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

Barrow Neurological Institute | Dignity Health. St. Joseph's Hospital and Medical Center

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

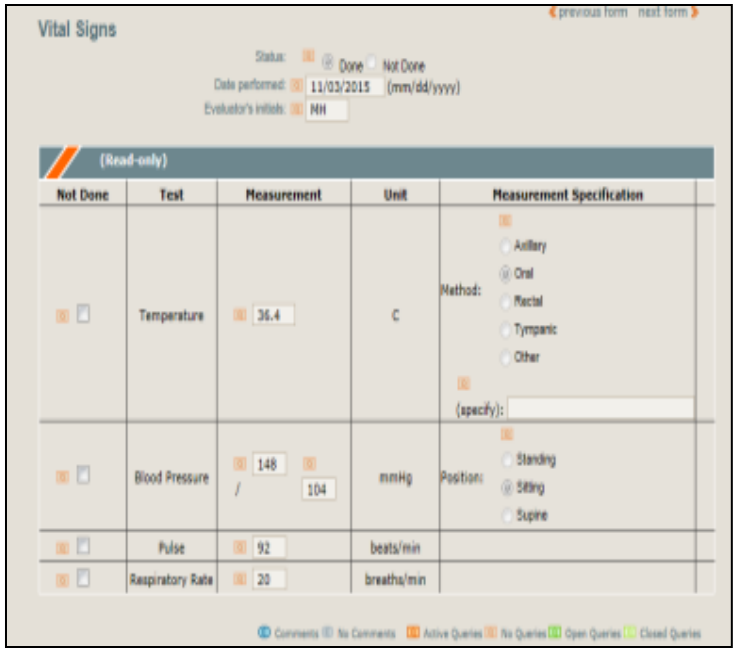
Regulatory Expectations

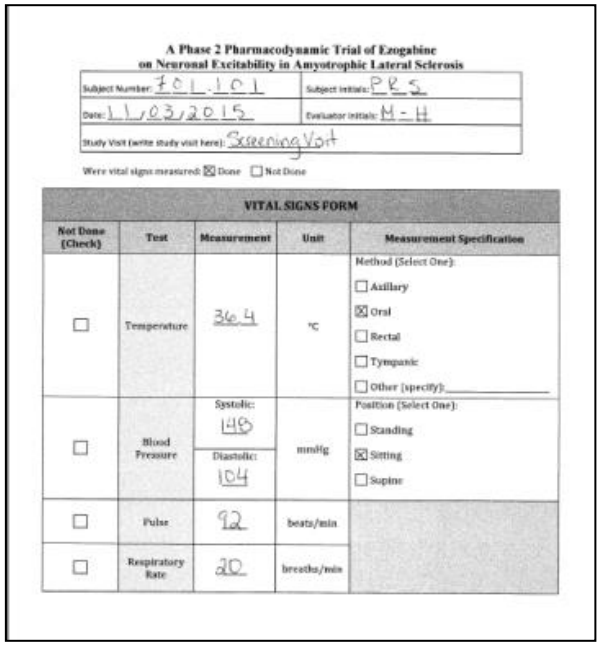
FDA Guidance: **ALCOA-C**

“ 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 **A**ttributable, **L**egible, **C**ontemporaneous, **O**riginal, and **A**ccurate- **C**omplete”.

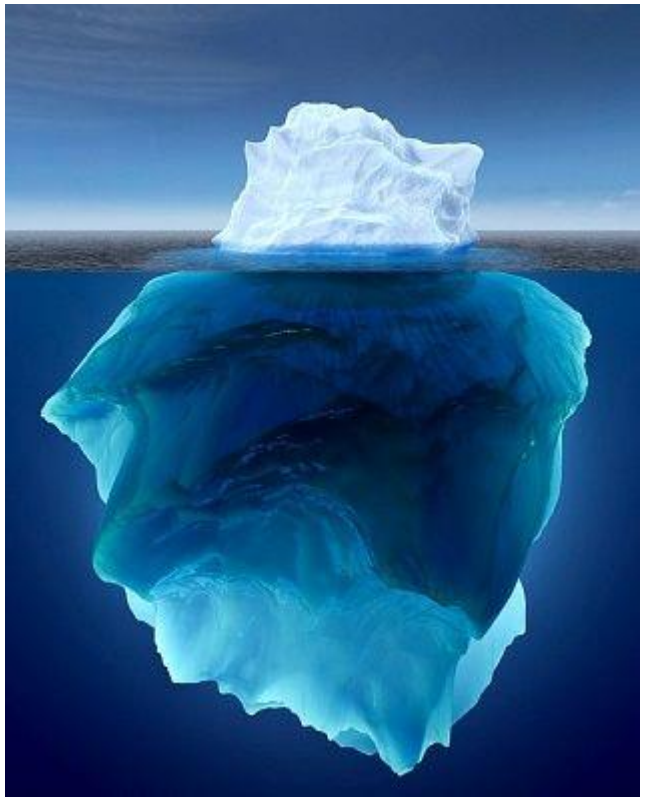
Source Data Verification (SDV)

