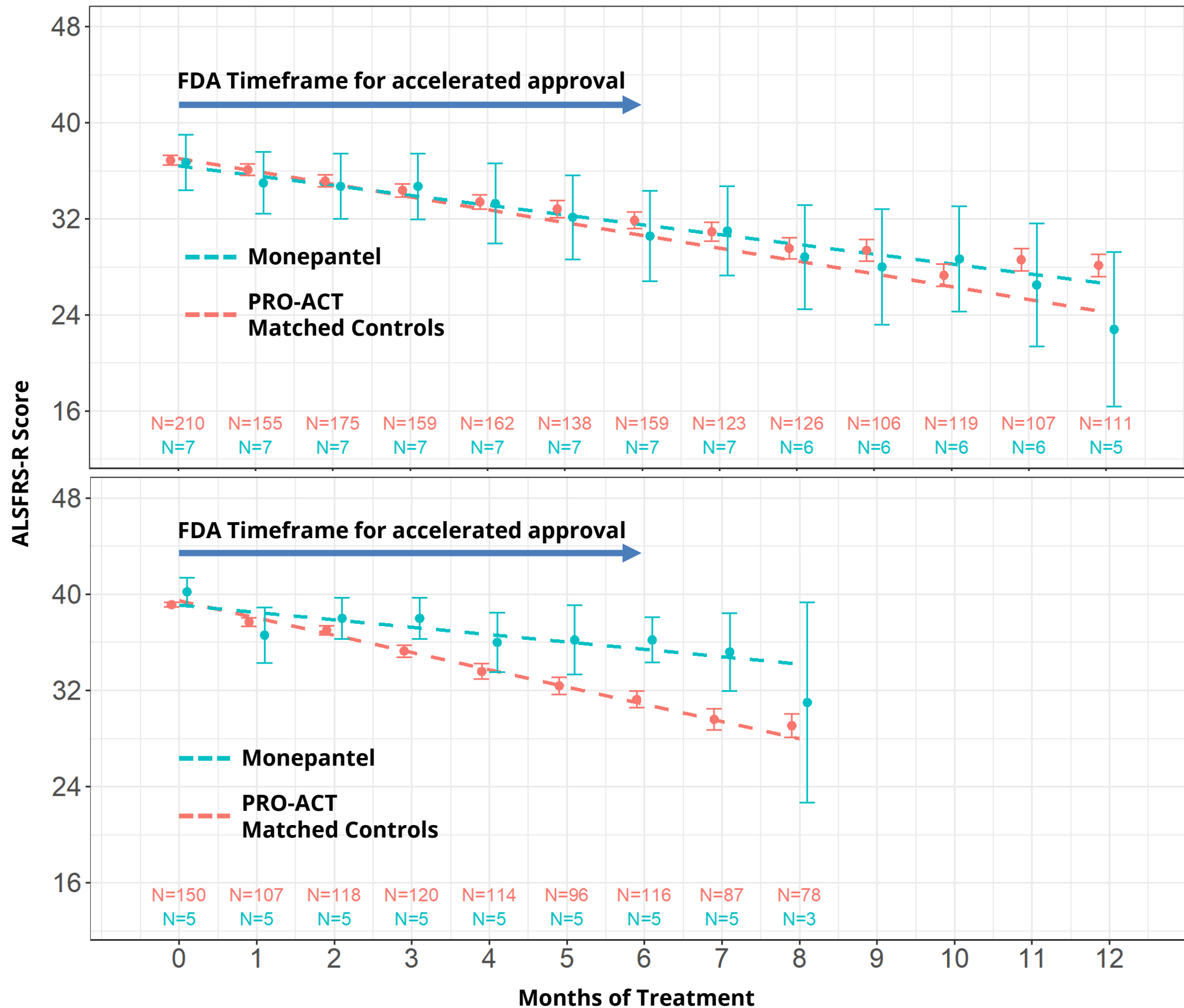
The PRO-ACT database is the largest publicly available repository of merged ALS clinical study data. Data were pooled from 16 completed Phase 2/3 ALS clinical studies and one observational study. Over 8 million de-identified longitudinally collected data points from more than 8,600 persons with ALS, including demographics, family histories, and longitudinal clinical and laboratory data.

