

# HEALEY ALS Platform Trial

Weekly Q&A – March 17, 2022



## Healey Center

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



The AMG Foundation

# Guest Speaker

**Edward Kasarskis, MD, PhD**

Platform Trial Site Principal Investigator  
University of Kentucky, Lexington KY



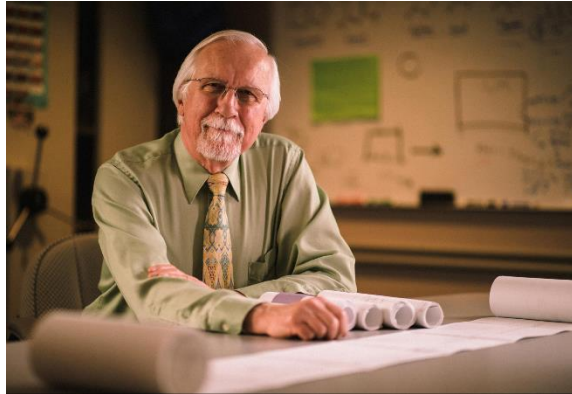
# University of Kentucky ALS Center

Edward J. Kasarskis MD, PhD  
Cynthia Shaw Crispen Chair for ALS Research  
Professor, Neurology

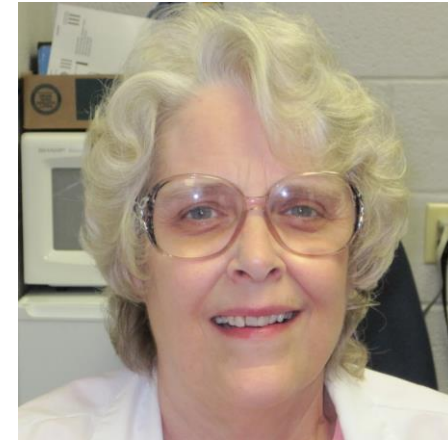
# Where Are We?



