

# HEALEY ALS Platform Trial

<https://www.massgeneral.org/neurology/als/research/platform-trial>



## Healey Center

Sean M. Healey & AMG Center  
for ALS at Mass General



alsFINDINGaCURE



THE ARTHUR M. BLANK  
FAMILY FOUNDATION



The AMG Foundation



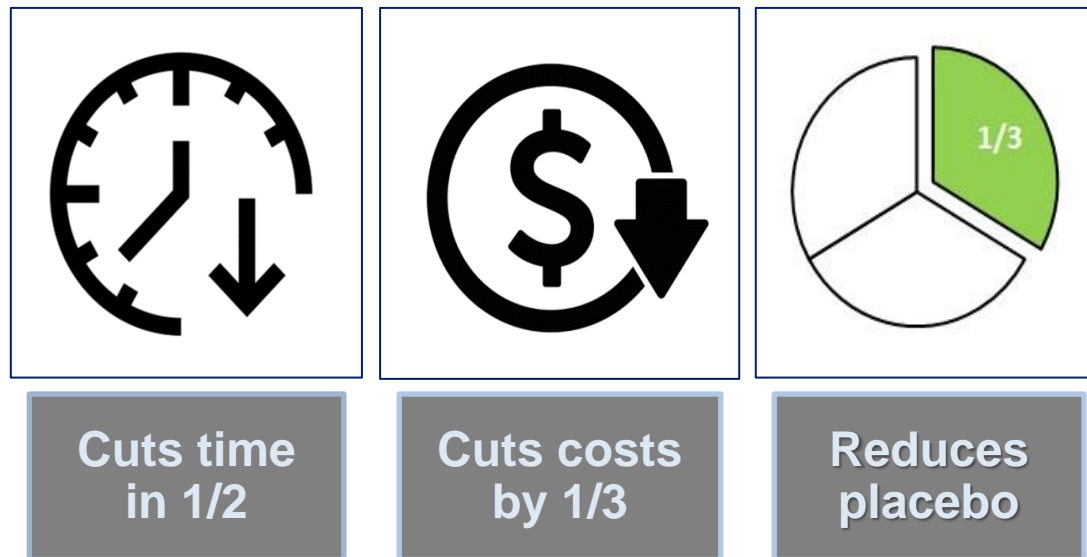
*Accelerating innovation for a cure*

*Merit Cudkowicz, MD, MSc  
Sean M. Healey*

# Traditional Clinical Trial

Vs.

