HEALEY ALS Platform Trial

Weekly Q&A – April 28, 2022

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

Northeast Amyotrophic Lateral Sclerosis Consortium

HARVARD MEDICAL SCHOOL
MASSACHUSETTS GENERAL HOSPITAL

NEALS

BADROW Neurological Institute

Barrow Consultants

Biohaven Pharmaceuticals
Cleene Nanomedicine

The Arthur M. Blank Family Foundation

AMG 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

- **Screening**
  - Regimen Assignment
    - (n=160 for each regimen)

- **Regimen A**
- **Regimen B**
- **Regimen C**
- **Regimen D**
- **Regimen E**

- **3:1 Randomization within each Regimen**

- **Regimen Assignment**
  - Regimen A: Zilucoplan (n=120 for active drug; n=40 for placebo)
  - Regimen C: Verdiperstat
  - Regimen E: Pridopidine
  - Regimen B: CNM-Au8
  - Regimen D: Trehalose

- **Open Label Extension**
  - Placebo

- **Shared Placebo**
Study Results Timelines – Regimens A-D

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

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!
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

as of 4/28/22
## 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.*

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

Patient Navigation
Central resource for people living with ALS

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)
Send us webinar questions and ideas!

Travel around the US meeting Platform Trial Site Investigators

Send us webinar questions and ideas!

Guest speakers from partner foundations

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


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