

HEALEY ALS Platform Trial

Weekly Q&A – Aug 26, 2021



Healey Center

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



The AMG Foundation

Guest Speaker

Niraja Suresh, MD
University of South Florida, FL
Platform Trial Site Investigator



University of South Florida



- ALS Clinic at the University of South Florida (USF) is located at the Carol & Frank Morsani Center for Advanced Healthcare
- Established in 2010, the USF ALS Clinic provides multidisciplinary care for patients with ALS in the Tampa Bay and surrounding regions.
- We serve average 250 to 350 patients per year, we are the only academic, tertiary referral center on the west coast of Florida

University of South Florida



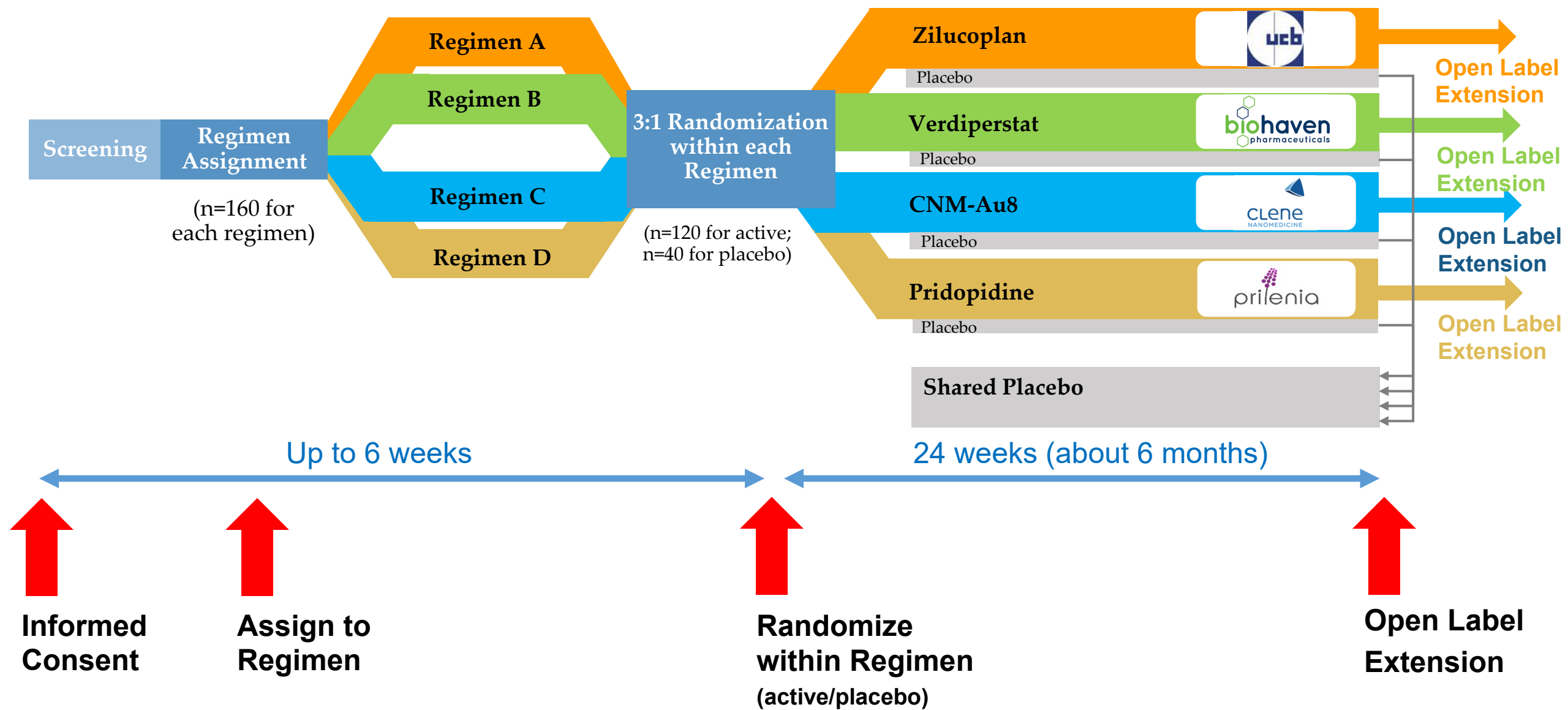
- Dr. Tuan Vu, Dr. Niraja Suresh, and Jerrica Farias
- Dr. Mark Armanious, Dr. Anthony Bradshaw, Dr. Rebecca Hurst, Dr. Cliff Gooch, Dr. Hera Stephens, Dr. Rossitza Chichkova, and Dr. Katie Murray
- Coordinators: Lucy Lam, Laura Alfonso



