

HEALEY ALS Platform Trial

Weekly Q&A – Feb 17, 2022



Healey Center

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

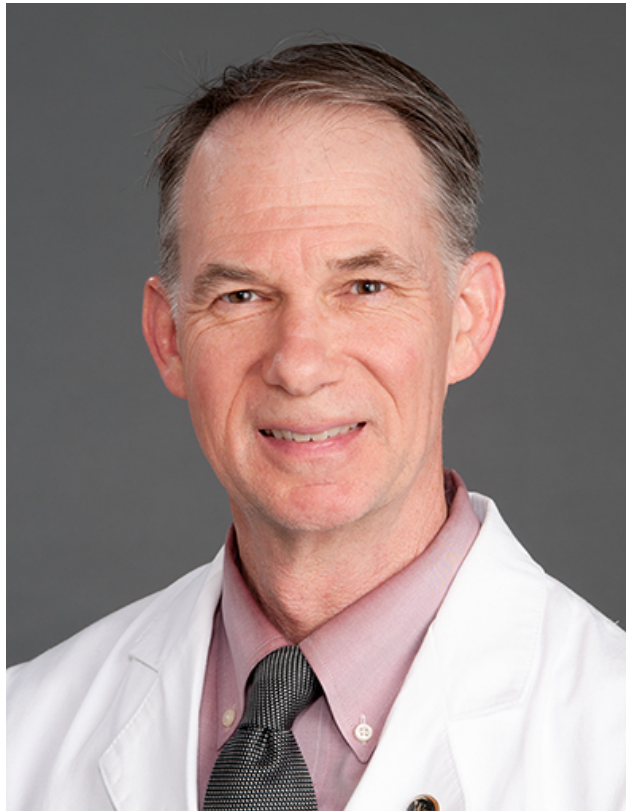


The AMG Foundation

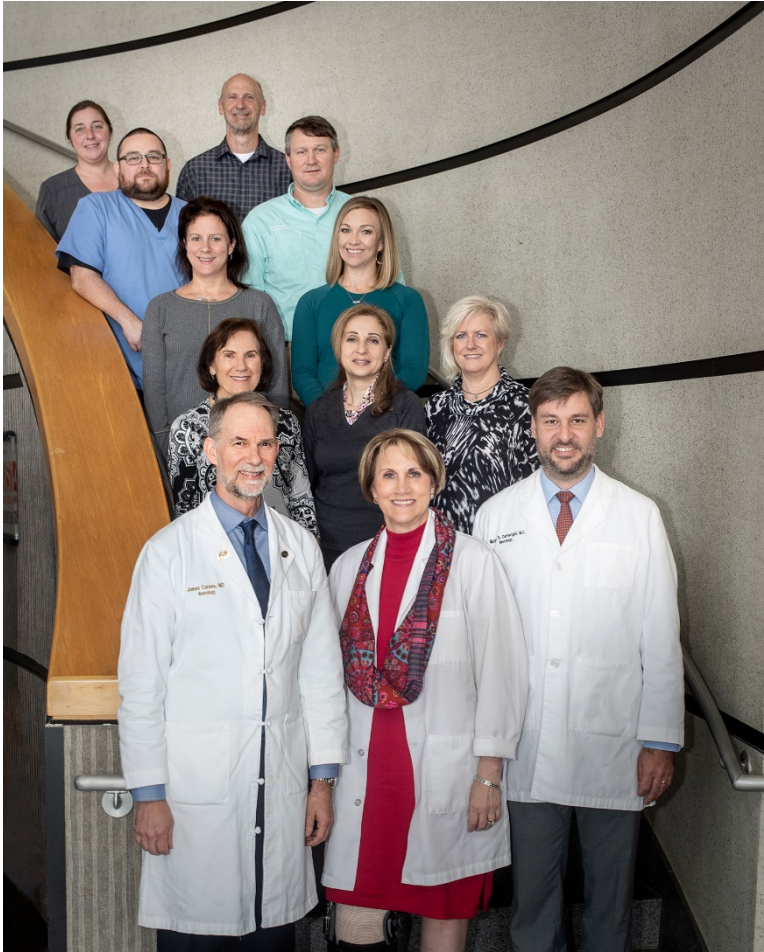
Guest Speaker

Jim Caress, MD

Platform Trial Site Principal Investigator
Wake Forest University, Winston-Salem NC



Wake Forest Team Unmasked (!)



