Thank you!
The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial

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

- **Regimen A**
  - Placebo

- **Regimen B**
  - 3:1 Randomization within each Regimen
  - Verdiperstat
    - Placebo
  - CNM-Au8
    - Placebo

- **Regimen C**
  - (n=120 for active drug; n=40 for placebo)

- **Open Label Extension**
The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial

Regimen A (n=160 for each regimen)

Regimen B

Regimen C

Regimen D

3:1 Randomization within each Regimen

Screening

Assignment

Pridopidine

Placebo

Verdiperstat

Placebo

Zilucoplan

Placebo

CNM-Au8

Placebo

Open Label Extension

Open Label Extension

Open Label Extension

Open Label Extension

(n=120 for active drug, n=40 for placebo)
The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial

- **Regimen A**: (n=160 for each regimen)
  - Placebo
  - Verdiperstat
  - CNM-Au8
  - Pridopidine
  - Zilucoplan

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

- **Regimen C**: (n=120 for active drug; n=40 for placebo)
  - Placebo

- **Regimen D**: Placebo

- **Regimen E**: Placebo

**Shared Placebo**
Regimens A, B, C and D completed enrollment!

- 162 individuals were randomized within Regimen A
- 167 individuals were randomized within Regimen B
- 161 individuals were randomized within Regimen C
- 163 individuals were randomized within Regimen D

> 300 participants have entered the Open Label Extension (OLE)
Completing enrollment for the fourth regimen in the Platform Trial provides further hope towards discovering treatments for people living with ALS using this novel approach to therapy development in the disease. We are grateful for our partnerships with people with ALS who provide advice and input and without whose participation we could not develop effective ALS treatments.

Merit Cudkowicz, MD, MSc
Director, Sean M. Healey & AMG Center for ALS, Massachusetts General Hospital

“The exceptional enrollment rates we are seeing are a testament to the power of the platform approach”

Sabrina Paganoni, MD, PhD
physician-scientist at the Healey & AMG Center and co-lead investigator of the trial
Our first collaborative paper was accepted for publication!

**Adaptive Platform Trials to Transform ALS Therapy Development**

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Plans for Expansion: 22 New Sites Coming Soon

- Atrium Health
- Cleveland Clinic
- George Washington University
- University of California, San Francisco
- Yale University
- Indiana University
- Stony Brook University
- Georgetown University
- University of Arkansas
- University of Pittsburgh
- University of Southern California
- University of Utah
- Augusta University
- University of Cincinnati
- Virginia Commonwealth University
- Swedish Medical Center
- Las Vegas Clinic
- Kaiser, Los Angeles
- Lehigh Valley Health Network
- St. Alphonsus Regional Medical Center
- Hackensack University
- Essentia Health

Note: Only those sites highlighted in blue are actively enrolling at this time. Additional sites will be activated on a rolling basis.
Patient Navigation
Central resource for people living with ALS

Phone: 833-425-8257 (HALT ALS)
E-mail: healeyalsplatform@mgh.harvard.edu

- Enrollment updates shared weekly
- Over 1,000 questions about ALS research answered live
- Over 13,000 viewers from 43 countries
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Weekly webinar registration:

ALS Link sign-up:
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Upcoming Guest Speaker:
Jan 13th- Sharon Hesterlee, PhD of the Muscular Dystrophy Association


ALS Link sign-up: https://bit.ly/3EWEp38