

HEALEY ALS Platform Trial

Weekly Q&A – April 14, 2022



Healey Center

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



The AMG Foundation



Guest Speaker

Kuldip Dave, PhD
Vice President of Research
The ALS Association



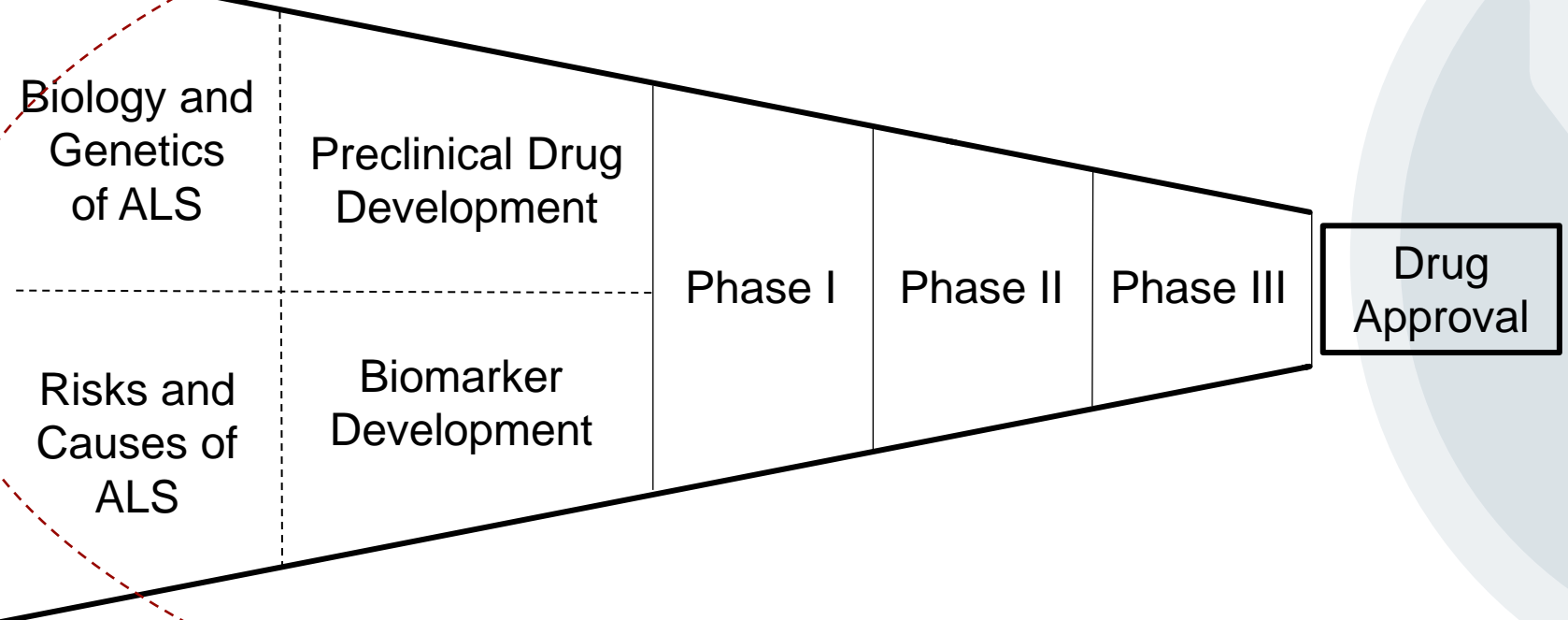


Kuldip Dave, Ph.D.

