HEALEY ALS Platform Trial

Weekly Q&A – Sept 1, 2022

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

Northeast Amyotrophic Lateral Sclerotic Consortium

Harvard Medical School
Massachusetts General Hospital
NEALS
Neurological Clinical Research Institute
Barrow Neurological Institute
The Arthur M. Blank Family Foundation
Biohaven Pharmaceuticals
Clene Nanomedicine
Prelenia
SeeLos Therapeutics
Tambourine
AMG
ALS ONE
ALS Association
MIDA
Muscular Dystrophy Association
AADC
FINDING A CURE
JAM ALS
ALS Association
R2R
RUN2REVIVE
Baseball Winter Meetings
Berry Consultants
Seated Inspiration
AMG The AMG Foundation
James Berry, MD, MPH
Regimen co-Lead (Regimen C)
Massachusetts General Hospital
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

(n=120 for active drug; n=40 for placebo)

Regimen Assignment

Regimen A

Regimen B

Regimen C

Regimen D

Regimen E

3:1 Randomization within each Regimen

(n=120 for active drug; n=40 for placebo)

Zilucoplan

Verdiperstat

CNM-Au8

Pridopidine

Trehalose

Shared Placebo

Open Label Extension

Open Label Extension

Open Label Extension

Open Label Extension

Open Label Extension
ENDPOINTS

Primary Endpoint
Change in disease severity - **ALS Functional Rating Scale-Revised (ALSFRS-R)**

Secondary Endpoints
1. Change in respiratory function - slow vital capacity (SVC)
2. Change in muscle strength - hand held dynamometry (HHD)
3. Survival
4. Treatment-specific biomarkers as applicable

Exploratory Endpoints

Safety Endpoints
Exploratory Endpoints

Endpoint Development Engine

- DNA
- Neurofilaments
- Biomarkers (Blood, Urine, CSF)
- Speech Analysis
- Home Spirometry
Enrollment Updates (as of Sept 1, 2022)

• 131 individuals have signed informed consent

• 88 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

(as of 9/1/22)

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

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

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

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 8th - HEALEY ALS Platform Trial Update
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)