#### **WE WILL BEGIN SHORTLY**

 To submit text questions to today's presenters, please type your questions into the 'Q&A' box

 Today's presentation is being recorded and will be posted on the Healey & AMG Center for ALS website

https://www.massgeneral.org/neurology/als/news/webinars

# **HEALEY ALS Platform Trial**

Weekly Q&A - Feb 11, 2021













BARROW

Neurological Institute\*



### **Healey Center**

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



































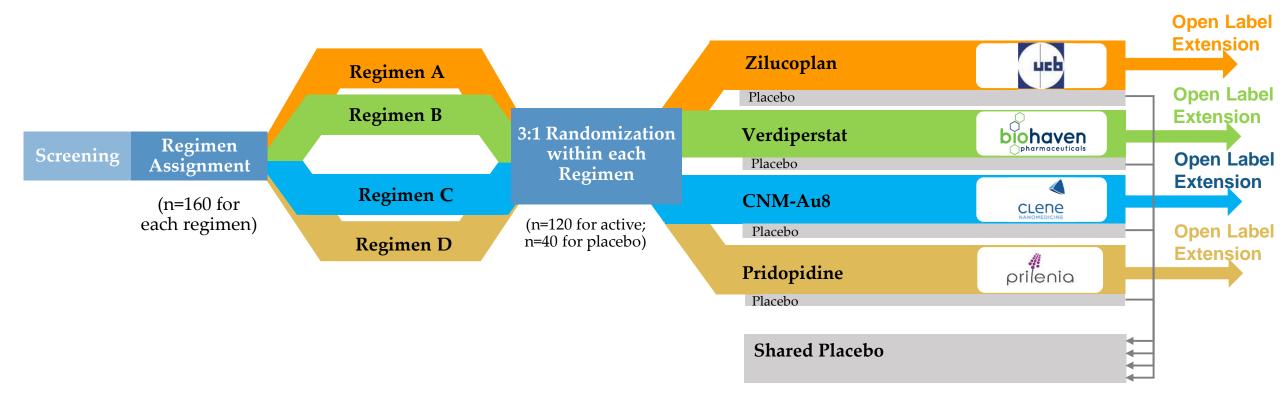


#### **AGENDA**

- Updates on the HEALEY ALS Platform Trial
  - Investigational Products
  - Activated Sites
  - Enrollment
- How to stay in touch and find a site near you

## YOUR QUESTIONS

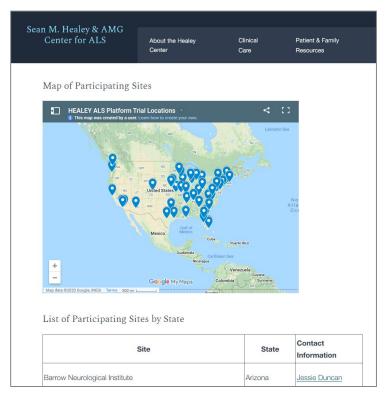
### Perpetual Adaptive Trial Randomization Ratio 3:1; Shared Placebo Open Label Extension offered



Screening

24 weeks on investigational product (active:placebo = 3:1)

#### How to Find a Center Near You



42 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 Feb 11, 2021 42 sites are enrolling

- **™** 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 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

# Enrollment Updates (as of Feb 11, 2021)

- 323 individuals with ALS signed informed consent
- 257 individuals were assigned to a regimen
- 217 are receiving investigational product (active or placebo)

We will continue to update the ALS community on enrollment (website, webinars)

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

https://bit.ly/3p4PrLL

## **Patient Navigator**

# Catherine Small Allison Bulat

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## 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/

#### Coming up: Drug mechanism of action and science webinars

Jan 21- Prilenia/Pridopidine (Recording available)

https://bit.ly/3ad3qel

**Feb 4 -** Clene/CNM-Au8 (Recording available)

https://bit.ly/3jB3WWt

Feb 18- Biohaven/Verdiperstat

Feb 25- UCB Ra Pharma/Zilucoplan