

HEALEY ALS Platform Trial

Weekly Q&A – Aug 19, 2021



Healey Center

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



The AMG Foundation

Guest Speaker

Laura Foster, MD
University of Colorado Anschutz, CO
Platform Trial Site Investigator





University of Colorado **Anschutz Medical Campus**

Laura Foster, MD





University of Colorado – Healey ALS Platform Trial Research Team



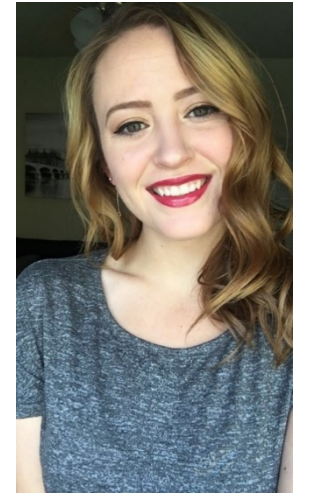
Laura Foster, MD
Assistant Professor



Stacy Dixon, MD, PhD
Assistant Professor



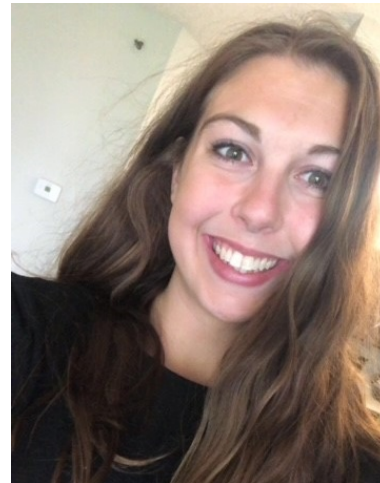
Brianna Blume, MS, CCRP
*Neuromuscular Research Development
Program Manager*



Brenna Baines, BS
*Clinical Research
Coordinator*



Haley Simpson, BS
*Clinical Research
Recruitment Specialist
& Coordinator*



Emily Hyslop, BA
*Clinical
Research
Coordinator*



Diana
Santacruz, BS
*Clinical
Research
Coordinator*





CU's participation in the Healey ALS Platform Trial

- Well-established research team
- First participant enrolled October 2020
- 14 participants (as of Aug 18, 2021)



CU study visit sites



How to help screening process

- Tell us about every medication or supplement, including over-the counter products
- Know where prior medical records are



