

HEALEY ALS Platform Trial

Weekly Q&A – June 10, 2021



Healey Center

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



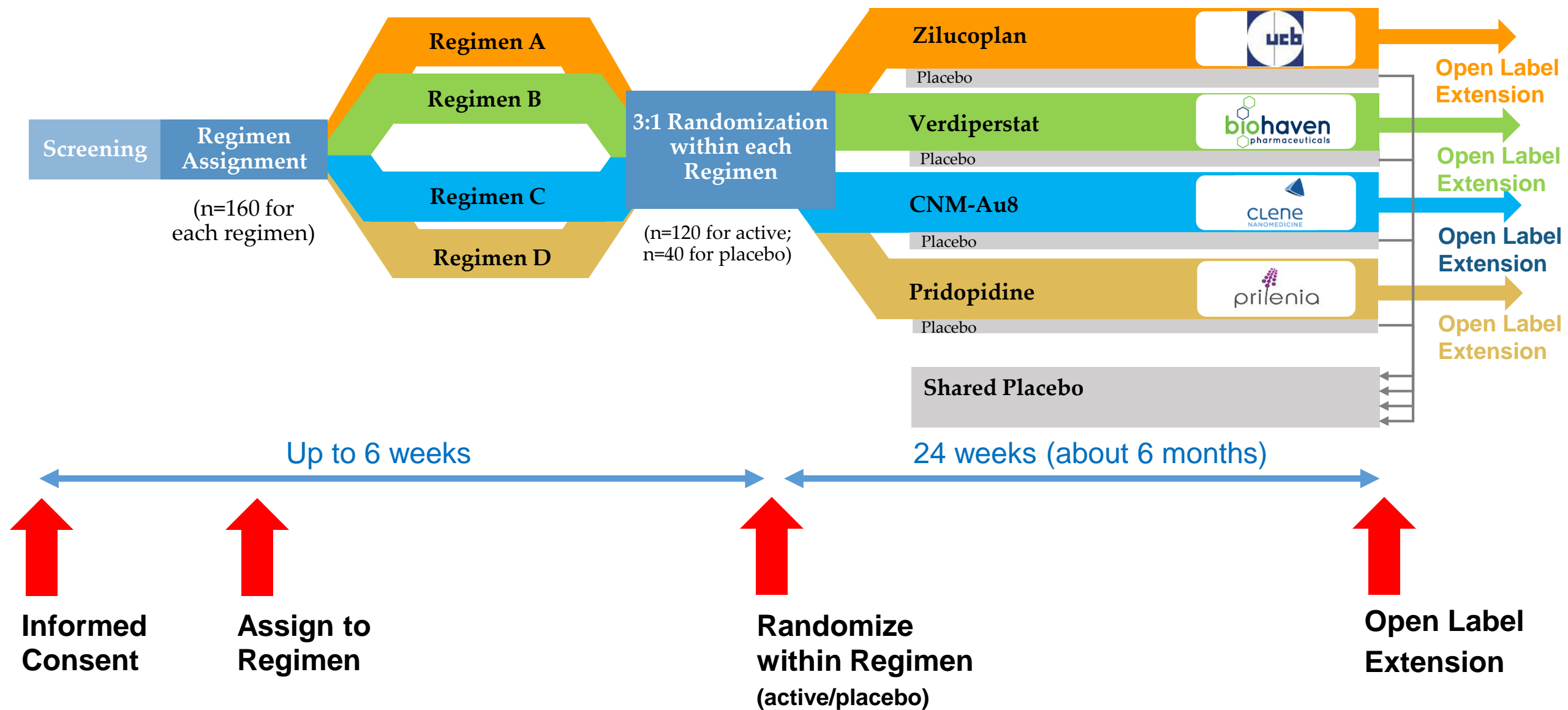
The AMG Foundation



Perpetual Adaptive Trial

Shared Placebo; Randomization Ratio 3:1

Open Label Extension (OLE) offered



Enrollment Updates (as of June 10, 2021)

- **582** individuals with ALS signed informed consent
- **452** individuals were assigned to a regimen
- **403** individuals were randomized within a regimen (active or placebo)
- **112** have entered the Open Label Extension (OLE)

- **110** individuals were randomized within Regimen A
- **127** individuals were randomized within Regimen B
- **127** individuals were randomized within Regimen C
- **39** individuals were randomized within Regimen D

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



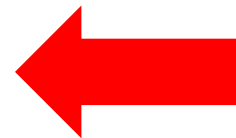
HEALEY ALS Platform Trial Locations
This map was created by a user. Learn how to create your own.

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>

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 (view recording- <https://bit.ly/3ad3qel>)

Feb 4 - Clene/CNM-Au8 (view recording- <https://bit.ly/3jB3WWt>)

Feb 18- Biohaven/Verdiperstat (view recording- <https://bit.ly/301qdDR>)

Feb 25- UCB/Zilucoplan (view recording- <https://bit.ly/3b3cpyS>)



Drug Development NEALS Webinar Series

This series will include talks on the preclinical development of potential therapies, the regulatory pathway, the science of trials, the clinical trial site level overview, and the importance of patient engagement.

Part 1: Scientific Considerations (Recorded April 7)

Part 2: Regulatory Considerations (Recorded April 9)

Part 3: Ethical Considerations (Recorded May 5)

Part 4: Site Considerations (Recorded May 14)

Part 5: Preclinical Development of Potential Therapies

June 23, 2021 @ 3:30-4:30pm ET

Part 6: Research Engagement by People Living with ALS

Sept 15, 2021 @ 4:00-5:00pm ET

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



An Update on the CENTAUR Trial: a Trial of AMX0035 in ALS **Recording Available**



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



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

Monday, May 17, 2021
2:00 - 3:00 PM EDT

ALS Clinical Trial Pipeline Webinar **Recording Available**



Merit Cudkowicz, MD, MSc
Sean M. Healey & AMG Center for ALS at Mass General Hospital
Monday, June 7, 2021
5:00 - 6:00 PM EDT

Dr. Cudkowicz will review the ALS clinical trial pipeline and answers questions about the current state of clinical research.

View recordings here:

www.neals.org

Send us webinar ideas!

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



Next Week's Guest: Dr. Lauren Elman of UPenn

A screenshot of a mobile application interface. The top bar is red with a back arrow, the text "University of Pennsylvania Com...", and a home icon. Below the bar, there is a text box with the following information:

name
University of Pennsylvania Comprehensive Neuroscience Center

description
Contact: Adreeja GuhaRay,
Adreeja.Guharay@Penmedicine.upenn.edu
u

To the right of the text is a map showing the location in Philadelphia, with labels for King of Prussia, Trenton, Hamilton Township, Philadelphia, Cherry Hill, and Wilmington. The map shows major roads like I-276 and I-295.

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 online:

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