



# 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*

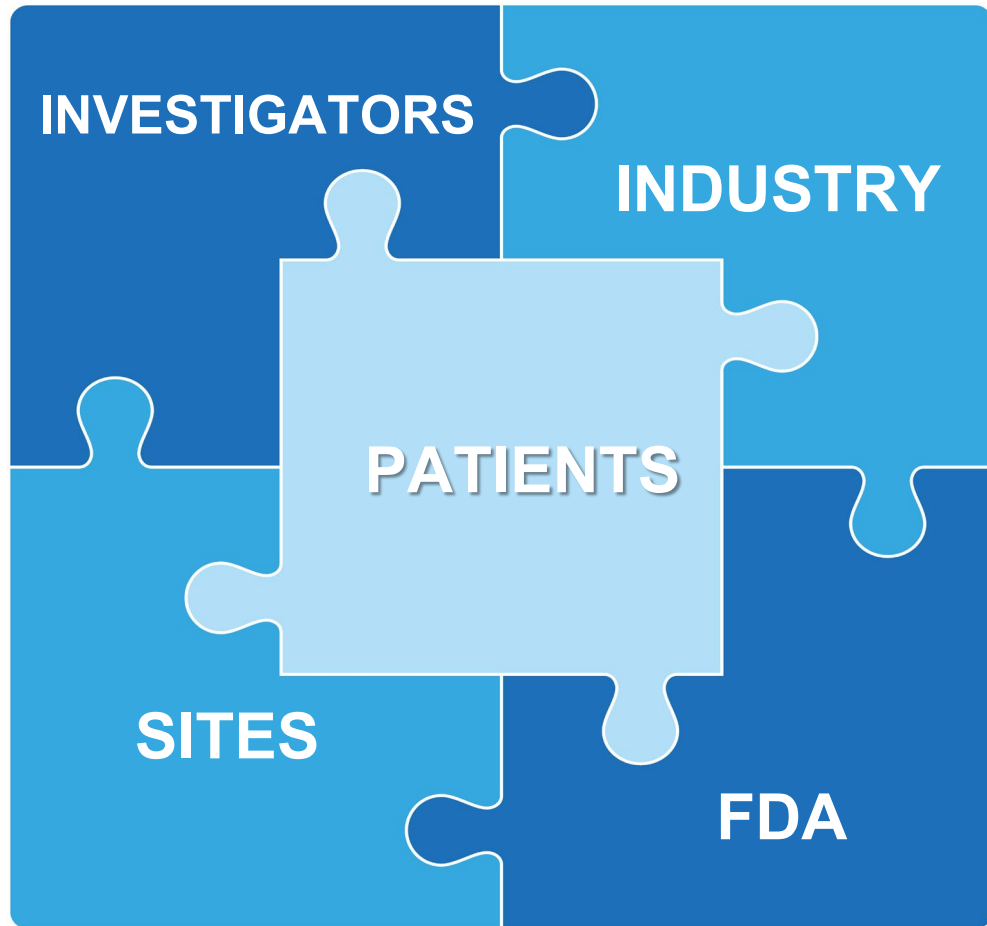


**Peter  
Foss**  
*ALS Finding a Cure*



**Troy  
Green**  
*Tackle ALS*

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



Concept to Launch



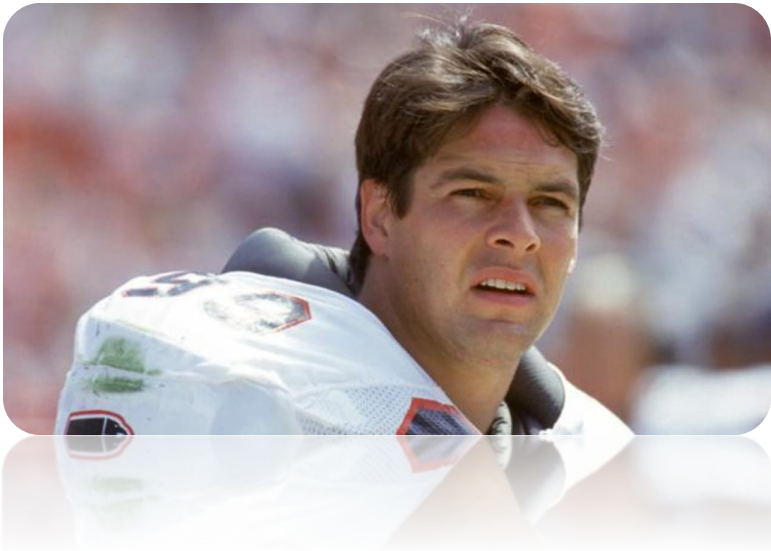
**Healey Center**

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