Then Came the Pandemic



Wake Forest ALS Center

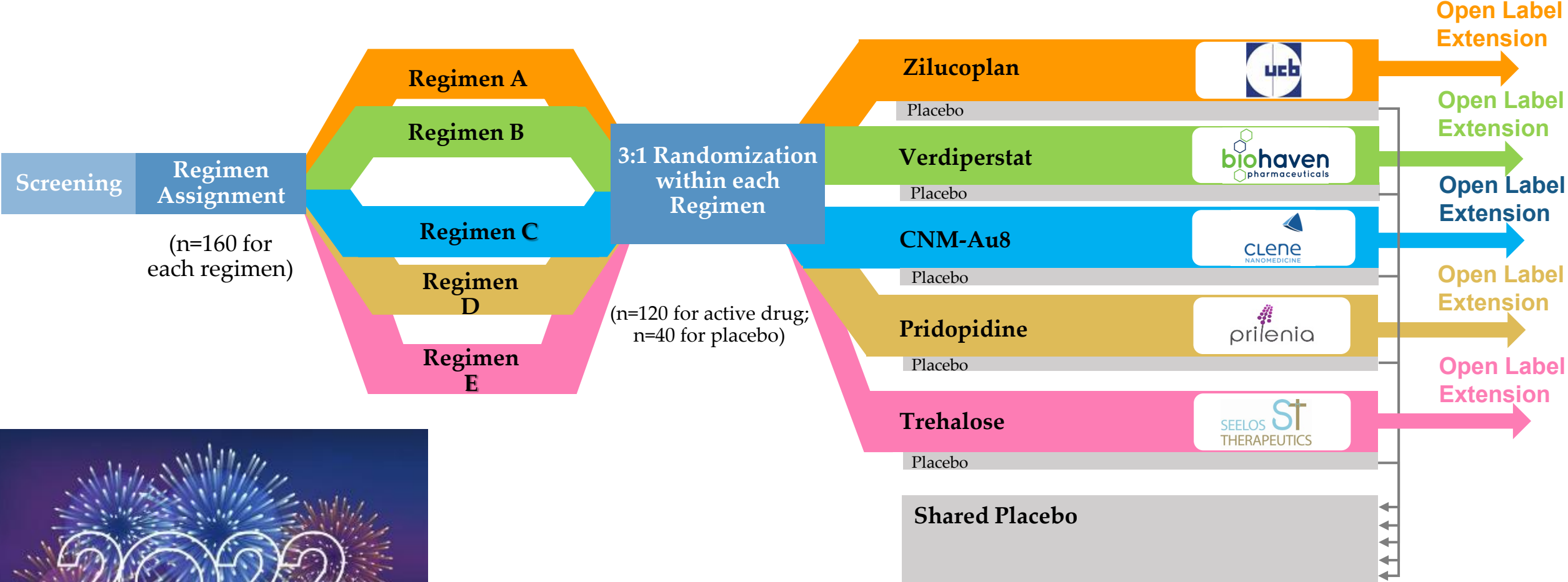
- Patient directed therapy sessions
- Patients move not clinicians
- ALS toolkit tracks ALSFRS, CBS, PFTs, neuro exam
- Free Lunch!



Wake Forest Baptist Health Sciences ALS Center provides state of the art medical care and offers hope through research to all patients diagnosed with ALS.

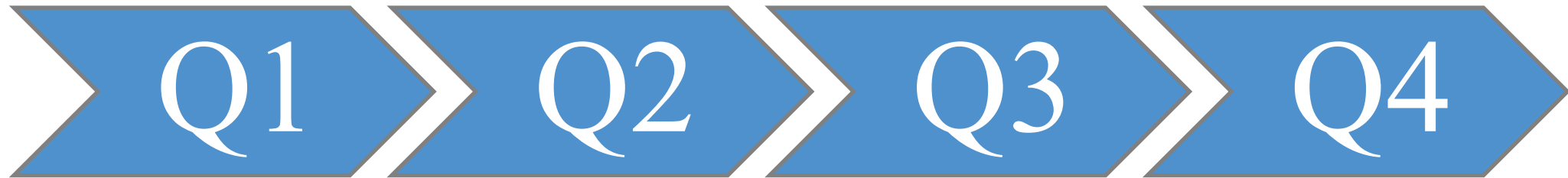
- Aim to have an available research study for all ALS patients
- Aim to have an open-label study
- Always have enrolling treatment trial
- Platform study should allow for continuous enrollment

