Guest Speaker

Hani A. Kushlaf, MD, FAAN, FANA, FAANEM
Platform Trial Site Investigator
University of Cincinnati, OH
Hani Kushlaf, MD, FAAN

UC Health ALS Center and Research

8/25/2022
ALS Center Team

- Clinicians: Robert Neel, Jacqueline Janecek
- Physical therapist: Abbie Homan
- Occupational therapist: Connie Budda
- Speech therapist: Courtney Anness
- Social worker: Yvonne Dressman
- Nurse Coordinator: Kaitlin Dixon
- Respiratory therapist: Ann Compton

- We follow, on average, 170-180 ALS patients
Our research team:

- Site investigator: Hani Kushlaf
- Subinvestigator: Jacki Janecek
- Senior research coordinator: Ashley Smith
- Research coordinator: Elizabeth Gehlmann

- Healey ALS Platform Trials, Regimen E
  - 8 patients in screening/screened for master protocol
  - 4 patients enrolled in regimen E

- Two retrospective studies:
  - The Prevalence of genetic variants causing ALS in our UC ALS cohort
  - The Correlation of pulmonary function with prognosis in our UC ALS cohort
The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial

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

- **3:1 Randomization within each Regimen**
  - Zilucoplan
    - Placebo
  - Verdiperstat
    - Placebo
  - CNM-Au8
    - Placebo
  - Pridopidine
    - Placebo
  - Trehalose
    - Placebo

- **Shared Placebo**

- **Open Label Extension**
  - Regimen A
  - Regimen B
  - Regimen C
  - Regimen D
  - Regimen E
Enrollment Updates (as of Aug 25, 2022)

• 122 individuals have signed informed consent

• 81 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.
47 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
- Providence Brain and Spine
- 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

(as of 8/25/22)

Sites in blue participated in previous regimens. Sites in green (underlined to the side) are new additions to the Platform Trial!
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 1st- Hosted by James Berry, MD, MPH
September 15th- Tawfiq Al-Lahham, MD (Site Investigator at UPMC in PA)
September 22nd- Mariah Connolly and Meghan Hall (Site Monitors for the Platform Trial)