

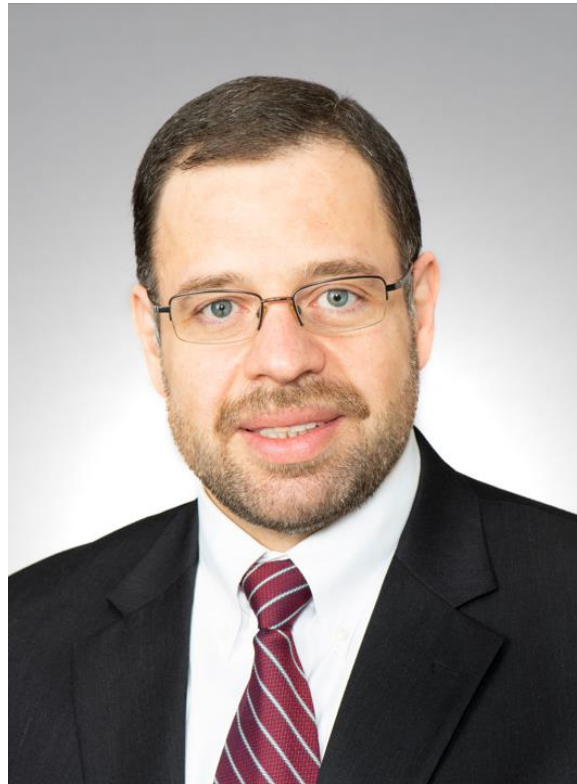
HEALEY ALS Platform Trial

Weekly Q&A – Sept 15, 2022



Guest Speaker

Tawfiq Al-Lahham, MD
University of Pittsburgh Medical Center, PA
Platform Trial Site Investigator





UPMC ALS Center

- 150-160 patients actively following in our center.
- Clinic supported by UPMC, ALS Association, MDA.



Our Team

- Speech Therapy/ Augmentative Communication:
Katya Hill, PhD, CCC-SLP
- Registered Dietician:
Jacquelyn Klunk, MS, RDN, LDN
- Physical Therapist:
Kathleen Brandfass, MS PT
- Social Worker:
Monica L. Schirm, MSW LSW
- Clinic Coordinator:
Michelle Hamilton, RN
- Physicians:
David Lacomis MD. and Tawfiq Al Lahham MD.
- Speech Therapy/ Swallow Evaluation and Management:
James L. Coyle, PhD, CCC-SLP, BCS-S
- Respiratory Therapist & Research Coordinator:
Melissa Ilnicki, RRT, RPSGT
- Occupational Therapist:
Alexandra Beschorner, OTR/L
- Clinical Psychologist:
Elizabeth Radomsky, PhD
- Neuromuscular Nurse Practitioner:
Rebecca Molczan, MSN, CRNP

ALS Research

- NIH
 - Identification of TDP-43 modifiers through single-cell transcriptional and epigenomic dissection of ALS and FTLD-MND.
Christopher Donnelly Ph.D., LiveLikeLou Center for ALS
Broad/MIT, Mayo, Pitt/UPMC.
 - SWITCH Trial: Early feasibility study of BCI for augmentative communication. Stentrode™ feasibility & Safety in pts with neurologic disorders and severe weakness (ALS or Other)
CMU, Pitt, Mt. Sinai, Synchron.



