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We are admitting audience members from the waiting room.

Please allow a few moments for the webinar to begin.



Patient Navigation

Central resource for people living with ALS

Two webinars
per month



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ALS Link sign-up:



<https://bit.ly/3CqGbhb>

Weekly webinar
registration:



<https://bit.ly/3r6Nd2L>

Upcoming Webinars:

August 28 – HEALEY ALS Platform Trial Biomarker Learnings with James Berry, MD, MPH and Eric Macklin, PhD (Mass General Hospital)

September 11 – Monthly EAP Discussion



IBUDILAST MN-166

NEALS

BJÖRN OSKARSSON, MD, FAAN
MAYO CLINIC FLORIDA

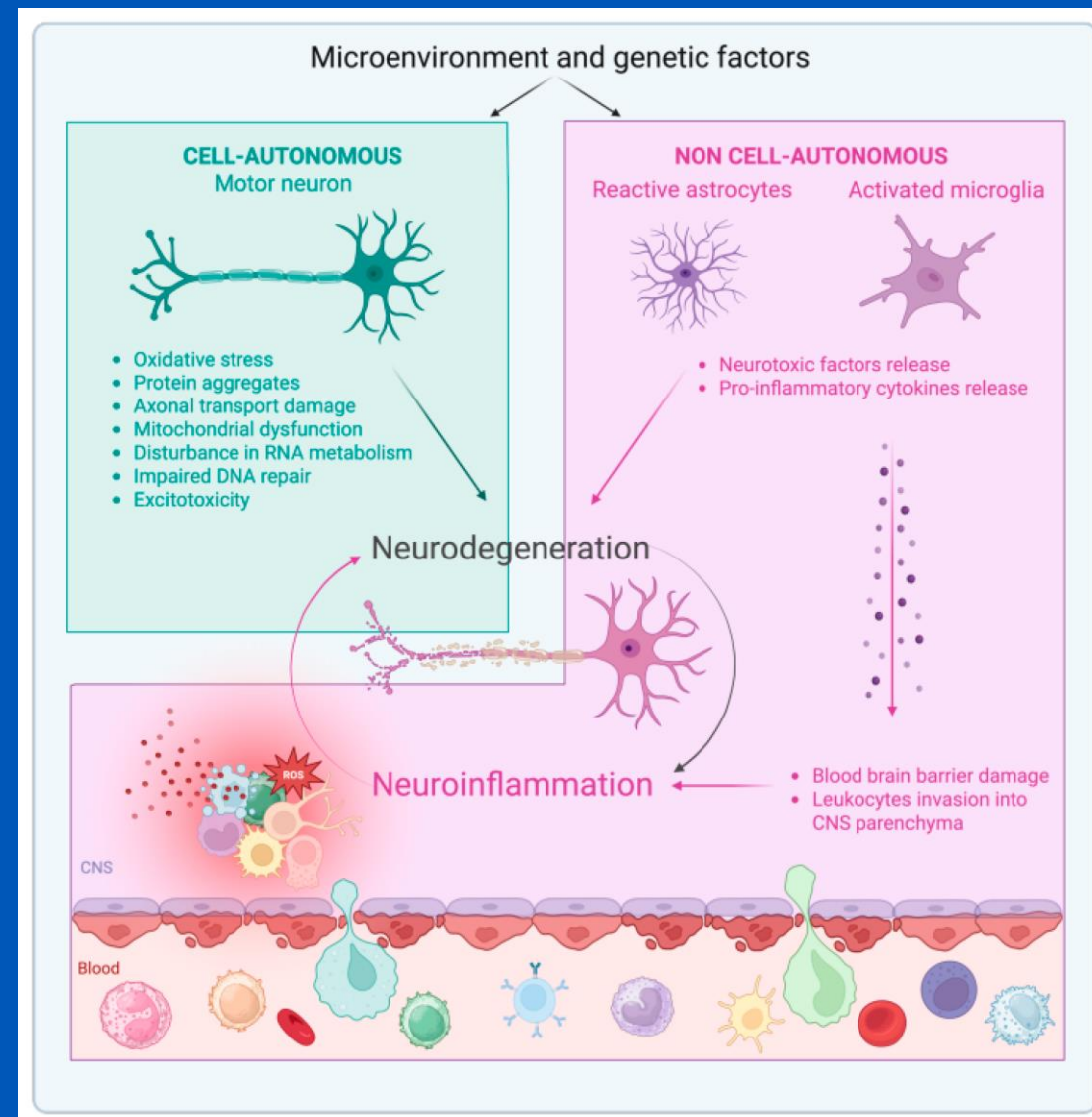


DISCLOSURES

- Consultant: MediciNova*, Tsumura, UniQure, VectorY, Phenonet
- Research grants: MediciNova*, Biogen/Ionis, Tsumura, AnnJi, TARGET ALS, UniQure, NINDS
- * relevant

