

# HEALEY ALS Platform Trial

Biostatistics Webinar Series and Weekly Q&A – July 28, 2022



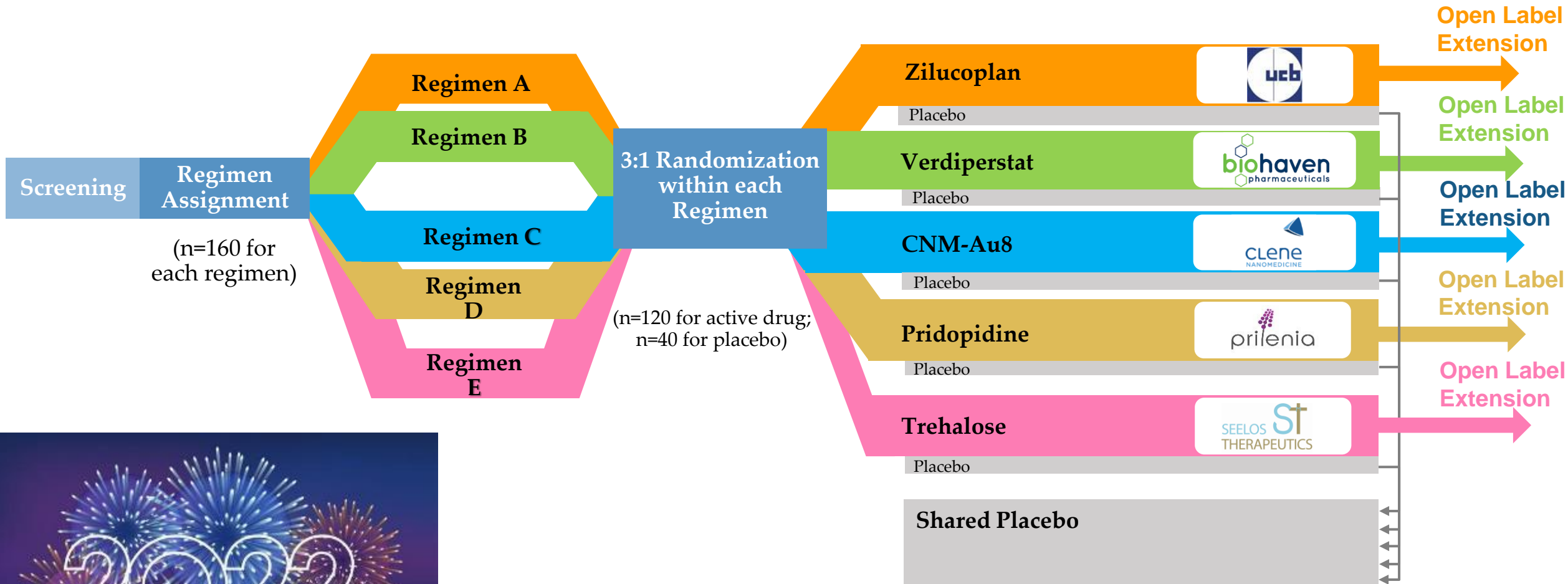
## Healey Center

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



The AMG Foundation

# The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial



# Enrollment Updates (as of July 28, 2022)

- **96** individuals have signed informed consent
- **66** individuals have been randomized within Regimen E

