

# 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>



# Healey Center

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

## HEALEY ALS Platform Trial

*Updates – Nov 30, 2020*



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



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



**Jennifer  
DiMartino**  
*ALS ONE*

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



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



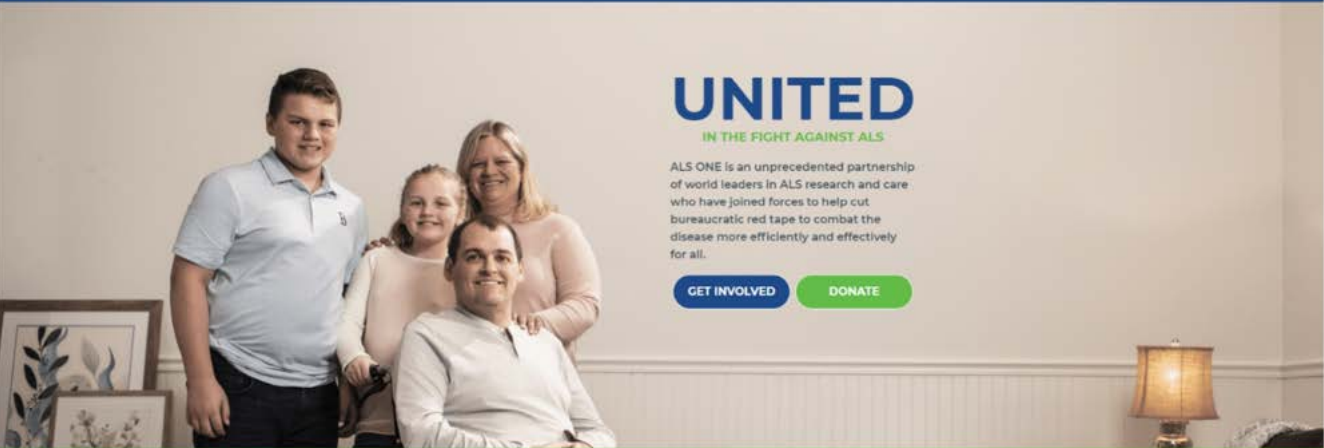
THE ARTHUR M. BLANK  
FAMILY FOUNDATION



# ALS ONE ↑



ALS ONE ↑ Our mission ▾ Our team Events & Initiatives



## UNITED


IN THE FIGHT AGAINST ALS

ALS ONE is an unprecedented partnership of world leaders in ALS research and care who have joined forces to help cut bureaucratic red tape to combat the disease more efficiently and effectively for all.

[GET INVOLVED](#) [DONATE](#)

ALS ONE provides care today, while uniting research for a cure tomorrow


### ALS ONE provides care today, while uniting research for a cure tomorrow



#### Research

A partnership of world-leading ALS researchers focused on a cure


[LEARN MORE](#)



#### Care

Providing care and essential resources for those who need it most

[LEARN MORE](#)



#### Fundraising

Critical funding is necessary to further our united research and care

[LEARN MORE](#)

