Guest Speaker

Jennifer DiMartino
Executive Director
ALS ONE
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.
UNITY makes us stronger

The linking of minds and resources of our partners, who are renowned world-leaders in ALS research and care, from The Healey Center at MGH, The University of Massachusetts Medical School, ALS Therapy Development Institute, and Compassionate Care ALS, is unique for its leadership in efficiency, dedication to the sharing and advancement of innovative research, and commitment to increasing access to essential equipment, care resources, and treatment.

- Kevin lost his battle to ALS just 7 months after ALS ONE's launch but we have grown stronger each year, making measurable and meaningful advancements.
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 are living their best life possible.

RESEARCH

<table>
<thead>
<tr>
<th>Merit Cudkowicz, MD, MSc</th>
<th>Robert Brown MD, DPhil</th>
<th>James Berry, MD, MPH</th>
<th>Fernando Vieira, MD</th>
</tr>
</thead>
<tbody>
<tr>
<td>Chief of Neurology &amp; The Healey Ctr. for ALS at MGH</td>
<td>Chair of Neurology UMass Medical School</td>
<td>Chief, Div. of ALS &amp; Director, NCRI at MGH</td>
<td>Chief Science Office and CEO at ALS TDI</td>
</tr>
</tbody>
</table>

CARE

<table>
<thead>
<tr>
<th>Darlene Sawicki, MSN, NP-BC</th>
<th>Jen Scalia, MSN, NP</th>
<th>Judi Carey, RN</th>
<th>Diane McKenna-Yasek, RN, BSN</th>
<th>Ron Hoffman</th>
</tr>
</thead>
<tbody>
<tr>
<td>Director of Advanced Practice Providers at MGH</td>
<td>Assoc. Director of The Healey Ctr. at MGH</td>
<td>Clinical Research Access Nurse at MGH</td>
<td>Neurology Research Nurse at UMass Med.</td>
<td>Founder and Executive Director at CCALS</td>
</tr>
</tbody>
</table>

LEARN MORE AT ALSONE.org
RELATIONSHIPS are the key

Through our partnership and the relationship model that ALS ONE has created, we continue to make measurable and meaningful advancements in the following areas, among others:

- THE HEALEY ALS Platform Trial, for which we are proud to be a founding partner and benefactor.
- Expanded Access Program (EAP).
- ALS Parenting at a Challenging Time (PACT) & ALS House Call program at MGH,
- Full-time ALS Research Access Nurse
- SOD-1 and C-9 orf gene silencing trials at UMass Medical School
- Preclinical research for the AT-1501 trial at ALS TDI.
- Annual ALS ONE ALS Research Symposium with over 300 researchers sharing critical data.
- Over $1 Million to help CCALS purchase new/refurbished equipment to assist individuals living w/ ALS.
- Helping to complete the CCALS Retreat Center for pALS and families.
- Helping pALS and families connect to the best providers, get the best resources they need, and supporting them through their journey emotionally.

LEARN MORE AT ALSONE.org
COMMITMENT and community

Since ALS ONE was launched in January of 2016, we have raised over $12 Million to advance key initiatives in ALS research and care on behalf of our partners.

- Over two-thirds of what we have raised to date has come from grassroots fundraising. We hold events large and small continually throughout the year and we try to infuse as much joy into the events and to those participating in them as we do gratitude for the funding that comes out of them.

- The incredible community that is fostered through our events and supportive outreach to pALS and families is an organic and essential pillar of our mission.

LEARN MORE AT ALSONE.org
Perpetual Adaptive Trial
Shared Placebo; Randomization Ratio 3:1
Open Label Extension (OLE) offered

Regimen A
(n=160 for each regimen)

Regimen B

Regimen C

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

3:1 Randomization within each Regimen

Zilucoplan
Placebo

Verdiperstat
Placebo

CNM-Au8
Placebo

Pridopidine
Placebo

Shared Placebo

Informed Consent
Assign to Regimen
Randomize within Regimen (active/placebo)
Up to 6 weeks

24 weeks (about 6 months)

Open Label Extension
Enrollment Updates (as of September 9, 2021)

- 740 individuals with ALS signed informed consent
- 583 individuals were assigned to a regimen
- 529 individuals were randomized within a regimen (active or placebo)
- 200 have entered the Open Label Extension (OLE)
- 144 individuals were randomized within Regimen A
- 158 individuals were randomized within Regimen B
- 160 individuals were randomized within Regimen C
- 67 individuals were randomized within Regimen D

Total randomized + Participants in screening

= 155
= 166
= 161
= 71

Thank You
This breakthrough trial would not be possible without your partnership
How to Find a Center Near You

52 sites are actively enrolling

Contact Info of Participating Sites by State

https://www.massgeneral.org/neurology/als/research/platform-trial-sites
Patient Navigator

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

To see whether you might qualify, view the list of eligibility criteria:
Send us webinar ideas!

- Biomarkers
- Biostatistics / Trial Design
- Get to know our sites

Upcoming Guest Speakers:

Sept 16th- No webinar this week in observance of Yom Kippur
Sept 23rd- Namita Goyal, MD (University of CA Irvine, CA)
Oct 7th- No webinar this week