HEALEY ALS Platform Trial

Weekly Q&A – April 15, 2021









Berry Consultants









Healey Center

Sean M. Healey & AMG Center for ALS at Mass General































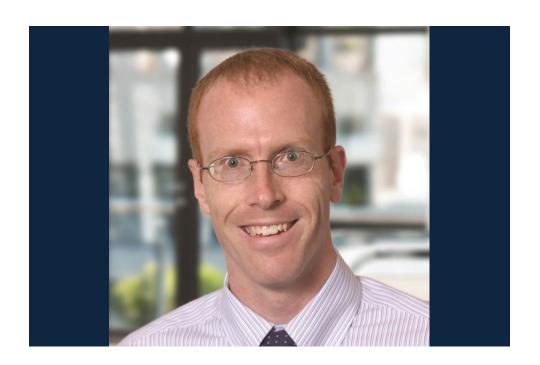






Guest Speaker

Adam Quick, MD
The Ohio State University Wexner Medical Center, Columbus OH
Platform Trial Site Investigator





Sarah Heintzman, CNP

Healey Platform Trial Site #762 Columbus Ohio Stephen Kolb, MD, PhD

Contact us via any of the following:

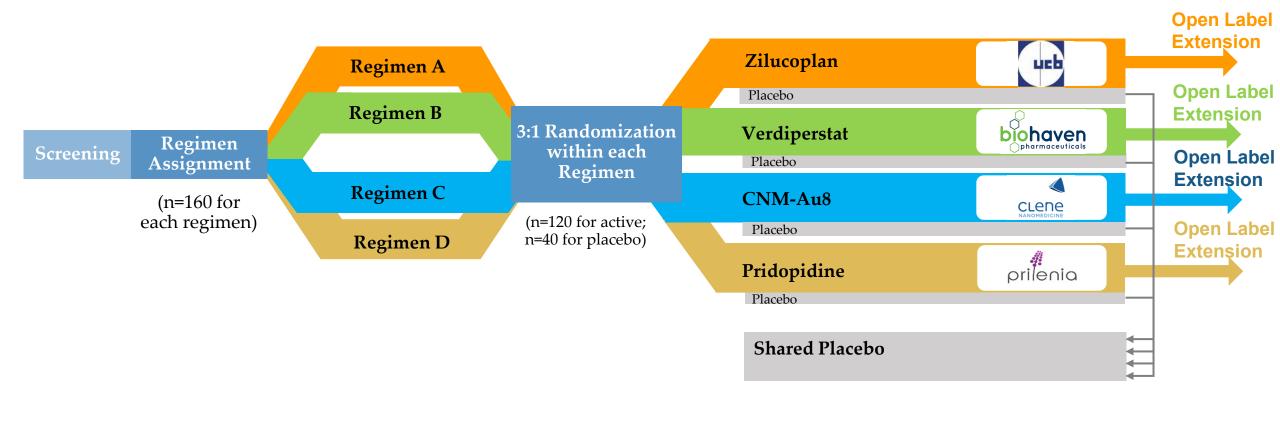
Email: Erin.Cohen@osumc.edu; Sarah.Heintzman@osumc.edu;

Adam.Quick@osumc.edu

Phone: 614-685-5661



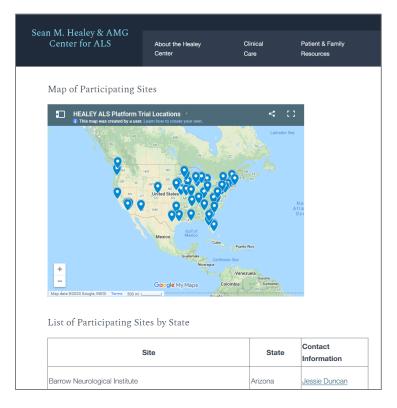
Perpetual Adaptive Trial Randomization Ratio 3:1; Shared Placebo Open Label Extension (OLE) offered



Screening

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

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
- **™** Holy Cross Hospital
- **™** Thomas Jefferson
- **™** Houston Methodist
- **☑** Henry Ford Health System
- **Mathematical Series** Barrow Neurological Institute
- **☑** Ohio State University
- **☑** Northwestern University
- **☑** University of Chicago
- **™** Wake Forest
- **☑ University of Nebraska**
- **☑** 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
- **☑** Cedars-Sinai
- **✓ University of Florida**
- ✓ University of South Florida
- **☑** Columbia University
- **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 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)



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

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Jan 21- Prilenia/Pridopidine (view recording- https://bit.ly/3ad3qel)

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

Feb 18- Biohaven/Verdiperstat (view recording- https://bit.ly/301qdDR)

Feb 25- UCB/Zilucoplan (view recording- https://bit.ly/3b3cpyS)
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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

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. Daragh Heitzman from Texas Neurology



Patient Navigator



Catherine Small Allison Bulat

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

https://bit.ly/3p4PrLL