

Sign up for
ALS Link:



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

Thank you for joining the webinar!

We are admitting audience members from the waiting room

Please allow a few moments for the webinar to begin



Monthly EAP community Q&A webinar

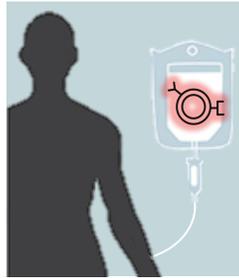
RAPA-501 EAP Update

February 12, 2026

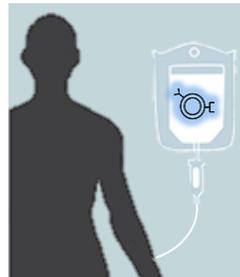
Dr. James Berry, MGH, Overall Study Principal Investigator
Brian Radecki, CEO, Sponsor (Rapa Therapeutics)
Dr. Daniel Fowler, Chief Medical Officer, Rapa

RAPA-501 T Stem Cell Infusion in People Living With ALS

Step 1
Patient T-Cells are harvested through apheresis and shipped to RAPA



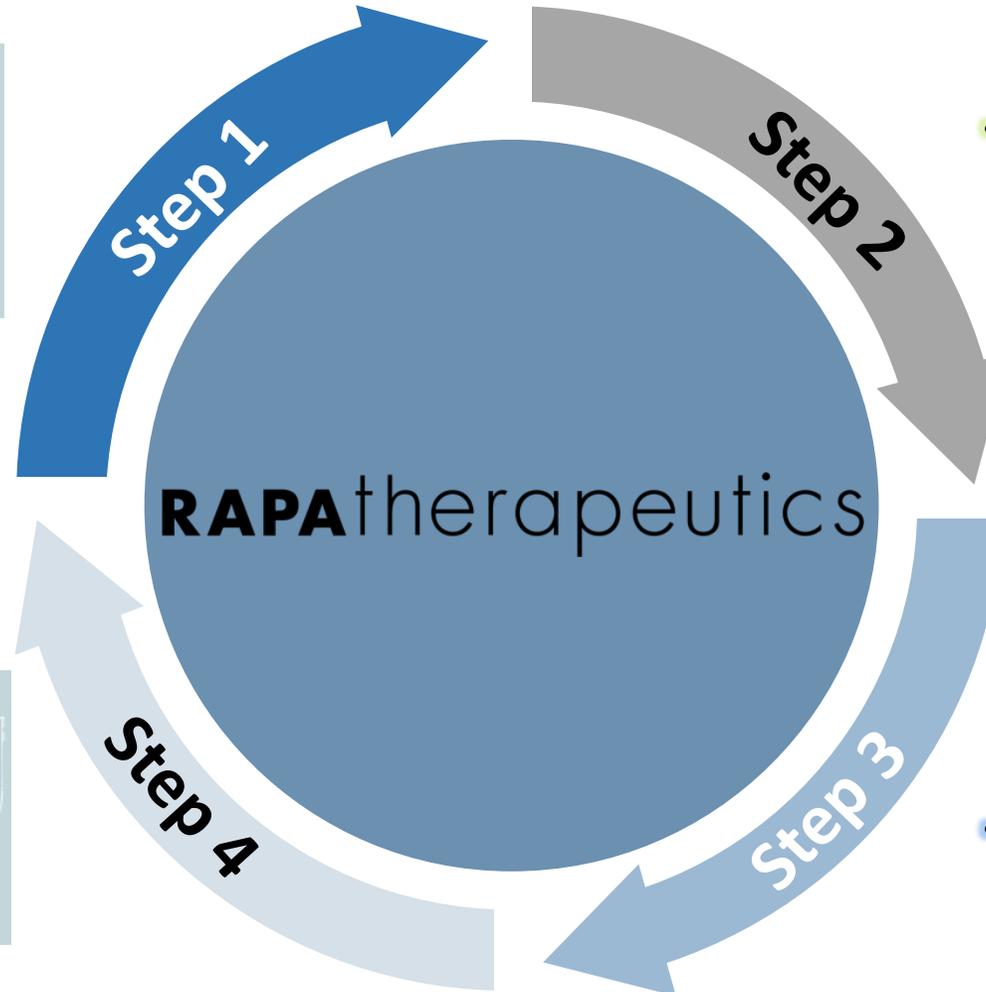
Step 4
RAPA-501 are infused (IV) back into patient for anti-inflammatory treatment.