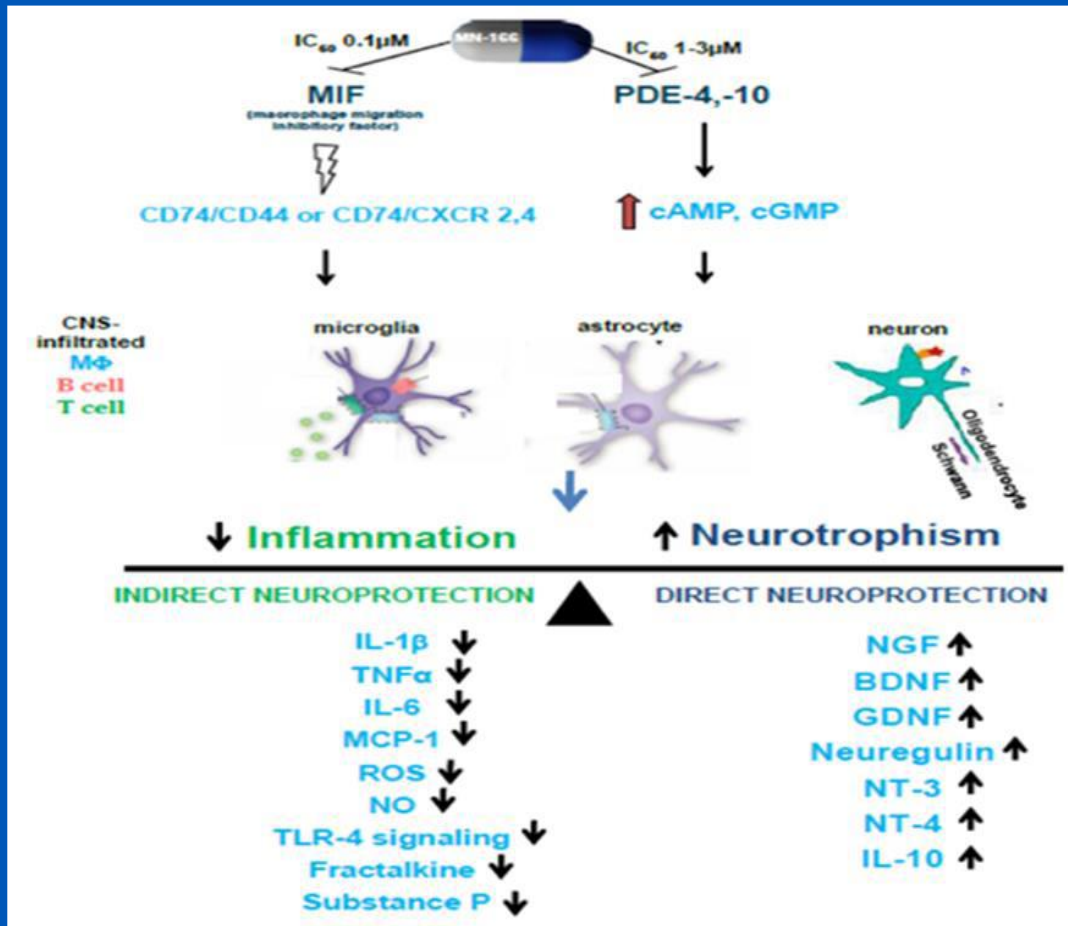
INFLAMMATION IN ALS

- Covered in prior talk
- Many aspects
- Activated monocytes/ microglia
- Proinflammatory cytokines



