Guest Speaker

James Berry, MD, MPH
Massachusetts General Hospital
Regimen co-Lead (Regimen C)
Perpetual Adaptive Trial
Shared Placebo; Randomization Ratio 3:1
Open Label Extension (OLE) offered

Screening
Regimen Assignment
(n=160 for each regimen)

Regimen A
Regimen B
Regimen C
Regimen D
(n=120 for active; n=40 for placebo)

3:1 Randomization within each Regimen

Regimen Assignment
Assign to Regimen

Informed Consent
Randomize within Regimen (active/placebo)

Open Label Extension

Up to 6 weeks
24 weeks (about 6 months)
Endpoints

Primary Endpoint
Change in disease severity - ALS Functional Rating Scale-Revised (ALSFRS-R)

Secondary Endpoints
1. Change in respiratory function - slow vital capacity (SVC)
2. Change in muscle strength - hand held dynamometry (HHD)
3. Survival
4. Treatment-specific biomarkers as applicable

Exploratory Endpoints

Safety Endpoints
Exploratory Endpoints

Endpoint Development Engine

- DNA
- Neurofilaments
- Biomarkers (Blood, Urine, CSF)
- Speech Analysis
- Home Spirometry
Regimens A, B and C completed enrollment!

- **162** individuals were randomized within Regimen A
- **167** individuals were randomized within Regimen B
- **161** individuals were randomized within Regimen C
- **146** individuals were randomized within Regimen D
- **282** have entered the Open Label Extension (OLE)

Thank You

This breakthrough trial would not be possible without your partnership
Plans for Expansion: 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 active at this time. Additional sites will be activated on a rolling basis.
Patient Navigator

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

Eligibility criteria:  
Enrolling sites:
# Finding Other Clinical Trial Opportunities

<table>
<thead>
<tr>
<th>Source</th>
<th>Contact Information</th>
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<tbody>
<tr>
<td>clinicaltrials.gov</td>
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<td>CDC National ALS Registry</td>
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<tr>
<td>NEALS Website</td>
<td>ALS Clinical Trial Liaison contact info</td>
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<tr>
<td></td>
<td>(855) 437- 4823</td>
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<td><a href="mailto:alstrials@neals.org">alstrials@neals.org</a></td>
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<tr>
<td>ALS Signal from I AM ALS</td>
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Send us webinar ideas!

- Biostatistics
- Get to know our sites

Upcoming Guest Speakers:

Nov 25th - No Webinar, Happy Thanksgiving!
Dec 2nd - Neil Thakur, PhD (The ALS Association)
Dec 9th - Nicholas Olney, MD (Providence ALS Clinic, OR)
Dec 16th - Lori Chibnik, PhD, MPH (Biostatistics)
Dec 23rd - No Webinar, Happy Holidays!
Dec 30th - No Webinar, Happy Holidays!
Join the ALS Link!

- Sign up to receive news and updates via email from the Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital
- Get the latest information about ALS clinical trials, clinical care, and webinar opportunities