Step 2
T-cells undergo DE-DIFFERENTIATION through RAPA's proprietary epigenetic reprogramming



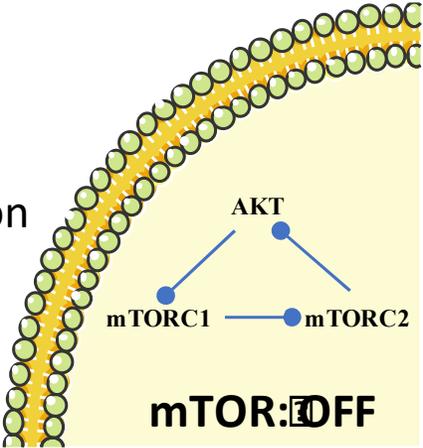
Step 3
RAPA's proprietary RE-DIFFERENTIATION process is completed in 1 week; T cells are sent back to treatment facility



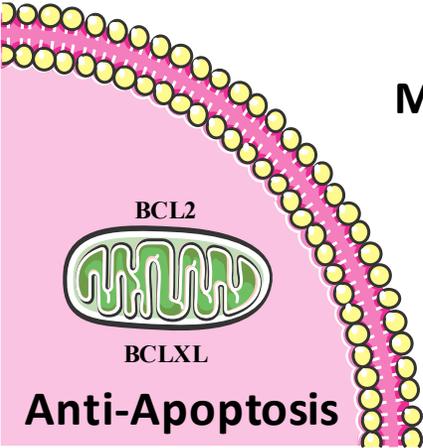
Ex Vivo Manufactured RAPA-501: T Cell Reprogramming

RAPA-501 HYBRID T_{REG}/Th2 Cells

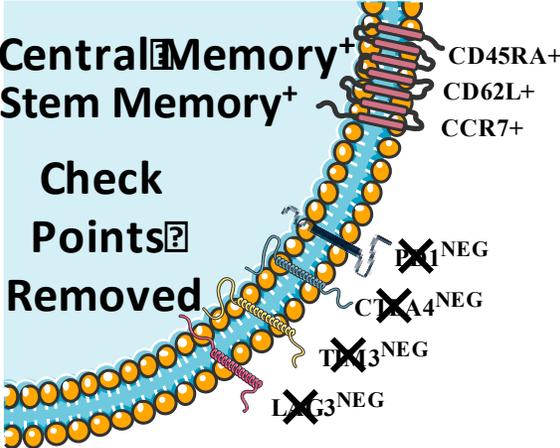
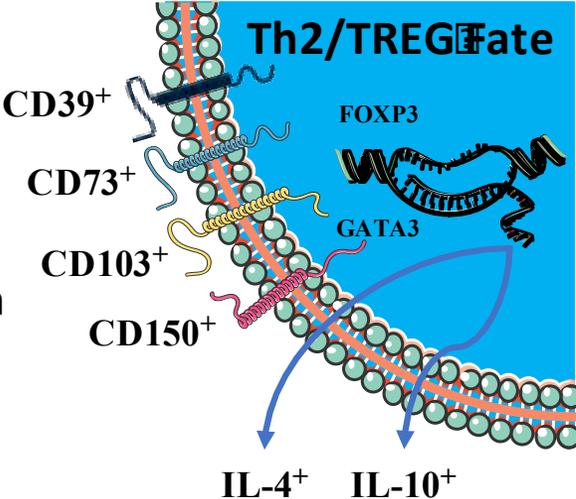
mTORC1 and mTORC2 Blocked
Erases Inflammatory Fate
Permits Reprogramming/Rejuvenation