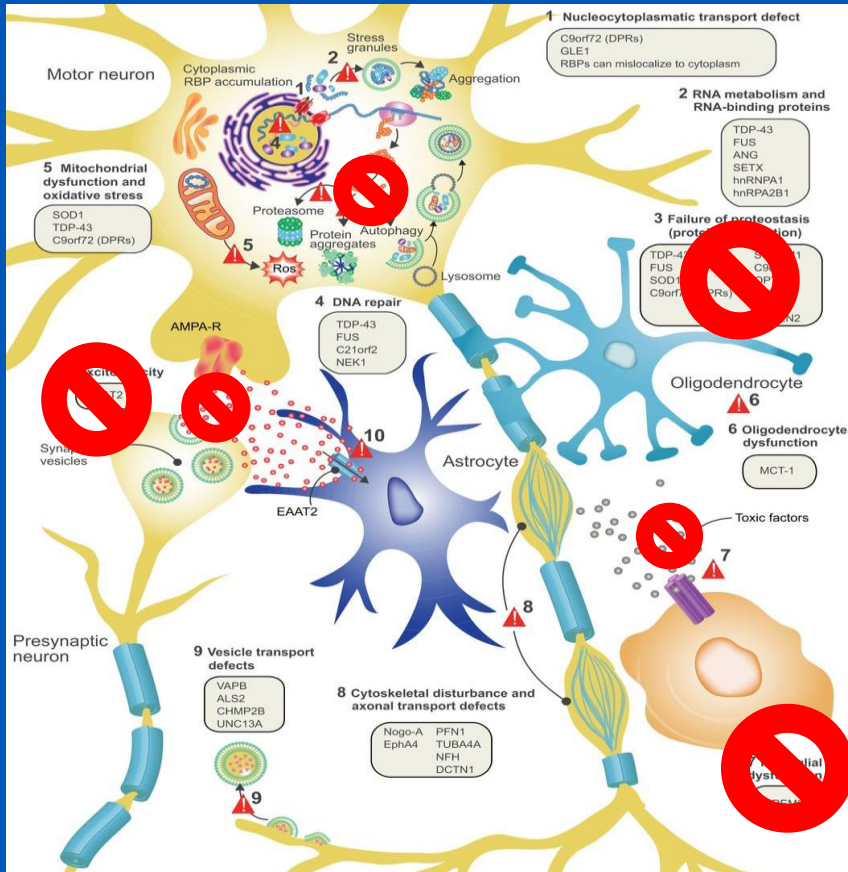
Stacchiotti, C.; et al. Neuroinflammation and Amyotrophic Lateral Sclerosis: Recent Advances in Anti-Inflammatory Cytokines as Therapeutic Strategies. *Int. J. Mol. Sci.* **2025**,

IBUDILAST MN-166



- orally available small molecule
- penetrates the CNS well
- Inhibits pro inflammatory cytokine macrophage migration inhibitory factor (MIF) and PDE 3, 4, 10
- Demonstrated neuroprotective action and glial cell attenuation
- 1989 Approved in Japan
- Excellent safety

ALS : pathology and proposed disease mechanisms.



1. Nucleocytoplasmic transport defect
2. Altered RNA metabolism and RNA-binding proteins
3. **Failure of proteostasis (protein aggregation)**
4. Impaired DNA repair
5. Mitochondrial dysfunction and oxidative stress
6. Oligodendrocyte dysfunction
7. **Neuroinflammation / microglia dysfunction**
8. Cytoskeletal dysfunction and axonal transport defect
9. Vesicle transport defects
10. **Excitotoxicity**

PHASE 1B/2A PROJECT

- NCT 02238626 - B. Brooks
- Single-center, randomized double blind (6 months), placebo-controlled trial followed by OLE (6 months).
- This study showed no overall change in disease progression between placebo and MN-166
- More patients receiving MN-166 remained stable or improved ALSFRS-R, ALSAQ-5 and average muscle strength in the post-hoc analysis.

Responder Analysis in MN-166-ALS-1201

Parameter	Responder category	Placebo (n=16)	MN-166 (n=33)
ALSFRS-R Total score	Stable or improved	2/16 (12.5%)	7/33 (21.2%)
ALSAQ-5		4/16 (25%)	17/33 (51.5%)
MMT		4/16 (25%)	11/33 (33.3%)

ALSFRS-R Score

NCT02238626

EC PP population

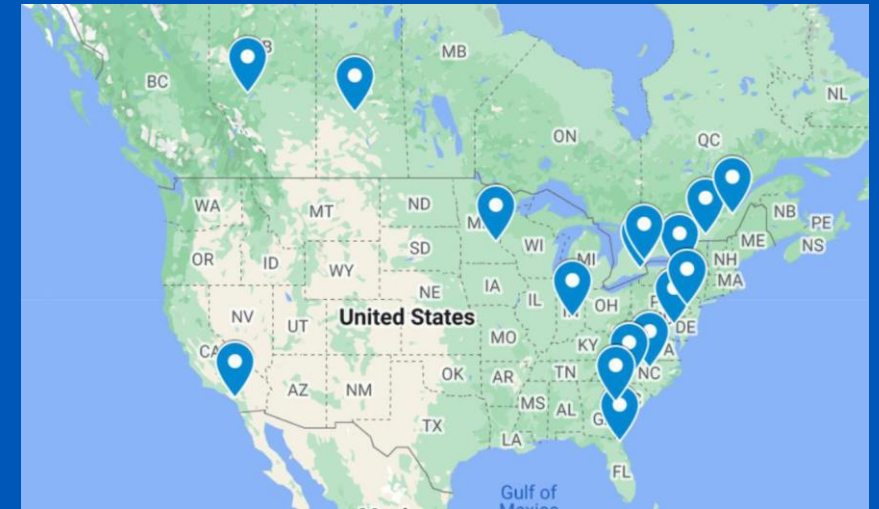
% of subjects with less than 1 unit change in 6 months
stable or improved on 6 or 12 months Ibudilast

