

HEALEY ALS Platform Trial

Weekly Q&A – April 28, 2022



Healey Center

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

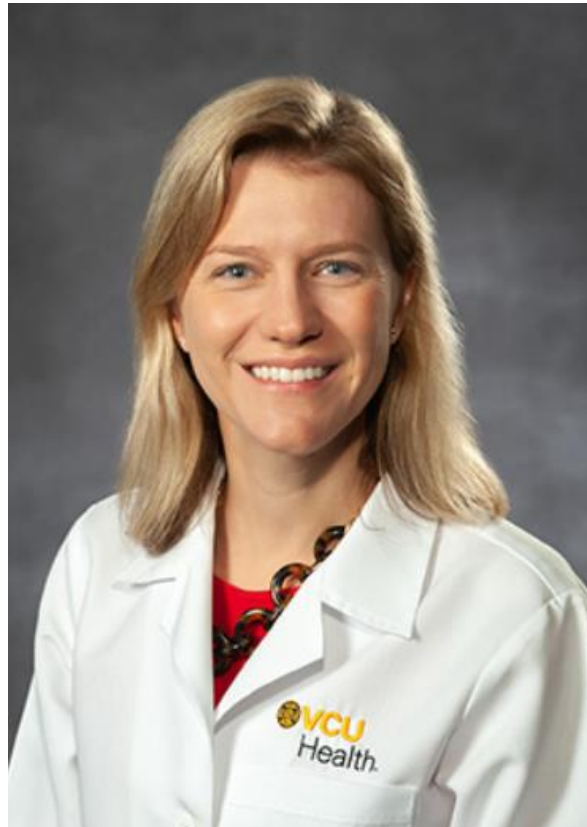


The AMG Foundation

Guest Speaker

Kelly G. Gwathmey, MD

Platform Trial Site Principal Investigator
Virginia Commonwealth University, Richmond VA



