HEALEY ALS Platform Trial

Weekly Q&A – April 8, 2021
Guest Speaker

Jeremy Shefner, MD, PhD
Barrow Neurological Institute Phoenix, AZ
Regimen Lead (Regimen D: Pridopidine)
Perpetual Adaptive Trial
Randomization Ratio 3:1; Shared Placebo
Open Label Extension (OLE) offered

Screening
Regimen Assignment
(n=160 for each regimen)

Regimen A
Regimen B
Regimen C
Regimen D

3:1 Randomization within each Regimen
(n=120 for active; n=40 for placebo)

Zilucoplan
Placebo

Verdiperstat
Placebo

CNM-Au8
Placebo

Pridopidine
Placebo

Shared Placebo

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

Open Label Extension

—as of 4/8/21—
Endpoints

Primary Endpoint
Change in disease severity - ALS Functional Rating Scale-Revised (ALSFRS-R)

Secondary Endpoints
1. Change in respiratory function - slow vital capacity (SVC)
2. Change in muscle strength - hand held dynamometry (HHD)
3. Survival
4. Treatment-specific biomarkers as applicable

Exploratory Endpoints

Safety Endpoints
Exploratory Endpoints

Endpoint Development Engine

- DNA
- Neurofilaments
- Biomarkers (Blood, Urine, CSF)
- Speech / Digital
How to Find a Center Near You

50 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 April 8, 2021
50 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
Enrollment Updates (as of April 8, 2021)

- 420 individuals with ALS signed informed consent
- 329 individuals were assigned to a regimen
- 280 are receiving investigational product (active or placebo)
- 56 have entered Open Label Extension (OLE)

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

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

  Feb 4  - Clene/CNM-Au8     (view recording- https://bit.ly/3jB3WWt)
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 7th 12:00-1:00pm EDT

Part 2: Regulatory Considerations
April 9th 12:30-1:30pm EDT

Send us webinar ideas!

• Biomarkers
• Biostatistics / Trial Design
• Get to know our sites

Next Week’s Guest: Dr. Adam Quick from Ohio State University