Double-Blind Phase		Open Label Phase		Overall
Placebo	Ibudilast	Ibudilast 0-6 mon treatment	Ibudilast 6-12 mon treatment	Ibudilast 0-12 mon treatment combined
(N = 15)	(N = 29)	(N = 12)	(N = 23)	(N = 35)
3 / 15 (20.0 %)	10 / 29 (34.5 %)	6 / 12 (50.0 %)	3 * / 23 (13.0 %)	19 * / 35 (54.3 %)
PP p = 0.4884		PP p = 0.1266		PP p = 0.0325

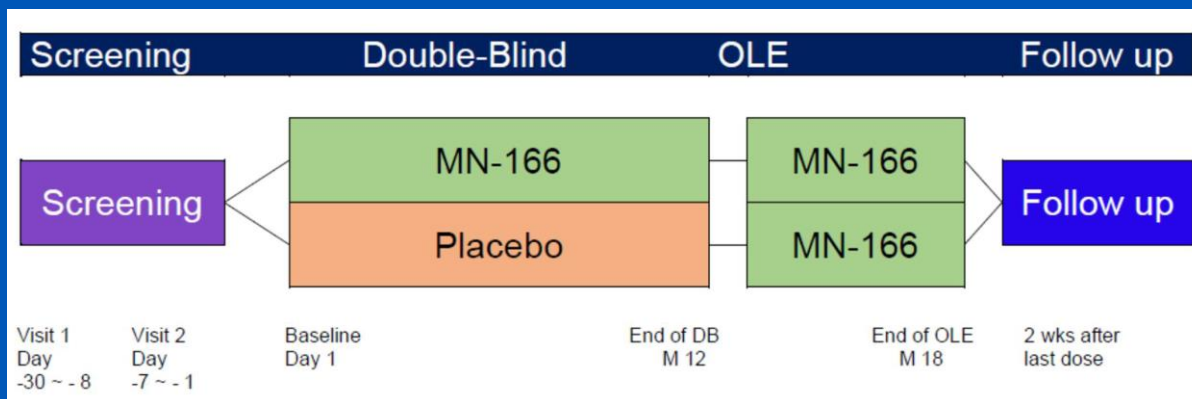
From Ben Brooks

COMBAT-ALS (NCT04057898)

- Phase 2b/3 Trial of MN-166 (Ibudilast) in ALS
- N=230 (4 left to randomize)
- 50 (or 30) mg BID



Bedlack R, Bodkins C, Dionne A, Elliott M, Genge A, Gosselin S, Goyal N, Johnston W, Maiser S, Maragakis N, Meyer JA, Rivner M, Schellenberg K, Turnbull J, Walsh A, Zinman L, Oskarsson B




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Available



Scalable Expanded Access with Analysis of Neurofilament and Other Biomarkers for Ibudilast in (SEANOBI-ALS)

ClinicalTrials.gov ID NCT06743776

Sponsor Mayo Clinic

Information provided by Bjorn E. Oskarsson, Mayo Clinic (Responsible Party)

Last Update Posted 2025-03-25



Study Details

Researcher View

No Results Posted

Record History

widetrial.com

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SEANOBI-ALS


An Expanded Access Program (EAP) for MN-166 in ALS

WideTrial is pleased to support the NIH-funded Expanded Access Program to provide treatment-use of MN-166 (ibudilast) for people with ALS who cannot enroll in the drug's ongoing research trial. In partnership with a leading academic medical center, WideTrial is establishing an open network of healthcare providers who seek to bring this investigational treatment option to their patients. If you would like to learn more about participating in this EAP, please register your interest here.

For Physicians

For Patients

[Read our recent announcement →](#)



More Sites, More Patients, More Data

The Leader in Pragmatic Expanded Access Trials

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Updates About the Expanded Access Program for MN-166 in ALS

Program to provide investigational drug to ALS patients who would otherwise be ineligible for clinical trials

SANTA CLARA, California — July 10, 2025: In March of this year, WideTrial launched an Expanded Access program (EAP) in ALS in partnership with a large academic medical center. This EAP -- "Scalable Expanded Access with Analysis of Neurofilament and Other Biomarkers in ALS" (SEANOBI-ALS) -- provides MN-166 (ibudilast) to people with ALS who cannot take part in regular clinical trials. To support this program, the National Institute of Neurological Disorders and Stroke (NINDS) has awarded the EAP's Sponsor -- the academic medical center -- a grant under the ACT For ALS of 2021.