# als FINDING a CURE®



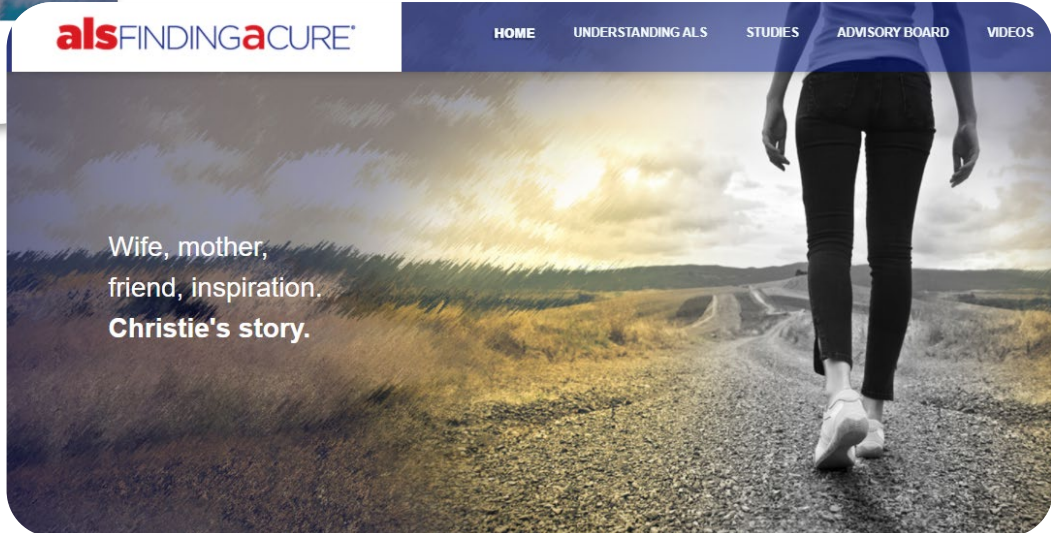
Research brings results.  
Be part of our  
solution.

[READ MORE](#)

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

als FINDING a CURE®

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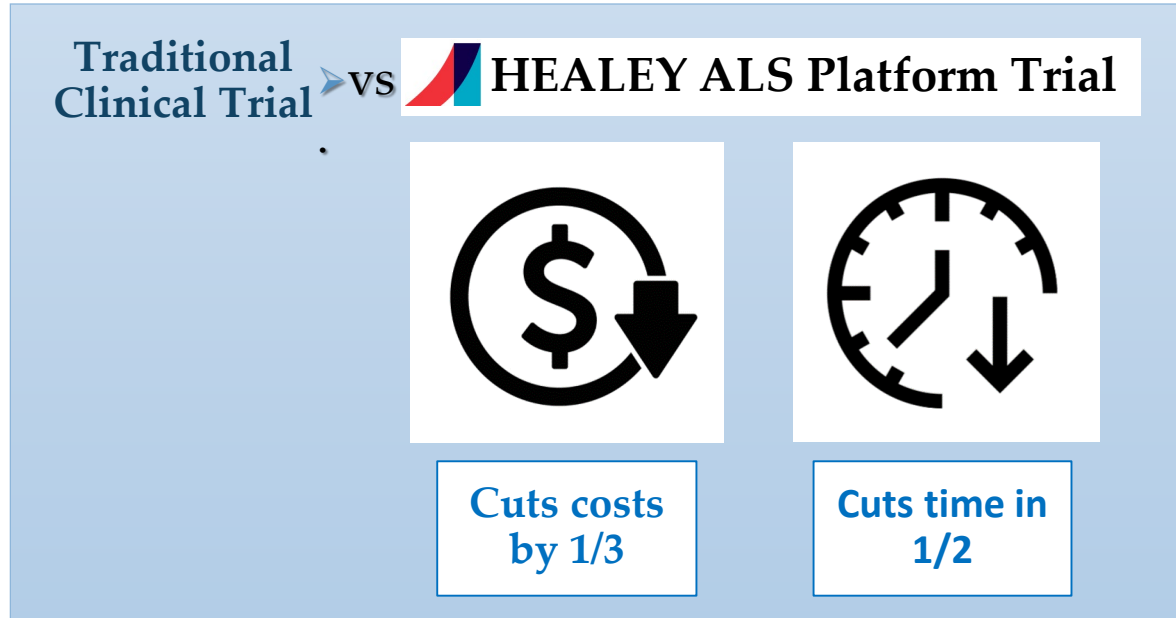
Wife, mother,  
friend, inspiration.  
**Christie's story.**

