

# HEALEY ALS Platform Trial

Weekly Q&A – June 17, 2021



## Healey Center

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



The AMG Foundation

# Guest Speaker

**Lauren Elman, MD**  
**University of Pennsylvania, Philadelphia**  
**Platform Trial Site Investigator**

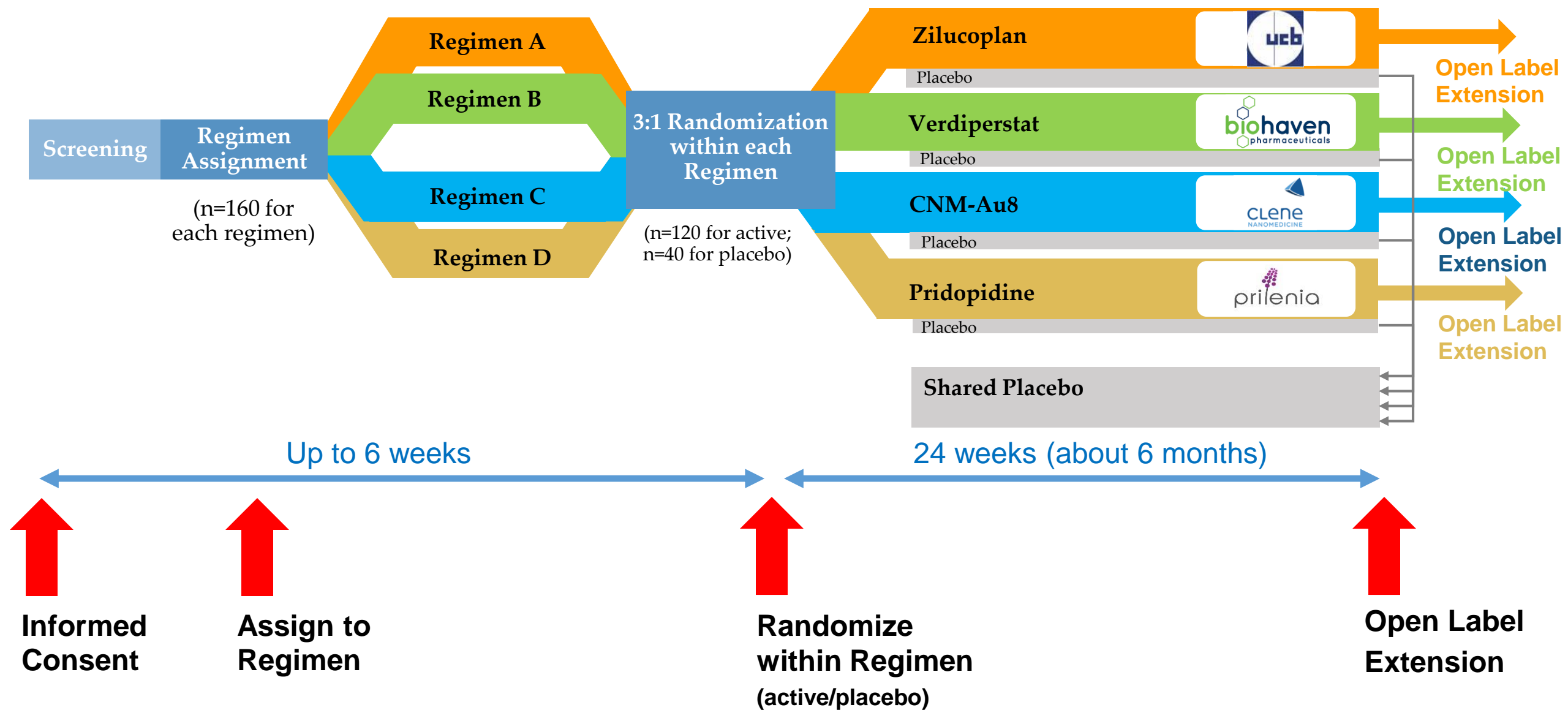




# Perpetual Adaptive Trial

## Shared Placebo; Randomization Ratio 3:1

### Open Label Extension (OLE) offered



# Enrollment Updates (as of June 17, 2021)

- **591** individuals with ALS signed informed consent
- **469** individuals were assigned to a regimen
- **410** individuals were randomized within a regimen (active or placebo)
- **116** have entered the Open Label Extension (OLE)
  
- **111** individuals were randomized within Regimen A
- **130** individuals were randomized within Regimen B
- **130** 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

# 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>

# Send us webinar ideas!

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



## Upcoming Guest Speakers:

**July 1<sup>st</sup>- Jeffrey Rosenfeld, MD (Loma Linda University, CA)**

**July 15<sup>th</sup>- Shafeeq Ladha, MD (Barrow Neurological Institute, AZ)**

**July 22<sup>nd</sup>- Richard Bedlack, MD, PhD, MS (Duke University, NC)**

# 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>)





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