The NINDS grant allows SEANOBI-ALS to include a total 200 patients, enrolled across multiple ALS clinics in the U.S. over a 4-year period, with each participating patient receiving MN-166 for a duration of up to 6 months.

The first treatment location, Mayo Clinic Jacksonville, has enrolled 15 patients as of the end of June 2025. Two (2) additional locations, Mayo Clinic Rochester and Mayo Clinic Arizona, will begin enrolling patients in July. Five (5) more clinical sites have been invited to join and each of the five has begun the onboarding process. An additional eight (8) pre-selected clinics will be invited this summer, and each clinic that completes its onboarding steps by the end of 2025 will be granted openings for at least 5 patients. The list of clinics to be invited in 2025 includes all nine (9) U.S. sites of the COMBAT-ALS Phase 2/3 research trial for MN-166.

As expected, the demand for access to this EAP far surpasses the budgeted capacity of 200 patients. Therefore, WideTrial and the Sponsor continue to work together to get the program scope increased. This includes continuous dialog with the outside parties who determine the maximum number of patients and the duration of treatment permitted under this EAP.

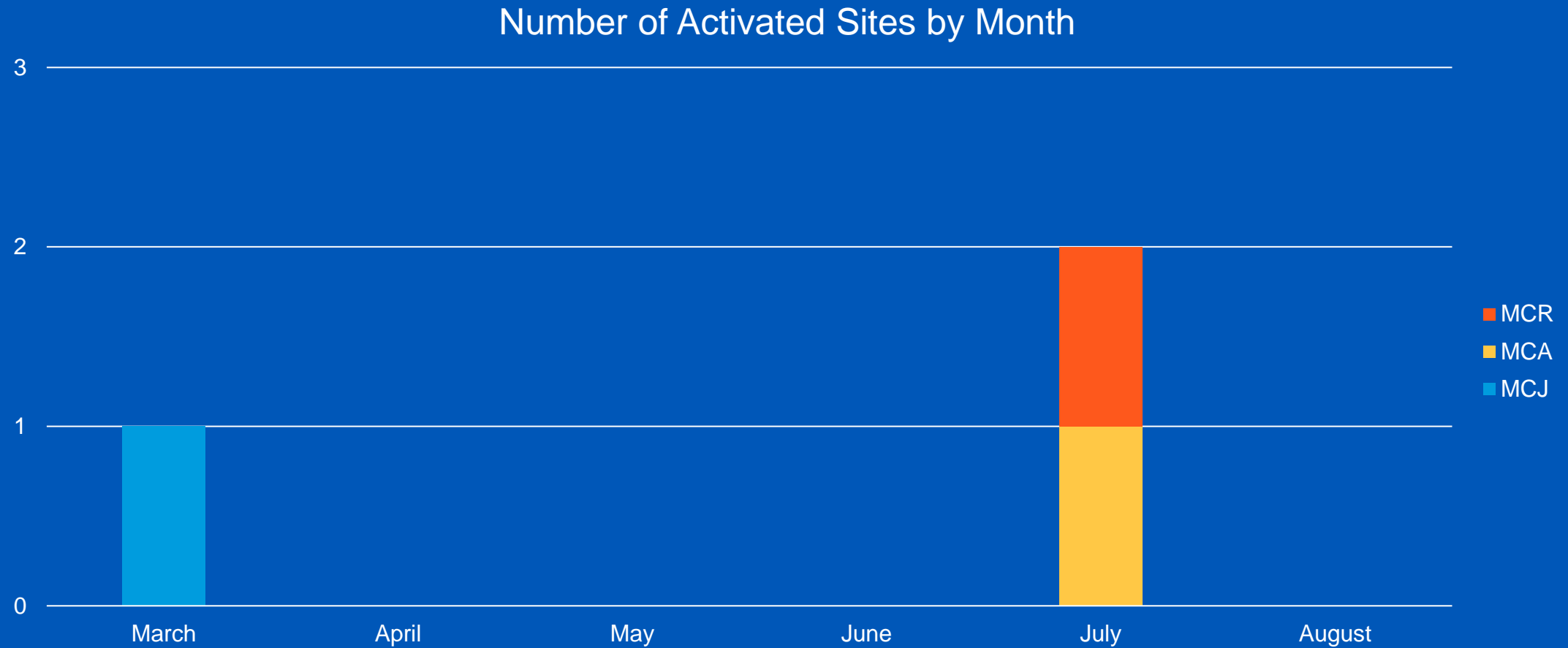
Although each participating clinic makes its own enrollment decisions, WideTrial will provide participating clinics with the contact details of patients in their region who have expressed interest via the WideTrial website and who have consented to information sharing.