¹Atassi N, Berry J, Shui A, Zach N, Sherman A, Sinani E, Walker J, Katsovskiy I, Schoenfeld D, Cudkowicz M, Leitner M. The PRO-ACT database: design, initial analyses, and predictive features. Neurology. 2014 Nov 4;83(19):1719-25. doi: 10.1212/WNL.0000000000000951. Epub 2014 Oct 8. PMID: 25298304; PMCID: PMC4239834.



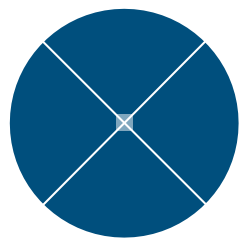
Phase 1 Preliminary Efficacy Amyotrophic Lateral Sclerosis Function Rating Score – Revised (ALSFRS-R)

Dose response observed between Cohort 1 and 2. Rate of disease progression in Cohort 2 was slowed by an impressive 58% compared to the PRO-ACT database matched controls



Cohort 1
-0.83 points per month
23% Slower Decline in ALSFRS-R

Cohort 2
-0.60 points per month
58% Slower Decline in ALSFRS-R



Phase 1
Comparison to Leading FDA-Approved Product

Monepantel displays a superior safety, tolerability and preliminary efficacy profile to the leading FDA approved drug Relyvrio®

Estimated Additional Life Expectancy for MPL

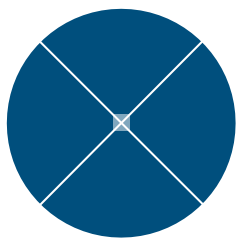
| | Estimated rate of ALSFRS-R Decline (points per month) | Slowing in ALSFRS-R Decline (%) | Slope Change from PRO-ACT matched controls | Additional Life Expectancy (months) |
|----------------------------------|---|---------------------------------|--|-------------------------------------|
| Combined Cohort 1 & 2 | -0.74 | 39% | 0.48 | 13.5 – 56.5 |
| Cohort 1 (Low Dose) | -0.83 | 23% | 0.24 | |
| Cohort 2 (High Dose) | -0.60 | 58% | 0.83 | |
| Relyvrio® | -1.24 | 25% | | 8 – 9 |

Median Survival Table¹

| ALSFRS-R Slope (points per month) | Median Survival (Months) | Increase in Life Expectancy (Months) |
|-----------------------------------|--------------------------|--------------------------------------|
| <-0.25 | 78.5 | 56.5 |
| -0.25-0.45 | 44.0 | 22.0 |
| -0.50-0.99 | 35.5 | 13.5 |
| >-1.0 | 22.0 | |

Treatment with monepantel may prolong a patient’s life expectancy by 13.5-56.5 months

¹Elamin M, Bede P, Montuschi A, Pender N, Chio A, Hardiman O. Predicting prognosis in amyotrophic lateral sclerosis: a simple algorithm. J Neurol. 2015 Jun;262(6):1447-54. doi: 10.1007/s00415-015-7731-6. Epub 2015 Apr 11. PMID: 25860344; PMCID: PMC4469087.