ALS Research

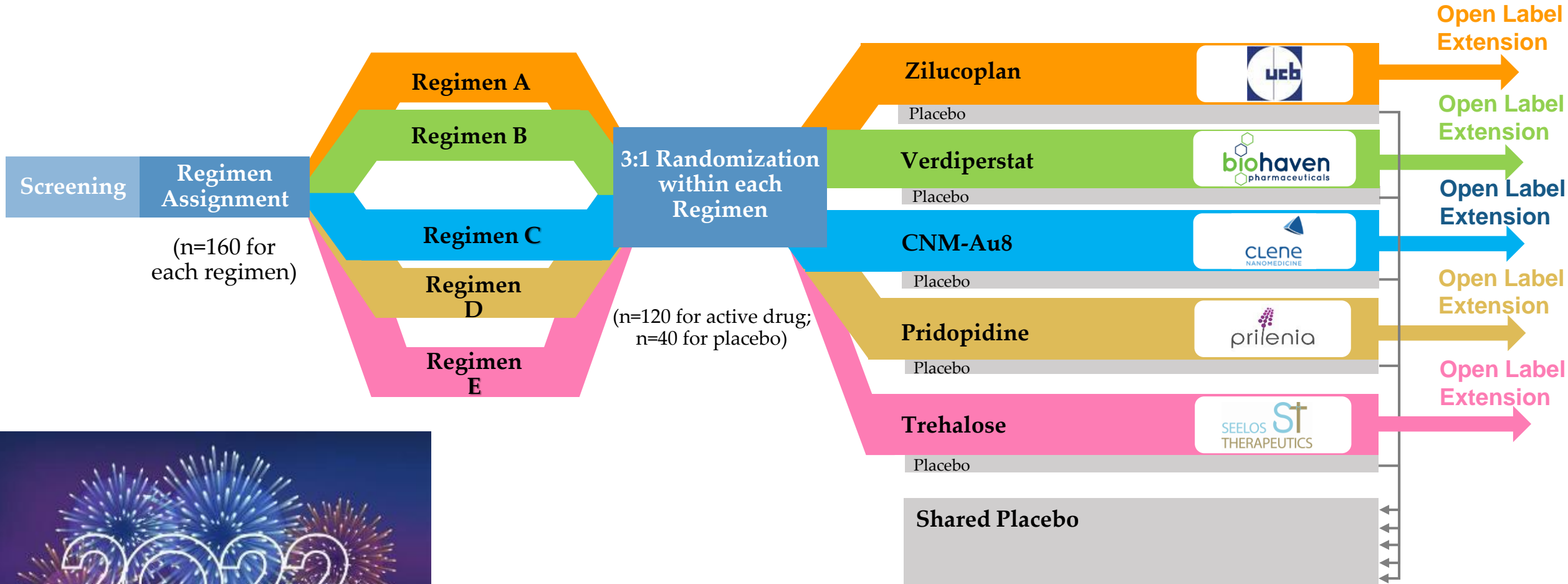
- Clinical trials
 - HEALEY ALS Platform Trial, Regimen E:
 - UPMC site activated in July 2022
 - 3 patients enrolled
 - 5 patients in screening process.
 - Contact: Melissa Innicki,
ilnickima@upmc.edu, 412-864-0865
 - Oral Edaravone
- Unfunded
 - High-definition Fiber Tractography (HDFT): A novel imaging modality to detect and quantify neuronal injury, UMN biomarker.



Thank you



The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial



Enrollment Updates (as of Sept 15, 2022)

- **138** individuals have signed informed consent
- **95** individuals have been randomized within Regimen E