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SEANOBI

- 200 patients
- 16 sites are selected
 - 3 Mayo clinic + US COMBAT + 4 other (Semmes Murphey, UCSF satellites, NSEU, HSC)
- 3 sites active
- 1st patient 1st visit March 2025
- Each site has their own patient list
- Mayo Florida alone has a 200+ patient list
- No patients who can participate in other trials
- Otherwise, broad inclusion criteria

SITE ACTIVATIONS



SITES IN DEVELOPMENT



Fully Executed
Expanded Access
Agreement

- Semmes
Murphy
Foundation
(Bertorini)



Expanded Access
Agreement
Under Review

- Duke University (Bedlack)
- Lehigh Valley Health
Network (Walsh)
- Augusta University
(Barnes)
- Hospital for Special Care
(Felice)
- UC San Francisco
(Goslinga)
- Nova Southeastern
University (Locatelli)
- UC Irvine (Goyal)



Fully Executed
Mutual NDA

- Indiana
University
(Bodkin)

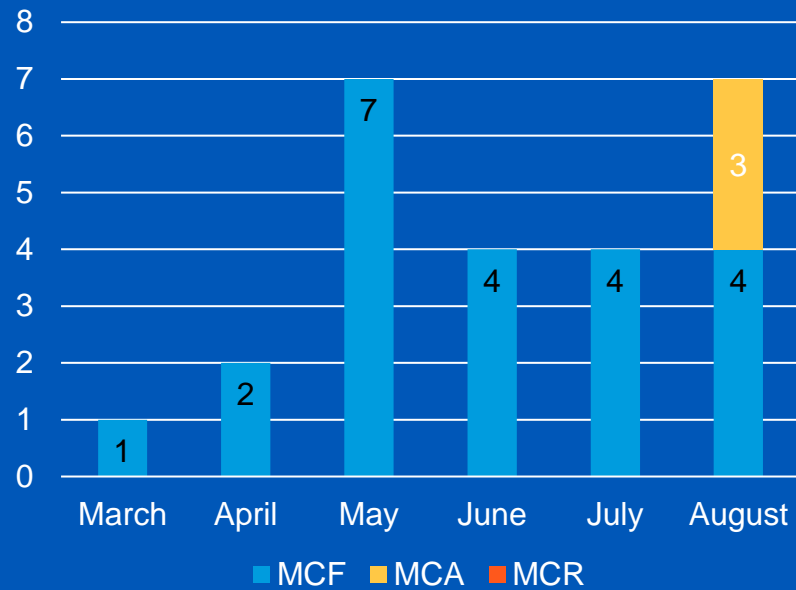


Mutual NDA
Under Review

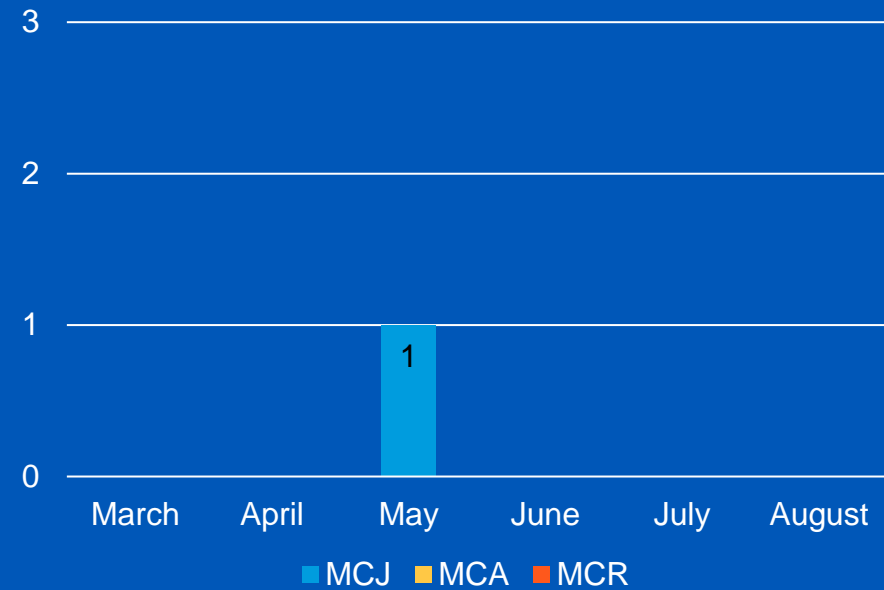
- Hennepin County
Med (Maiser)
- University of
Virginia (Elliott)
- SUNY Upstate
(Young)
- Johns Hopkins
University
(Maragakis)

