HEALEY ALS Platform Trial

Weekly Q&A – Dec 15, 2022

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

Northeast Amyotrophic Lateral Sclerosis Consortium

ALS Association
MUSCULAR DYSTROPHY ASSOCIATION
ALSOne
ALS FINDING A CURE
TAMBOURINE
THE ARTHUR M. BLANK FAMILY FOUNDATION
AMG
RUN2REVIVE
THE AMG FOUNDATION
The goal of the HEALEY ALS Platform Trial is to screen drugs rapidly and efficiently, get solid answers, and determine next steps.
Informational Webinars about Regimen E

Trehalose/SLS-005 Drug Science and Mechanism of Action Q&A Webinar
Hosted by Seelos Therapeutics on 10 March 2022

Recording Available!

Recording available under “science and mechanism of action series”

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

Recording Available!

https://bit.ly/3X2u004
Recording available under “educational webinars” on neals.org
60 Sites Currently Activated for Regimen E

(as of 12/15/22)

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
Stony Brook University
Kaiser, Los Angeles
Cleveland Clinic
Medical College of Wisconsin
University of Michigan
Las Vegas Clinic
George Washington University
Mayo Clinic Florida
University of Kentucky
Houston Methodist
Hackensack University

https://bit.ly/3g2NZr5
Enrollment Updates (as of Dec 15, 2022)

• 185 individuals have signed informed consent

• 132 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.
Regimen A
Regimen B
Regimen C
Regimen D
Regimen E
Regimen F
Regimen G
ABBV-CLS-7262 targets eIF2B, a key regulator of the integrated stress response (ISR). In neurons exposed to cellular stressors, inhibition of the ISR by ABBV-CLS-7262 restores protein synthesis and dissolves pre-formed TDP-43 containing stress granules. This effect of ABBV-CLS-7262 is of clinical interest because TDP-43 containing stress granules are thought to lead to TDP-43 inclusions, a hallmark of ALS pathology.
Regimen G

DNL343 is being developed by Denali Therapeutics. It targets eIF2B, a key regulator of the integrated stress response, to restore protein synthesis and dissolve pre-formed TDP-43 containing stress granules which are thought to lead to TDP-43 inclusions, a hallmark of ALS pathology.

"By adding one more drug to the platform, we continue to push research forward in hopes of soon finding many more effective treatments for ALS."

Merit Cudkowicz, MD, MSc
Director, Sean M. Healey & AMG Center for ALS, Massachusetts General Hospital
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/3o2Ds3m
ALS Link sign-up: https://bit.ly/3r6Nd2L

Upcoming Webinars:
December 22nd - Canceled for holidays
December 29th - Canceled for holidays
See you in the New Year!
New Expanded Access Video

Sean M. Healey & AMG Center for ALS Expanded Access Program, with TechVsALS

How can we grow the EAP?

FUNDING ➔ INFRASTRUCTURE ➔ ACCESS

leads to ➔ leads to ➔

➢ Sean M. Healey & AMG Center for ALS Expanded Access Program, with TechVsALS

Watch Video:

• Direct YouTube Link: https://www.youtube.com/watch?v=25-EeCwpEnU
• Mass General’s EAP Webpage: https://www.massgeneral.org/neurology/als/research/expanded-access

https://bit.ly/3HCwGw3