## HEALEY ALS Platform Trial



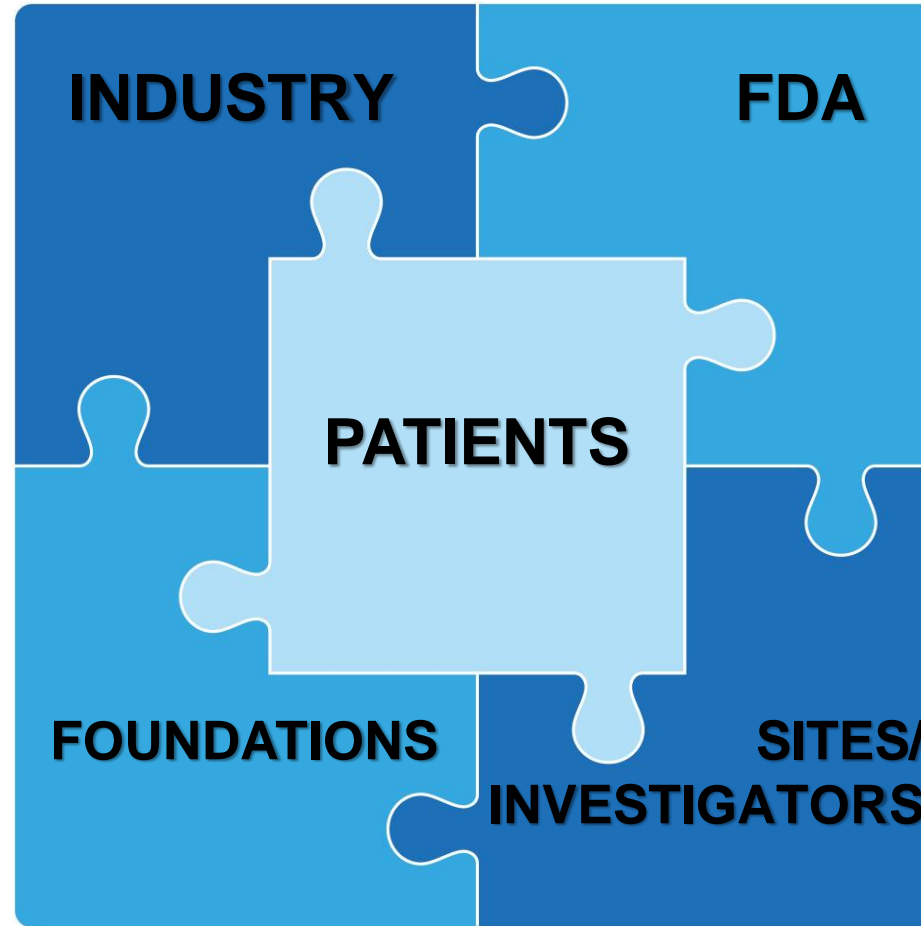
**“I lost the privilege of working on the human time clock  
on January 6, 2018  
The ALS clock is a lot faster”**

*Sandy – Person with ALS*

# Working with the entire ALS Community to launch the trial rapidly and efficiently

## ALS Platform Trial Industry Workshop

➤ “Platform trials may  
possibly be the best thing I  
have seen since diagnosis!”

