

HEALEY ALS Platform Trial

Weekly Q&A – Sept 22, 2022



Healey Center

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



The AMG Foundation



Guest Speakers

Mariah Connolly BS, CCRA
Senior Clinical Research Associate
Barrow Neurological Institute



Meghan Hall BS, CCRA, CCRP
Manager-Research Operations
Barrow Neurological Institute



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

Vital Signs

Status: Done Not Done
Date performed: 11/03/2015 (mm/dd/yyyy)
Evaluator's initials: NH

(Read-only)

Not Done	Test	Measurement	Unit	Measurement Specification
<input checked="" type="checkbox"/>	Temperature	36.4	C	Method: <input type="radio"/> Axillary <input checked="" type="radio"/> Oral <input type="radio"/> Rectal <input type="radio"/> Tympanic <input type="radio"/> Other (specify):
<input checked="" type="checkbox"/>	Blood Pressure	148 / 104	mmHg	Position: <input type="radio"/> Standing <input checked="" type="radio"/> Sitting <input type="radio"/> Supine
<input checked="" type="checkbox"/>	Pulse	92	beats/min	
<input checked="" type="checkbox"/>	Respiratory Rate	20	breaths/min	

Comments: No Comments | Active Queries: No Queries | Open Queries: Closed Queries

A Phase 2 Pharmacodynamic Trial of Ezogabine on Neuronal Excitability in Amyotrophic Lateral Sclerosis

Subject Number: FOL101 | Subject Initials: P.R.S.
Date: 11/03/2015 | Evaluator Initials: M-H
Study Visit (write study visit here): Screening Visit

Were vital signs measured? Done Not Done

VITAL SIGNS FORM

Not Done (Check)	Test	Measurement	Unit	Measurement Specification
<input type="checkbox"/>	Temperature	36.4	°C	Method (Select One): <input type="checkbox"/> Axillary <input checked="" type="checkbox"/> Oral <input type="checkbox"/> Rectal <input type="checkbox"/> Tympanic <input type="checkbox"/> Other (specify):
<input type="checkbox"/>	Blood Pressure	Systolic: 148 Diastolic: 104	mmHg	Position (Select One): <input type="checkbox"/> Standing <input checked="" type="checkbox"/> Sitting <input type="checkbox"/> Supine
<input type="checkbox"/>	Pulse	92	beats/min	
<input type="checkbox"/>	Respiratory Rate	20	breaths/min	

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

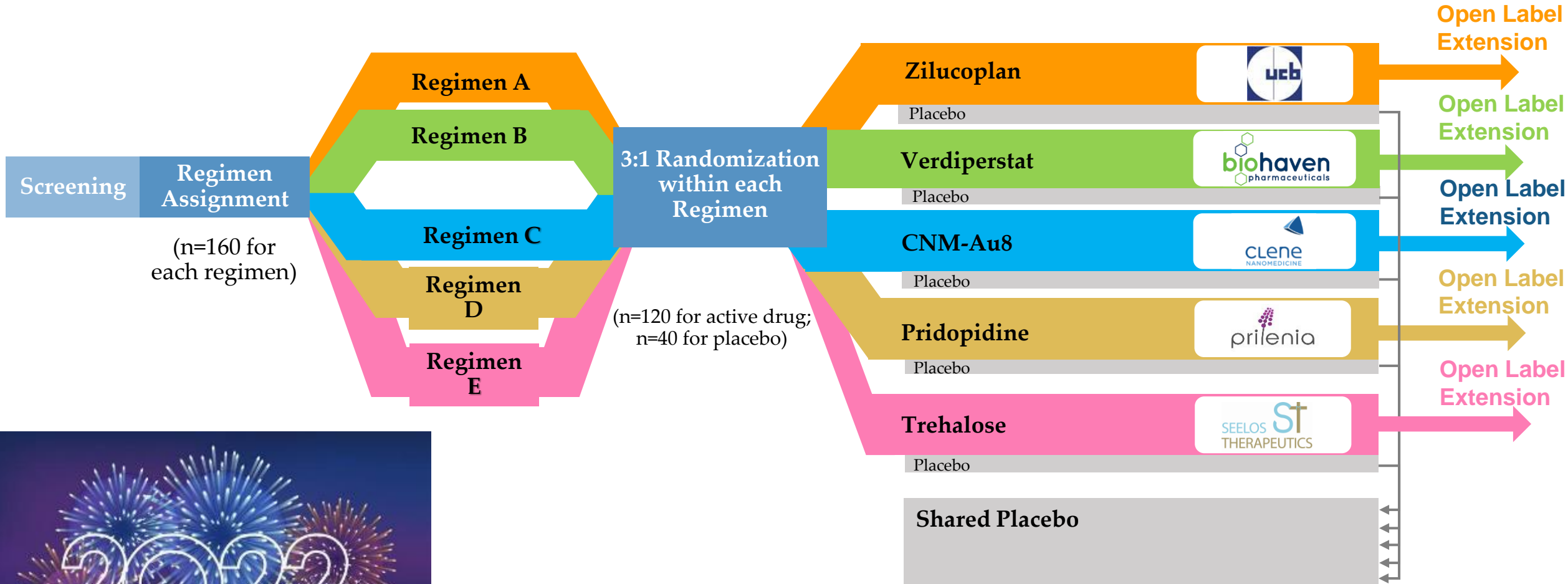
- CRAs located all across the US and visit clinical sites approx. every two months to monitor study data!
- Create monitoring reports and work with the sites to address any identified issues.
- Work very closely with sites and Project Managers to provide day to day operations



Thank you!