# AGENDA

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

**YOUR QUESTIONS**

# Accelerating ALS Therapy Development

## Traditional



	Intervention
Disease	Treatment A

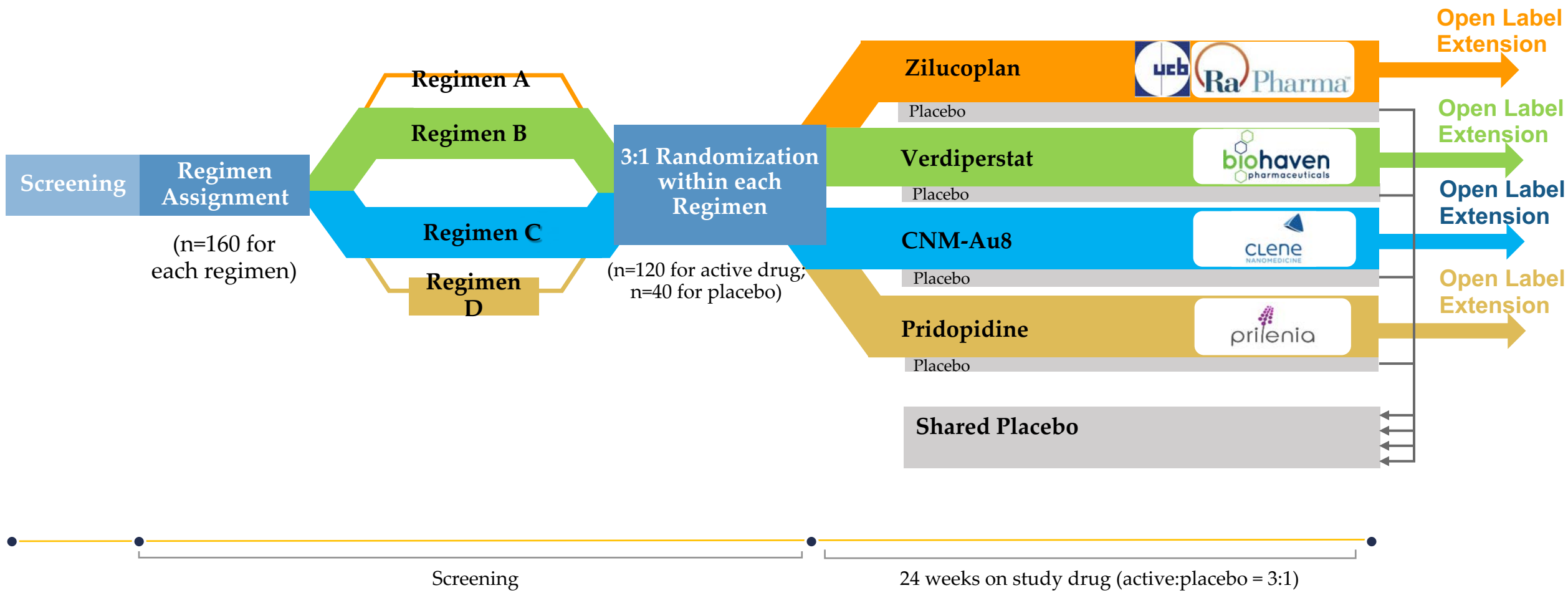


## Platform



	Intervention			
Disease	Treatment A	Treatment B	Treatment C	Treatment D

# Perpetual Adaptive Trial Randomization Ratio 3:1; Shared Placebo Open Label Extension offered



# 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 is being added (pridopidine)**
- **We are in discussions with 3 additional companies for 2021!**

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



# Platform Trial – Target Population

## *Key Inclusion Criteria*

- Sporadic or familial ALS (possible, probable, lab-supported probable, or definite by revised EEC)
- Time since onset of weakness due to ALS  $\leq 3$  years (36 months)
- Slow vital capacity (SVC)  $\geq 50\%$  of predicted capacity for age, height, and sex
- Able to swallow
- Participants must either not take riluzole or be on a stable dose of riluzole for  $\geq 30$  days
- Participants must either not take edaravone or have completed at least one cycle of edaravone

**Sample Size:** 160 per regimen (120 on active and 40 on placebo)

**Treatment Duration:** 24 weeks + Open Label Extension

# Platform Trial – Endpoints

## Primary Endpoint

Change in disease severity - **ALS Functional Rating Scale-Revised (ALSFRS-R) + Mortality**

## Secondary Endpoints

1. Change in respiratory function - slow vital capacity (SVC)
2. Change in muscle strength - hand held dynamometry (HHD)
3. Survival
4. Treatment-specific biomarkers as applicable

## Safety Endpoints

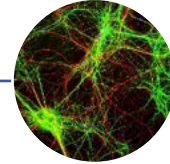
## Exploratory Endpoints

# Exploratory Endpoints

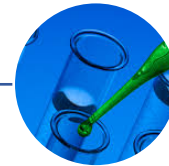
**Endpoint  
Development  
Engine**



**DNA**



**Neurofilaments**



**Biomarkers** (Blood, Urine, CSF)



**Speech Analysis**



**Home Spirometry**

# List of enrolling sites as of Nov 30, 2020

## 31 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
- ✓ University of South Florida
- ✓ Columbia University
- ✓ University of Virginia
- ✓ Emory


# 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.

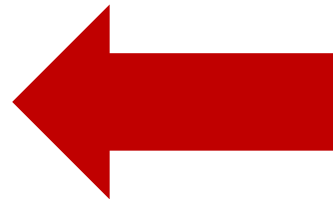


List of Participating Sites by State

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

\* 31 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](mailto:healeyalsplatform@mgh.harvard.edu)

# 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](#)



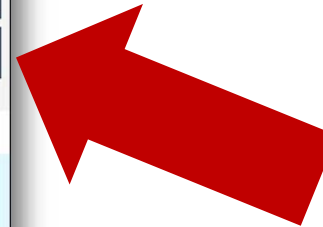
[Enrolling ALS Trials & Studies](#)

[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>

# Enrollment Updates (as of Nov 30, 2020)

- **209** individuals with ALS signed informed consent
- **162** individuals were assigned to a regimen
- **131** are currently receiving study drug (active or placebo)

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



# INITIAL 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 tests)
- **Regional variability means that some sites have experienced different barriers at different times which affected 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 in the spring**
  - Training, contracts, and administrative tasks to enable site activation
- **Protocol amendments made the trial “COVID resilient”**
  - Home spirometry
  - Telemedicine for some visits
  - Home labs for some visits

**Enrollment Started in July 2020 and has been exceeding expectations at sites that were able to open**

# CURRENT COVID-19 IMPACT

- Many centers are experiencing a second surge
  - staff redeployment
  - some institutions are limiting research activities
- Supply chain problems (central vendor)
- Cyberattacks
- Home nursing shortages

# For More Updates

- **Weekly 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/research/platform-trial-news/>

**Coming up: webinars about each drug's mechanism of action and science**