

HEALEY ALS Platform Trial

Weekly Q&A – June 23, 2022



Healey Center

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



The AMG Foundation

Guest Speaker

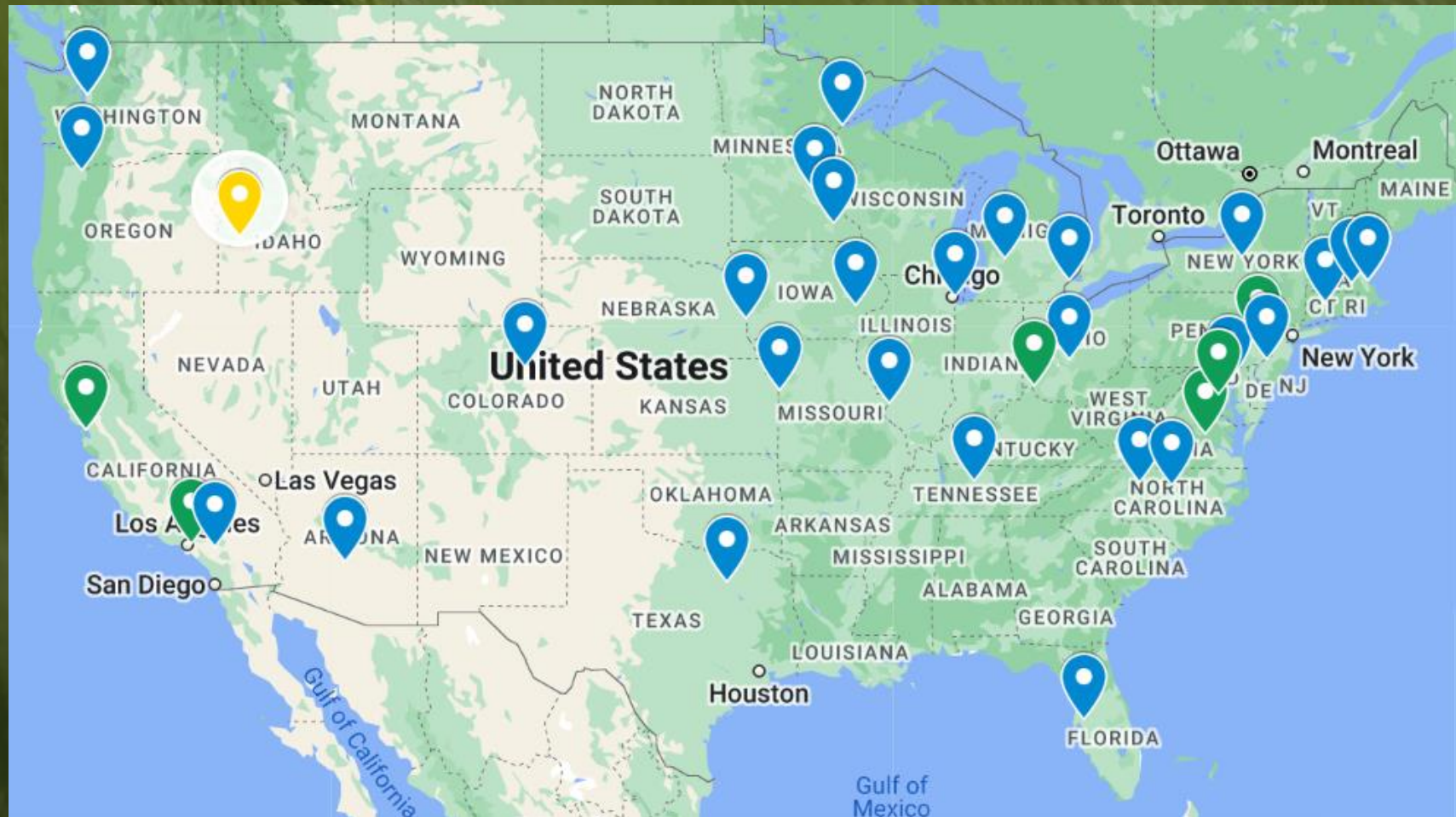
Jackie Whitesell, MD

Platform Trial Site Investigator

Saint Alphonsus Regional Medical Center, Boise ID



SAINT ALPHONSUS NEUROLOGY ALS CLINIC



ABOUT US

Our clinic serves patients in Idaho, Oregon, and Nevada. We serve a rural population.

We have 7 neurologist plus 2 neuro-hospitalists.

We have a monthly multidisciplinary ALS team

We have a dedicated neurology research coordinator.