ENROLLMENT UPDATE

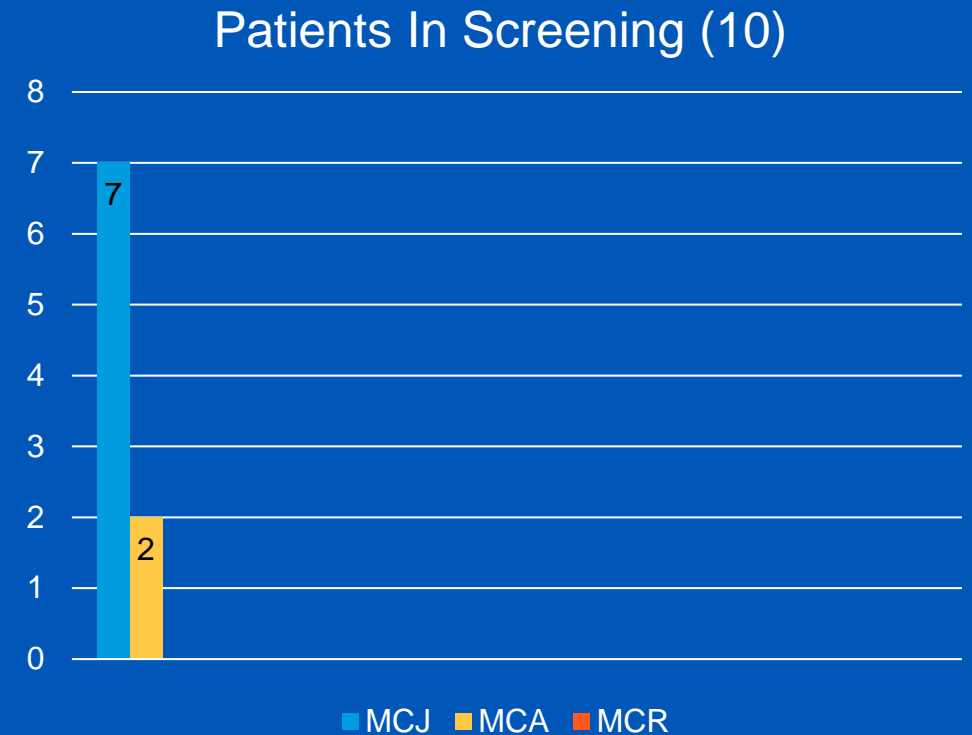
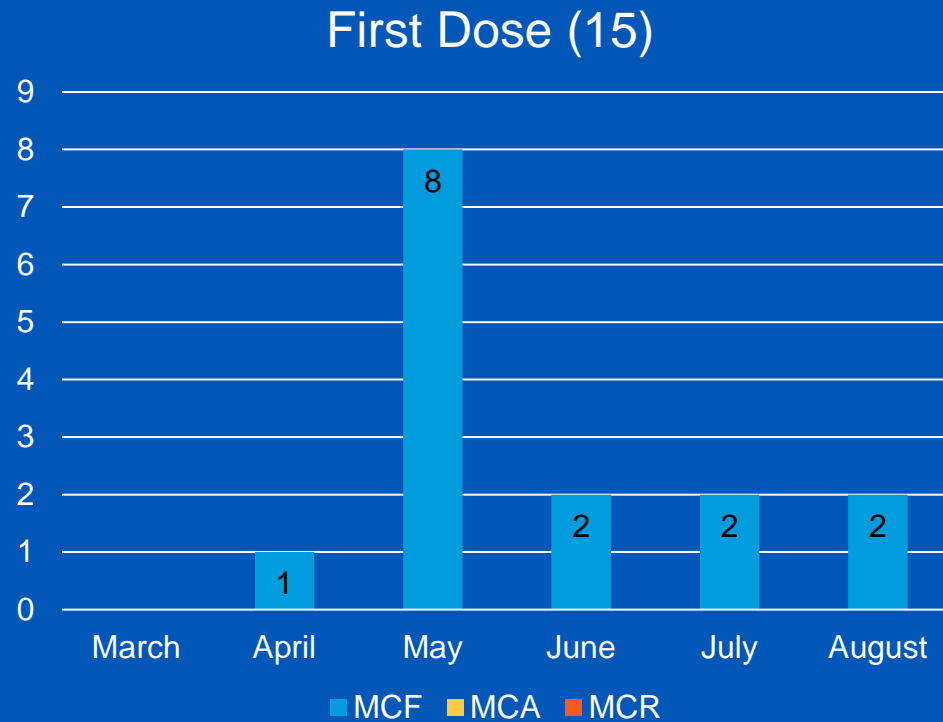
Participating Patients (25)



Screen Failures (1)



