



Healey Center

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

HEALEY ALS Platform Trial

Updates – Sept 29, 2020



**Merit
Cudkowicz**
*Healey & AMG
Center*



**Sabrina
Paganoni**
*Healey & AMG
Center*

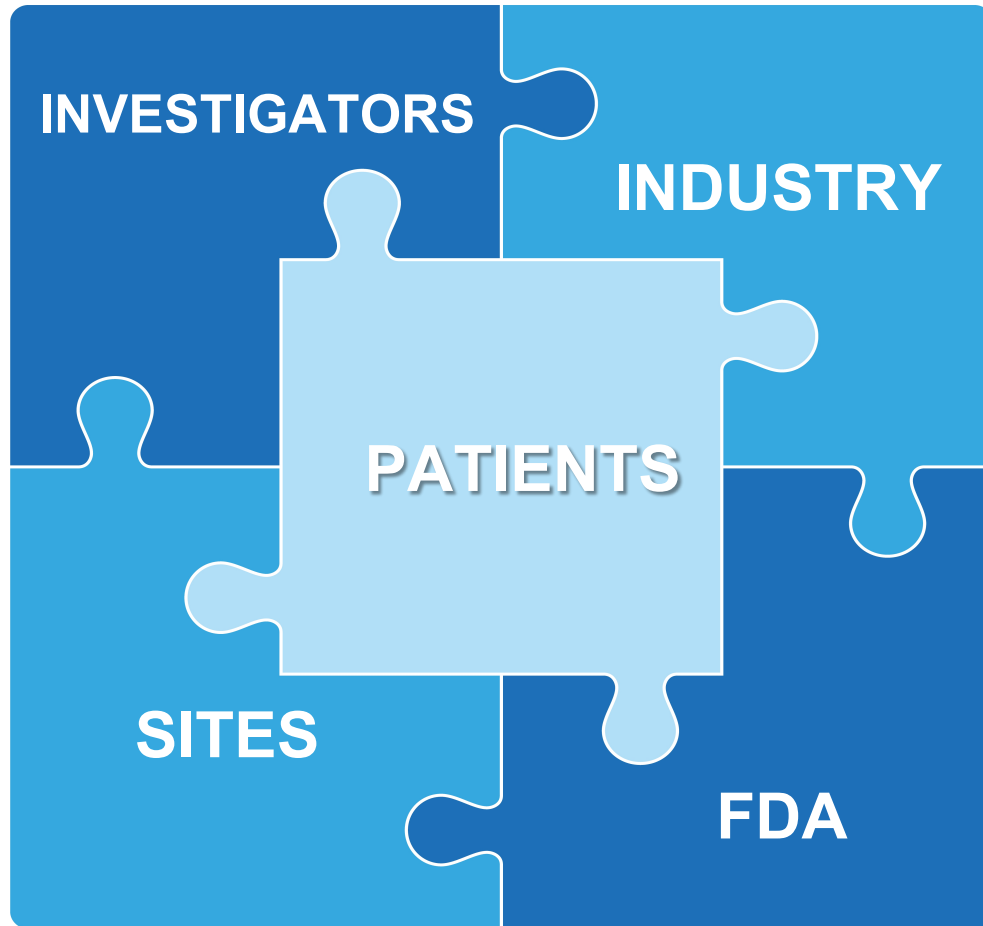


**Kuldip
Dave**
*The ALS
Association*

AGENDA

- **Updates on the HEALEY ALS Platform Trial**
 - *Enrollment*
 - *Study Treatments*
 - *Sites*
- **How to stay in touch and find a site near you**
- **What's next and EAPs**

BRINGING TOGETHER A COMMUNITY to launch the first platform trial for ALS



Concept to Launch

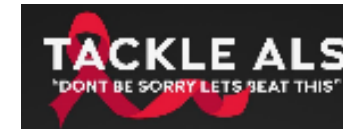


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for ALS at Mass General



BRINGING TOGETHER A COMMUNITY to launch the first platform trial for ALS



COVID-19 IMPACT

- Most centers experienced major disruptions to normal workflow
- Limited to no ability to start new studies / enroll new research participants (prohibited at many sites)
- Staff redeployment and furloughs
- Substantial restrictions on staff access to the clinic and research areas with most staff working remotely
- Restrictions on certain procedures (respiratory function testing still prohibited at many sites)
- Regional variability means that some sites have experienced different barriers at different times which might affect their timelines

