

# HEALEY ALS Platform Trial

Weekly Q&A – May 6, 2021



HARVARD  
MEDICAL SCHOOL



MASSACHUSETTS  
GENERAL HOSPITAL



## Healey Center

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

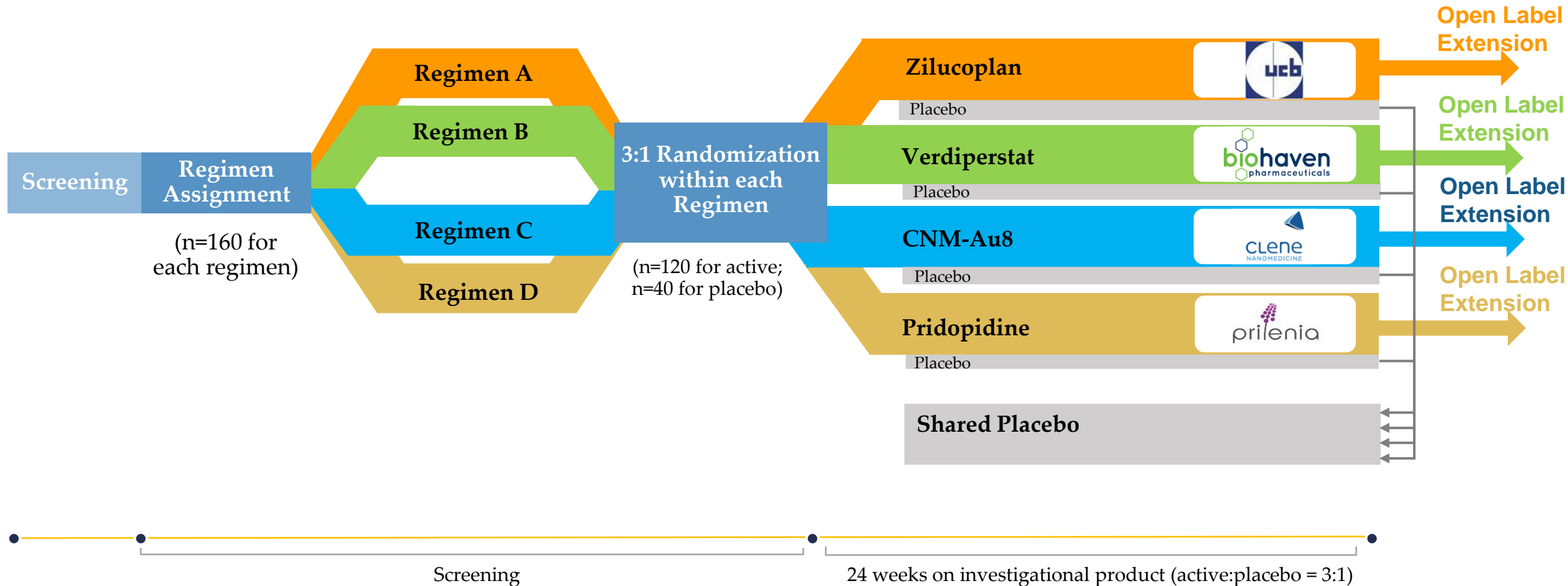


The AMG Foundation

# Perpetual Adaptive Trial

## Randomization Ratio 3:1; Shared Placebo

### Open Label Extension (OLE) offered



# Enrollment Updates (as of May 6, 2021)

- **485** individuals with ALS signed informed consent
- **369** individuals were assigned to a regimen
- **323** individuals were randomized within a regimen (active or placebo)
- **82** have entered the Open Label Extension (OLE)
  
- **90** individuals were randomized within Regimen A
- **104** individuals were randomized within Regimen B
- **106** individuals were randomized within Regimen C
- **23** 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.

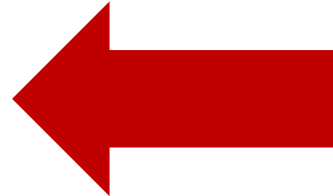


List of Participating Sites by State

Site	State	Contact Information
Barrow Neurological Institute	Arizona	<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>

# List of enrolling sites as of May 6, 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

# 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 14<sup>th</sup> 3:30-4:30pm EDT

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



## 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](http://www.neals.org)





## 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**

**Register to attend and view recordings here:**

**[www.neals.org](http://www.neals.org)**

# Send us webinar ideas!

- Biomarkers
- Biostatistics / Trial Design
- Get to know our 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 online:

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