Metabolic Fitness Selected
T Cell Therapy Without Chemotherapy



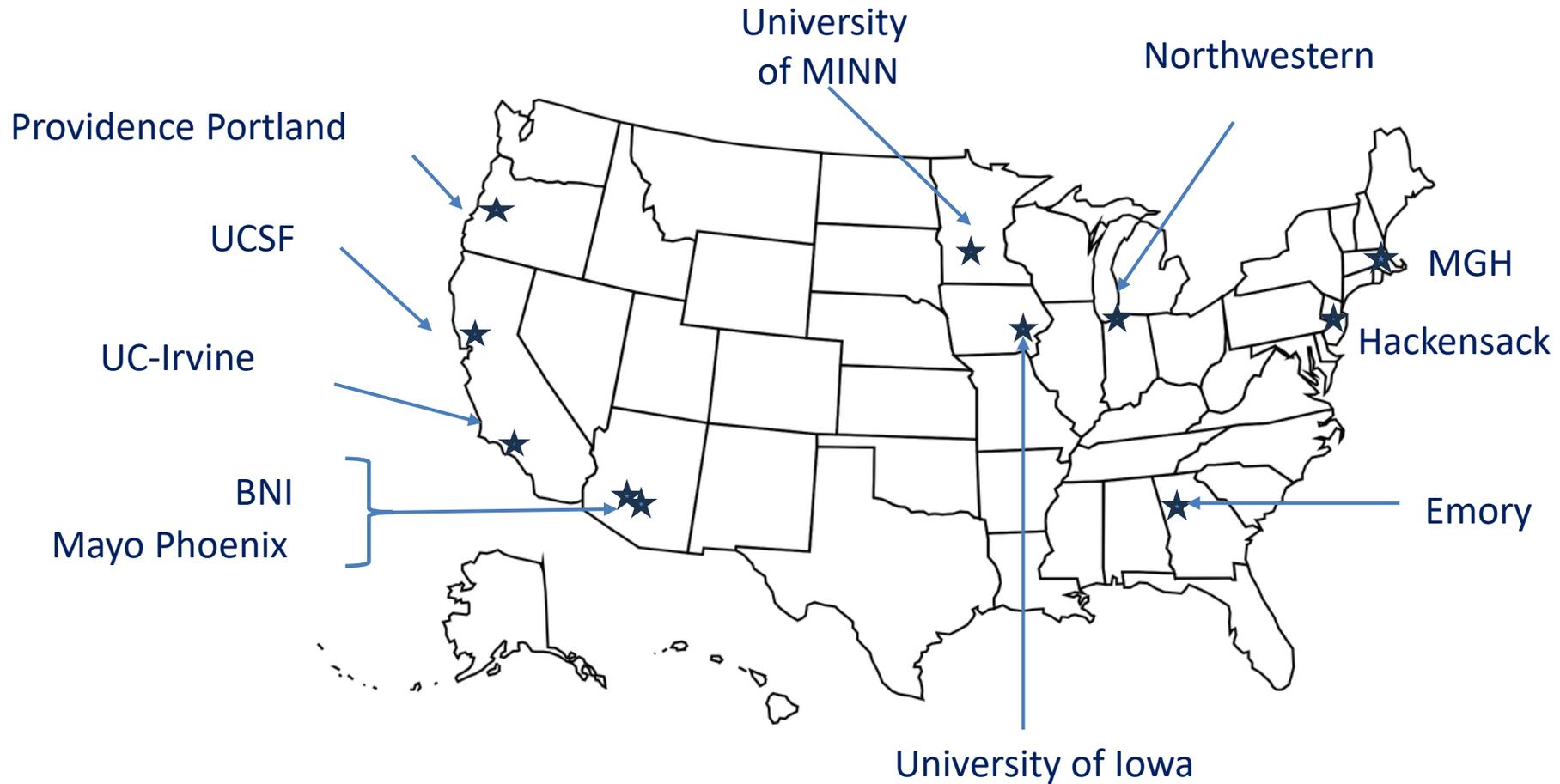
Key RAPA-501 Function
Anti-Inflammatory
Immune Reconstitution



