

HEALEY ALS Platform Trial

Weekly Q&A – Sept 30, 2021



Healey Center

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



The AMG Foundation

Guest Speaker

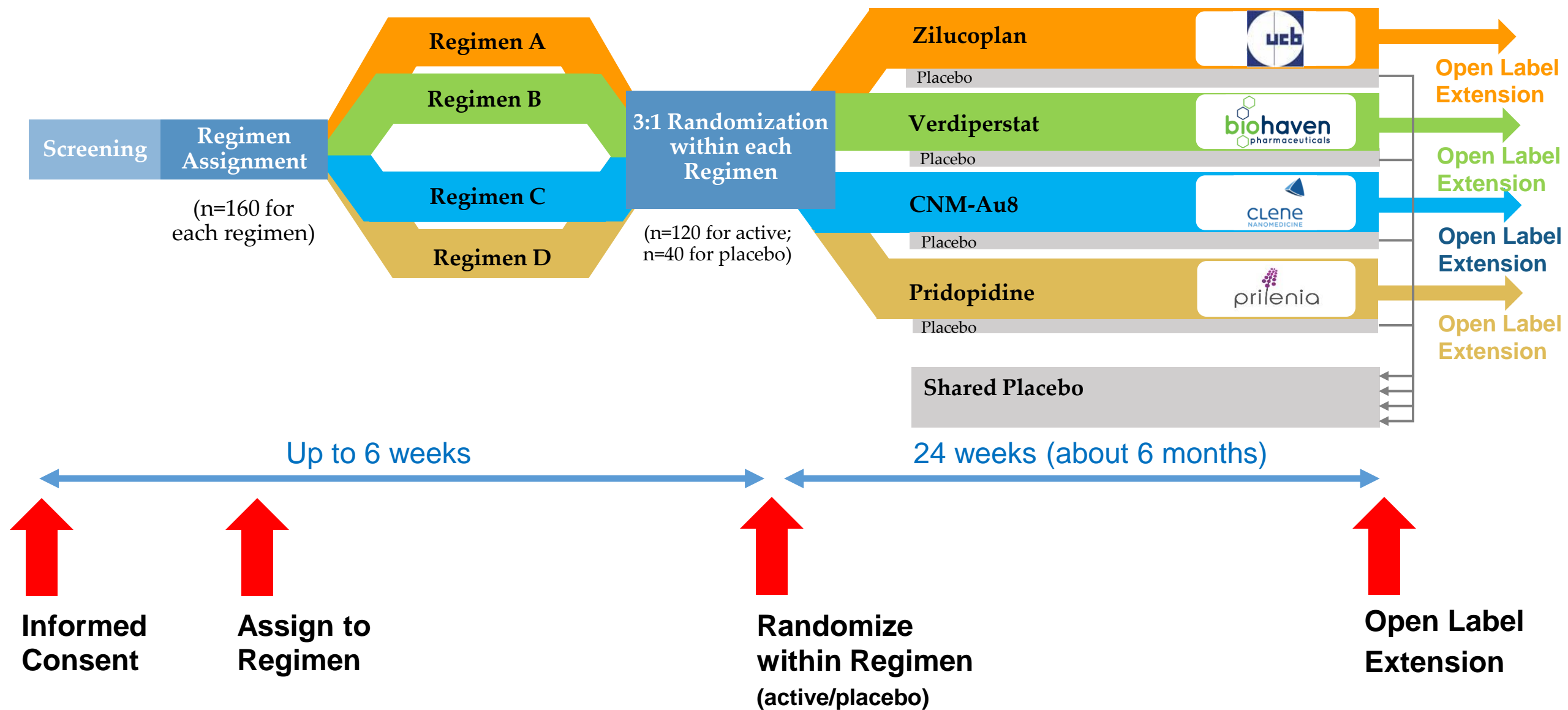
Irfan Qureshi, MD
Biohaven Pharmaceuticals
Vice President, Neurology



Perpetual Adaptive Trial

Shared Placebo; Randomization Ratio 3:1

Open Label Extension (OLE) offered



Enrollment Updates (as of September 30, 2021)

- **773** individuals with ALS signed informed consent
 - **615** individuals were assigned to a regimen
 - **554** individuals were randomized within a regimen (active or placebo)
 - **220** have entered the Open Label Extension (OLE)
- | | Total randomized +
Participants in screening |
|--|---|
|--|---|

Thank You

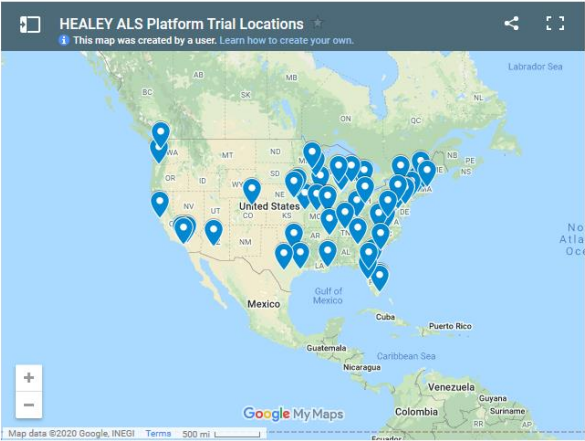
This breakthrough trial would not be possible without your partnership

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:

Oct 7th- No webinar this week

Oct 14th- David Walk, MD FAAN (University of Minnesota, MN)

Oct 21st- TBD

Oct 28th- Megan Miller, PhD (IAMALS Director of Science and Policy)



View recordings at
<https://bit.ly/3frQsfB>



An Update on AMX0035 and Design of the Phase 3 PHOENIX Study



Merit Cudkowicz, MD, MSc
Sean M. Healey & AMG Center for
ALS at Mass General Hospital



Sabrina Paganoni, MD, PhD
Sean M. Healey & AMG Center for
ALS at Mass General Hospital

Thursday, September 23, 2021
1:00 - 2:00 PM EDT

ALS Drug Development Webinar Series Part 6: Research Engagement by People Living With ALS



Richard Bedlack, MD, PhD
Duke ALS Clinic



Merit Cudkowicz, MD, MSc
Sean M. Healey & AMG Center for
ALS at Mass General Hospital

Wednesday, September 15, 2021
4:00 - 5:00 PM EDT

Recorded

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>)