Thank you for joining the weekly webinar!
We are admitting audience members from the waiting room.
Please allow a few moments for the webinar to begin.
HEALEY ALS PLATFORM TRIAL WEEKLY WEBINAR

September 14, 2023

Jennifer DiMartino, Executive Director, ALS ONE
jen@alsONE.org
ALS ONE was founded in 2016 by Massachusetts resident, Kevin Gosnell, a prominent business leader who was diagnosed with ALS at the prime of his life at age 46.

- Kevin’s vision was to break down the barriers that traditional silos created and open the door for unity in the fight against a disease that needed it so desperately. To create a research and care team, joining as ONE, to help reduce redundancy, advance science more effectively & efficiently, and to help individuals living with ALS navigate the complexities of the disease by supporting them as a team. ALS ONE was formed, uniting the Massachusetts leaders, among the world-leaders in ALS research and care:
COMMUNICATION is everything

Regular meetings are held with our research and care teams, fostering a unified approach to advancing critical ALS studies while ensuring that individuals living with ALS have the equipment, resources, and support needed and deserved to live their best life while navigating the complexities of the disease.
COMMITMENT to our mission

Our founder lost his battle to ALS just 7 months after our launch, but since then, we have raised almost $17 Million to advance key initiatives in ALS research with and for our partners.

Over two-thirds of what we have raised to date has come from grassroots fundraising - from events big, small & everything in between.

A small staff with low overhead yet with a very large footprint.

LEARN MORE AT ALSONE.org
**Funding: ways we have and continue to make a difference**

We continue to help make significant advancements by funding the following initiatives, among others:

**MGH**
- THE HEALEY ALS Platform Trial (founding partner and benefactor).
- Expanded Access Program (EAP)
- Parenting at a Challenging Time (PACT) Program
- ALS House Call program.
- Full-time ALS Research Access Nurse
- Nutrition and Data studies
- Biomarker studies
- Neutotechnology initiatives

**UMASS**
- Preclinical studies of microRNA-based suppression of SOD1 and divalent siRNA suppression of SOD1
- Preclinical studies of ASO afinersen to suppress C9orf72
- Preclinical studies of ASO to modify splicing of cryptic STMN2
- Single-cell transcriptomic analysis of SOD1 mice

**CCALS**
- $2M+ in equipment
- Care Resources & emotional support
- Completion of the Retreat Center
- Expanded resources in NY/NJ area

**ALS TDI**
- Completion of AT-1501
- Phase 1 trial of anti-CD40-Ligand Antibody
- Preclinical mice studies

LEARN MORE AT ALSONE.org
Research & Care joint initiatives: Profound examples of unity

- Bi-Weekly team conference calls
- In-Services to major hospitals to train on caring for individuals with ALS – Hospital Grand Rounds, VNA’s, hospice & palliative groups.
- Webinars for patients and families.
WAYS to get involved

Host an Event
Events as small as a car wash and as large as a golf tournament are held regularly by pALS, families, and supporters and mean the world to us - and we help every step of the way!

Drive with our Plate
Massachusetts drivers can support ALS research and care every single day while driving with the Mass. ALS License Plate.

Attend one of our events
9.16.23: Roy-ALS Ride for ALS
9.16.23: 1/2 Way to St. Patrick’s Day Party
9.25.23: cALSea Crew Golf Tourney
10.1.23: Rise Against ALS: Decades Party
10.2.23: Cars & Coffee
10.7-9.23: Southborough Golf Weekend
10.9.23: Team NSF Drive Golf Tourney
10.21.23: Tucker Toss Cornhole Tourney
10.26.23: Go On Be Brave! Movie Screening
11.5.23: NYC Marathon Team
11.16-20.23: ALS ONE Research Symposium

LEARN MORE AT ALSONE.org or email jen@alsone.org
HEALEY ALS Platform Trial:

Common Protocol and Shared Infrastructure

Regimen A
Regimen B
Regimen C
Regimen D
Regimen E
Regimen F
Regimen G

Screen for eligibility → Randomization 3:1 → Active
→ Placebo

Screening Period → Randomized Period (24 weeks)
→ Open-Label Extension Period (Active Treatment Extension)

