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

UPMC ALS Center
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 Ilnicki,
    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

- **Screening**
  - Regimen A (n=160 for each regimen)
  - Regimen B
  - Regimen C
  - Regimen D
  - Regimen E

- **Regimen Assignment**

- **3:1 Randomization within each Regimen**
  - Regimen A
  - Regimen B
  - Regimen C
  - Regimen D
  - Regimen E

- **Zilucoplan**
  - Placebo

- **Verdiperstat**
  - Placebo

- **CNM-Au8**
  - Placebo

- **Pridopidine**
  - Placebo

- **Trehalose**
  - Placebo

- **Shared Placebo**
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

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

(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!

https://bit.ly/3g2NZr5
Patient Navigation
Central resource for people living with ALS

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)
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 & AMC CENTER FOR ALS AT MGH

Shafeeq Ladha, MD
BARROW NEUROLOGICAL INSTITUTE

TUESDAY 4 OCTOBER 3 PM EDT

Register Below:

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