April 2022

ALS Research Ecosystem

Drug Development



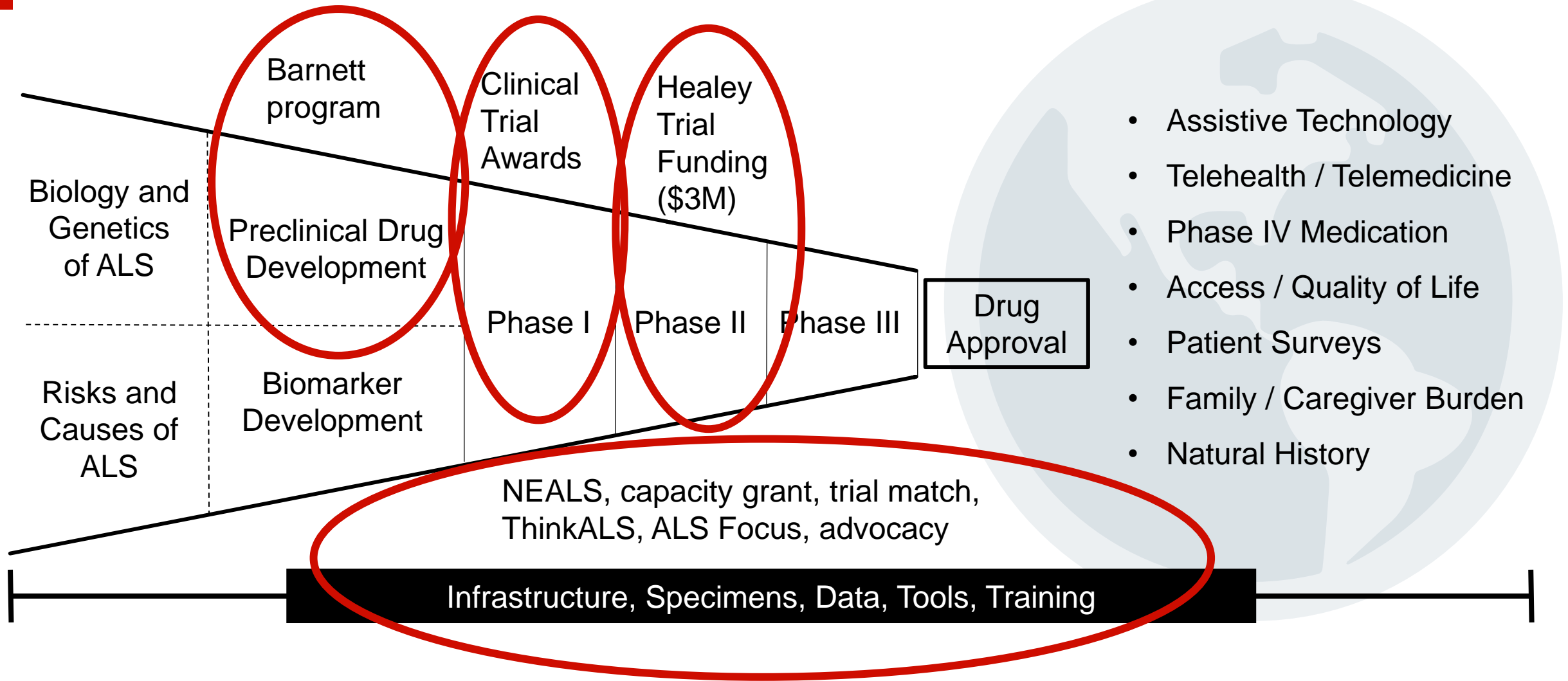
Managing ALS

- Assistive Technology
- Telehealth / Telemedicine
- Phase IV Medication
- Access / Quality of Life
- Patient Surveys
- Family / Caregiver Burden
- Natural History

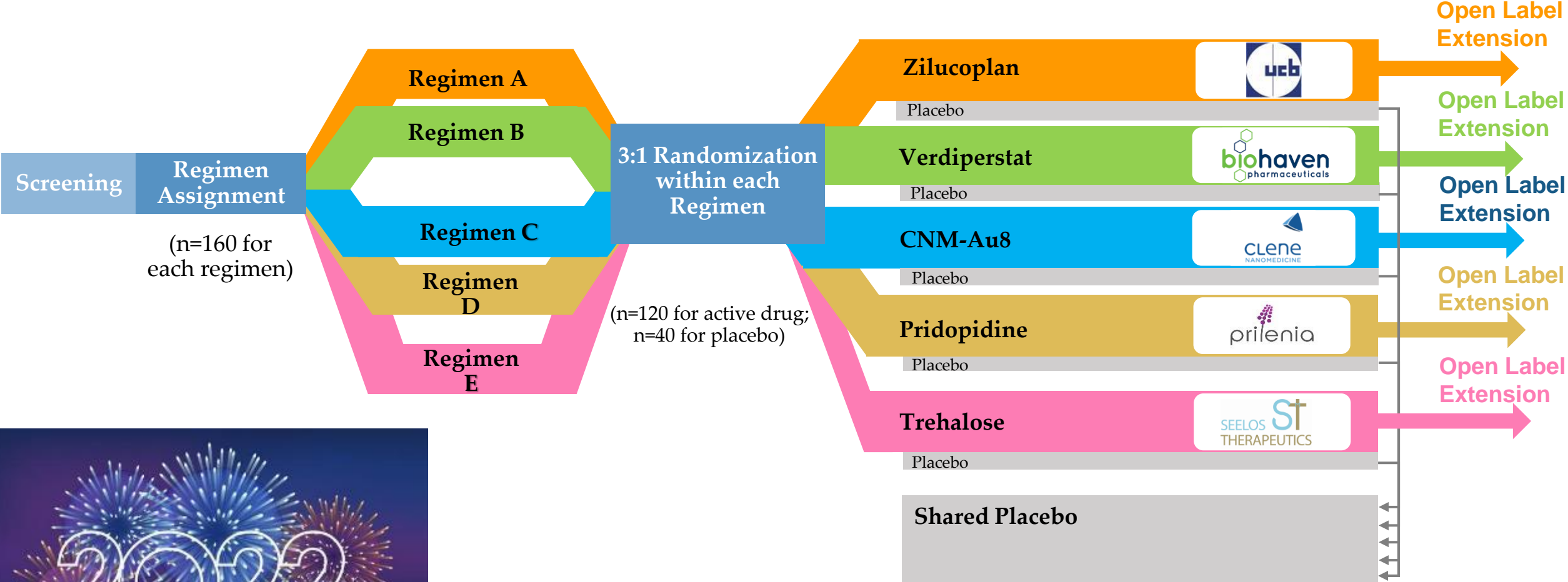
Infrastructure, Specimens, Data, Tools, Training

