

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 – Jan 7, 2021



AGENDA

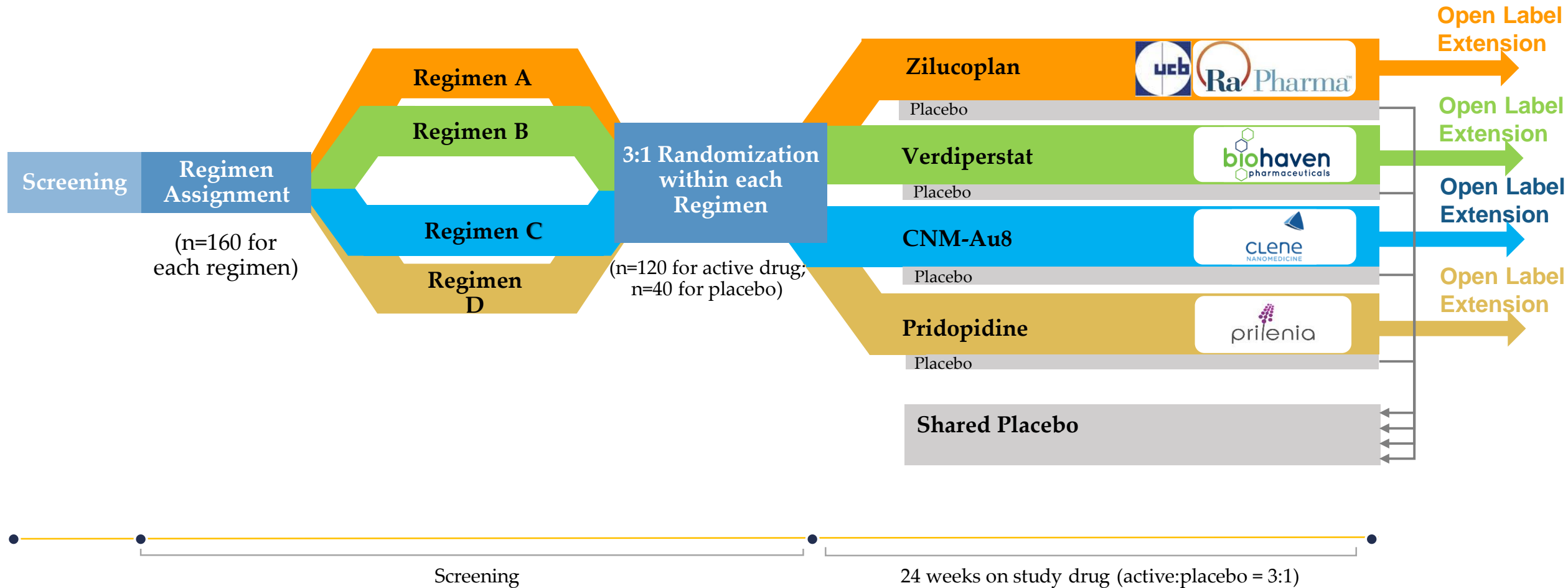
- **Updates on the HEALEY ALS Platform Trial**
 - *Study Treatments*
 - *Sites*
 - *Enrollment*
- **How to stay in touch and find a site near you**

YOUR QUESTIONS

Perpetual Adaptive Trial

Randomization Ratio 3:1; Shared Placebo

Open Label Extension offered




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.

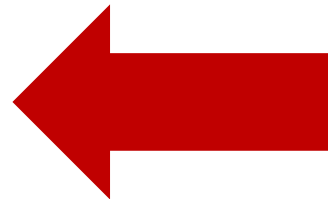


List of Participating Sites by State

Site	State	Contact Information
Barrow Neurological Institute	Arizona	Jessie Duncan

34 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 Jan 7, 2021

34 sites are enrolling

- ✓ Texas Neurology
- ✓ MGH
- ✓ 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 Medical Center
- ✓ Loma Linda
- ✓ University of Washington
- ✓ University of Iowa
- ✓ Washington University
- ✓ University of Pennsylvania
- ✓ University of Michigan
- ✓ Forbes Norris MDA/ALS Research Center, CPMC
- ✓ Penn State Hershey
- ✓ UMass
- ✓ 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 Medical Center

Enrollment Updates (as of Jan 7, 2021)

- **257** individuals with ALS signed informed consent
- **203** individuals were assigned to a regimen
- **181** are currently receiving study drug (active or placebo)

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

Expanded Access Program (EAP)

- **Great interest in EAPs from the patient community**
- **Long term goal is to build an EAP in parallel to the Platform Trial**
 - The idea came from the Patient Advisory Committee
 - The EAP would allow access to patients who are not eligible for the trial
- **We are working to achieve this goal**
 - We are actively fundraising to support this effort
 - One grant has been received from I AM ALS to start building the EAP

<https://www.massgeneral.org/neurology/als/news/2020-12-18-iamals-eap-grant>

Patient Navigator

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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

Feb 4 - Clene/CNM-Au8

Feb 18- Biohaven/Verdiperstat

Feb 25- Ra Pharma/Zilucoplan