# AGENDA

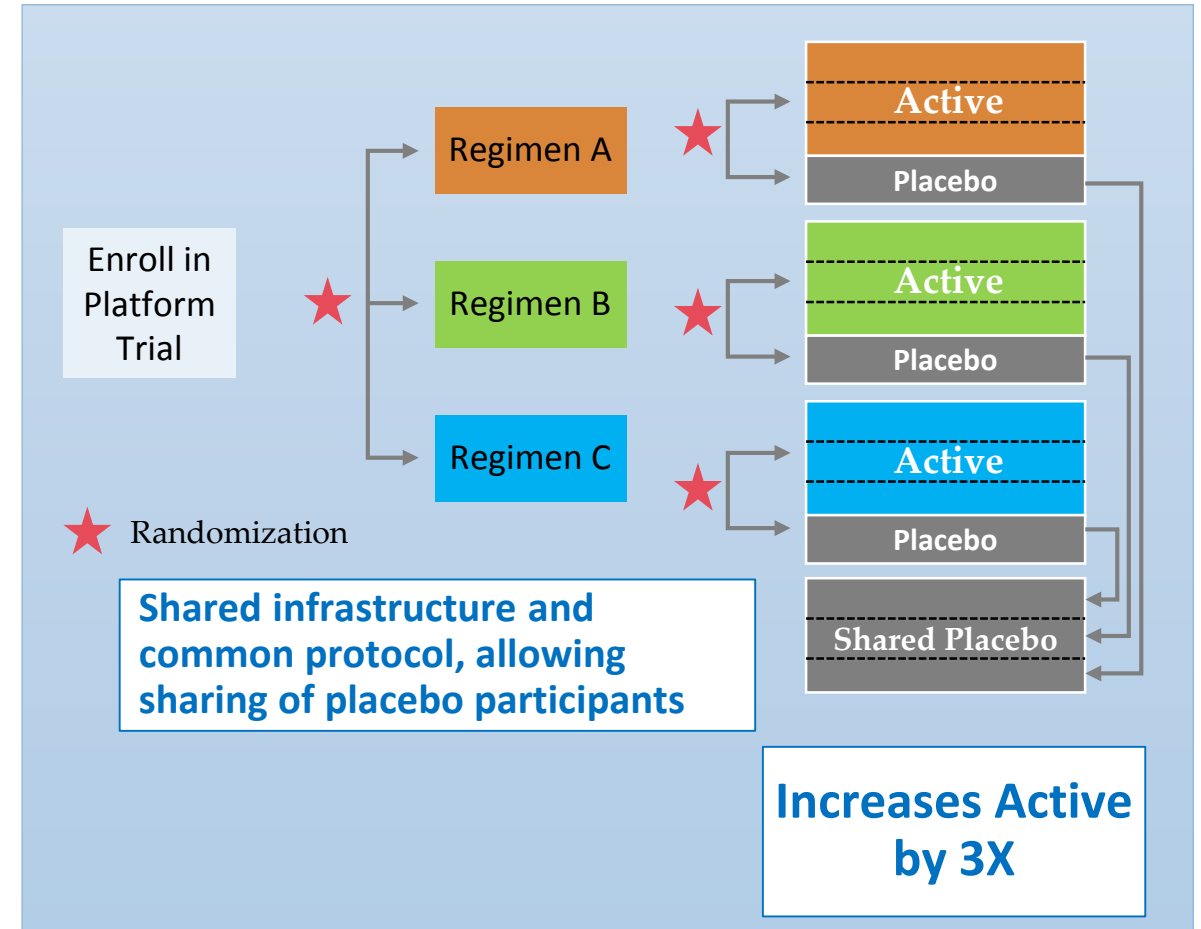
- **Updates on the HEALEY ALS Platform Trial**
  - *Study Treatments*
  - *Enrollment*
  - *Sites*
- **How to stay in touch and find a site near you**

# 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 4<sup>th</sup> 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**

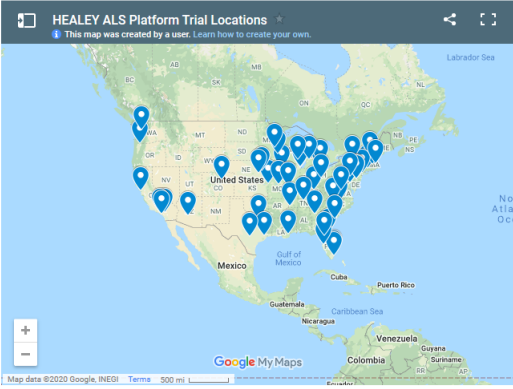
Phone: 833-425-8257 (HALT ALS)

# 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



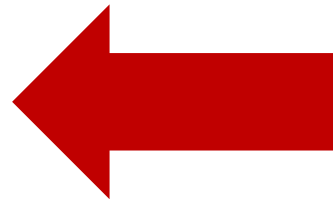
HEALEY ALS Platform Trial Locations  
This map was created by a user. Learn how to create your own.

List of Participating Sites by State

Site	State	Contact Information
Barrow Neurological Institute	Arizona	<a href="#">Jessie Duncan</a>

- \* 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>