



Healey Center

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

HEALEY ALS Platform Trial

Updates – October 29, 2020



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



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

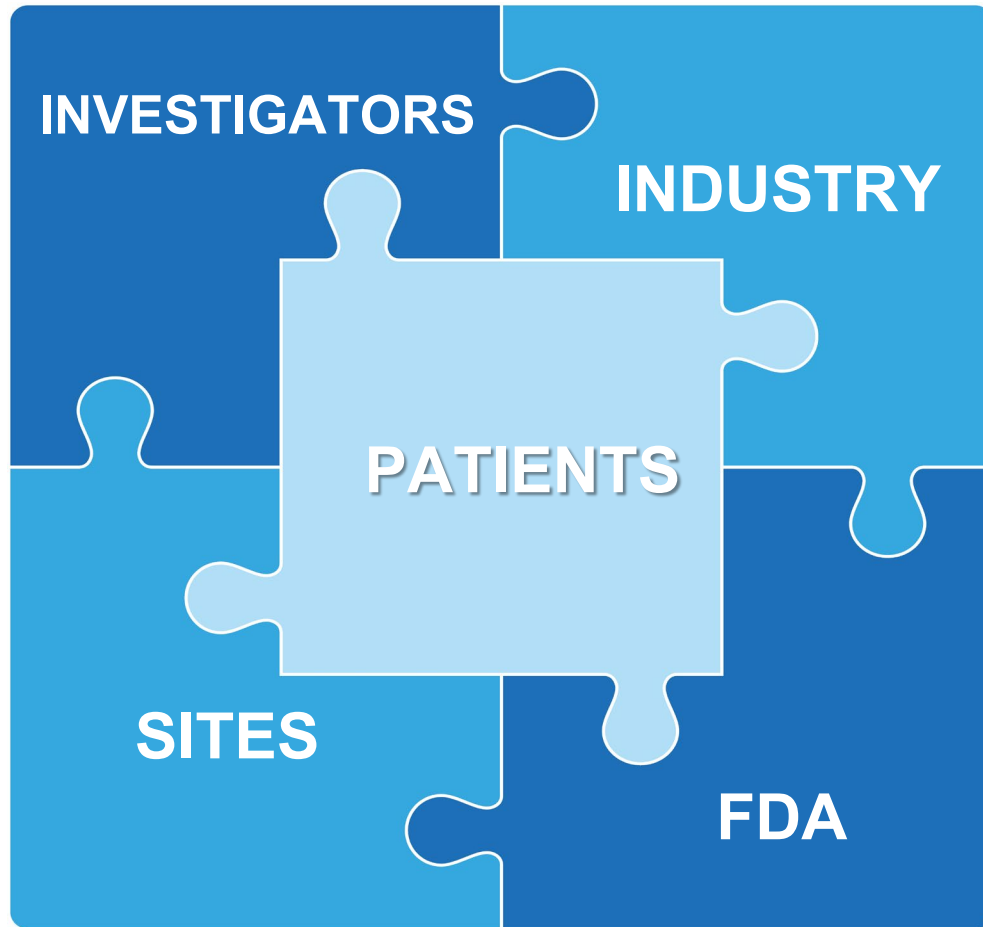


**Peter
Foss**
ALS Finding a Cure

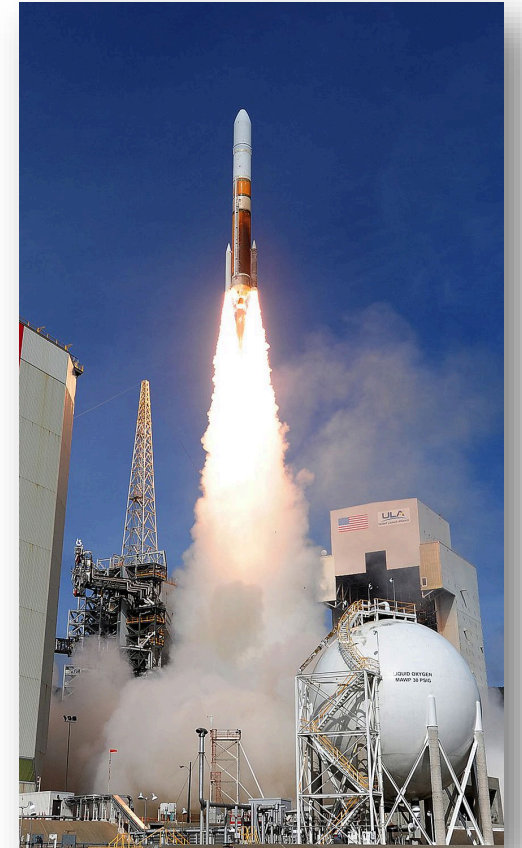


**Troy
Green**
Tackle ALS

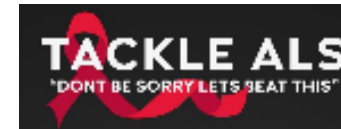
BRINGING TOGETHER A COMMUNITY to launch the first platform trial for ALS