VCU Health ALS Clinic



ALS Clinic Team



Short Pump Pavilion
Richmond, Virginia

Our team of experts

1. Kelly Gwathmey, M.D., Chair, Neuromuscular Medicine Division; Assistant Professor, Neurology

2. Qihua Fan, MD
Assistant Professor, Neurology

3. Michelle Gebhardt
Nurse Navigator

4. Paula Brockenbrough
Respiratory Therapist

5. Rebecca Rhodes
Registered Dietitian

6. Kiera Berggren
Speech Language Pathologist

7. Amanda Butler
Physical Therapist

8. Ashley Moneymaker
Occupational Therapist

9. Anne Shields
Social Worker

10. LaVon Smith
Clinical Research Coordinator

11. Kim Claytor
Clinical Research Coordinator (Not pictured)

12. Chelsea Chambers
Genetic Counselor

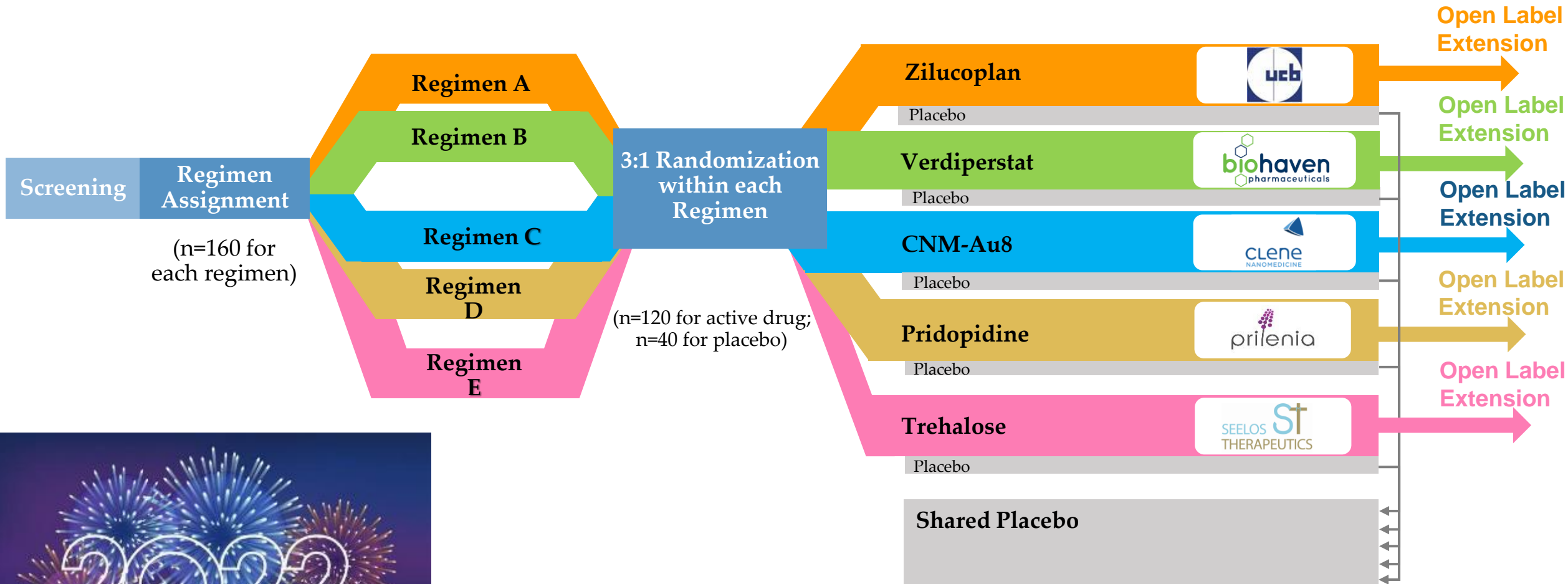


VCU Health ALS Clinic Quick Facts



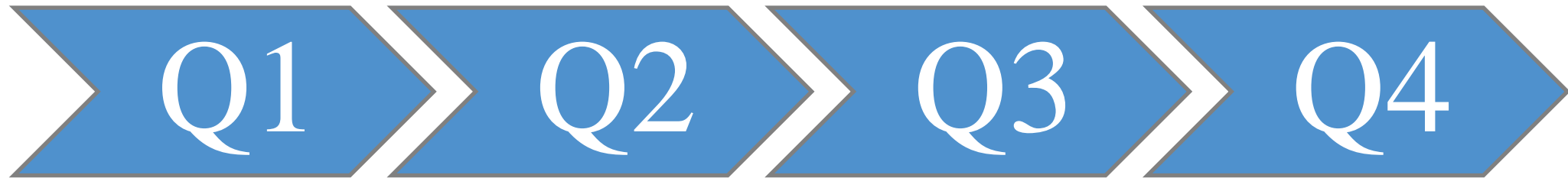
- On average 160-175 active patients (including PLS)
- Full day every Tuesday and one “Rapid Access” clinic monthly
- Clinic supported ALS Association, MDA, and our VCU ALS foundation, Harper’s Hope
- 3 active drug trials
 - **Master Platform Study and Regimen E activated 2/4/22**
 - 1 patient enrolled and 1 patient in screening
- 2 ongoing natural history studies (1 ALS, 1 PLS)
- Several investigator-initiated studies including an epidemiological study of ALS in Virginia in collaboration with the ALS Association

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 28, 2022)

- **45** individuals have signed informed consent
- **31** individuals have been randomized within Regimen E

