

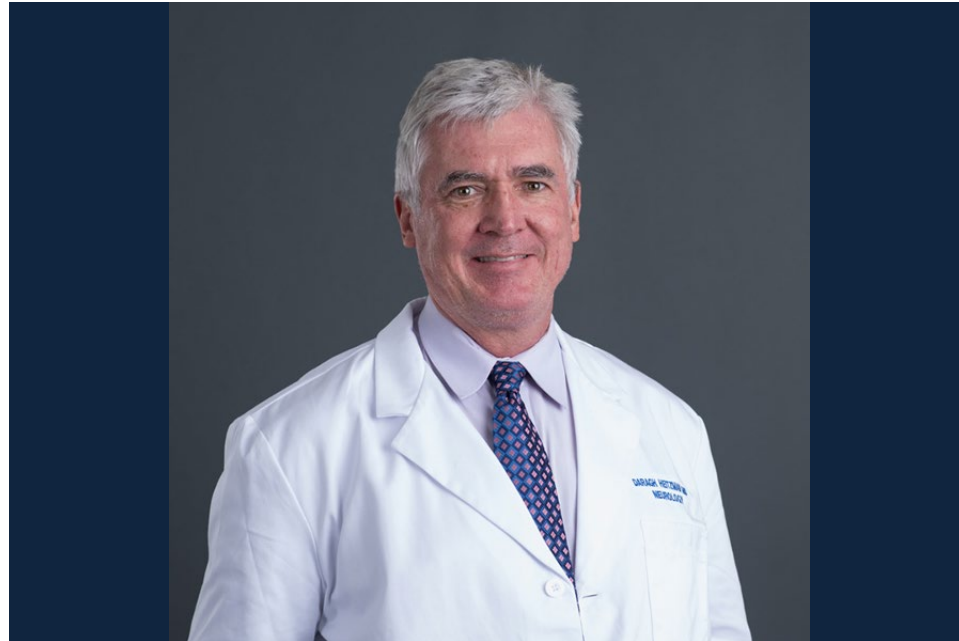
HEALEY ALS Platform Trial

Weekly Q&A – April 22, 2021



Guest Speaker

Daragh Heitzman, MD, FAAN
Texas Neurology, Dallas TX
Platform Trial Site Investigator