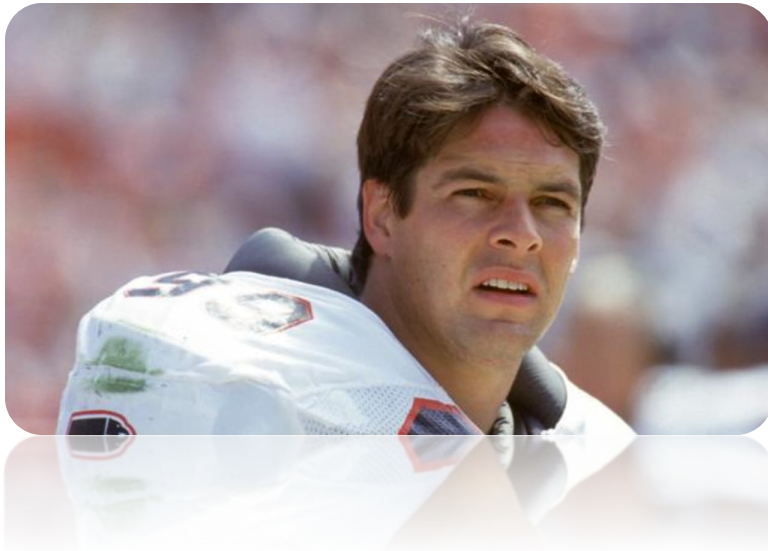


Concept to Launch



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





als FINDING a CURE®

A group of scientists in white lab coats and safety goggles are working in a laboratory. They are gathered around a computer monitor, looking at the screen with interest. One scientist is pointing at the screen, while others are looking on. The background shows laboratory equipment and a framed picture on the wall.

Research brings results.
Be part of our
solution.

[READ MORE](#)

Our sole **mission** is to fund ALS research to find a cure.

als FINDING a CURE®

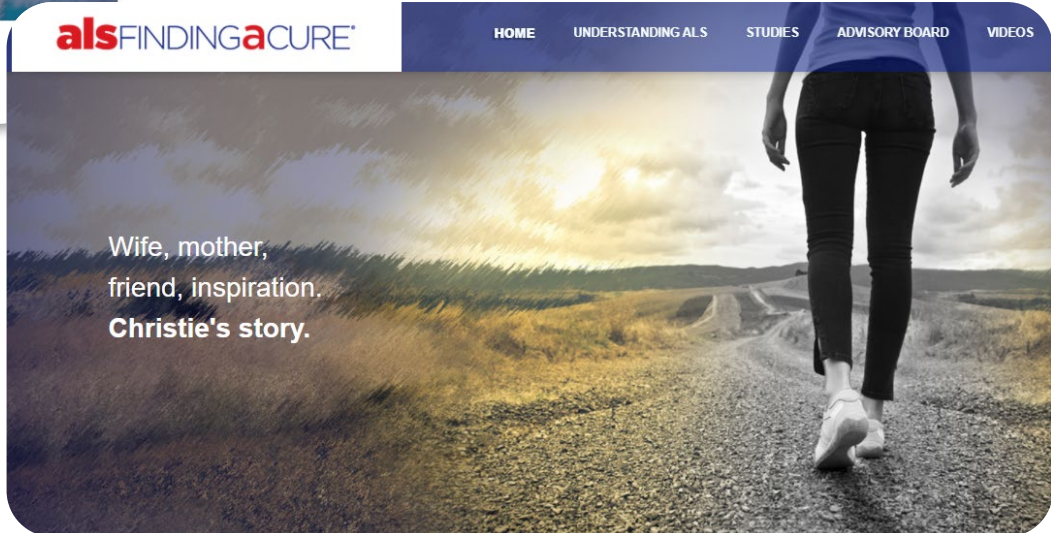
[HOME](#)

[UNDERSTANDING ALS](#)

[STUDIES](#)

[ADVISORY BOARD](#)

[VIDEOS](#)

A person is walking away from the camera on a gravel path that leads into a vast, open landscape. The path is flanked by dry grass and shrubs. In the distance, there are rolling hills under a dramatic sky with a bright sun or moon low on the horizon, creating a golden glow. The person is wearing dark pants and white sneakers.