We remained committed to the trial through COVID and were able to start the trial despite the pandemic

- **Lots of activities happened remotely**
 - Training, contracts, and administrative tasks to enable site activation
- **Protocol amendments made the trial “COVID proof”**

Enrollment Started in July 2020!

Enrollment Updates

- **96** individuals with ALS signed informed consent
- **48** are currently on study drug

We will continue to update the ALS community on enrollment (website, webinars)

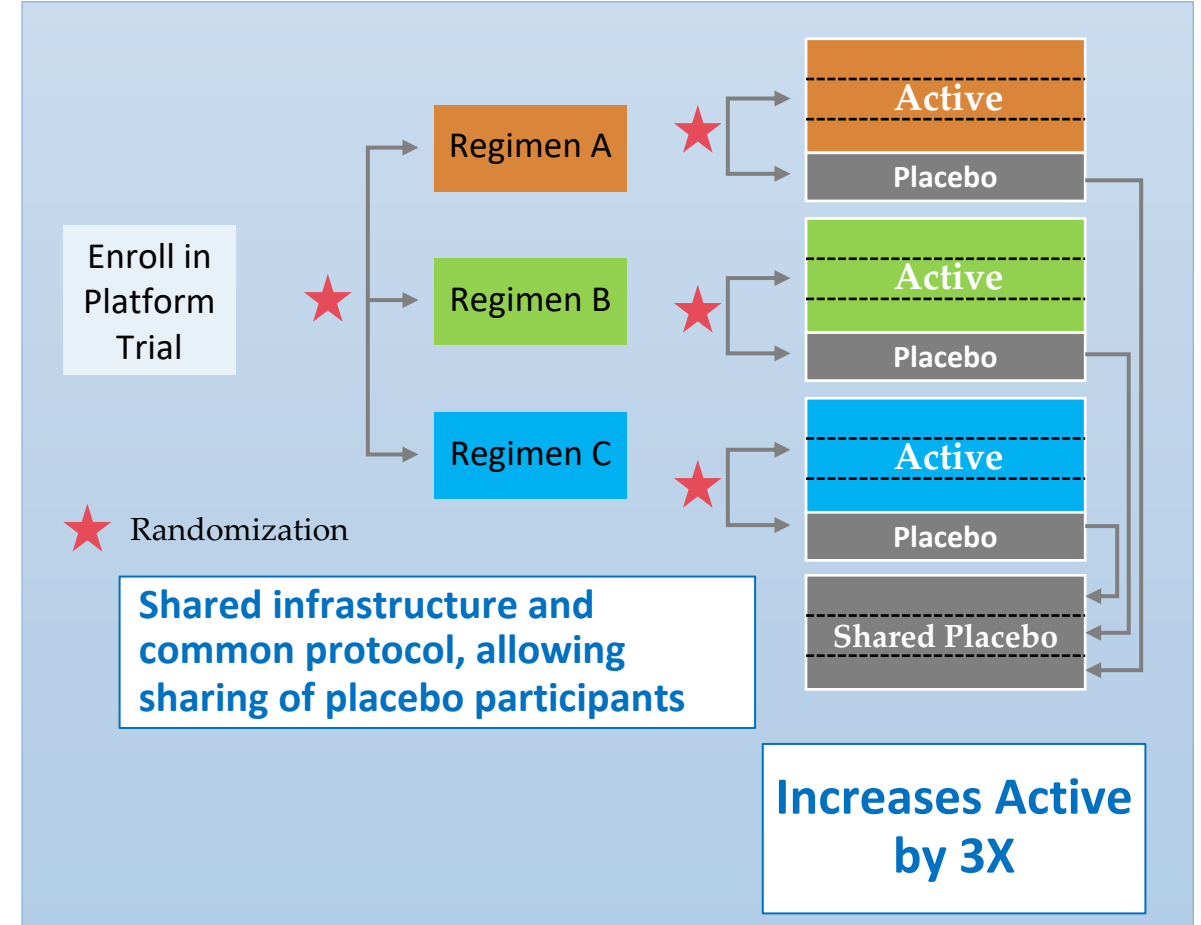
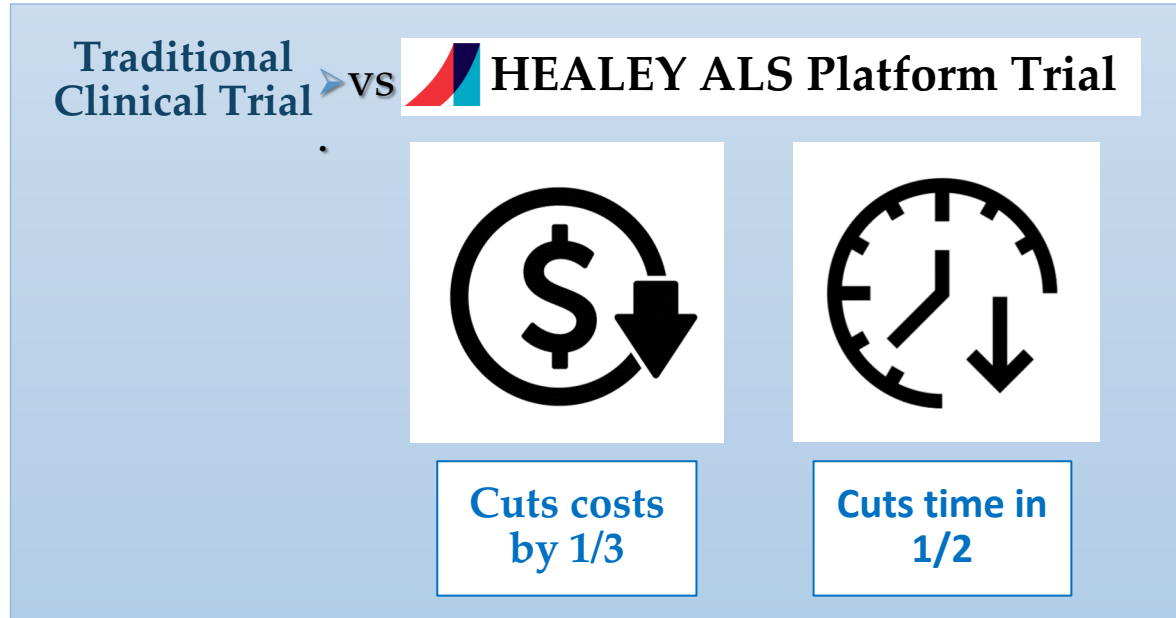
Questions about enrollment? Please contact the Patient Navigator

