Guest Speaker

Edward Kasarskis, MD, PhD
Platform Trial Site Principal Investigator
University of Kentucky, Lexington KY
University of Kentucky
ALS Center

Edward J. Kasarskis MD, PhD
Cynthia Shaw Crispen Chair for ALS Research
Professor, Neurology
Where Are We?
Other ALS Research at UK

• Nutrition in ALS
  – Rup Tandan; University of Vermont

• FUS familial ALS
  – Haining Zhu; University of Arizona
  – Ziyuan Guo; University of Cincinnati
The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial

Regimen A (n=160 for each regimen)
Regimen B
Regimen C
Regimen D
Regimen E

3:1 Randomization within each Regimen

Zilucoplan
Placebo

Verdiperstat
Placebo

CNM-Au8
Placebo

Pridopidine
Placebo

Trehalose
Placebo

Shared Placebo

(n=120 for active drug; n=40 for placebo)
Enrollment Updates (as of March 17, 2022)

- **15** individuals have signed informed consent
- **8** 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.
18 Sites Currently Activated for Regimen E

(as of 3/17/22)

Sites in blue participated in previous regimens. Sites in green (also underlined above) are new additions to the Platform Trial!
Regimen E Site Activation

<table>
<thead>
<tr>
<th>Sites closest to activation:</th>
</tr>
</thead>
<tbody>
<tr>
<td>➢ Spectrum Health</td>
</tr>
<tr>
<td>➢ University of Colorado</td>
</tr>
<tr>
<td>➢ SUNY Upstate</td>
</tr>
<tr>
<td>➢ Henry Ford Hospital</td>
</tr>
<tr>
<td>➢ University of South Florida</td>
</tr>
</tbody>
</table>
# Checking Site Status Online

[https://bit.ly/3g2NZr5](https://bit.ly/3g2NZr5) as of 3/17/22

## 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>
The ALS Association/Northeast ALS Consortium Educational Webinar

**The Latest on Expanded Access Protocols in ALS**

Register Below:


Recording will later be available under “educational webinars” on neals.org
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 Speaker:
March 24th - Jaimin Shah, MD (Mayo Clinic, Jacksonville FL)
Send us webinar questions and ideas!

Travel around the US meeting Platform Trial Site Investigators

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

Guest speakers from partner foundations

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