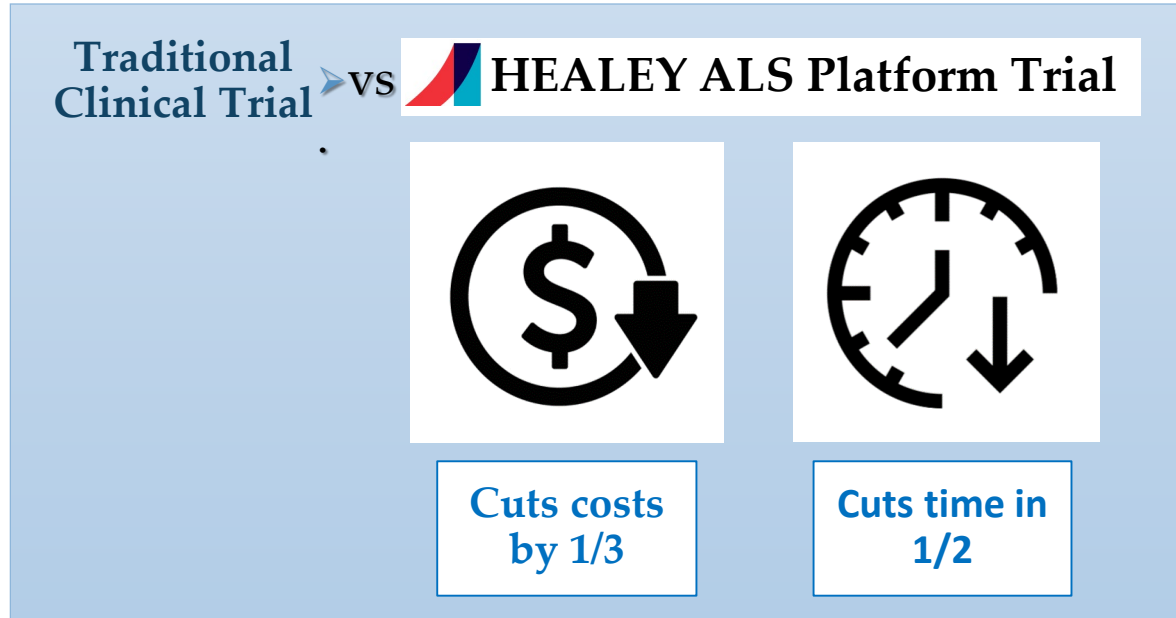
Wife, mother,
friend, inspiration.
Christie's story.

AGENDA

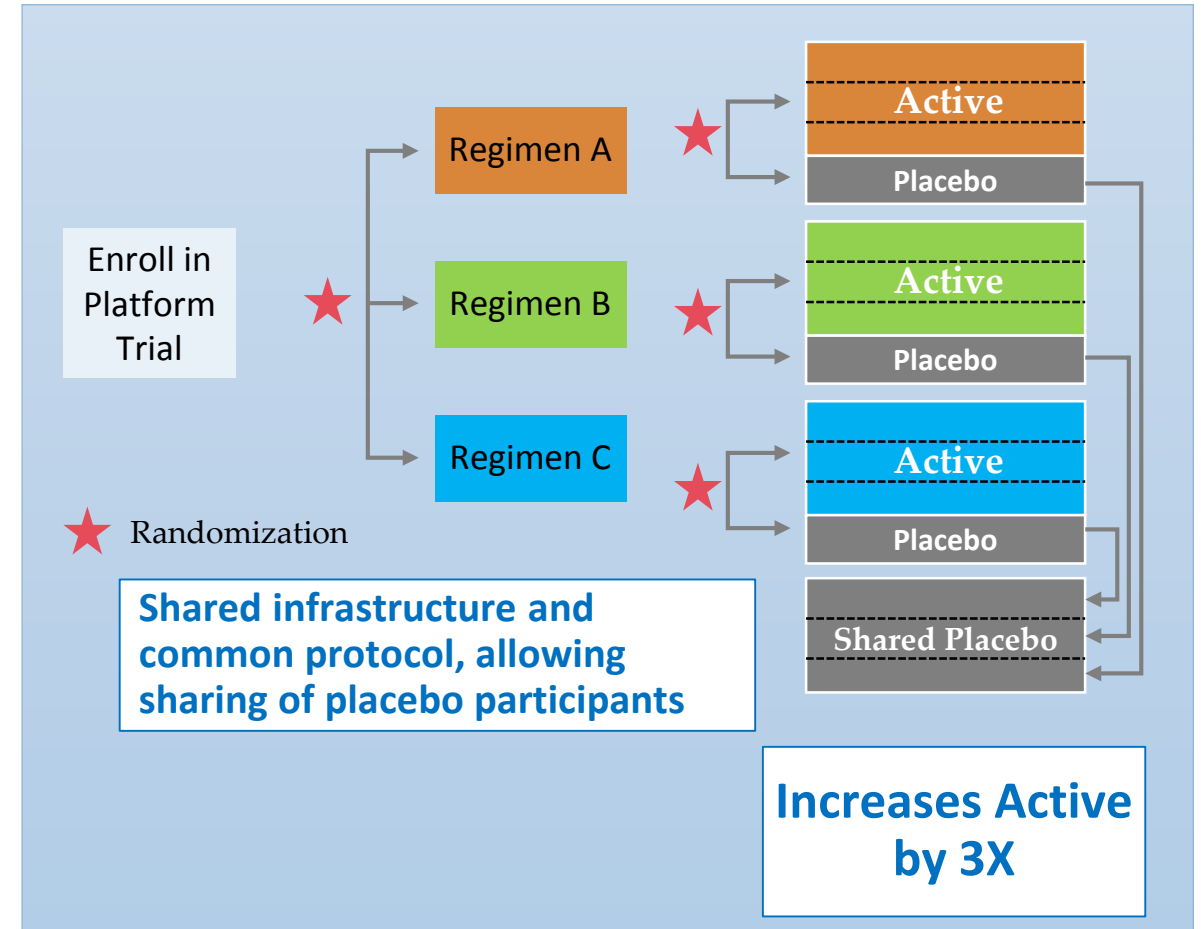
- **Updates on the HEALEY ALS Platform Trial**
 - *Study Treatments*
 - *Enrollment*
 - *Sites*
- **How to stay in touch and find a site near you**