ENROLLMENT UPDATE



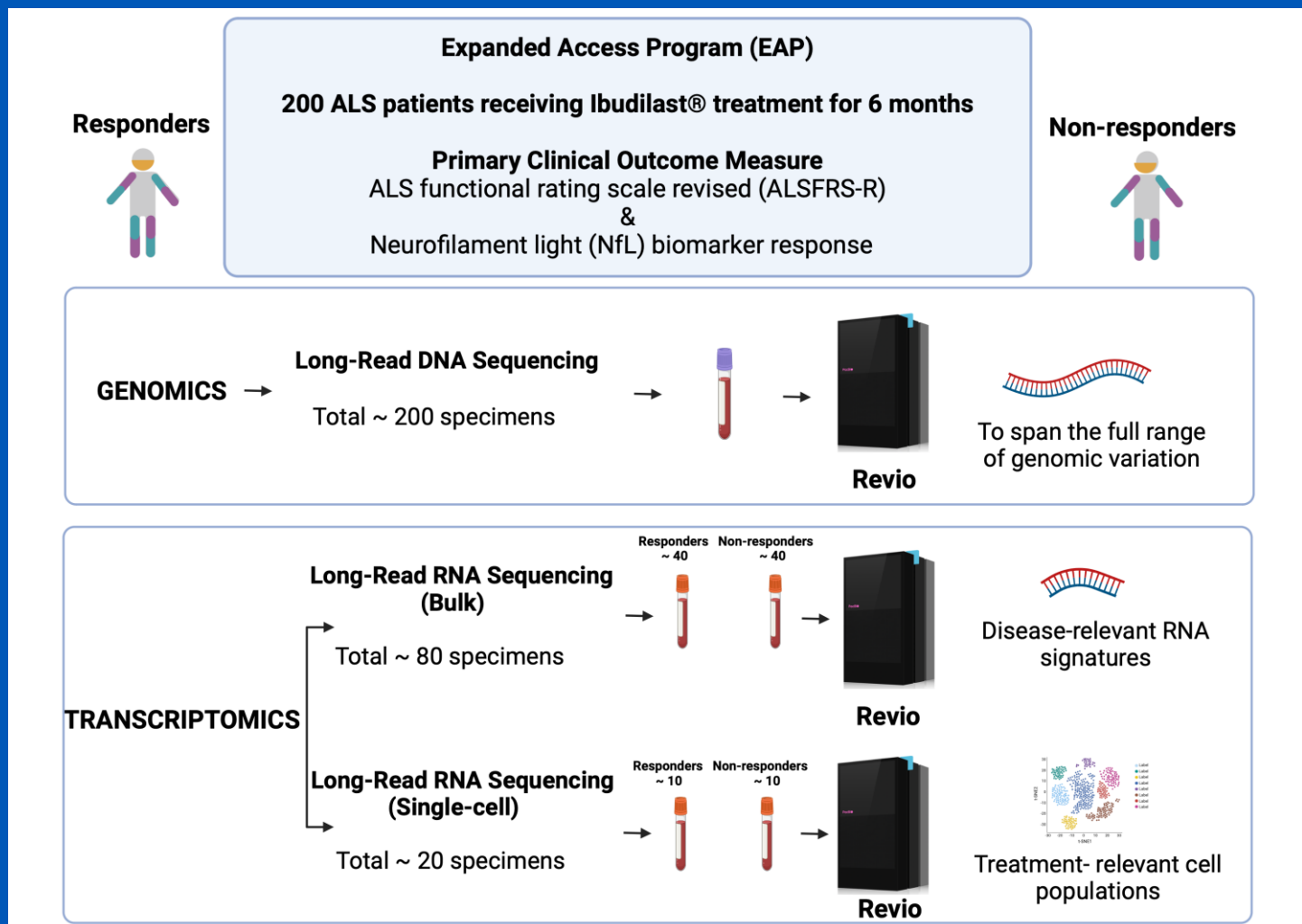
NEUROFILAMENT LIGHT AND OTHER OUTCOMES

- Neurofilament light (NfL)
 - levels are elevated in ALS
- levels are stable over time
- a person can serve as their own control
- CLIA certified results will be provided to patients and their physicians
- ALSFRS_r, ALSAQ5 and NeuroQOL
- inflammatory biomarkers

MULTI-OMIC STUDIES



Dr. Marka van Blitterswijk
MD, PhD



Using long-read sequencing to generate multi-omic profile in MN-166 treatment responders

ACKNOWLEDGEMENTS

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 - DSMB Terry Heiman-Patterson, MD, Temple
 - DSMB Americo Fernandes, MD, U Nebraska
 - DSMB Robert Silbergleit, MD U Michigan
 - DSMB Renee Martin, PhD MUSC
- Clinical Research team
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 - Huy Tran, CRC
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QUESTIONS & ANSWERS