T Stem Memory Induced Checkpoints Removed
Long-Lasting *In Vivo* Effects

RAPA-501-EAP Study

Rapid Accrual Implemented at Eleven Centers of Excellence



RAPA-501-EAP Study

Dual Focus for Expanded Access Protocols Through Act for ALS

Objective #1: Access

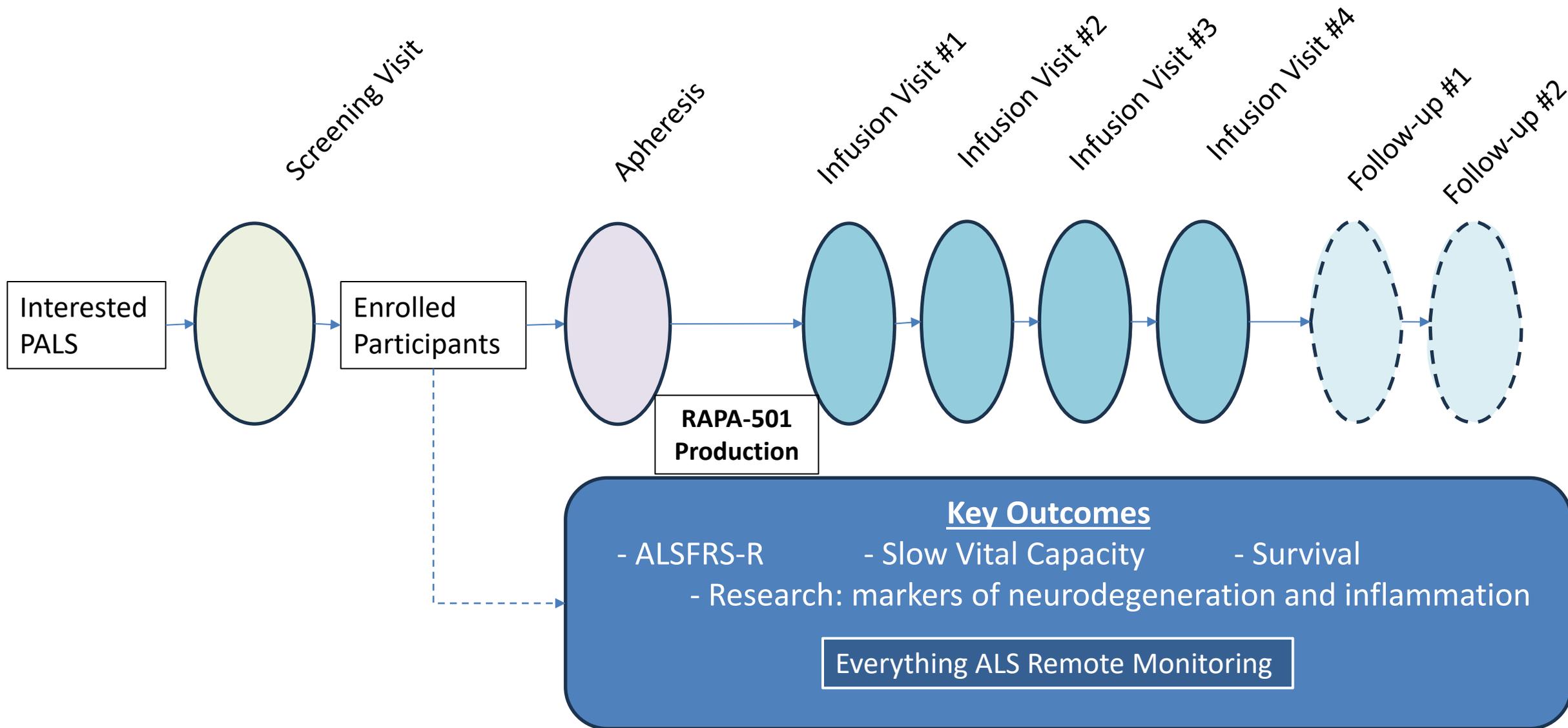
- Participants not eligible for conventional trials (including other RAPA-501)
- Treat only “advanced-stage” ALS → VC < 50% of expected