NEUROLOGY CLINIC TRIALS

ALS

Healey Platform trial

MS

MS registry COREVITAS



OUR TEAM



PHYSICAL THERAPIST (LIZ ARMSTRONG)

RESPIRATORY THERAPY (JAMIE GRANDSTAFF)

SPEECH THERAPY (SUZANNE NEIDERMEYER)

MEDICAL ASSISTANT (TRISHIA STREET)

RESEARCH COORDINATOR (JOSHUA HARRISON)

MEDICAL EQUIPMENT/LOAN CLOSET (SHANE DAVIS
AND MIKE MCHARGUE)

ALSA CARE SERVICE COORDINATOR (HEIDI FRANTZ)

FUTURE DIRECTIONS/GOALS

GRANTS

We are applying for grant funding to help with patient travel expenses to clinical trial sites

OUTREACH CLINIC

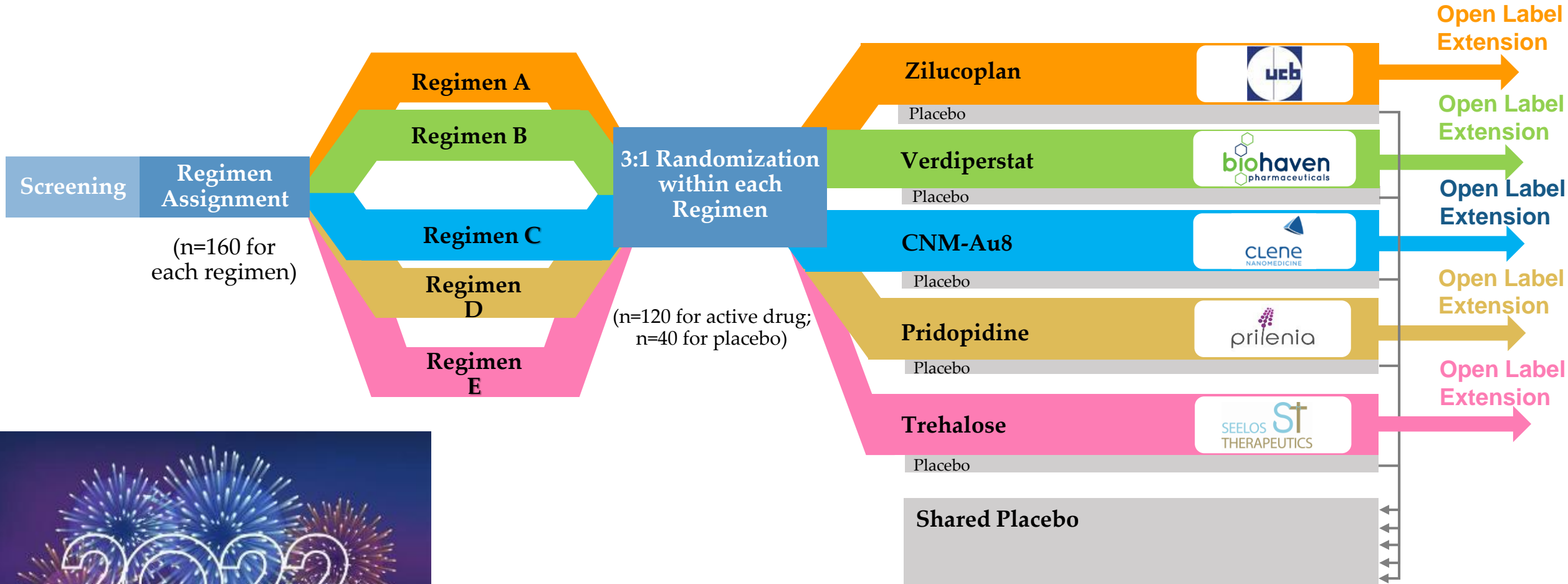
I have taken a part-time position at VA Boise and see ALS there

DIETICIAN AND SOCIAL WORK

We are working to fill both needs



The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial



Enrollment Updates (as of June 23, 2022)

- **82** individuals have signed informed consent
- **50** 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

New Resource

- Printer-friendly “Evergreen” Platform Trial General Info Brochure is available online!

The HEALEY ALS Platform Trial was designed to decrease the amount of time it will take to find successful therapies, improve active study medication to placebo ratio, and increase access for participants. One of the innovative features of the Platform Trial is that enrollment will continue perpetually as more investigational medications are added over time.

Am I Eligible?

What to Expect in the Trial Process

Download Trial Brochure



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



HEALEY ALS Platform Trial

A nationwide network of research centers in the Northeast ALS (NEALS) Consortium are partnering with the Sean M. Healey & AMG Center to conduct this first-of-its-kind platform trial for people living with ALS. The mission is to find answers as quickly as possible by testing multiple drugs using a shared platform.

Trial Highlights:

- **Multicenter Trial**
More than 70 sites across the US are participating in the Platform Trial, working together to enroll 160 participants for each regimen.
- **3:1 Active Drug to Placebo Ratio**
Participants have a 75% chance of receiving active study drug, and a 25% chance of receiving placebo.
- **Open Label Extension (OLE)**
Upon completion of the 24-week trial, participants may have the option to enroll in the OLE for their regimen. During OLE, participants know with 100% certainty that they are receiving active study drug.

View map and contact info for participating research centers:
<https://bit.ly/3lICv9t>

For general questions about the HEALEY ALS Platform Trial, Contact the Patient Navigator
healeyalsplatform@mgh.harvard.edu
833-425-8257 (HALT ALS)

Visit our website to learn more about current and future regimens:
<https://bit.ly/31EK198>



Frequently Asked Questions

What is a platform trial?
The platform trial tests multiple investigational products in different participants using a common master protocol. New investigational products can be added to the platform; this means more opportunities to find groundbreaking therapies in a faster timeframe.