149 active projects, across 10 countries, \$53M commitment

Holistic Strategy to Increase # of Clinical Trials in ALS

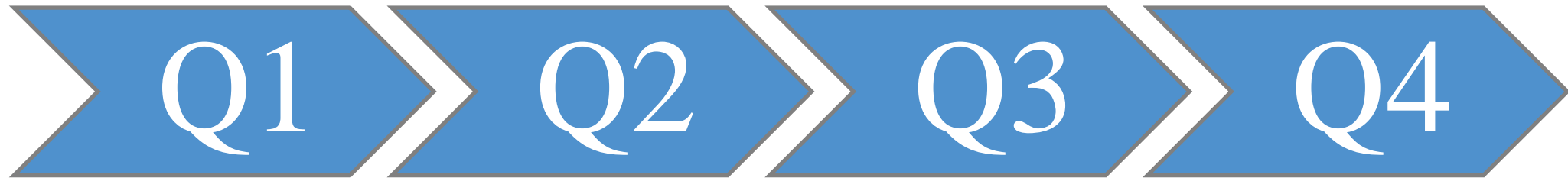


The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial



Study Results Timelines – Regimens A-D

***** Note: COVID-19 might impact these estimates**



LPLV

(Last Participant Last Visit in the Randomized,
Placebo Controlled Period)

Database Lock

Top Line Results

Study Reports

Expected LPLV: March-July 2022 (depending on the regimen and OLE choice)

Expected Database Lock: May-Sept 2022

Top Line Results: July-Sept 2022

Study Reports: Oct-Dec 2022

Enrollment Updates (as of April 14, 2022)

- **32** individuals have signed informed consent
- **23** 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

24 Sites Currently Activated for Regimen E



(as of 4/14/22)

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 Alphonus 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

Regimen E Site Activation

Sites closest to activation:

- University of Colorado
- Providence Brain and Spine
- Mayo Rochester
- University of Cincinnati
- Swedish Medical Center
- Thomas Jefferson
- Loma Linda University
- Ohio State University

Checking Site Status Online

List of Participating Sites

Sites marked "Recruiting" are currently enrolling participants in regimen E.

Sites marked "Active, Not recruiting" are active in the platform (for example, they are following participants in regimens A-D) but are not enrolling new participants at this time. Most active sites are expected to start enrolling in regimen E soon.

Site	State	Enrollment Status	Contact Information
Jefferson Weinberg ALS Center, Thomas Jefferson University	PA	Active, Not recruiting	Stephanie Hansen
Lehigh Valley Health Network	PA	Recruiting	Andrew Orzel (610-402-9543)
Penn State Hershey	PA	Active, Not recruiting	Heidi Runk



<https://bit.ly/3g2NZr5>

Patient Navigation

Central resource for people living with ALS



Catherine Small

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>



Allison Bulat

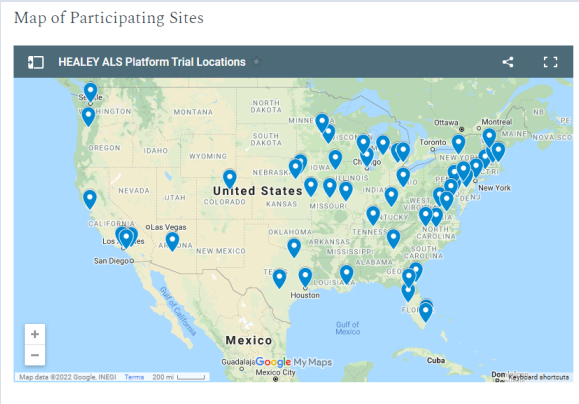
Upcoming Guest Speakers:

April 28th- Kelly Gwathmey, MD (Site PI at Virginia Commonwealth University)

May 5th- Peter Foss (President, ALS Finding a Cure)

Send us webinar questions and ideas!

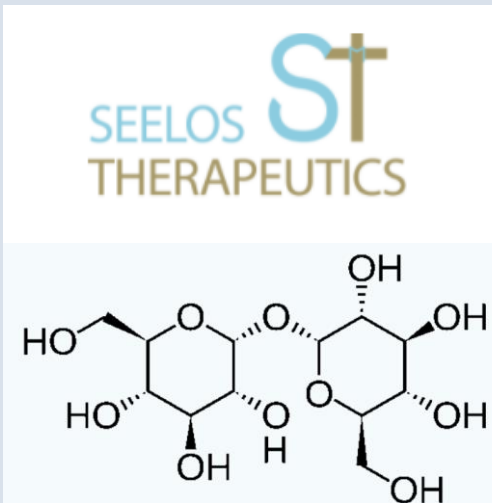
Travel around the
US meeting
Platform Trial Site
Investigators



Guest speakers
from partner
foundations

Trehalose/SLS-005
drug science and
mechanism of action
webinar with Seelos
Therapeutics

<https://bit.ly/3L4hrdB>



Special Topics:
Biomarkers, EAPs,
Biostats, Science
topics (epigenetics,
epidemiology)