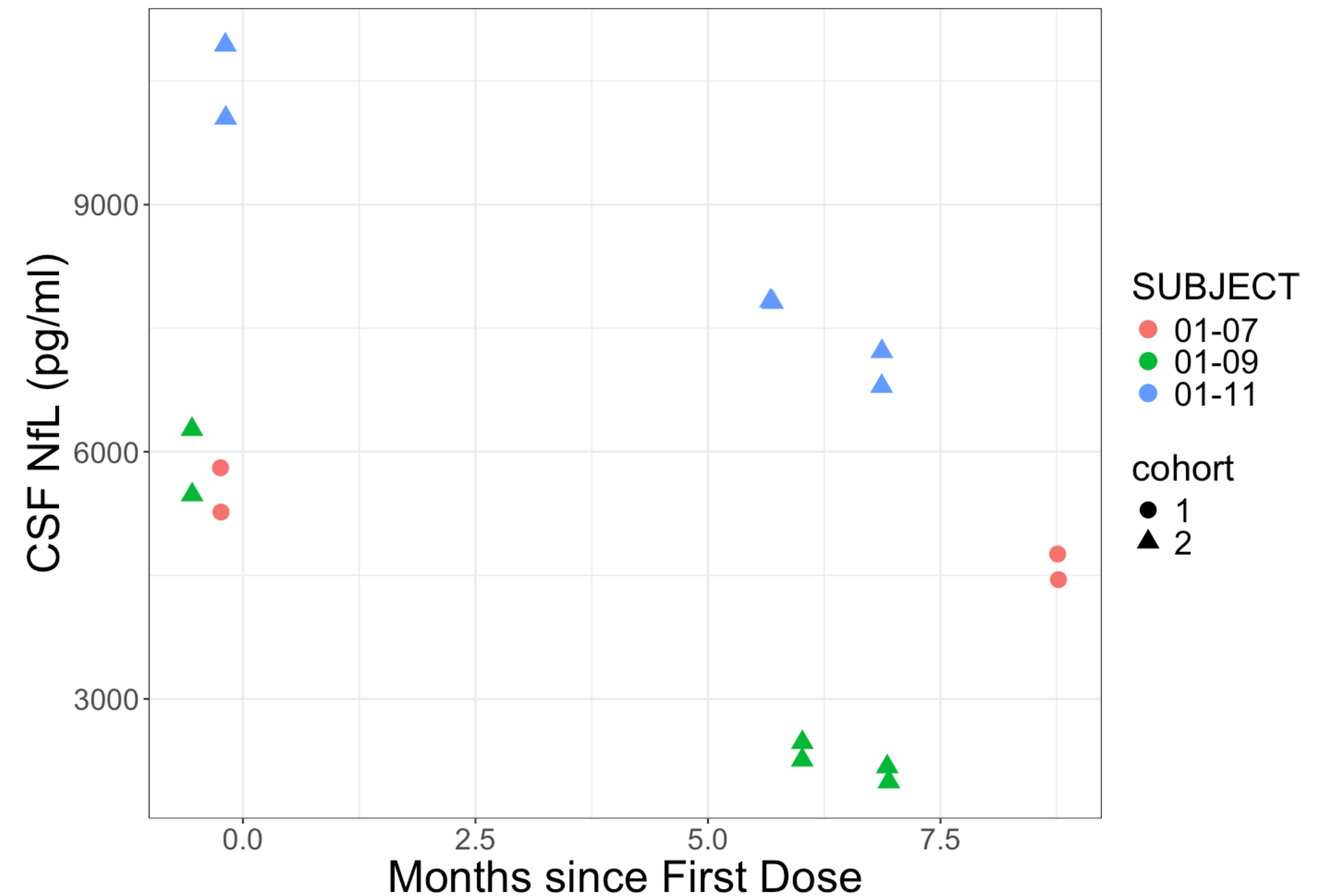
Phase 1 Biomarker Assessment

Neurofilament levels correlate with disease progression rate in ALS and higher levels of neurofilament are associated with faster/greater decline of ALSFRS-R over time¹

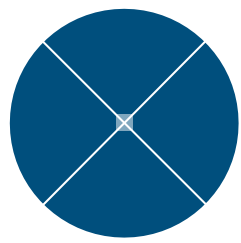
- **Decline of 6.9% (14.0 %, 0.9%) per month in CSF NfL levels**
- **Although limited data, the CSF NfL data is encouraging**

Biomarker data important for receiving accelerated approval

Change in CSF NfL Levels



¹Brodovitch A, Boucraut J, Delmont E, Parlanti A, Grapperon A-M, Attarian S, et al. Combination of serum and CSF neurofilament-light and neuroinflammatory biomarkers to evaluate ALS. Sci Rep. 2021;11(1):703. doi: 10.1038/s41598-020-80370-6.



