

HEALEY ALS Platform Trial

Weekly Q&A – May 13, 2021



Healey Center

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

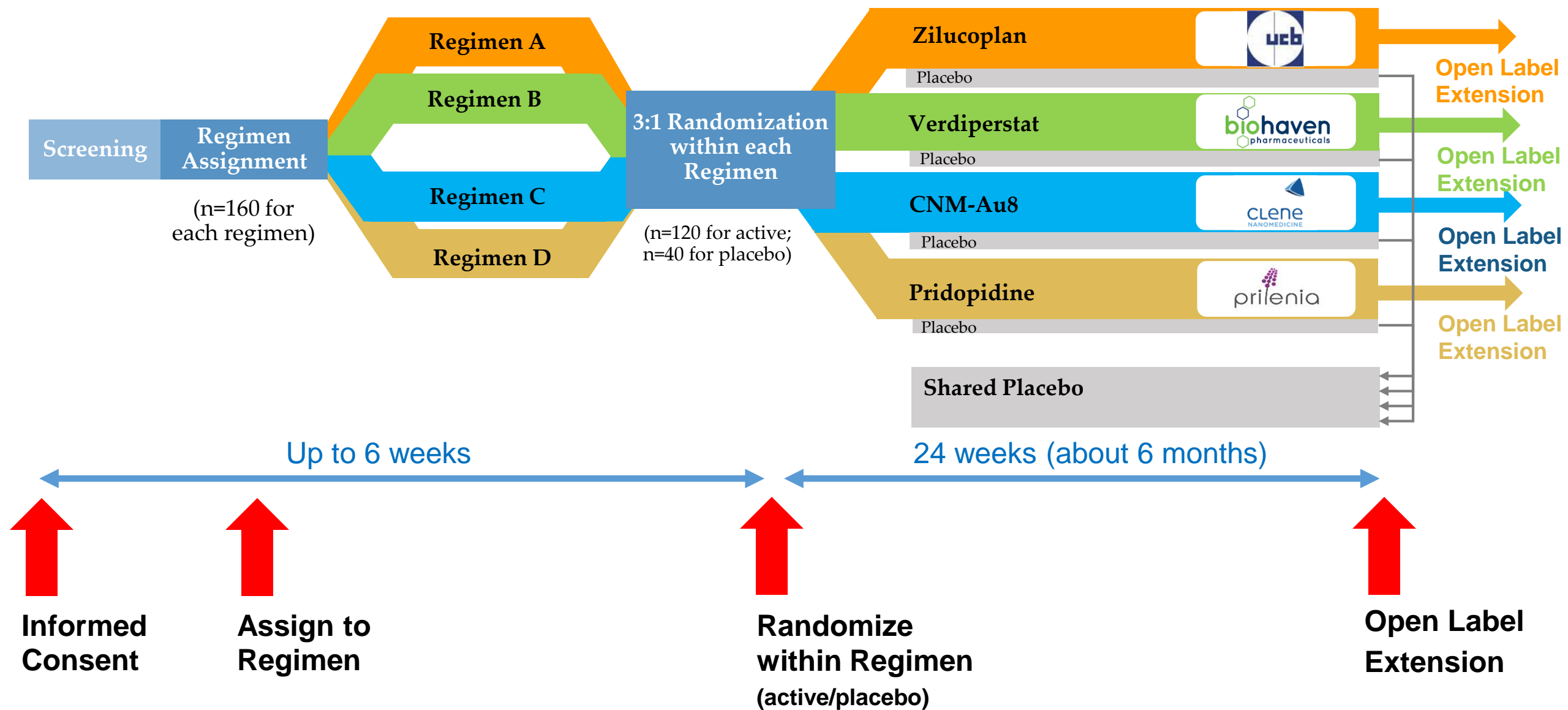


The AMG Foundation

Perpetual Adaptive Trial

Shared Placebo; Randomization Ratio 3:1

Open Label Extension (OLE) offered



Enrollment Updates (as of May 13, 2021)

- **512** individuals with ALS signed informed consent
- **391** individuals were assigned to a regimen
- **338** individuals were randomized within a regimen (active or placebo)
- **89** have entered the Open Label Extension (OLE)

- **93** individuals were randomized within Regimen A
- **111** individuals were randomized within Regimen B
- **109** individuals were randomized within Regimen C
- **25** 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



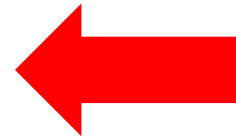
HEALEY ALS Platform Trial Locations
This map was created by a user. Learn how to create your own.

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>

List of enrolling sites as of May 13, 2021

52 sites are enrolling

- ✓ Texas Neurology
- ✓ Mass General Hospital
- ✓ UTHSCSA
- ✓ Hospital for Special Care
- ✓ Holy Cross Hospital
- ✓ Thomas Jefferson
- ✓ Houston Methodist
- ✓ Henry Ford Health System
- ✓ Barrow Neurological Institute
- ✓ Ohio State University
- ✓ Northwestern University
- ✓ University of Chicago
- ✓ Wake Forest
- ✓ University of Nebraska
- ✓ Loma Linda University
- ✓ University of Washington
- ✓ University of Iowa
- ✓ Washington University
- ✓ University of Pennsylvania
- ✓ University of Michigan
- ✓ California Pacific Medical Center
- ✓ Penn State Hershey
- ✓ UMass Worcester
- ✓ University of Miami
- ✓ University of Colorado
- ✓ Cedars-Sinai
- ✓ University of Florida
- ✓ University of South Florida
- ✓ Columbia University
- ✓ University of Virginia
- ✓ Emory University
- ✓ University of Maryland
- ✓ SUNY Upstate
- ✓ Beth Israel Deaconess
- ✓ Temple University
- ✓ Dartmouth-Hitchcock
- ✓ Medical College of Wisconsin
- ✓ Spectrum Health
- ✓ University of Missouri
- ✓ University of Minnesota
- ✓ Johns Hopkins University
- ✓ University of CA Irvine
- ✓ University of Kansas
- ✓ Vanderbilt University
- ✓ University of Kentucky
- ✓ Mayo Rochester
- ✓ Duke University
- ✓ Neurology Associates
- ✓ Ochsner Health System
- ✓ Mayo Clinic Florida
- ✓ St. Louis University
- ✓ Providence Brain and Spine

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).

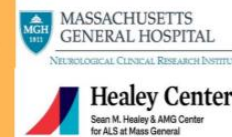
Learn More:

[What does this drug do?](#)

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



Regimen
A



HEALEY ALS
Platform Trial

Study
Drugs



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?](#)



Regimen
B

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?](#)



Regimen
C

Regimen
D



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
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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



FAQ:

Which supplements/interventions are exclusionary?

Guiding Principles:

- Any supplements/medications that are in a trial are considered exclusionary (as they might affect the efficacy of the treatment under evaluation)
 - Additional supplements/medications might be exclusionary based on safety and interactions with the treatment under evaluation
 - Examples of exclusionary agents: TUDCA, sodium phenylbutyrate, curcumin, very high doses of methylcobalamin
- Most trials have a list of exclusionary supplements/medications, but each trial has a different list
- **The list might change as new trials open and others end**
- Note: a washout period prior to Master Protocol Screening is required (30 days or 5 half-lives if known, whichever is longer)

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 –Friday, May 14th 3:30-4:30pm EDT

Register to attend and view recordings at <https://bit.ly/3frQsfB>



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



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

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