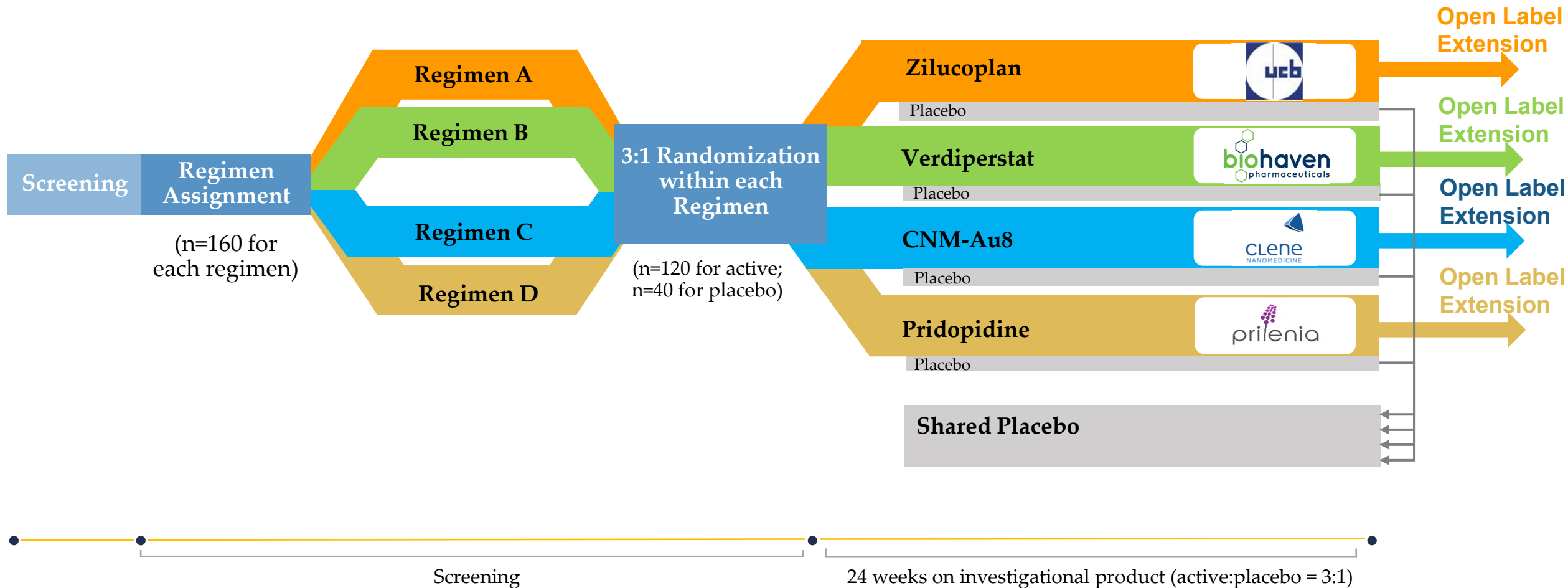


ALS Center at Texas Neurology in Dallas, Texas



- 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




How to Find a Center Near You

Sean M. Healey & AMG
Center for ALS

About the Healey Center Clinical Care Patient & Family Resources

Map of Participating Sites

HEALEY ALS Platform Trial Locations
This map was created by a user. Learn how to create your own.

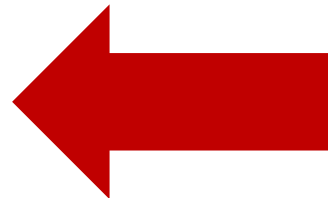


List of Participating Sites by State

Site	State	Contact Information
Barrow Neurological Institute	Arizona	Jessie Duncan

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

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>)



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

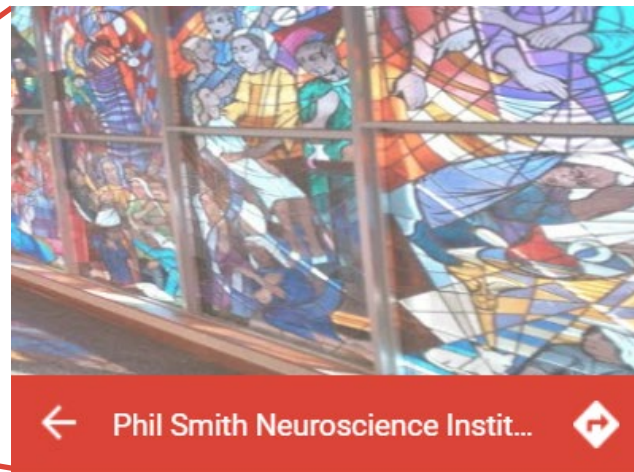
Register to attend and view recordings at <https://bit.ly/3frQsfB>


Send us webinar ideas!

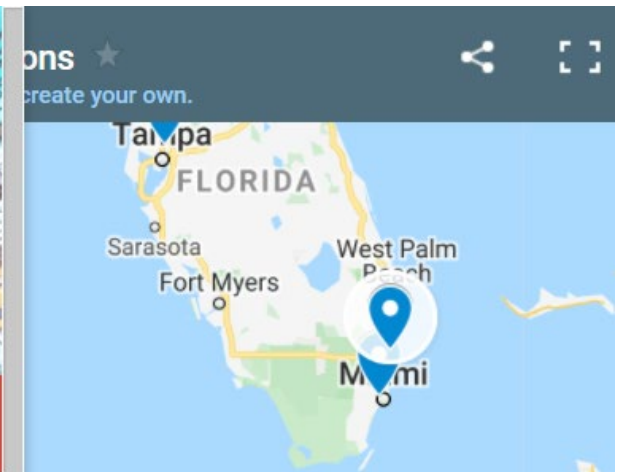
- Biomarkers
- Biostatistics / Trial Design
- Get to know our sites



Next Week's Guest: Dr. Eduardo Locatelli of Holy Cross Hospital



← Phil Smith Neuroscience Instit... 



Patient Navigator



Catherine Small

Allison Bulat

Phone: 833-425-8257 (HALT ALS)

E-mail: healeyalsplatform@mgh.harvard.edu



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

<https://bit.ly/3p4PrLL>