Travel tips

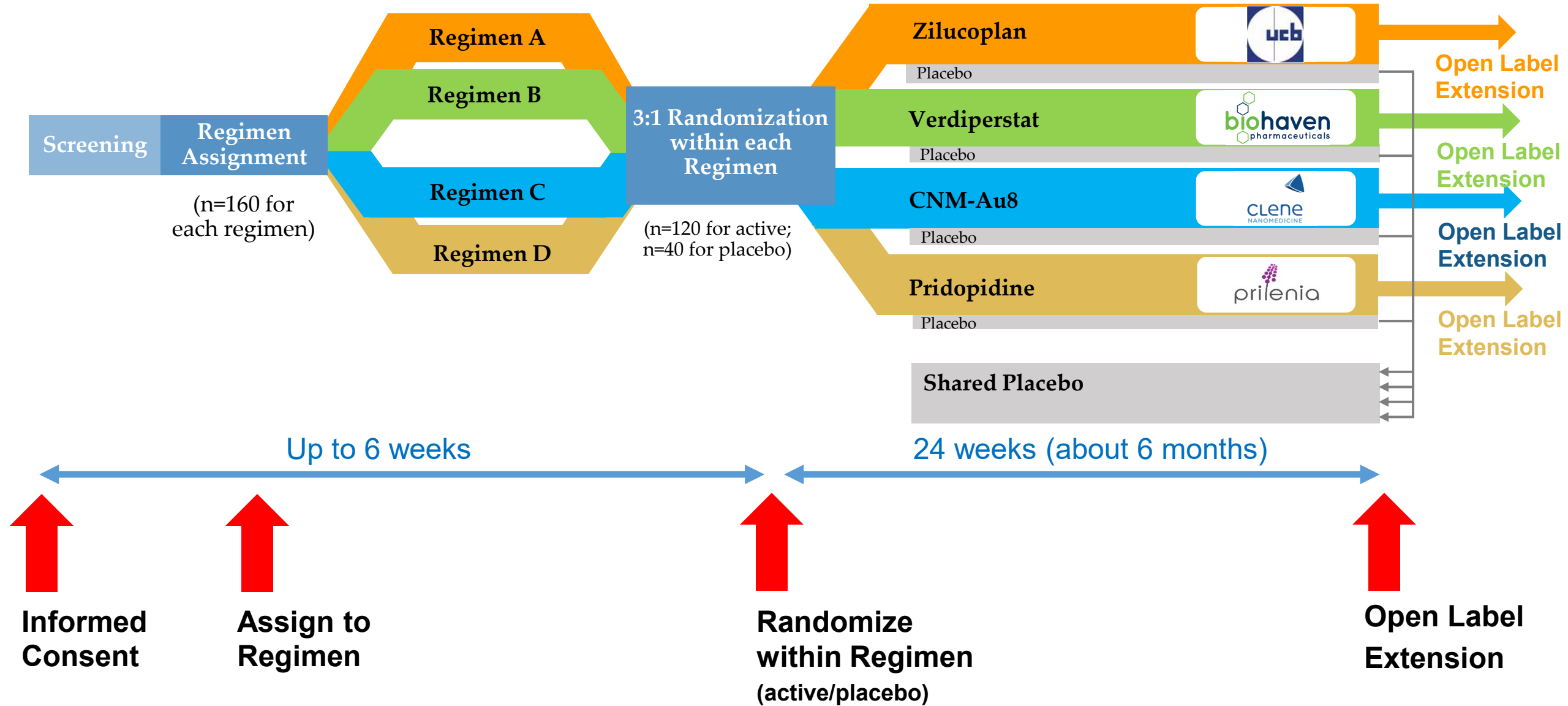
- Bring a travel buddy
- Bring snacks
- Stay at a hotel nearby



Perpetual Adaptive Trial

Shared Placebo; Randomization Ratio 3:1

Open Label Extension (OLE) offered



Enrollment Updates (as of August 19, 2021)

- **705** individuals with ALS signed informed consent
 - **558** individuals were assigned to a regimen
 - **510** individuals were randomized within a regimen (active or placebo)
 - **180** have entered the Open Label Extension (OLE)
- | | Total randomized +
Participants in screening |
|---|---|
| • 139 individuals were randomized within Regimen A | = 150 |
| • 154 individuals were randomized within Regimen B | = 157 |
| • 155 individuals were randomized within Regimen C | = 158 |
| • 62 individuals were randomized within Regimen D | = 67 |

Thank You

This breakthrough trial would not be possible without your partnership

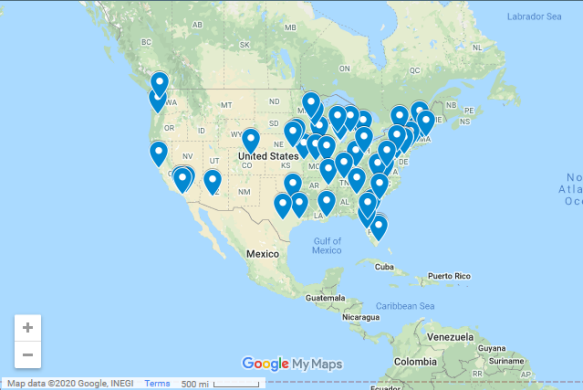
How to Find a Center Near You

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:

Aug 26th- Niraja Suresh, MD (University of South Florida, FL)

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)

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>)