How are investigational products chosen for this trial?
The investigational products are selected by a team of experts after careful review of the pharmaceutical company and the science behind the study drug. Each investigational product is believed to have an equal chance of success for all forms of ALS based on available scientific evidence.

Why use a platform trial approach?
Platform trials are designed to decrease the time it takes to find effective therapies, reduce the number of participants on placebo, and increase access to research by conducting the same trial at multiple locations. The platform trial will remain open until safe and effective treatments are found for all people living with ALS.

What is meant by the term 'regimen'?
A regimen is designed to test the safety and efficacy of one specific investigational product. Participants in the trial are randomly assigned to one regimen, and then randomized to active drug or placebo within that regimen.

What phase is this research trial?
The Platform Trial is a late-stage trial (Phase 2/3), so data derived from the trial, if positive, could be used to support the approval of new medication.

What is open label extension?
Open Label Extension (OLE) allows participants to receive the active drug for the regimen to which they were assigned after completing the 24-week placebo-controlled trial. The duration of OLE may vary.

Stay Connected to the Platform Trial

Investigational products will be added to the HEALEY ALS Platform Trial through support by pharma, foundation partners, philanthropy, federal, and other fundraising initiatives.

Learn more about what to expect in the trial process:



<https://bit.ly/3ExRa18>

Sign up for the ALS Link to receive research news and updates via email:



<https://bit.ly/3xC6RXX>

Additional questions? Register to attend the Weekly Q&A Webinars:



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

Boosting Awareness

- On 5/23/22, a research notification about the Platform Trial was sent out to 11,440 registrants on the CDC's National ALS Registry
- To view the CDC website or sign up for the National ALS Registry Newsletter:



<https://bit.ly/2DObSSU>



Dear ALS Registry Participant,

The [HEALEY ALS Platform Trial](#) is enrolling people living with Amyotrophic Lateral Sclerosis (ALS) to test the efficacy of multiple investigational products. The platform trial is designed to decrease the time it takes to test new potential therapies and increase access to research for people living with ALS. One of the innovative features of the Platform Trial is that enrollment will continue as more investigational products are added.

We partner with more than 50 research centers across the USA (view [site map](#)). If you would like to enroll in this trial, please contact the research team at any local participating center (HEALEY ALS Platform Trial sites [contact information](#)). Please note that the Platform Trial involves about 6 to 8 in-person study visits over the course of 6 months, so we encourage participants to pursue enrollment at conveniently located centers.

If you have questions about the HEALEY ALS Platform Trial or would like assistance connecting with a local site, please contact the Patient Navigator [via email](#) or phone 833-425-8257 (HALT ALS).

Additionally, our weekly Q&A [Webinars](#), held on Thursdays at 5pm ET, are a great way to stay up to date about the Platform Trial and submit questions in real time.

For more information about the Platform Trial:

- Register to attend upcoming webinars or view recordings of previous webinars:
 - [Webinars](#)
- Overview of the Platform Trial:
 - [General Information: Platform Trial](#)
 - [Something New is Here: HEALEY Platform Trial](#)
 - [Frequently Asked Questions](#)
 - [What to Expect in the Trial Process](#)
- Investigational Products in the Platform Trial:
 - [Study Drugs](#)
- Find out if you or a loved one may be eligible to participate:
 - [Online Eligibility Checking Tool](#)

Thank you,
The HEALEY ALS Platform Trial Patient Navigation Team

Please note: The National ALS Registry and ATSDR are not part of this project and have no control over it. Please directly contact the research staff listed in the attachment to this email.

If you want us to stop sending these notices, you can update the preferences on your registry account on the [National ALS Registry](#) website (www.cdc.gov/als) by logging into your account and clicking on the "Update Account" link. Then uncheck the "I Agree" box under ALS Research Notification Consent form.

Patient Navigation

Central resource for people living with ALS



Catherine Small

Phone: 833-425-8257 (HALT ALS)

E-mail: healeyalsplatform@mgh.harvard.edu

Weekly webinar
registration:



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

ALS Link sign-up:



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



Allison Bulat

Upcoming Guest Speakers:

June 30th- Gale Kittle, RN, MPH (Director, Outcomes Measurement Training)

July 14th- Eufrosina Young, MD (Investigator at SUNY Upstate, New York)



The ALS Association/Northeast ALS Consortium Educational Webinar

Healey ALS Platform Trial Regimen E: A Participant's Guide

Friday, June 17, 2022
5:00 - 6:00 PM EDT

The purpose of this webinar is to provide an overview of how SLS-005 (trehalose) may help to slow ALS and illustrate what potential participants can expect when enrolling in Regimen E of the Healey ALS Platform Trial. The presenters will review some of the unique aspects of the regimen related to the intravenous administration.

Recording Available:



<https://bit.ly/39Hs3SI>