Perpetual Adaptive Trial

Shared Placebo; Randomization Ratio 3:1

Open Label Extension (OLE) offered



Enrollment Updates (as of August 19, 2021)

- **717** individuals with ALS signed informed consent
 - **565** individuals were assigned to a regimen
 - **518** individuals were randomized within a regimen (active or placebo)
 - **187** have entered the Open Label Extension (OLE)
- | | Total randomized +
Participants in screening |
|---|---|
| • 140 individuals were randomized within Regimen A | = 150 |
| • 158 individuals were randomized within Regimen B | = 160 |
| • 156 individuals were randomized within Regimen C | = 160 |
| • 64 individuals were randomized within Regimen D | = 67 |

Thank You

This breakthrough trial would not be possible without your partnership

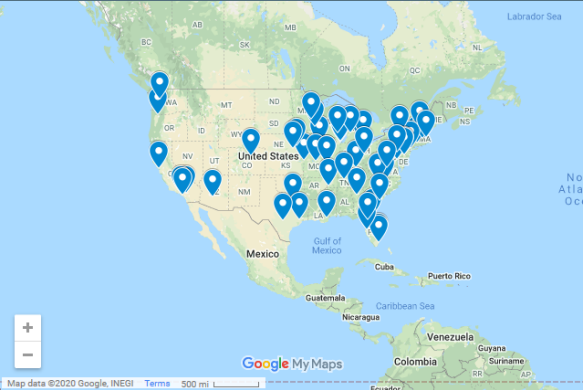
How to Find a Center Near You

Sean M. Healey & AMG
Center for ALS

About the Healey Center Clinical Care Patient & Family Resources

Map of Participating Sites

HEALEY ALS Platform Trial Locations
This map was created by a user. Learn how to create your own.



List of Participating Sites by State

Site	State	Contact Information
Barrow Neurological Institute	Arizona	Jessie Duncan

52 sites are actively enrolling

Contact Info of Participating Sites by State



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

Patient Navigator



Catherine Small

Allison Bulat

Phone: 833-425-8257 (HALT ALS)

E-mail: healeyalsplatform@mgh.harvard.edu



To see whether you might qualify,
view the list of eligibility criteria:

<https://bit.ly/3p4PrLL>



Send us webinar ideas!

- Biomarkers
- Biostatistics / Trial Design
- Get to know our sites



Upcoming Guest Speakers:

Sept 2nd- Terry Heiman-Patterson, MD (Temple University, PA)

Sept 9th- Jennifer DiMartino (Executive Director, ALS ONE)

Sept 16th- No webinar this week in observance of Yom Kippur

Sept 23rd- Namita Goyal, MD (University of CA Irvine, CA)

Oct 7th- No webinar this week

For More Updates

- **Weekly webinars**

The idea of came from our Patient Advisory Committee: we are excited to be talking with you on a weekly basis and take any questions you might have

- **Find the schedule and registration links on our website**

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

Previously: Drug mechanism of action and science webinars



- Jan 21- Prilenia/Pridopidine (recorded- <https://bit.ly/3ad3qel>)
- Feb 4 - Clene/CNM-Au8 (recorded- <https://bit.ly/3jB3WWt>)
- Feb 18- Biohaven/Verdiperstat (recorded- <https://bit.ly/301qdDR>)
- Feb 25- UCB/Zilucoplan (recorded- <https://bit.ly/3b3cpyS>)