

HEALEY ALS Platform Trial

Weekly Q&A – May 20, 2021



Healey Center

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

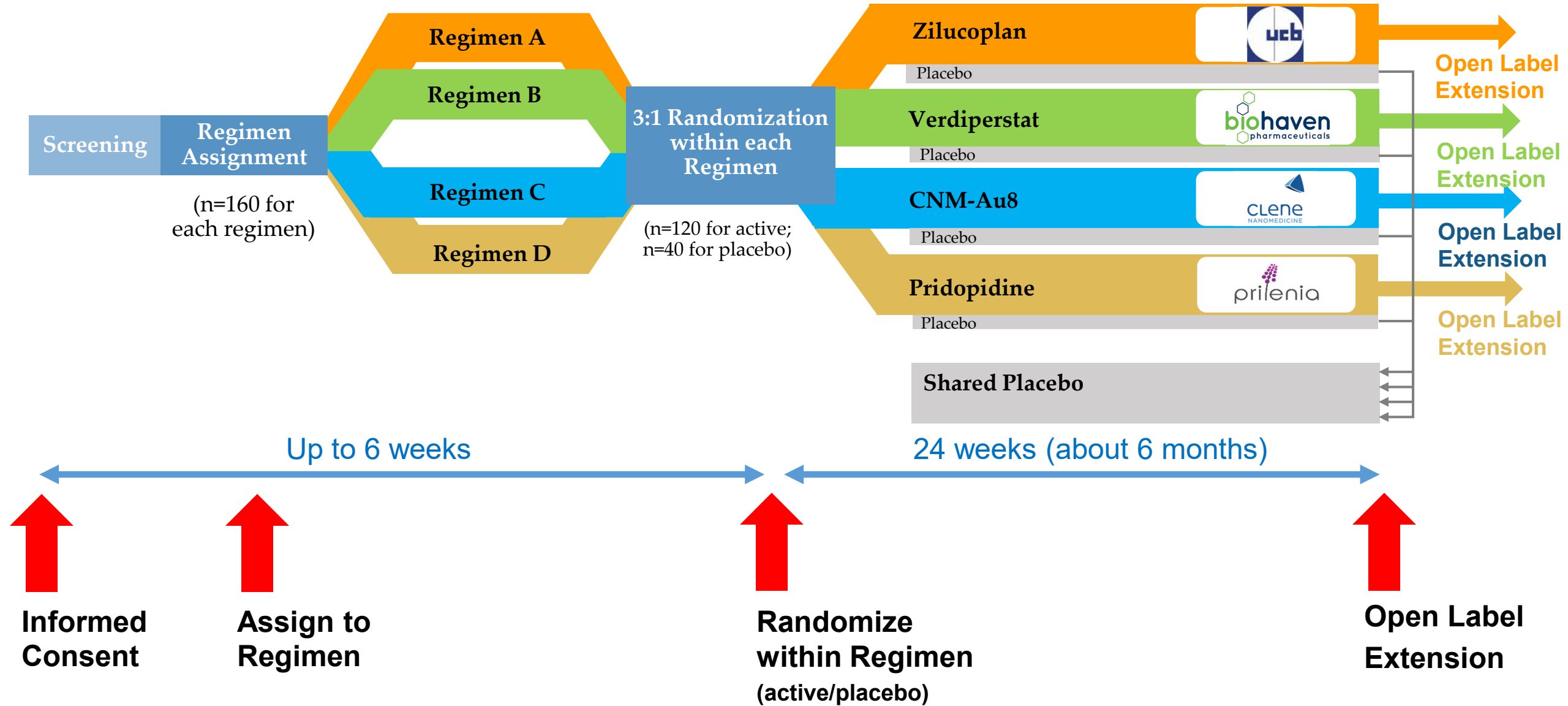


The AMG Foundation

Perpetual Adaptive Trial

Shared Placebo; Randomization Ratio 3:1

Open Label Extension (OLE) offered



Enrollment Updates (as of May 20, 2021)