The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial



Study Results Timelines – Regimens A-D

***** Note: COVID-19 might impact these estimates**



LPLV

(Last Participant Last Visit in the Randomized,
Placebo Controlled Period)

Database Lock

Top Line Results

Study Reports

Expected LPLV: March-July 2022 (depending on the regimen and OLE choice)

Expected Database Lock: May-Sept 2022

Top Line Results: July-Sept 2022

Study Reports: Oct-Dec 2022

Regimen E Site Activation

Activated!

- Lehigh Valley Health Network- **activated!**
- Mass General Hospital- **activated!**
- University of Kansas- **activated!**
- University of Maryland- **activated!**
- California Pacific Medical Center-**activated!**
- Northwestern University-**activated!**
- Virginia Commonwealth University-**activated!**
- University of Nebraska-**activated!**
- Washington University-**activated!**
- Wake Forest University-**activated!**

Sites closest to activation:

- Barrow Neurological Institute
- Saint Alphonsus Regional
- University of Massachusetts
- Hospital for Special Care
- University of Colorado
- Spectrum Health
- Essentia
- SUNY Upstate
- Duke University
- University of South Florida
- Beth Israel Deaconess
- Ohio State University
- Providence Brain and Spine

Checking Site Status Online

List of Participating Sites

Sites marked "Recruiting" are currently enrolling participants in regimen E.

Sites marked "Active, Not recruiting" are active in the platform (for example, they are following participants in regimens A-D) but are not enrolling new participants at this time. Most active sites are expected to start enrolling in regimen E soon.

Site	State	Enrollment Status	Contact Information
Jefferson Weinberg ALS Center, Thomas Jefferson University	PA	Active, Not recruiting	Stephanie Hansen
Lehigh Valley Health Network	PA	Recruiting	Andrew Orzel (610-402-9543)
Penn State Hershey	PA	Active, Not recruiting	Heidi Runk



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

Patient Navigation

Central resource for people living with ALS



Catherine Small

Phone: 833-425-8257 (HALT ALS)

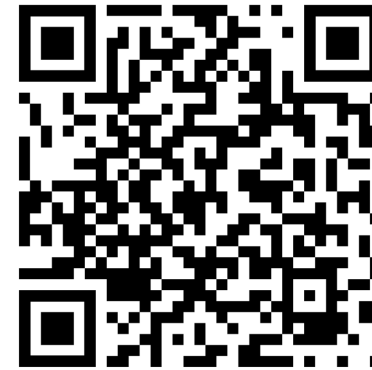
E-mail: healeyalsplatform@mgh.harvard.edu

Weekly webinar
registration:



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

ALS Link sign-up:



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

Upcoming Guest Speakers:

March 3rd- Andrea Swenson, MD (University of Iowa, Iowa City IA)

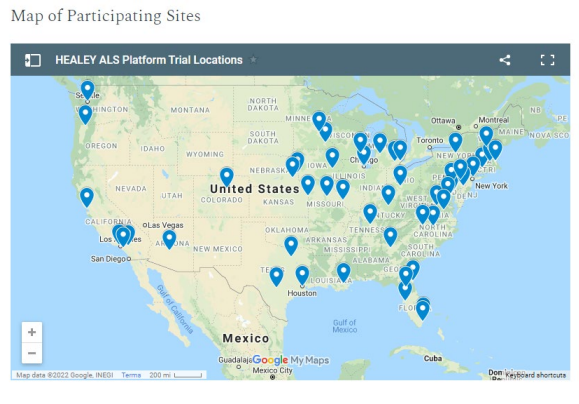
March 24th- Jaimin Shah, MD (Mayo Clinic, Jacksonville FL)



Allison Bulat

Send us webinar questions and ideas!

Travel around the
US meeting
Platform Trial Site
Investigators



Guest speakers
from partner
foundations

Trehalose/SLS-005
drug science and
mechanism of action
webinar with Seelos
Therapeutics



Special Topics:
Biomarkers, EAPs,
Biostats, Science
topics (epigenetics,
epidemiology)