ENROLLMENT COMPLETE
ENROLLING
ENROLLING
Enrollment Update: Regimen F and Regimen G

328
Participants consented to Master Protocol since RGF and RGG initiated

267
Participants assigned to RGF or RGG

226
Participants randomized within RGF or RGG

Thank You
for your partnership in ALS research

(as of 9/14/23)
62 Sites Currently Active

Nova Southeastern University
Essentia Health
Texas Neurology
Mass General Hospital
University of Nebraska
Hospital for Special Care
Henry Ford Hospital
Augusta University
Beth Israel Deaconess
University of Texas HSC
University of Colorado
Loma Linda University
Ohio State University
Cedars Sinai Medical Center
Duke University
Wake Forest University
Saint Alphonsus
UMass Worcester
Lehigh Valley
Thomas Jefferson
University of South Florida
University of Pennsylvania
SUNY Upstate
University of Iowa
California Pacific Med Center
Houston Methodist
Vanderbilt University
University of Minnesota
Washington University
Barrow Neurological Institute
University of Miami

(as of 9/14/23)

Temple University
University of Virginia
Johns Hopkins University
University of Southern CA
Holy Cross Hospital
University of Washington
University of Utah
Penn State Hershey
University of Michigan
University of Kansas
Stony Brook University
University of Cincinnati
Mayo Clinic Rochester
Northwestern University
Georgetown University
Kaiser, Los Angeles
University of Pittsburgh
Virginia Commonwealth
Med College Wisconsin
University of CA, San Fran
University of Florida, Gainesville
Providence Brain and Spine
Cleveland Clinic
George Washington University
Hackensack University
Swedish Medical Center
University of Kentucky
Dartmouth Hitchcock
University of Maryland
University of Chicago
University of CA, Irvine
Checking Site Status Online

List of Participating Sites

Sites marked "Recruiting" are currently enrolling participants. Sites marked "Active, Not recruiting" are active in the Platform Trial (for example, they are following participants in ongoing regimens that have already completed enrollment) but are not enrolling new participants at this time.

<table>
<thead>
<tr>
<th>Site</th>
<th>State</th>
<th>Enrollment Status</th>
<th>Trial Contact Information</th>
</tr>
</thead>
<tbody>
<tr>
<td>Barrow Neurological Institute</td>
<td>AZ</td>
<td>Recruiting</td>
<td>Whitney Dailey</td>
</tr>
<tr>
<td>Cedars-Sinai Medical Center</td>
<td>CA</td>
<td>Recruiting</td>
<td>Sophia Mostowy</td>
</tr>
<tr>
<td>Forbes Norris MDA/ALS Research Center,</td>
<td>CA</td>
<td>Recruiting</td>
<td>Teji Dulai</td>
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<td>California Pacific Medical Center</td>
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<tr>
<td>Kaiser Permanente, Los Angeles Medical</td>
<td>CA</td>
<td>Recruiting</td>
<td>Mary H. Berganza</td>
</tr>
<tr>
<td>Medical Center</td>
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</tbody>
</table>

Contact a study team near you to discuss enrollment opportunities

https://bit.ly/3g2NZr5
Study Drugs Webpage

This public webpage provides:

1. General Brochure and Biomarkers Brochure
2. Overview of science behind Regimen F and Regimen G
3. Links to download relevant brochures, including the Lumbar Puncture Brochure
4. Links to view relevant videos and webinar recordings

Regimen F: ABBV-CLS-7262, by Calico and AbbVie - Now Recruiting

- Watch a webinar about the science behind ABBV-CLS-7262
- Watch this video for more information on the mechanism of action behind ABBV-CLS-7262.
- Download Regimen F Brochure
- Download Lumbar Puncture Brochure
- Step by Step Guide to Lumbar Punctures: Watch short video

Regimen G: DNL343, by Denali Therapeutics

- Download Regimen G Brochure
- Watch a webinar about the science behind DNL343

https://www.massgeneral.org/neurology/als/research/first-platform-trial-treatments

Healey & AMG Center
Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital
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 Webinars:
September 21st- weekly Q&A webinar with Amanda Lee of ALSA
September 28th- weekly Q&A webinar and EAP update
October 5th- canceled for NEALS conference