

HEALEY ALS Platform Trial

Weekly Q&A – Sept 9, 2021



Healey Center

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



als FINDING a CURE



THE ARTHUR M. BLANK
FAMILY FOUNDATION



The AMG Foundation

Guest Speaker



Jennifer DiMartino
Executive Director
ALS ONE



ALS
ONE 



UNITED
in the fight against ALS.



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.

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

 University of
Massachusetts
UMASS Medical School

CCALS
COMPASSIONATE CARE ALS
LEADING A NEW VISION IN ALS CARE SINCE 1998

ALS THERAPY DEVELOPMENT
INSTITUTE

LEARN MORE AT ALSONE.org



UNITED
in the fight against ALS.

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.



UNITED
in the fight against ALS.

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



Merit Cudkowicz, MD, MSc

Chief of Neurology & The Healey Ctr. for ALS at MGH



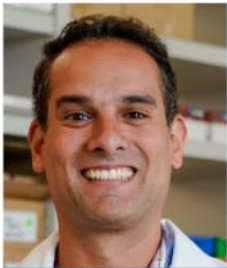
Robert Brown MD, DPhil

Chair of Neurology UMass Medical School



James Berry, MD, MPH

Chief, Div. of ALS & Director, NCRI at MGH



Fernando Vieira, MD

Chief Science Office and CEO at ALS TDI

CARE



Darlene Sawicki MSN, NP-BC

Director of Advanced Practice Providers at MGH



Jen Scalia, MSN, NP

Assoc. Director of The Healey Ctr. at MGH



Judi Carey, RN

Clinical Research Access Nurse at MGH



Diane McKenna-Yasek, RN, BSN

Neurology Research Nurse at UMass Med.



Ron Hoffman

Founder and Executive Director at CCALS



UNITED
in the fight against ALS.

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.



UNITED
in the fight against ALS.

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.

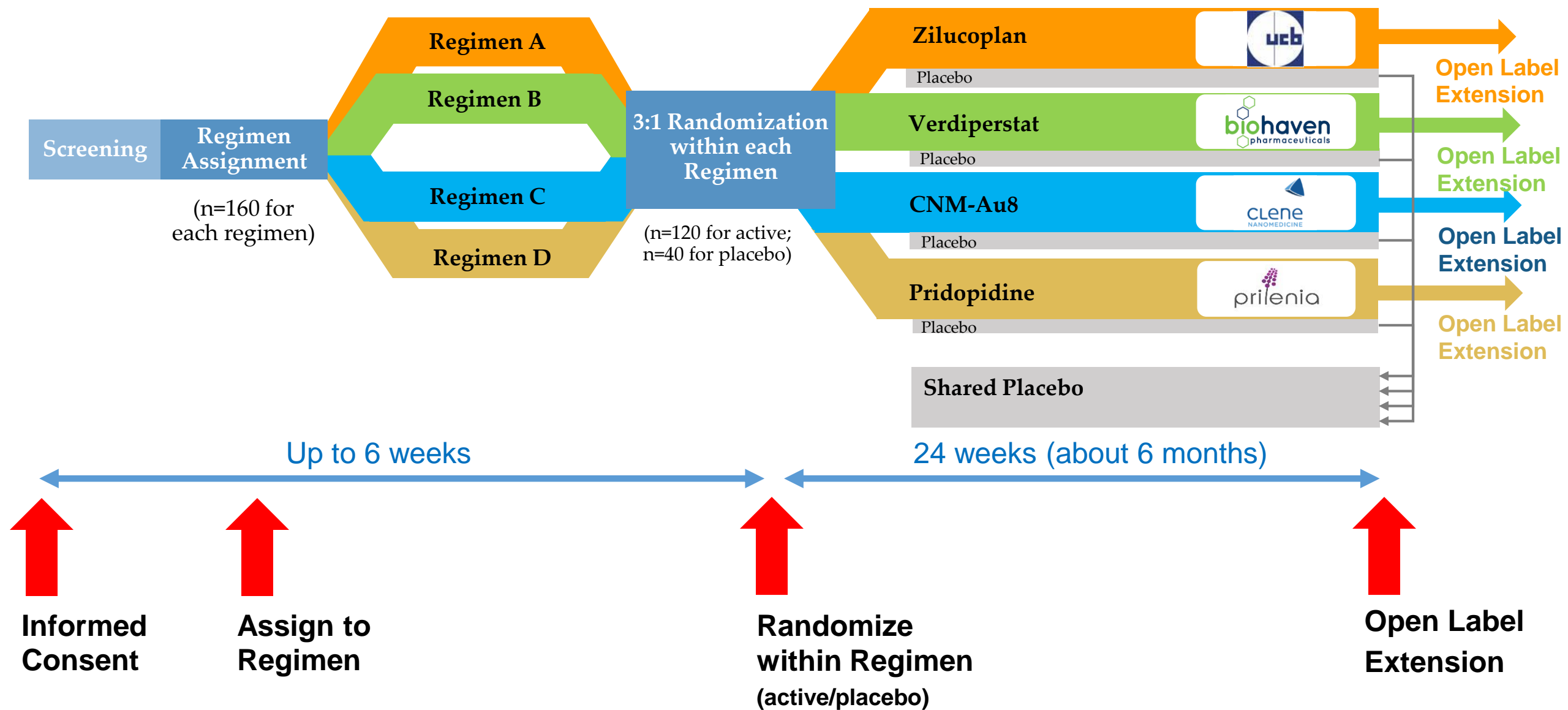
- **Over two-thirds of what we have raised to date has come from grassroots fundraising.** We hold events large and small continually throughout the year and we try to infuse as much joy into the events and to those participating in them as we do gratitude for the funding that comes out of them.
- **The incredible community** that is fostered through our events and supportive outreach to pALS and families is an organic and essential pillar of our mission.



Perpetual Adaptive Trial

Shared Placebo; Randomization Ratio 3:1

Open Label Extension (OLE) offered



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)
- | | Total randomized +
Participants in screening |
|---|---|
| • 144 individuals were randomized within Regimen A | = 155 |
| • 158 individuals were randomized within Regimen B | = 166 |
| • 160 individuals were randomized within Regimen C | = 161 |
| • 67 individuals were randomized within Regimen D | = 71 |

Thank You

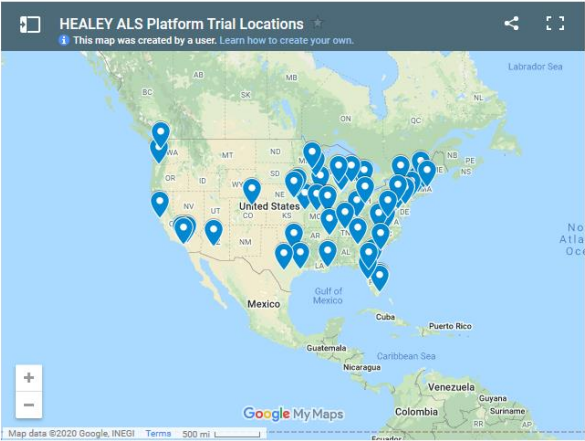
This breakthrough trial would not be possible without your partnership

How to Find a Center Near You

Sean M. Healey & AMG
Center for ALS

About the Healey Center Clinical Care Patient & Family Resources

Map of Participating Sites



HEALEY ALS Platform Trial Locations
This map was created by a user. Learn how to create your own.

List of Participating Sites by State

Site	State	Contact Information
Barrow Neurological Institute	Arizona	Jessie Duncan

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:

<https://bit.ly/3p4PrLL>



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