Phone: 833-425-8257 (HALT ALS)

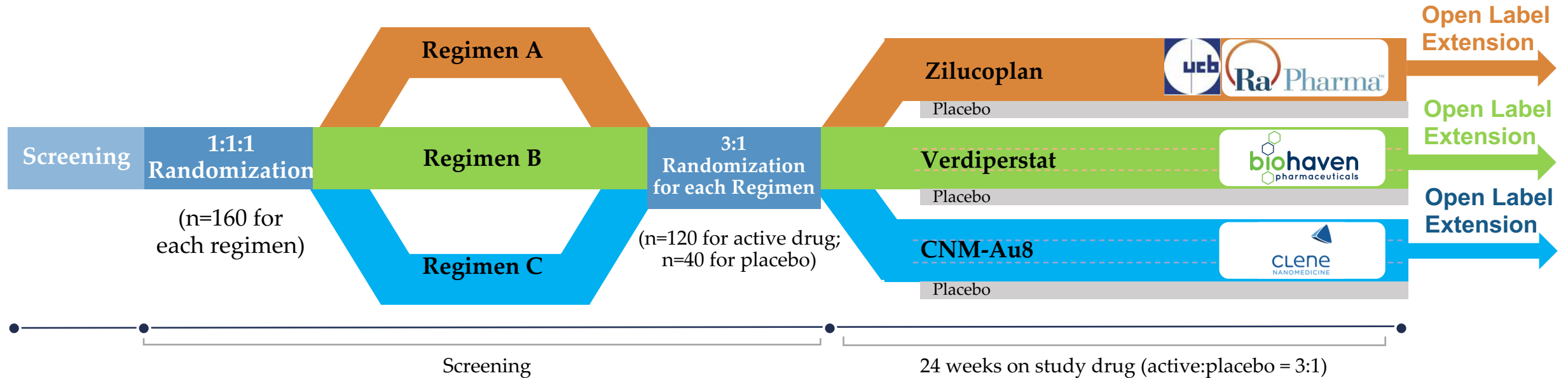
E-mail: healeyalsplatform@mgh.harvard.edu

