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 – Dec 17, 2020





- 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

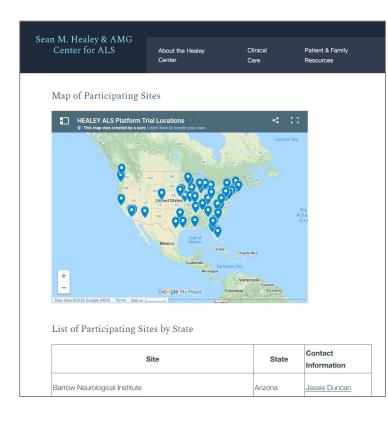
Screening

Open Label Extension Zilucoplan **Regimen** A Ra Pharma **Open Label** Placebo **Regimen B** Extension **3:1** Randomization Verdiperstat biohaven Regimen pharmaceuticals within each Screening **Open Label** Assignment Placebo Regimen Extension **Regimen C CNM-Au8** (n=160 for CLENE each regimen) (n=120 for active drug; Placebo **Open Label** Regimen n=40 for placebo) **Extension** D prilenia Pridopidine Placebo Shared Placebo

Open Label Extension starting Jan 2021 (A,B,C)

24 weeks on study drug (active:placebo = 3:1)

How to Find a Center Near You



All 54 sites are IRB approved! 33 sites are actively enrolling

Call for new sites! Suggestions?

Contact Info of Participating Sites by State

https://www.massgeneral.org/neurology/als/research/platform-trial-sites

List of enrolling sites as of Dec 17, 2020 33 sites are enrolling

- **Texas Neurology**
- 🗹 MGH
- **W** UTHSCSA
- **Markov Hospital for Special Care**
- **Markov Holy Cross Hospital**
- **Markov Thomas Jefferson**
- **Markov Methodist**
- **Henry Ford Health System**
- **Solution** Barrow Neurological Institute
- **Markov Ohio State University**
- **Morthwestern University**
- **W** University of Chicago
- Wake Forest
- **W** University of Nebraska Medical Center
- 🗹 Loma Linda
- **W** University of Washington

- **W** University of Iowa
- Washington University
- **W** University of Pennsylvania
- **W** University of Michigan
- **Forbes Norris MDA/ALS Research Center, CPMC**
- **Penn State Hershey**
- C UMass
- ✓ University of Miami
- **W** University of Colorado
- 🗹 Cedars-Sinai
- **W** University of Florida
- C University of South Florida
- Columbia University
- **W** University of Virginia
- **Emory**
- **W** University of Maryland
- SUNY Upstate

Enrollment Updates (as of Dec 17, 2020)

- 239 individuals with ALS signed informed consent
- 186 individuals were assigned to a regimen
- 164 are currently receiving study drug (active or placebo)

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

ONGOING COVID-19 IMPACT

- Many centers are experiencing a second surge
 - staff redeployment
 - some institutions are limiting research activities
- Supply chain problems (central vendor)
- Home nursing shortages

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/

Early 2021: webinars about each drug's mechanism of action and science