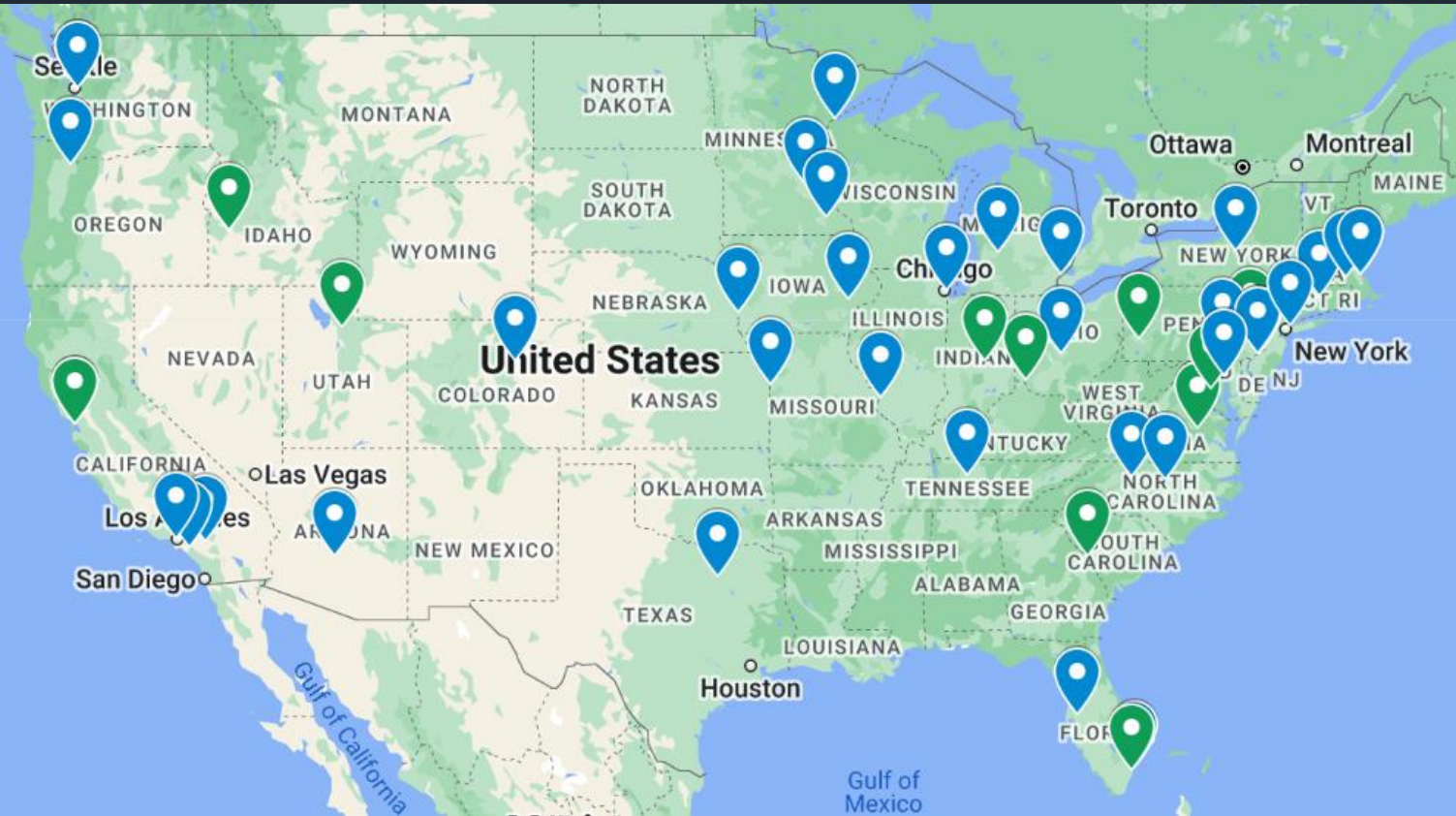
Thank You

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

49 Sites Currently Activated for Regimen E



(as of 9/15/22)

Sites in blue participated in previous regimens. Sites in green (underlined to the side) are new additions to the Platform Trial!

- Lehigh Valley Health Network
- Mass General Hospital
- University of Kansas
- University of Maryland
- California Pacific Medical Center
- Northwestern University
- Virginia Commonwealth University
- University of Nebraska
- Washington University
- Wake Forest University
- Hospital for Special Care
- Saint Alphonsus Regional
- University of Massachusetts
- Duke University
- Barrow Neurological Institute
- Georgetown University
- Texas Neurology
- Beth Israel Deaconess Medical Center
- SUNY Upstate
- Spectrum Health
- Henry Ford Hospital
- Essentia Health
- University of Southern California
- University of South Florida
- University of Colorado
- Providence Brain and Spine
- University of Minnesota
- Loma Linda University
- University of Iowa
- Swedish Medical Center
- Ohio State University
- University of Cincinnati
- Thomas Jefferson University
- UC San Francisco
- Mayo Rochester
- University of Washington
- Vanderbilt University
- UPMC
- Indiana University
- Augusta University
- University of Utah
- Holy Cross Hospital
- Penn State Hershey
- University of CA, Irvine
- Cedars Sinai Medical Center
- University of Pennsylvania
- Nova Southeastern University
- Johns Hopkins University**
- Columbia University**

Site Map & Contacts:



<https://bit.ly/3g2NZr5>

Patient Navigation

Central resource for people living with ALS



Catherine Small

Phone: 833-425-8257 (HALT ALS)

E-mail: healeyalsplatform@mgh.harvard.edu

Weekly webinar
registration:



<https://bit.ly/3r6Nd2L>

ALS Link sign-up:



<https://bit.ly/3o2Ds3m>

Upcoming Guest Speakers:

September 22nd- Mariah Connolly and Meghan Hall (Site Monitors for the Platform Trial)

September 29th- Michael Elliott, MD, FAAN (Site Investigator at Swedish Medical in WA)

October 6th- Catherine Douthwright, PhD, CCRP (University of Massachusetts, Worcester)



Allison Bulat



The ALS Association/Northeast ALS Consortium Educational Webinar

Update on Healey ALS Platform Trial Regimen E: Trehalose for ALS

**UPDATE ON
REGIMEN E**

TREHALOSE FOR ALS



Merit Cudkowicz, MD, MSc
SEAN M. HEALEY & AMG
CENTER FOR ALS AT MGH



Shafeeq Ladha, MD
BARROW NEUROLOGICAL
INSTITUTE

TUESDAY | 4 OCTOBER
3 PM EDT

Register Below:



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

*Recording will later be available under
“educational webinars” on neals.org*