- **531** individuals with ALS signed informed consent
- **404** individuals were assigned to a regimen
- **358** individuals were randomized within a regimen (active or placebo)
- **94** have entered the Open Label Extension (OLE)

- **99** individuals were randomized within Regimen A
- **115** individuals were randomized within Regimen B
- **114** individuals were randomized within Regimen C
- **30** individuals were randomized within Regimen D

Thank You

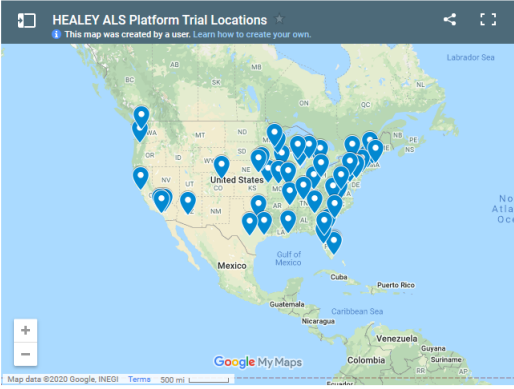
This breakthrough trial would not be possible without your participation

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



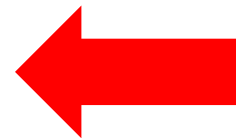
HEALEY ALS Platform Trial Locations
This map was created by a user. Learn how to create your own.

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>

New Online Resources!

Online Eligibility Checking Tool <https://bit.ly/3ezu4Qx>

Find Out If You're Eligible

We've prepared a short list of questions to help you find out if you might be eligible to participate in the HEALEY ALS Platform Trial.

Please note that the result of this survey does not serve as official confirmation of your eligibility, as many factors are considered prior to study participation (view a full list of inclusion / exclusion criteria [here](#)). Determining eligibility for the Platform Trial will depend on a thorough assessment of your clinical symptoms, review of past medical history, and lab work that can only be performed by an investigator (doctor or nurse practitioner) on the trial.



Eligibility questions

New Online Resources!

Platform Trial

Overview >

For Patients >

What to Expect >

About the Trial >

Study Drugs >

Study Drugs in the HEALEY ALS Platform Trial

Introducing the Study Drugs

A quick introduction to the trial and the study drugs, including information about earlier studies.

[Download brochure >](#)

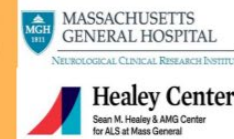
Trial of Zilucoplan

Developed By: UCB

Administration: Subcutaneous injection (prefilled syringe, self-administered once daily).

Learn More:

[What does this drug do?](#)
[Has this drug been studied before?](#)



HEALEY ALS
Platform Trial

Regimen
A

Regimen
B

Study
Drugs

Regimen
C

Regimen
D

Trial of CNM-Au8

Developed By: Clene Nanomedicine

Administration: Oral (ingestible liquid, 2oz once daily). Can be taken through G-tube.

Learn More:

[What does this drug do?](#)
[Has this drug been studied before?](#)



Trial of Verdiperstat

Developed By: Biohaven Pharmaceuticals

Administration: Oral (pill, 4 tablets daily).

Learn More:

[What does this drug do?](#)
[Has this drug been studied before?](#)



Trial of Pridopidine

Developed By: Prilenia Therapeutics

Administration: Oral (pill, 2 capsules daily).

Learn More:

[What does this drug do?](#)
[Has this drug been studied before?](#)



Download the Digital Drug Brochure from our website:

<https://bit.ly/2Q9N3aZ>

New Webpage! <https://bit.ly/3uGkhOa>

Platform
Trial

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What to Expect in the Trial Process

Expressing Interest in Participation



The Pre-Screening Process



Master Protocol Screening Visit and Informed Consent



Assignment to Regimen and Screening



Open Label Extension and Re-Assignment to Regimen



Trial Data and Results



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](https://bit.ly/3ad3qel))

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

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

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

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



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.

Register to attend and view recordings here:

www.neals.org

Patient Navigator



Catherine Small

Allison Bulat

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E-mail: healeyalsplatform@mgh.harvard.edu



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

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