Objective #2: Research

- EAP study designed to:
 - better understand immune system in patients living with ALS
 - better understand immune modulation mediated by RAPA-501
 - develop AI-driven model for survival prognostication
 - “Virtual Controls” ⇒ replace or augment use of placebo controls

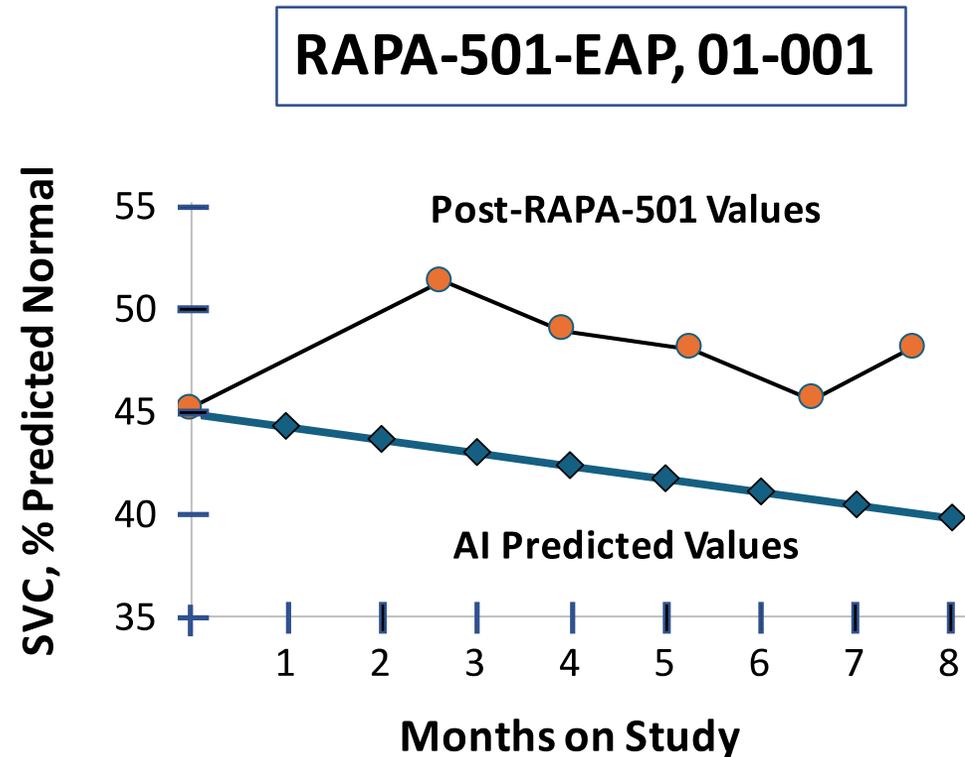
RAPA-501-EAP Study

Measurements and Study Flow (8 month duration)