- 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

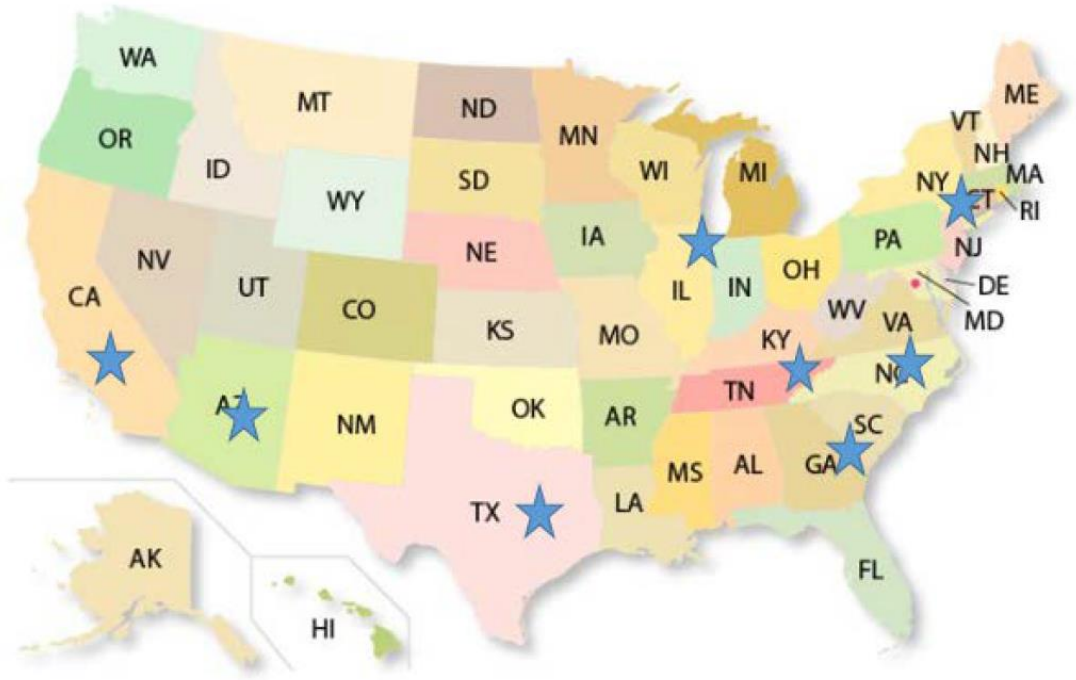


Monitoring also helps to identify any research misconduct!

- **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 **F**abricating 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!

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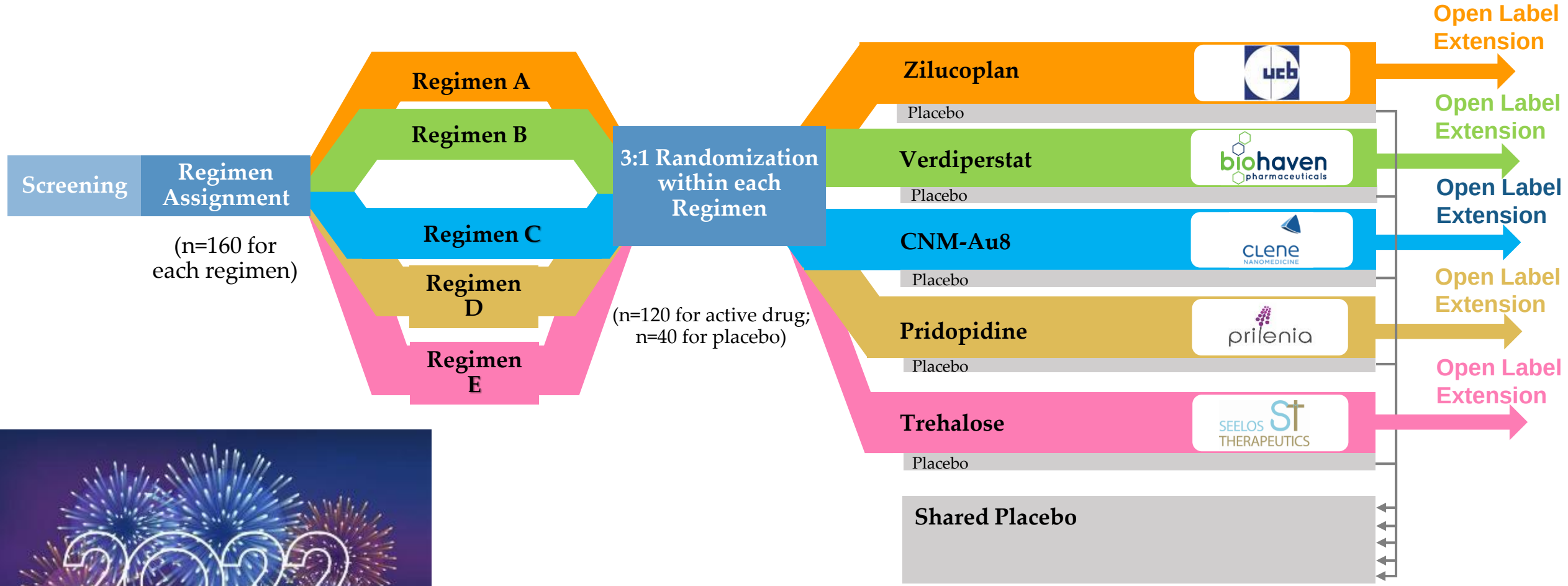
The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial



Enrollment Updates (as of Sept 22, 2022)

- **137 individuals have signed informed consent**

- **97 individuals have been randomized within Regimen E**

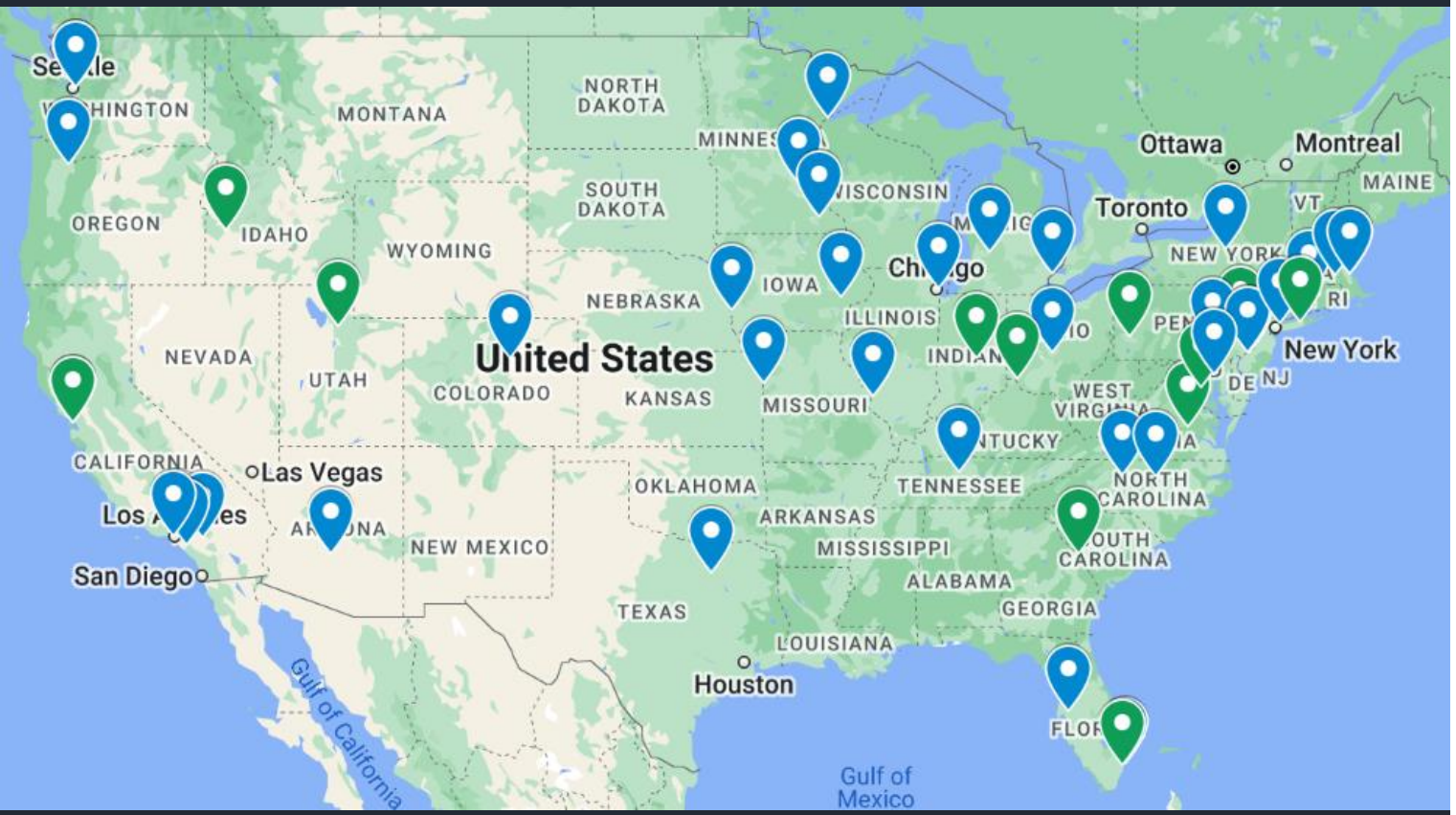
Thank You

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



(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!

- ☑ 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

Site Map & Contacts:

https://bit.ly/3g2NZr5

Patient Navigation
Central resource for people living with ALS

Catherine Small

Allison Bulat

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Weekly webinar registration:

https://bit.ly/3r6Nd2L

ALS Link sign-up:

https://bit.ly/3o2Ds3m

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)

The ALS Association/Northeast ALS Consortium Educational Webinar

Update on Healey ALS Platform Trial Regimen E: Trehalose for ALS

Register Below:

https://bit.ly/3dhQvff

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