Phase 1 Conclusion

Positive Phase 1 study results showing monepantel has an excellent safety profile and the ability to slow the progression of MND/ALS

Primary Objectives

Excellent safety and tolerability profile

Preliminary efficacy data

Slows the rate of progression by 58% (Cohort 2 High Dose)

Blood Brain Barrier

Monepantel and its active metabolite, MPLS, and is detectable in cerebrospinal fluid

Competitive Advantage

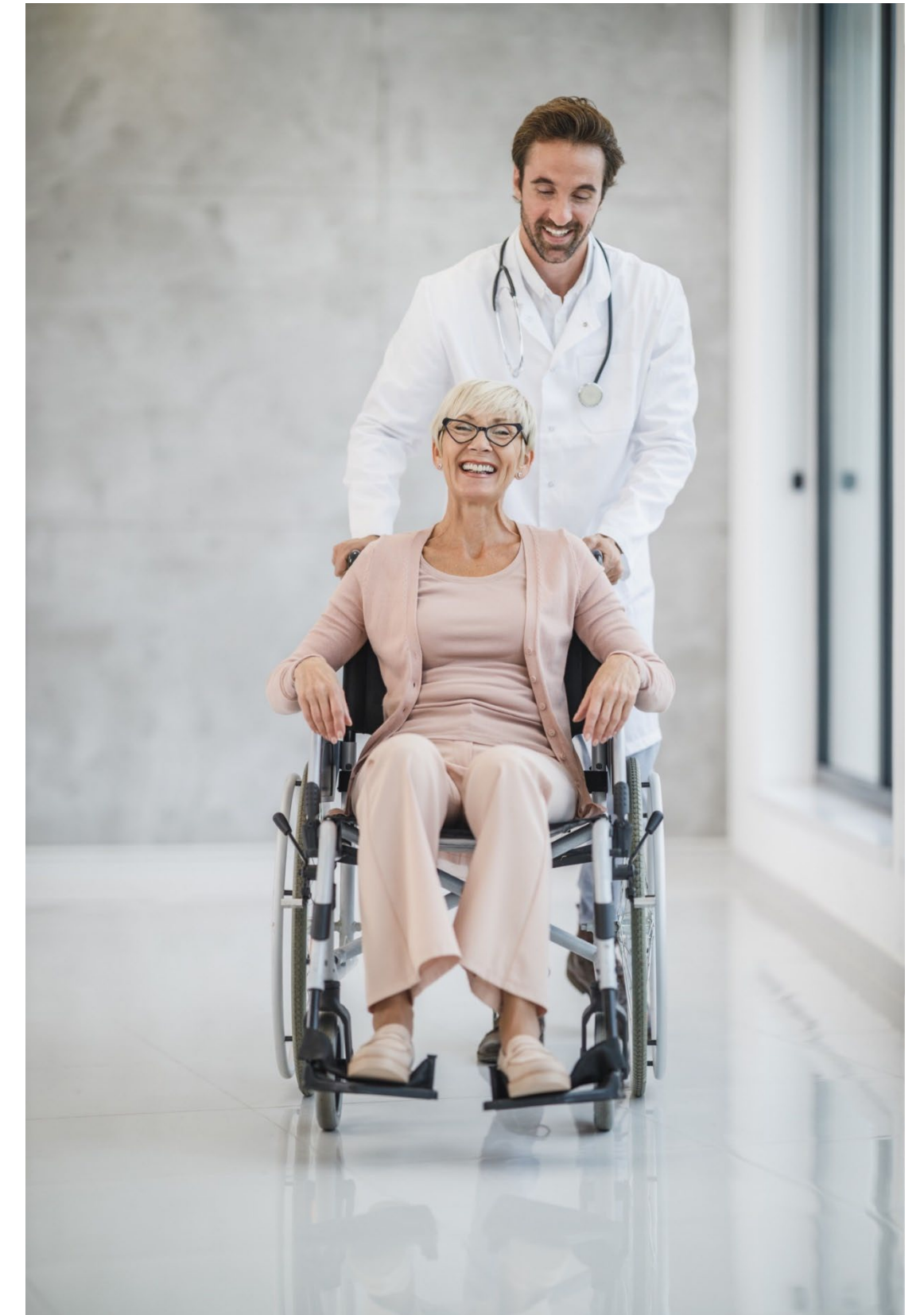
Superior safety, tolerability and preliminary efficacy profile to leading FDA approved drug Relyvrio®