# Who Are We?



Edward J. Kasarskis



Deborah Taylor

Emma Hanley

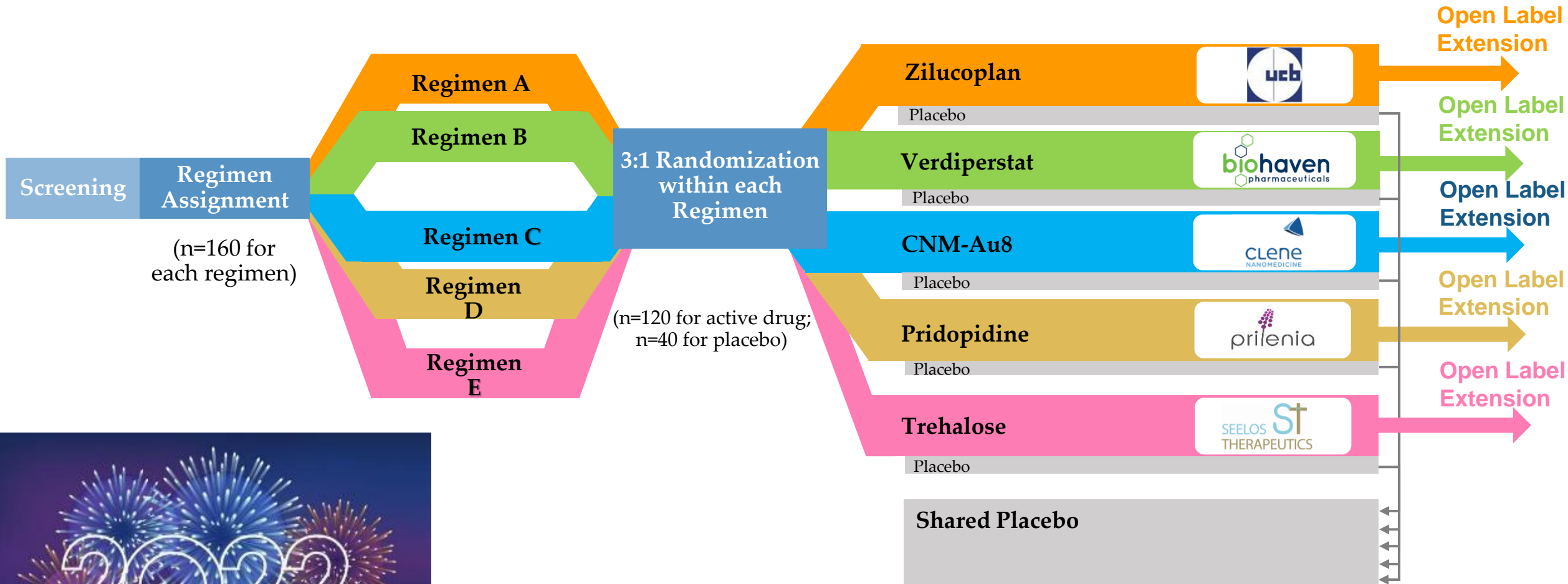


Vish Kumaraswamy

# 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



# Enrollment Updates (as of March 17, 2022)

- **15** individuals have signed informed consent
- **8** individuals have been randomized within Regimen E

*Thank You*

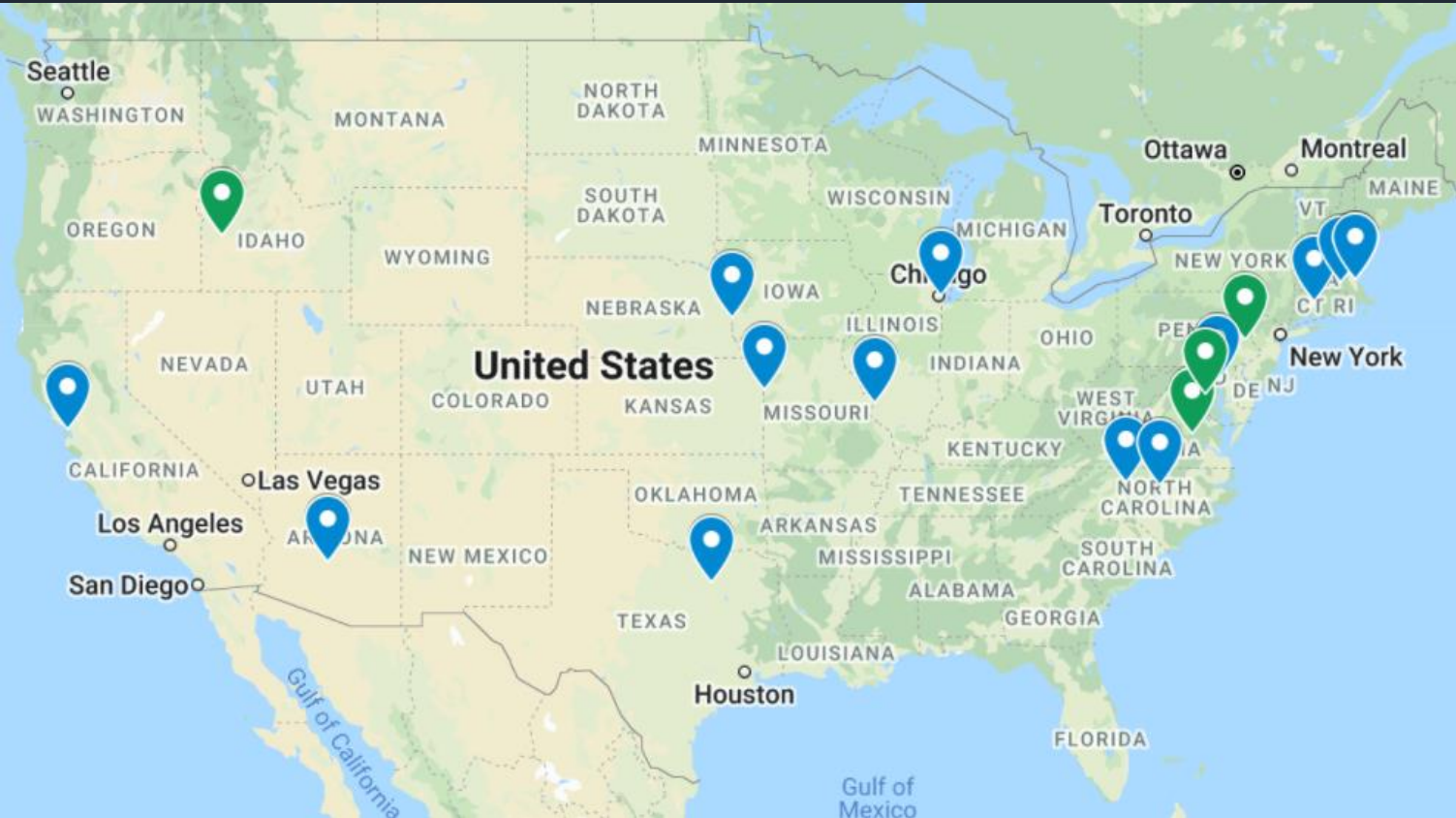
**This breakthrough trial would not be possible without your participation**