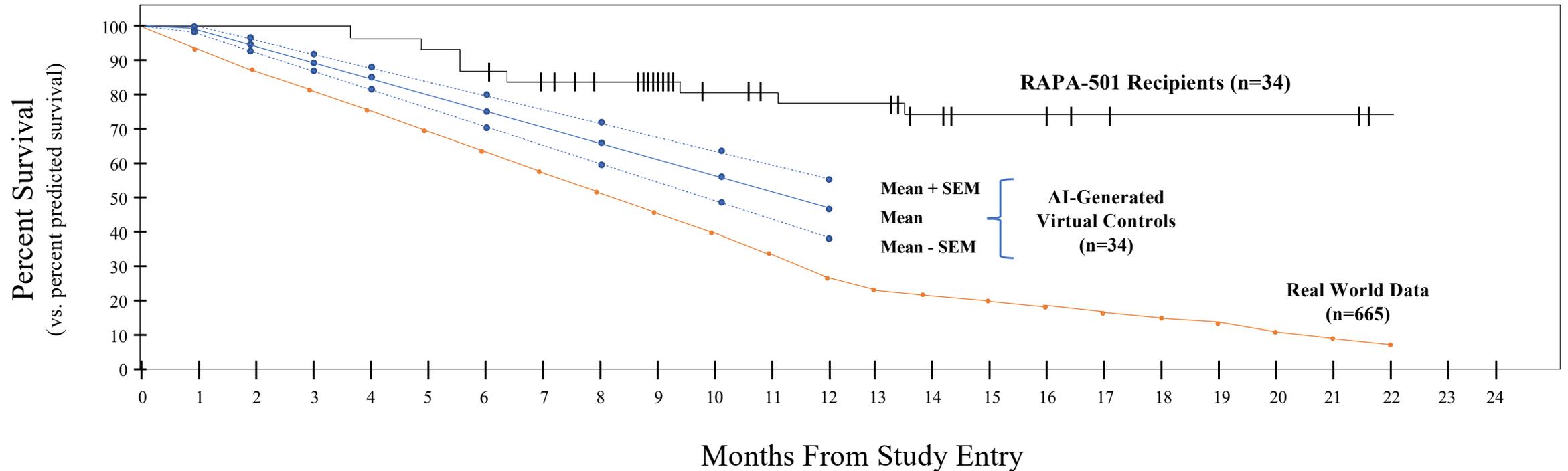
RAPA-501-EAP Study

Early Evidence for Preservation of Pulmonary Function



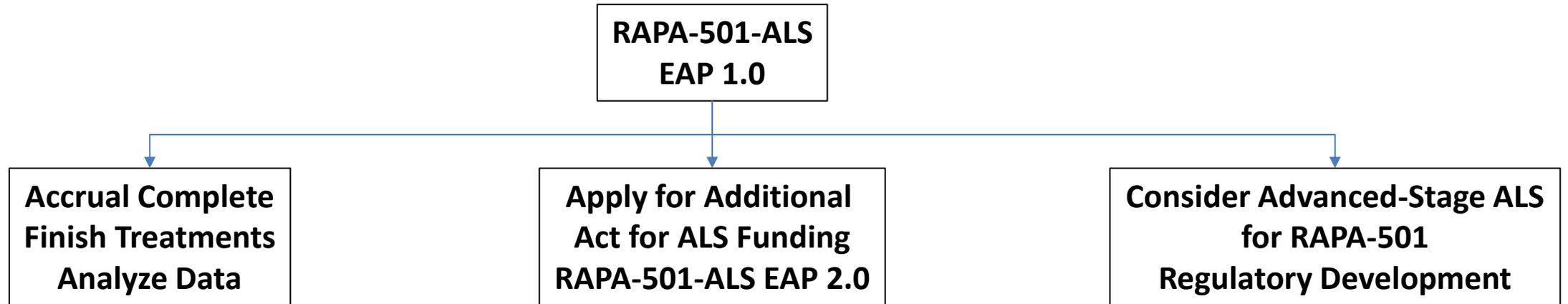
RAPA-501-EAP Study

Improved Survival Relative to AI Virtual Control Cohort ($p=0.01$)
Apparent Improved Survival Relative to Real World Data (PRO-ACT)



RAPA-501 in Advanced-Stage ALS

Next Steps



Patient Navigation

Central resource for people living with ALS



Catherine Small
Patient Navigator

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E-mail: healeyalsplatform@mgh.harvard.edu

**EAP webinars
second Thursday
each month!**

**Register for
webinars:**



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

Upcoming Webinars (Thurs, 5:00- 5:30pm Eastern Time)

February 26 – NfL Analysis in ALS Research

March 12 – Monthly EAP Discussion

March 19 – HEALEY ALS Platform Trial Update (Regimen I Drug Science with Neurizon)