HEALEY ALS Platform Trial

Offers Several Benefits Over Traditional Trials



- Regimen were selected by ALS scientists based on a competitive process



We want to keep adding great therapies

- Submissions accepted on a rolling basis
- We opened with 3 regimens (zilucoplan, verdiperstat, CNM-Au8)
- The 4th regimen will be added in 2020 (pridopidine)
- We are in discussions with 3 additional companies for 2021!

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

Enrollment Updates

- **152** individuals with ALS signed informed consent
- **111** 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)

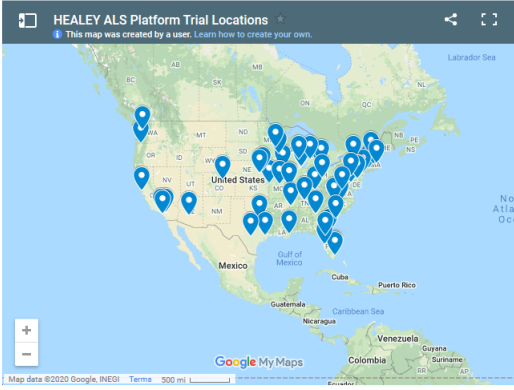
How to Find a Center Near You

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

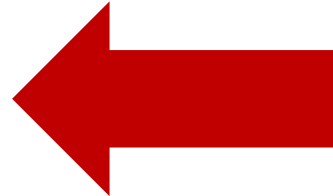


List of Participating Sites by State

Site	State	Contact Information
Barrow Neurological Institute	Arizona	Jessie Duncan

- * 28 sites are actively enrolling
- * A total of 45 sites are already IRB approved
- * Goal is to activate the other sites soon and add more in early 2021!

Contact Info of Participating Sites by State



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

List of enrolling sites as of Oct 29, 2020

28 sites are enrolling

- ✓ Texas Neurology
- ✓ MGH
- ✓ UTHSCSA
- ✓ Hospital for Special Care
- ✓ Holy Cross Hospital
- ✓ Thomas Jefferson
- ✓ Houston Methodist
- ✓ Henry Ford Health System
- ✓ Barrow Neurological Institute
- ✓ Ohio State University
- ✓ Northwestern University
- ✓ University of Chicago
- ✓ Wake Forest
- ✓ University of Nebraska Medical Center
- ✓ Loma Linda
- ✓ University of Washington
- ✓ University of Iowa
- ✓ Washington University
- ✓ University of Pennsylvania
- ✓ University of Michigan
- ✓ Forbes Norris MDA/ALS Research Center, CPMC
- ✓ Penn State Hershey
- ✓ UMass
- ✓ University of Miami
- ✓ University of Colorado
- ✓ Cedars-Sinai
- ✓ University of Florida
- ✓ Columbia University

For More Updates

- **Weekly informal Q&A + 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>

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**](https://www.massgeneral.org/neurology/als/research/platform-trial)