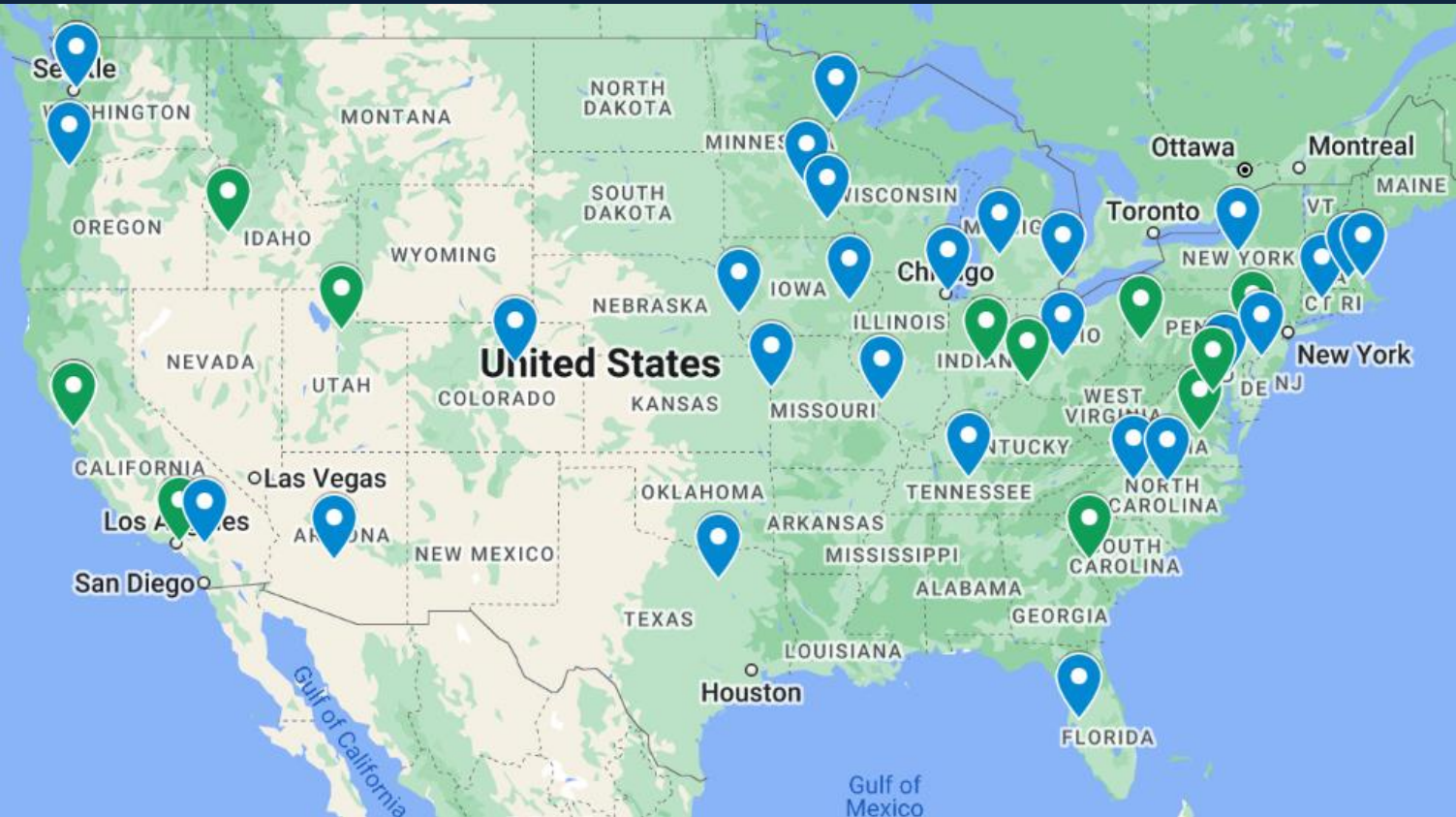
*Thank You*

**This breakthrough trial would not be possible without your participation**

Your **partnership** in research is what keeps us filled with passion, dedication, and the commitment to uncover new promising treatments for ALS

**Every research participant, whether on the active drug or placebo, plays a critical role in making the hope of finding a cure for ALS a reality**

# 41 Sites Currently Activated for Regimen E



(as of 7/28/22)

*Sites in blue participated in previous regimens. Sites in green (underlined to the side) are new additions to the Platform Trial!*

- Lehigh Valley Health Network
- Mass General Hospital
- University of Kansas
- University of Maryland
- California Pacific Medical Center
- Northwestern University
- Virginia Commonwealth University
- University of Nebraska
- Washington University
- Wake Forest University
- Hospital for Special Care
- Saint Alphonsus Regional
- University of Massachusetts
- Duke University
- Barrow Neurological Institute
- Georgetown University
- Texas Neurology
- Beth Israel Deaconess Medical Center
- SUNY Upstate
- Spectrum Health
- Henry Ford Hospital
- Essentia Health
- University of Southern California
- University of South Florida
- University of Colorado
- Providence Brain and Spine
- University of Minnesota
- Loma Linda University
- University of Iowa
- Swedish Medical Center
- Ohio State University
- University of Cincinnati
- Thomas Jefferson University
- UC San Francisco
- Mayo Rochester
- University of Washington
- Vanderbilt University
- UPMC
- Indiana University
- Augusta University
- University of Utah

