Guest Speaker

Daragh Heitzman, MD, FAAN
Texas Neurology, Dallas TX
Platform Trial Site Investigator
ALS Center at Texas Neurology in Dallas, Texas

- Private practice, since 2000, MDA and ALSA
- 2 multidisciplinary clinics per month, open forum, approximately 30 patients attend per month
- 3 neurologists, Social Worker, PT, OT, ST, RT, Dietary, Research Coordinator, RN, MA’s, Speech Communication Vendor, Durable Equipment Vendor, MDA and ALSA reps
- Follow approximately 220 patients with ALS, PLS and PMA
- Recruitment area; North and West Texas, Oklahoma and some parts of Louisiana, Arkansas and Mississippi
- Have participated in approximately 30 motor neuron disease trials (drug > observational), besides some basic science studies, since the early 1990’s
- Research department: 4 research coordinators
- Healey ALS Platform Trial Team: Daragh Heitzman, MD, Alan Martin, MD, Mohamad Nasri, MD/CCRC, Todd Morgan, CCRC
Perpetual Adaptive Trial
Randomization Ratio 3:1; Shared Placebo
Open Label Extension (OLE) offered

Screening

Regimen Assignment

(n=160 for each regimen)

Regimen A

3:1 Randomization within each Regimen

Regimen B

(n=120 for active; n=40 for placebo)

Regimen C

Regimen D

Zilucoplan

Verdiperstat

CNM-Au8

Pridopidine

Shared Placebo

Screening

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

as of 4/22/21
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 22, 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 22, 2021)

• 453 individuals with ALS signed informed consent
• 344 individuals were assigned to a regimen
• 304 are receiving investigational product (active or placebo)
• 70 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**

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 7)
Part 2: Regulatory Considerations  (Recorded April 9)
Part 3: Ethical Considerations  –Wednesday, May 5th 1-2pm EDT
Part 4: Site Considerations  –Friday, May 14th 3:30-4:30pm EDT

Register to attend and view recordings at https://bit.ly/3frQsfB
Send us webinar ideas!

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

Next Week’s Guest: Dr. Eduardo Locatelli of Holy Cross Hospital
Patient Navigator

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