Guest Speaker

James Berry, MD, MPH
Mass General Hospital Boston, MA
Regimen co-Lead (Regimen C)
Perpetual Adaptive Trial
Randomization Ratio 3:1; Shared Placebo
Open Label Extension (OLE) offered

Screening → Regimen Assignment

- Regimen A (n=160 for each regimen)
- Regimen B
- Regimen C
- Regimen D

3:1 Randomization within each Regimen

- Zilucoplan
  - Placebo
- Verdiperstat
  - Placebo
- CNM-Au8
  - Placebo
- Pridopidine
  - Placebo
- Shared Placebo

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

as of 4/1/21
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
How to Find a Center Near You

49 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 1, 2021
49 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
Enrollment Updates (as of April 1, 2021)

• 404 individuals with ALS signed informed consent
• 313 individuals were assigned to a regimen
• 275 are receiving investigational product (active or placebo)
• 48 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

Catherine Small
Allison Bulat
Phone: 833-425-8257 (HALT ALS)
E-mail: healeyalsplatform@mgh.harvard.edu

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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/

**Previously: Drug mechanism of action and science webinars**

- **Jan 21 -** Prilenia/Pridopidine  

- **Feb 4 -** Clene/CNM-Au8  
  (view recording- https://bit.ly/3jB3WWt)

- **Feb 18 -** Biohaven/Verdiperstat  

- **Feb 25 -** UCB/Zilucoplan  
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
April 7\textsuperscript{th} 12:00-1:00pm EDT

Part 2: Regulatory Considerations
April 9\textsuperscript{th} 12:30-1:30pm EDT

Send us webinar ideas!

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