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)

- ✓ 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
- Sites in blue participated in previous regimens. Sites in green (also underlined above) are new additions to the Platform Trial!*

# Regimen E Site Activation

## Sites closest to activation:

- Spectrum Health
- University of Colorado
- SUNY Upstate
- Henry Ford Hospital
- University of South Florida
- Providence Brain and Spine
- Ohio State University
- Essentia Health
- University of Cincinnati

# 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	<a href="#">Stephanie Hansen</a>
<b>Lehigh Valley Health Network</b>	<b>PA</b>	<b>Recruiting</b>	<b><a href="#">Andrew Orzel</a></b> <b><a href="#">(610-402-9543)</a></b>
Penn State Hershey	PA	Active, Not recruiting	<a href="#">Heidi Runk</a>



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



The ALS Association/Northeast ALS Consortium Educational Webinar

## The Latest on Expanded Access Protocols in ALS



*Moderator: Jinsy A. Andrews, MD, MSc, Columbia University Medical Center*

*Panelists: Merit Cudkowicz, MD, MSc, Sean M. Healey & AMG Center for ALS at Mass General Hospital, Sabrina Paganoni, MD, PhD, Sean M. Healey & AMG Center for ALS at Mass General Hospital, Philip Green, and Sandy Morris*

Friday, March 18, 2022  
12:00 - 1:00 PM EDT

Register Below:



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

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

# Patient Navigation

## Central resource for people living with ALS



Catherine Small

Phone: 833-425-8257 (HALT ALS)

E-mail: [healeyalsplatform@mgh.harvard.edu](mailto:healeyalsplatform@mgh.harvard.edu)

Weekly webinar  
registration:



<https://bit.ly/3r6Nd2L>

ALS Link sign-up:



<https://bit.ly/3o2Ds3m>

**Upcoming Guest Speaker:**

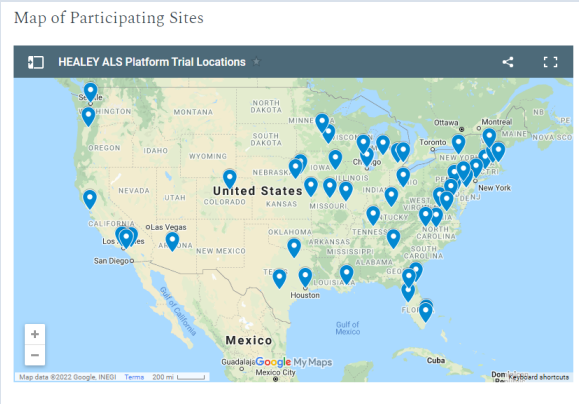
**March 24<sup>th</sup>- Jaimin Shah, MD (Mayo Clinic, Jacksonville FL)**



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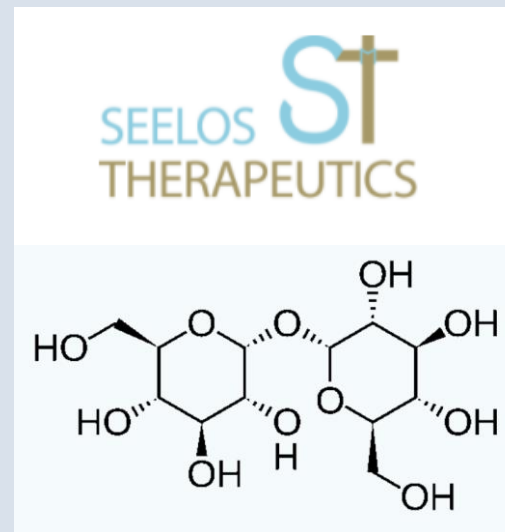
# Send us webinar questions and ideas!

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



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