

# 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

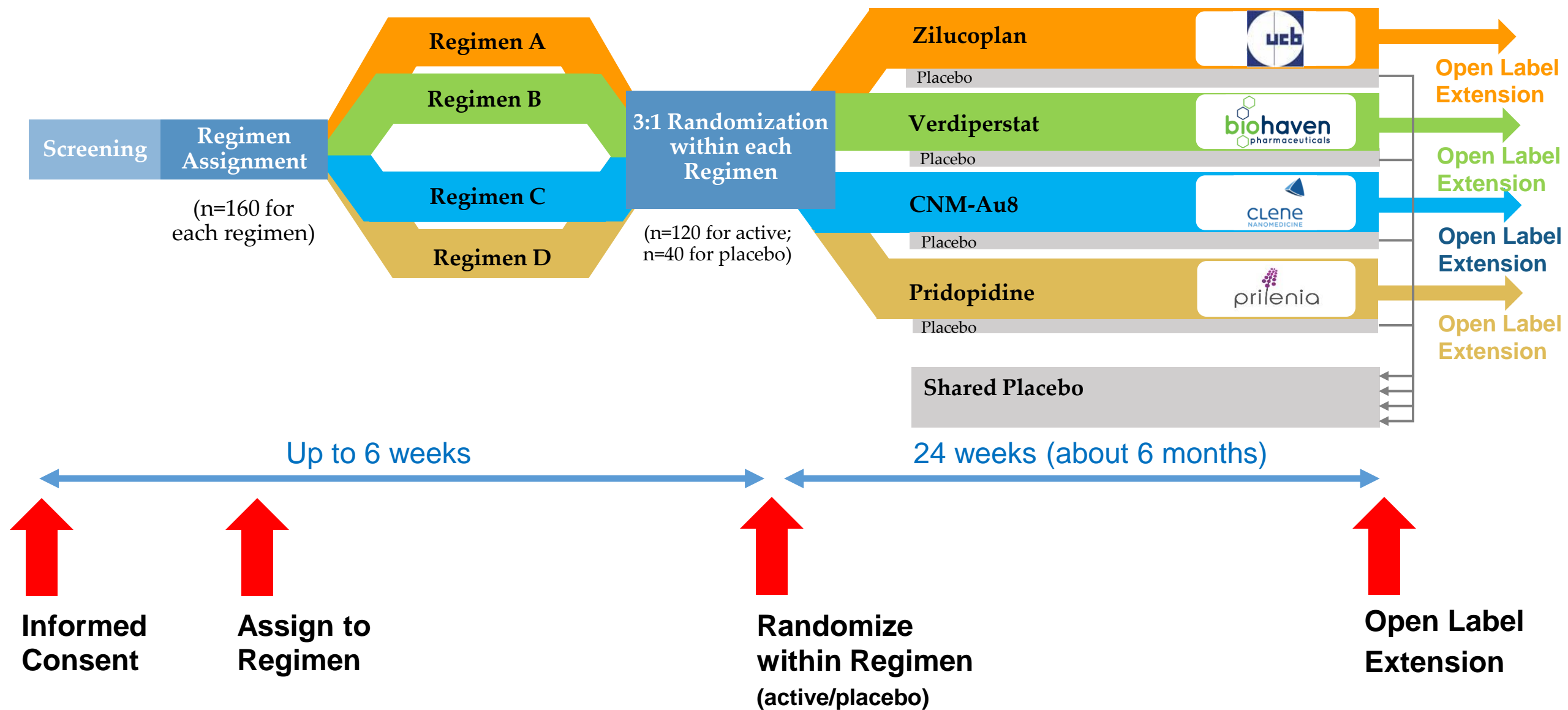
**Irfan Qureshi, MD**  
**Biohaven Pharmaceuticals**  
**Vice President, Neurology**



# 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 +<br>Participants in screening |
|--|---|
|--|---|

*Thank You*

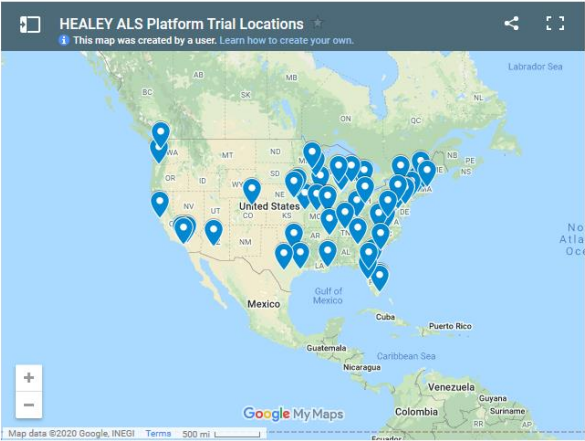
This breakthrough trial would not be possible without your partnership

# 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	<a href="#">Jessie Duncan</a>

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](mailto: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 7<sup>th</sup>- No webinar this week**

**Oct 14<sup>th</sup>- David Walk, MD FAAN (University of Minnesota, MN)**

**Oct 21<sup>st</sup>- TBD**

**Oct 28<sup>th</sup>- 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>)