HEALEY ALS Platform Trial

Offers Several Benefits Over Traditional Trials



Perpetual adaptive trial- Open Label Extension offered



- Treatments were selected by ALS scientists and experts based on a competitive process

We want to keep adding great therapies

- **Submissions accepted on a rolling basis**
- **We opened with 3 regimens**
- **The 4th regimen will be added in 2020 (pridopidine, Prilenia)**
- **We are in discussions with 3 additional companies for 2021!**

<https://www.massgeneral.org/neurology/als/research/platform-trial>


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.

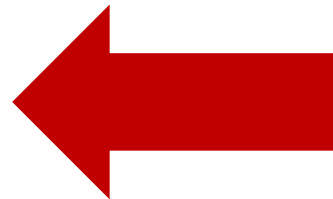


List of Participating Sites by State

Site	State	Contact Information
Barrow Neurological Institute	Arizona	Jessie Duncan

- 22 sites are actively enrolling!
- More will be added once the first 54 have been activated

Contact Info of Participating Sites by State




<https://www.massgeneral.org/neurology/als/research/platform-trial>

How to Receive Platform Trial Updates

Sign up for the ALS Link Newsletter (for info on this trial and other research opportunities)

Not sure which trial might be right for you? The Healey Center for ALS offers coordination services to help you understand your options and make the best choice for your individual situation.

[Introduction to Clinical Trials](#)



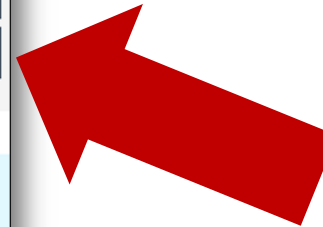
[Enrolling ALS Trials & Studies](#)

[ALS News and Publications](#)

[Sign Up for ALS Link](#)

ALS Platform Trial

The Healey Center is launching the first adaptive ALS Platform Trial to bring novel ALS therapies to patients more quickly.



<https://www.massgeneral.org/neurology/als/services/clinical-trials-enrollment-coordination>

For More Updates

- **Weekly informal Q&A (starting next week!) + monthly webinars**
- The idea of came from our Patient Advisory Committee: we are excited to be talking with you on a weekly basis and take any questions you might have
- **Find the schedule and registration links on our website**
<https://www.massgeneral.org/neurology/als/news/webinars>

EXPANDED ACCESS PROGRAM (EAP) PLANS

- **Goal is to build an EAP in parallel to the Platform Trial**
 - The idea came from the Patient Advisory Committee
 - The EAP would allow access to patients who are not eligible for the trial
- **We are working to achieve this goal**
 - We submitted one grant to start building the EAP program
 - We are actively fundraising to support this effort
 - Please visit our website for more information:

<https://www.massgeneral.org/neurology/als/research/expanded-access>

QUESTIONS

- **To submit text questions to today's presenters, please type your questions into the 'Q&A' box**
- **Today's presentation is being recorded and will be posted on the Healey & AMG Center for ALS website**

<https://www.massgeneral.org/neurology/als/research/platform-trial>