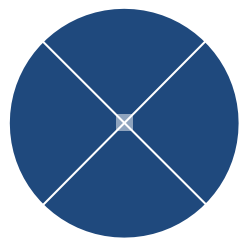
Survival

May prolong a patient's life expectancy by 13.5-56.5 months

Next Steps

Moving forward with pivotal registration Phase 2/3 clinical study

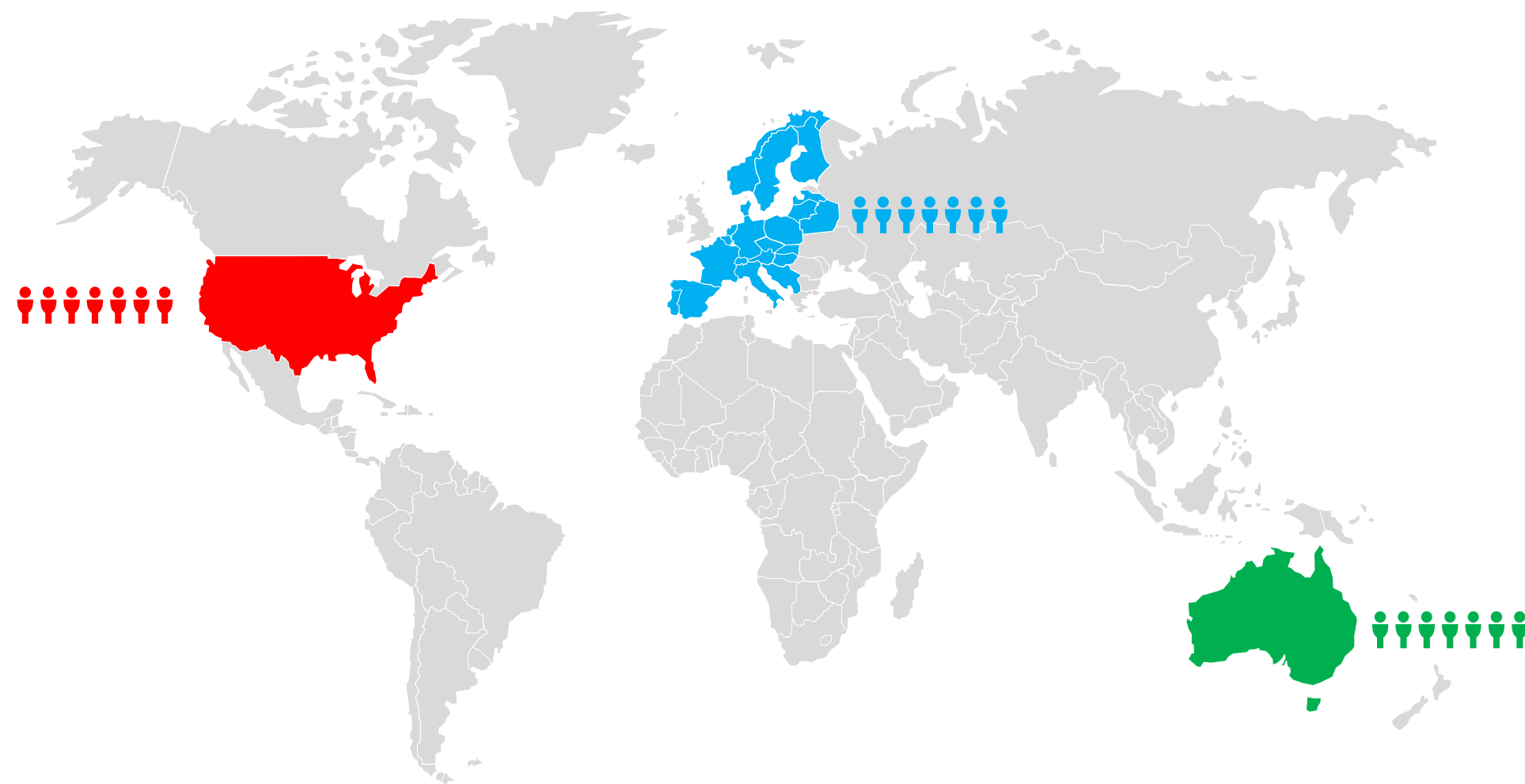




Pre-IND Meeting Response

Successfully completed a Pre-IND meeting with the FDA to confirm the details of the ongoing development program, including the requirements for non-clinical and clinical pharmacology, clinical, chemistry, and manufacturing controls

