HEALEY ALS Platform Trial

Weekly Q&A – Sept 22, 2022
Guest Speakers

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Clinical Study Monitoring

- What is Monitoring and Why is it Important to have in Clinical Trials?
- Monitoring for HEALEY ALS Platform Trial

Meghan Hall and Mariah Connolly
Barrow Neurological Institute
What is Monitoring?

“The act of overseeing the progress of a clinical trial, and of ensuring that it is **conducted**, **recorded**, and **reported** in accordance with the protocol, standard operating procedures (SOPs), Good Clinical Practices (GCP), and the applicable regulatory requirements.”

*ICH Guidance for Industry: E6 GCP Consolidated Guidance Sec 1.38*
Monitoring Activities

Role of the Clinical Monitor

• Communication with the Site Investigator and Study Site Staff

• Review of the Study Site’s Processes, Procedures, and Records

• Verification of the Accuracy of Data

• Site Management

• Eyes and Ears of the Study!

Goals of Monitoring

• Verify that the rights and well-being of human subjects are protected.

• Verify the reported trial data are accurate, complete and verifiable from source documents.

• Verify that the conduct of the trial is in compliance with the currently approved protocol amendment(s), with GCP, and applicable regulatory requirements
Regulatory Expectations

FDA Guidance: **ALCOA-C**

“To be acceptable the data (from clinical trials) should meet certain fundamental elements of quality whether collected or recorded electronically or on paper. Data should be **Attributable**, **Legible**, **Contemporaneous**, **Original**, and **Accurate- Complete**.”
Source Data Verification (SDV)

• A process by which data within the Case Report Form (CRF) or other data collection systems are compared to the original source of information
• Data should be verifiable and reproducible
Monitoring also helps to identify unforeseen risks and prevent data quality issues.
Monitoring also helps to identify any research misconduct!

• Falsification and fabrication = FRAUD!

• FDA is focused on identifying Research Misconduct:
  • Research misconduct means **Falsification** of **data** in proposing, designing, performing, recording, supervising or reviewing research, or in reporting research results.
    • Falsification includes acts of omission and commission.

• Acts of omission
  • consciously not revealing all data
    (e.g. reportable adverse events, concomitant meds., etc)

• Acts of commission
  • consciously altering data or **Fabricating** data (e.g. lab values, BP readings, bogus specimens)

• Data is interpreted broadly
  • individual facts, statistics, tissue samples, items of information, statements made by individuals
Monitoring for HEALEY ALS Platform Trial

• CRAs located all across the US and visit clinical sites approx. every two months to monitor study data!
• Create monitoring reports and work with the sites to address any identified issues.
• Work very closely with sites and Project Managers to provide day to day operations
Thank you!
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
Enrollment Updates (as of Sept 22, 2022)

• 137 individuals have signed informed consent

• 97 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
50 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
- University of Texas
- 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

(as of 9/22/22)

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:

Upcoming Guest Speakers:

September 29th- Michael Elliott, MD, FAAN (Site Investigator at Swedish Medical in WA)
October 6th- Catherine Douthwright, PhD, CCRP (University of Massachusetts, Worcester)

ALS Link sign-up:
https://bit.ly/3o2Ds3m
Register Below:

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