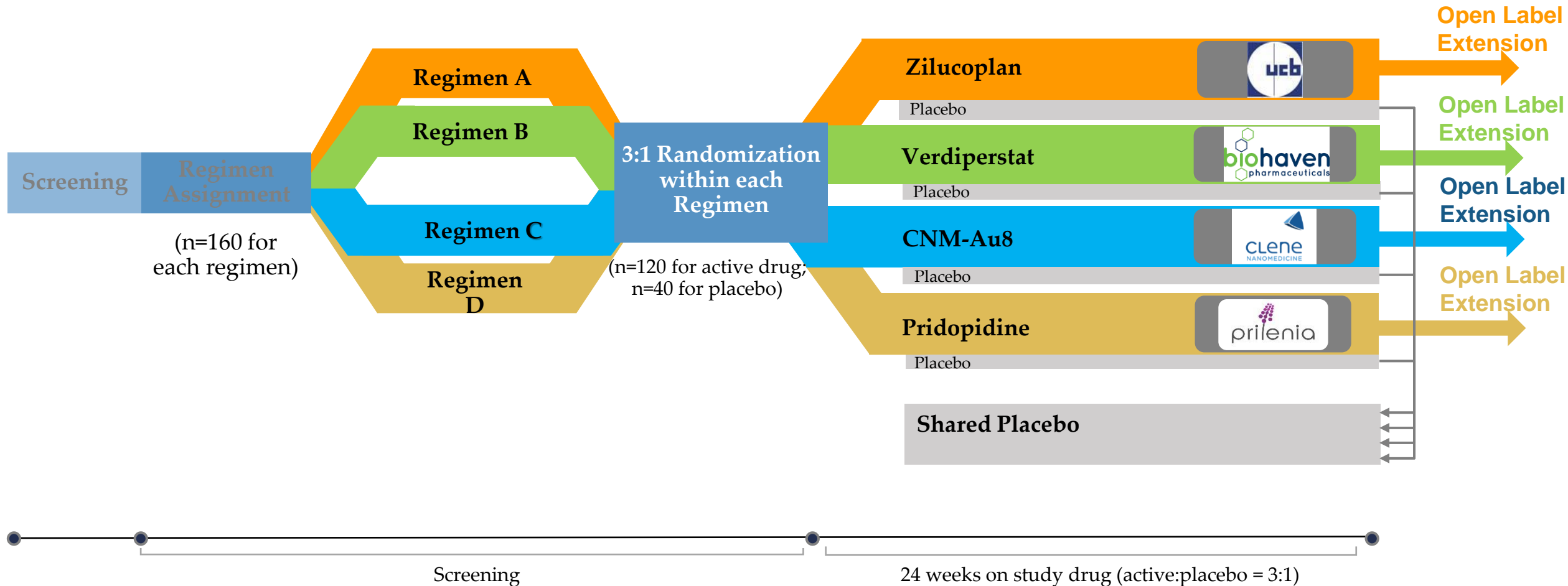


**54** TRIAL-READY SITES



**1** Central  
IRB

# The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial



# LEADERSHIP & TRAINING

## RGA Regimen Leads

- Sabrina Paganoni, MGH, Regimen Lead
- Christina Fournier, Emory, Regimen co-Lead
- Senda Ajroud-Driss, Northwestern, Regimen Steering Committee Member

## RGB Regimen Leads

- Jinsy Andrews, Columbia, Regimen Lead
- Suma Babu, MGH, Regimen co-PI
- Eufrosina Young, MD, SUNY Upstate, Regimen Steering Committee Member

## RGC Regimen Leads

James Berry, MGH, Regimen co-Lead  
Nicholas Maragakis, Johns Hopkins, Regimen co-Lead  
Elijah W. Stommel, MD, Hickcock, Regimen Steering Committee Member