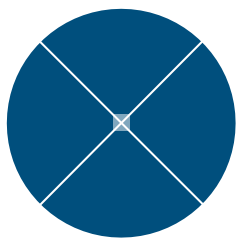
Global Phase 2/3 Clinical Study



Specific FDA Feedback and Guidance

- FDA provided **positive feedback** and outlined the **path required** to potentially receive **accelerated and full approval** of monepantel for the treatment of ALS
- PharmAust will **initiate requirements** requested by the FDA in the **preparation to open an IND application** for the adaptive Phase 2/3 clinical study
- PharmAust will now prepare to **launch clinical sites in Europe and Australia** where data can also be used to **support the FDA drug approval** process
- **Registration in Europe and Australia** now possible with this **global approach**

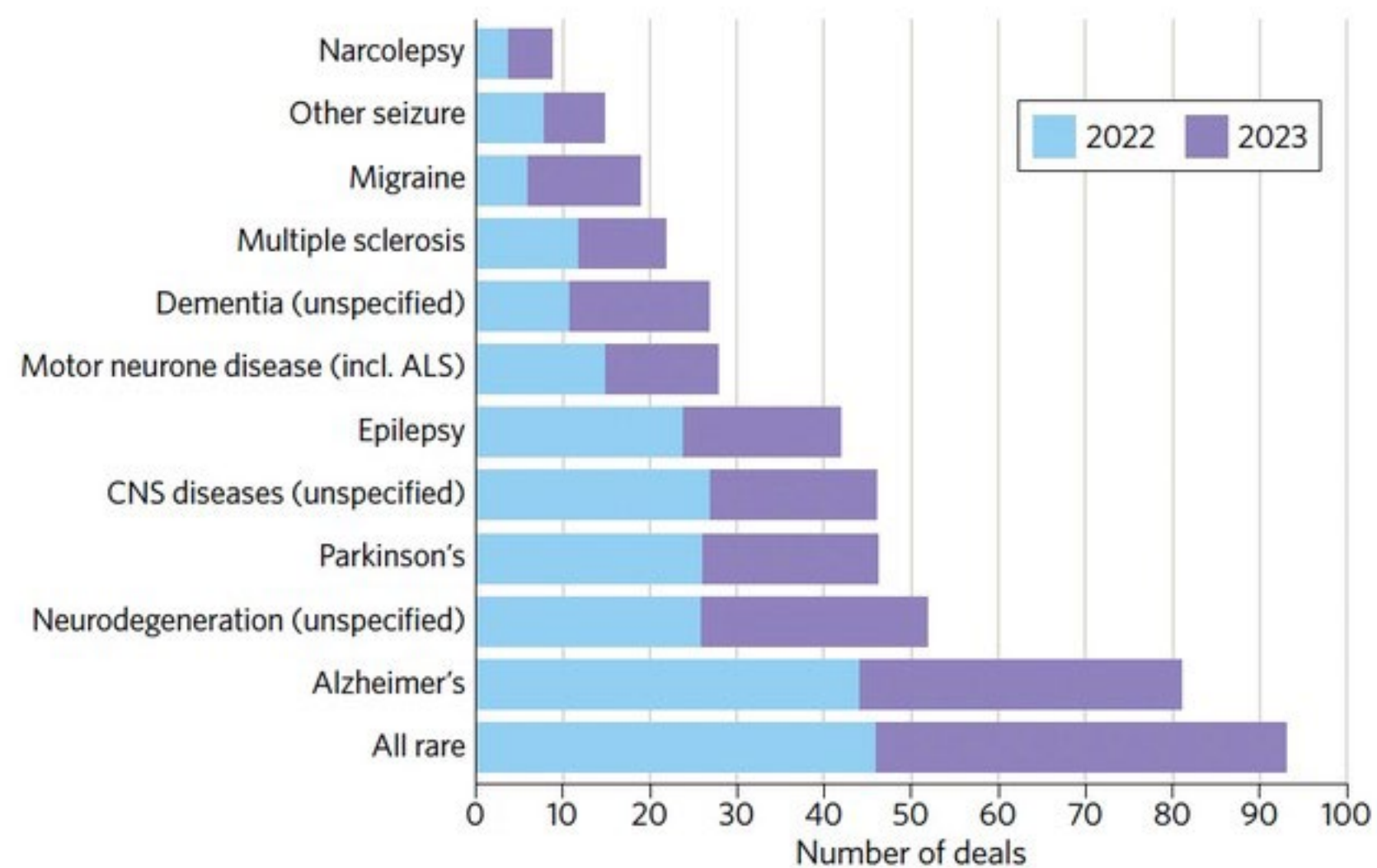
Single clinical study sufficient
subject to demonstrating substantial evidence of effectiveness and an adequate database supporting safety