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

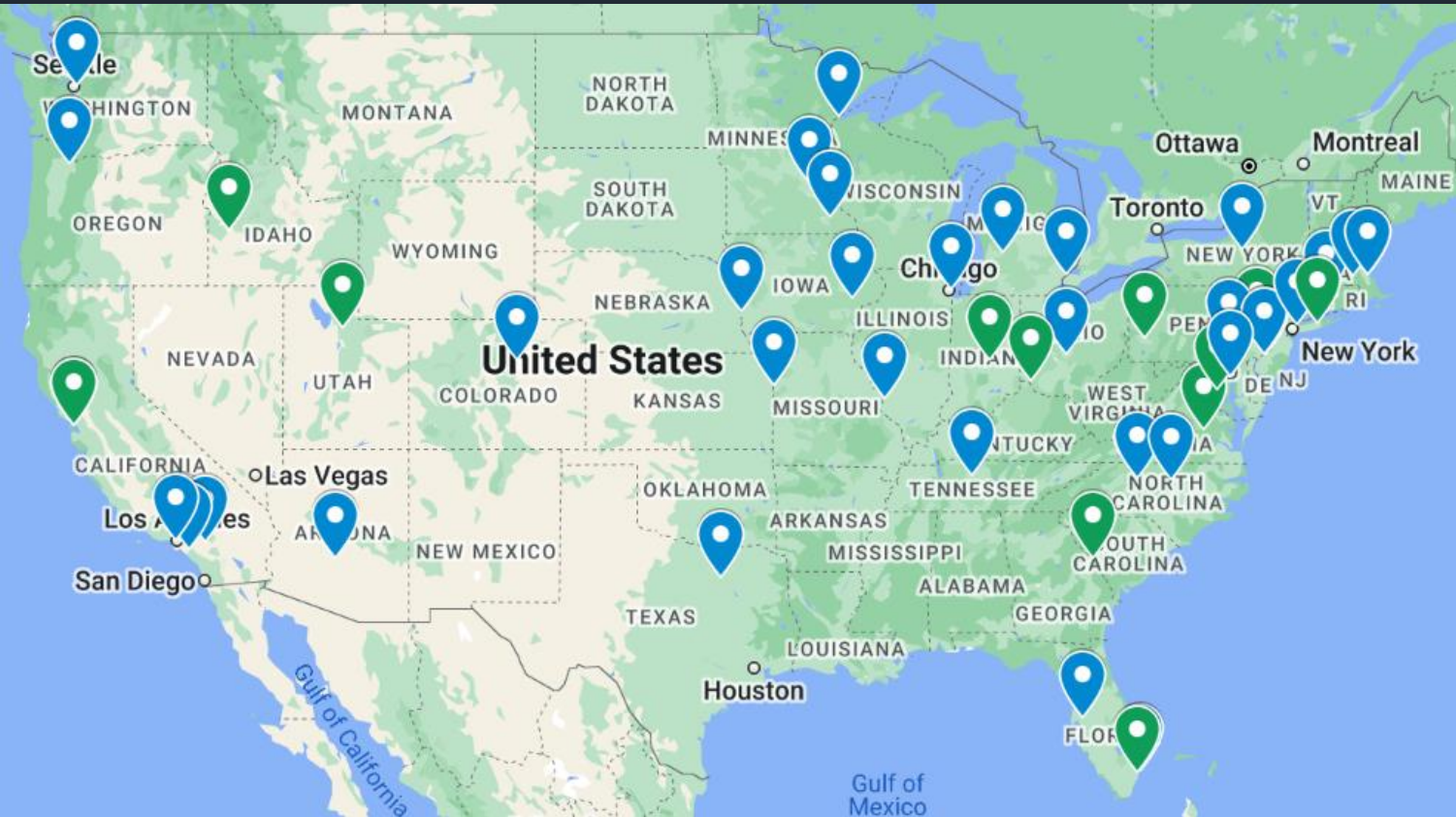
Thank You

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

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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)



Allison Bulat



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



Merit Cudkowicz, MD, MSc
SEAN M. HEALEY & AMG
CENTER FOR ALS AT MGH



Shafeeq Ladha, MD
BARROW NEUROLOGICAL
INSTITUTE

TUESDAY | 4 OCTOBER
3 PM EDT

Register Below:



<https://bit.ly/3dhQvff>

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