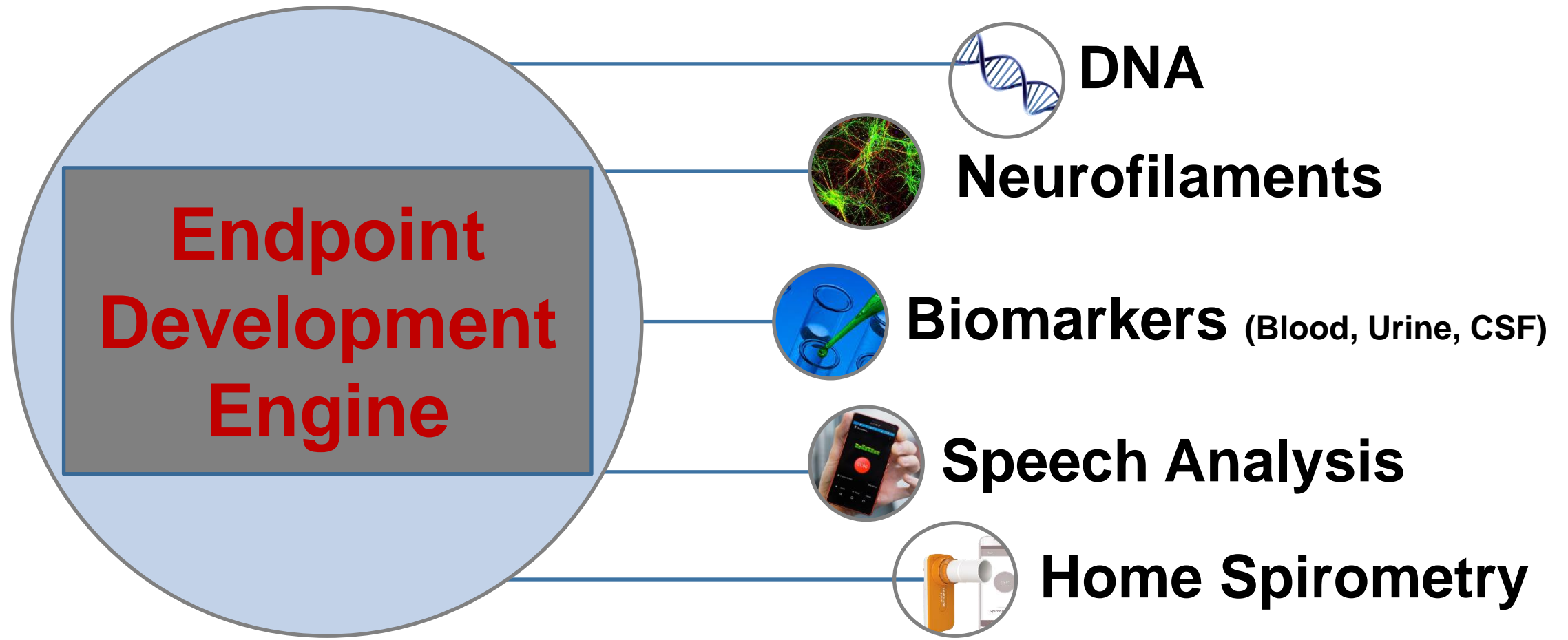
## RGD Regimen Leads

Jeremy Shefner, Barrow Neurological Institute, Regimen Lead  
Bjorn Oskarsson, Mayo Clinic, Regimen co-Lead  
Katie Nicholson, MGH, Regimen Steering Committee Member





# Exploratory Endpoints



# ENROLLMENT UPDATES

*As of 9/16/2021*

Total # Consented Master Protocol	746
Total # Assigned to Regimen	590
Total # Randomized within Regimen	529
RGA Total # Randomized	147
RGB Total # Randomized	160
RGC Total # Randomized	160
RGD Total # Randomized	69
Total # OLE Initiation	200

✓ **Enrollment  
complete for  
RGB & RBC**



*“I’m looking  
forward to  
helping find a  
cure for ALS.”*

*-Platform trial  
participant*



# Trial Updates

- **Adding 21 Expansion Sites**
- **Regimen E (5<sup>th</sup>) – Seelos**
  - 2021 Q3 Start-up
  - 2021 Q4 Enrolling
- **6<sup>th</sup> Revalesio in design/discussion**
- **Three more selected and pending contract**
- **Interim Analysis**
  - Began Q2 2021 and every 3 months





# Therapy Evaluation Committee

*3 NEW applications reviewed in August 2021*



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➤ 60 Companies Interested

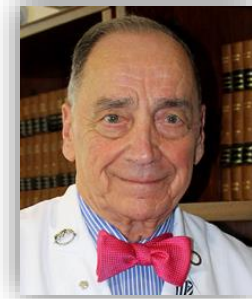
➤ 33 Applied & Reviewed

## Committee Recommendations:

➤ 9 Selected - **A. Immediate-ready for inclusion in platform trial**  
*(6 Contracted; 3 Pending Contract)*

➤ 13 - **B. Future-to be considered once additional data is obtained**  
*(10 launching trial in ALS outside of platform trial)*