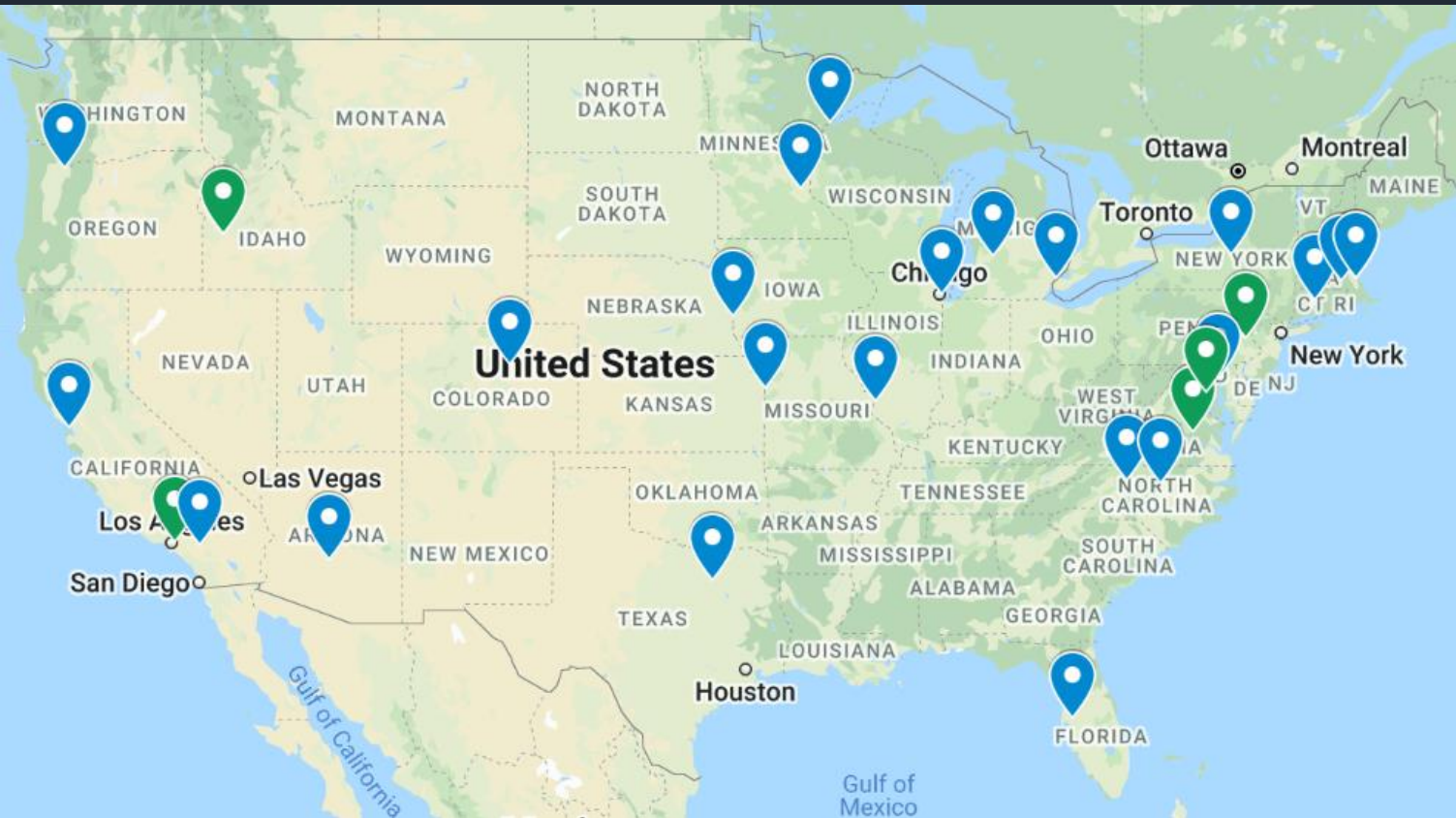
Thank You

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

28 Sites Currently Activated for Regimen E



(as of 4/28/22)

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

Regimen E Site Activation

Sites closest to activation:

- Mayo Rochester
- University of Cincinnati
- University of Pittsburgh
- Swedish Medical Center
- Thomas Jefferson
- University of Iowa
- 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>

Upcoming Guest Speakers:

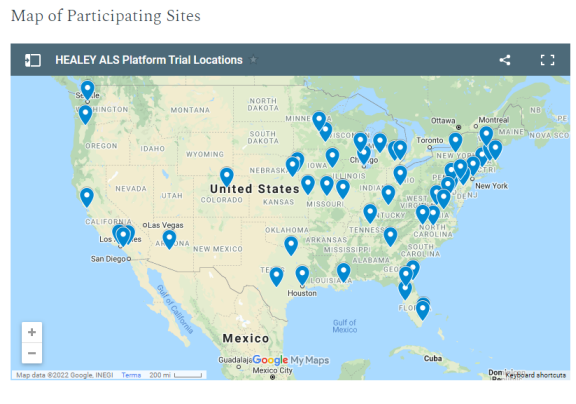
May 5th- Peter Foss (President, ALS Finding a Cure)



Allison Bulat

Send us webinar questions and ideas!

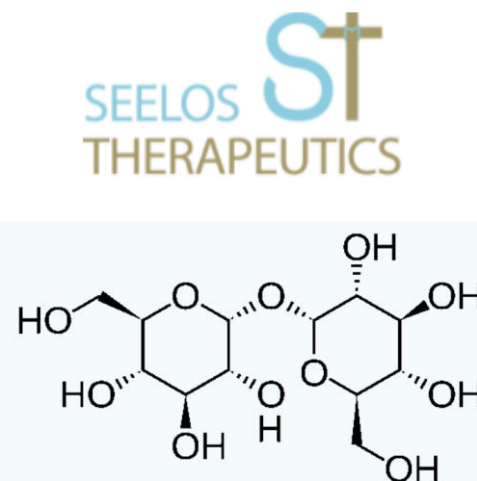
Travel around the
US meeting
Platform Trial Site
Investigators



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)