Site Map & Contacts:



<https://bit.ly/3g2NZr5>

# Patient Navigation

## Central resource for people living with ALS



Catherine Small

Phone: 833-425-8257 (HALT ALS)

E-mail: [healeyalsplatform@mgh.harvard.edu](mailto:healeyalsplatform@mgh.harvard.edu)

Weekly webinar  
registration:



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

ALS Link sign-up:



<https://bit.ly/3o2Ds3m>



Allison Bulat

### Upcoming Guest Speakers:

**August 4<sup>th</sup>- Cancelled**

**August 11<sup>th</sup>- TBD**

**August 18<sup>th</sup>- Amanda Peltier, MD, MS** (Site Investigator at Vanderbilt University in TN)

# Guest Speaker

**Lori Chibnik, PhD, MPH**

Assistant Professor & Biostatistician

Harvard TH Chan School of Public Health & MGH



# Trial Statisticians

## MGH Biostatistics



Eric Macklin, PhD; Lori B. Chibnik, PhD, MPH;  
Douglas Hayden, PhD; Marie-Abele Bind, PhD;  
PoYing Lai, MS



## Berry Consultants



Michelle Detry, PhD; Melanie Quintana, PhD;  
Ben Saville, PhD; Matteo Vestrucci, PhD



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# PLACEBOS



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- What is a placebo
  - Why do we need them?
  - What is a placebo effect (in ALS)?
  - Can we use placebos from previous trials?

# What is a placebo?

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- A “*sugar pill*”?
- Something that is identical to the active drug, but without the active ingredient.
  - Identical in look, weight, taste, smell, dose, all properties
  - Only way to tell it is placebo is by chemical analysis

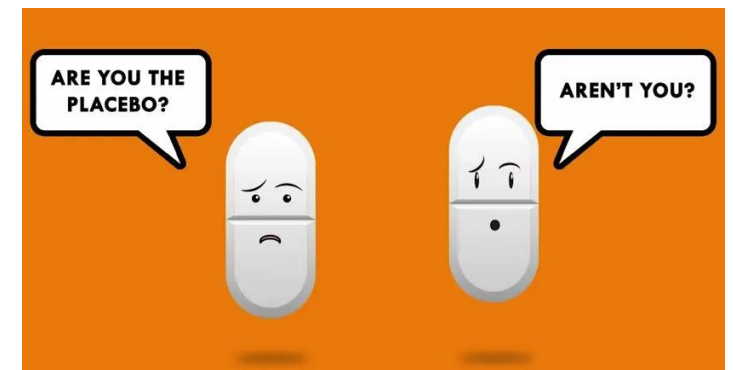
# Why do we need them?

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- The goal of a clinical trial is to show that a drug is **safe** and **effective**
- We need to show that any efficacy and adverse events are the result of the drug and only the drug
- The design of a double-blind clinical trial – where neither participant, nor researcher knows which drug someone is getting – is the best way to determine this.

# What is the Placebo Effect – in general

- a beneficial health outcome resulting from a person's anticipation that an intervention will help
- Placebo effect is seen more in *subjective* measures than *objective* measures
- Some interesting examples
  - *higher* dose placebos are more effective than *lower* dose placebos
  - *more* expensive placebos are more effective than *less* expensive placebos



# Placebo Effect in ALS

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- ALS progression has very little subjectivity, however the placebo effect can also be seen in objective measures
- Examples:
  - An open-label trial of lithium in a small number of patients suggested this drug helped slow the disease. But a larger, placebo-controlled, double-blind trial found no effect
  - Animal studies and open-label human trials suggested the antibiotic minocycline was beneficial. But a larger, placebo-controlled trial showed it was not, and may even have been harmful.

# Can we use placebos from previous trials?

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- Remember: We need to show that any efficacy and adverse events are the result of the drug and only the drug
- Any differences between previous study and current study mean we cannot know if it is the drug that is the reason for the differences
- Examples:
  - Differential selection of patients, different consent criteria
  - Changes in standard of care (e.g. edaravone)
  - Different studies have different data quality and completion

# Questions?



# HEALEY ALS Platform trial

Study design  
'clinically meaningful' results  
Sample Size  
Randomization