Rare Central Nervous System disease market

The global CNS rare disease treatment market is expected to reach US\$13.8 billion by 2027 (CAGR > 8.5%)¹

Neurological disease deals by therapy type in 2022 and 2023 (October)²



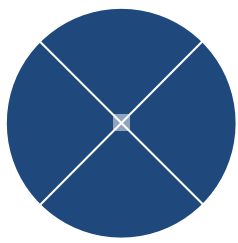
Selected partnering deals in the CNS field in 2023²

| Date | Stage | Companies | Deal Value | Target |
|----------|-------------|-------------------------------------|--|---|
| Jul 2023 | Phase 3 | Biogen / Reata Pharmaceuticals | US\$7.3 Billion Acquisition • Reata just launched Skyclarys (omaveloxolone) in US, under regulatory review in Europe to treat Friedreich's ataxia | Omaveloxolone • Possesses antioxidative and anti-inflammatory activities |
| Mar 2023 | Phase 1 | Biohaven / Hangzhou Highlightll | US\$970 Million License • US\$20 million in cash and equity upfront, development and commercial milestones. tiered royalties | BHV-8000 • Dual Tyrosine Kinase 2 (TYK2)/Janus Kinase 1 (JAK1) inhibitor |
| Sep 2023 | Preclinical | Takeda / Acurastem | US\$580 Million License • Combined upfront payment and milestones could reach US\$580 million in total, alongside royalties | AS-202 • PIKFYVE-targeted antisense oligonucleotide |

Over 49 deals were announced 2023 involving rare CNS diseases, with disclosed deal values totalling more than US\$13.2 billion

¹The Insight Partner March 2020

²Mark Zipkin, Neurodegeneration and rare diseases drive CNS therapy deals. Biopharma Dealmakers News Feature. 1 December 2023. doi: <https://doi.org/10.1038/d43747-023-00128-7>



MND R&D timeline

Timeline

- MEND Study Patient Completes
- ODD Submission
- Management Hires
- Compassionate-use Program
- FightMND Grant Invitation



Q4
CY2023

- Pre-IND Meeting Request
- Berry Consultants Partnership
- OLE Study HREC Approval
- MEND Study Top-line Results
- SAB Appointments



Q1
CY2024

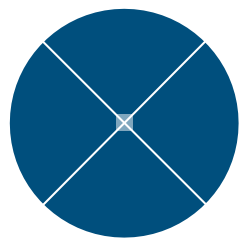
- ODD Response
- Phase 2/3 Study HREC Approval
- First Patient Dosed MND Study



Q2
CY2024



IND – Investigational New Drug; ODD – Orphan Drug Designation; OLE – Open Label Extension; SAB – Scientific Advisory Board



Thank you

**FIGHT
MIND.**
IT TAKES PEOPLE



Health Care Bethlehem





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