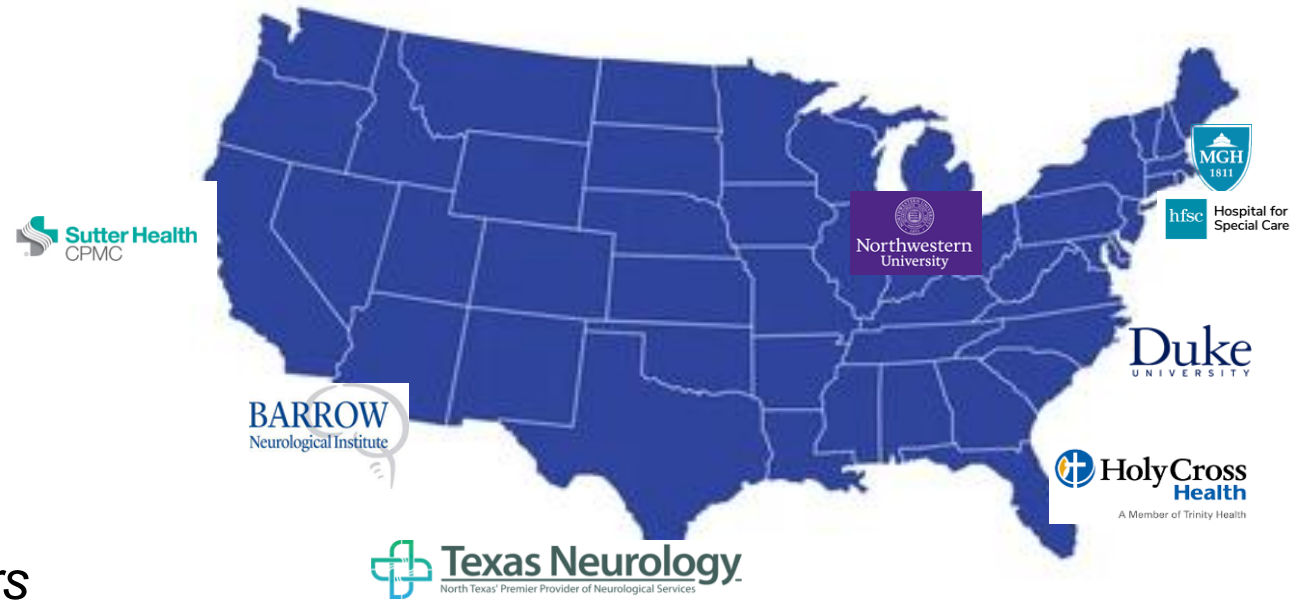
➤ 11 - **C. Should not be considered for this platform trial**  
*(2 launching trial in ALS outside of platform trial)*



Committee Members: Timothy Miller;  
Robert Brown; Merit Cudkowicz; Clotilde  
Lagier-Tourenne; Stanley Appel; Melanie  
Leitner

# LEADING INNOVATION THROUGH MULTI CENTER EXPANDED ACCESS PROGRAM *COMPANION TO HEALEY ALS PLATFORM TRIAL*

- Provide access to experimental treatments  
useful data on safety and biomarkers
- For participants ineligible for platform trial
- Three EAPs underway!
  1. Biohaven EAP 35 Participants at 3 centers
  2. Clene EAP 15 Participants at 3 centers
  3. Prilenia EAP 24 Participants at 3 centers





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## als FINDING a CURE

- 21 global applicants
- 6 countries represented (Australia, Canada, China, England, Switzerland, United States)

## 2021 ALS SCHOLARS IN THERAPEUTICS

### JULY 1, 2021 – JUNE 30, 2023



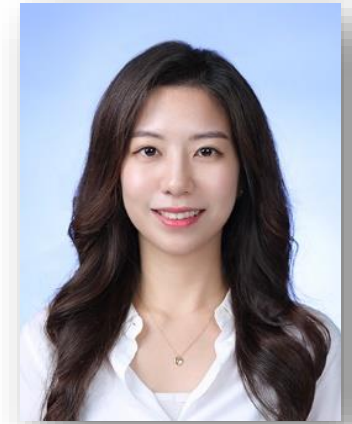
**Juliet Goldsmith, PhD**  
*University of Pennsylvania*  
**Autophagic regulation of  
mitochondrial DNA in ALS**



**Matthew Anthony White, PhD**  
*Kings College London*  
**Targeting TDP-43  
Autoregulation for  
Therapeutic Benefit in ALS-  
FTD**



**Brian Jude Joseph, PhD**  
*Harvard University*  
**Developing U1-inhibition as a  
treatment modality for SOD1  
ALS**



**Su Min Lim, PhD**  
*Massachusetts General Hospital/  
Harvard Medical School*  
**Multiplexed Image-based  
Platform for Therapeutic  
Development in ALS**