Guest Speaker

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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 (n=120 for active; n=40 for placebo)
- 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 4/15/21
How to Find a Center Near You

51 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 15, 2021
51 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
✅ St. Louis University
Enrollment Updates (as of April 15, 2021)

- 432 individuals with ALS signed informed consent
- 332 individuals were assigned to a regimen
- 294 are receiving investigational product (active or placebo)
- 68 have entered Open Label Extension (OLE)

Thank You

This breakthrough trial would not be possible without your participation.

Your partnership in research is what keeps us filled with passion, dedication, and the commitment to uncover new promising treatments for ALS.

Every research participant, whether on the active drug or placebo, plays a critical role in making the hope of finding a cure for ALS a reality.
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  
Previously: 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 (Recorded)
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. Daragh Heitzman from Texas Neurology
Patient Navigator

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