

# HEALEY ALS Platform Trial

Weekly Q&A – Nov 18, 2021



## Healey Center

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



# Guest Speaker

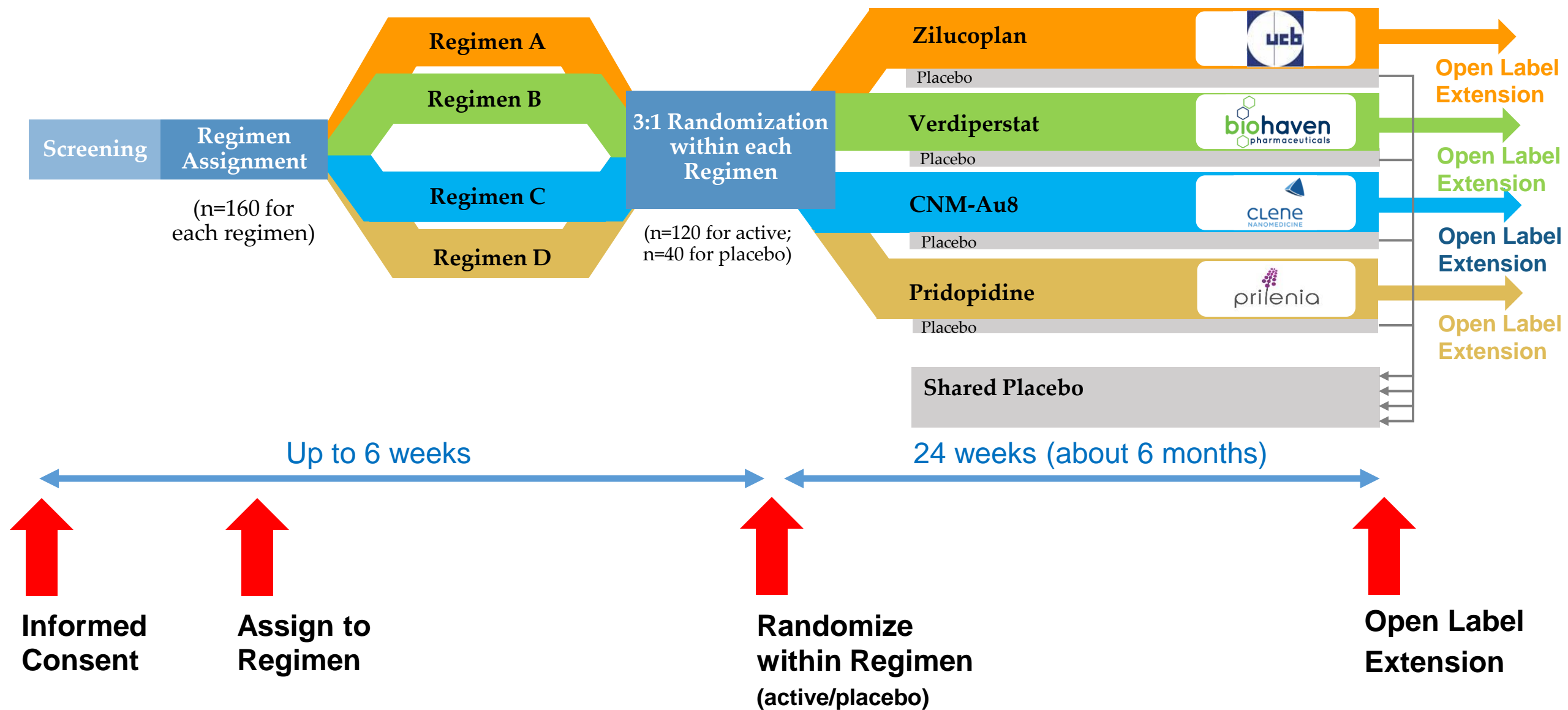
**James Berry, MD, MPH**  
**Massachusetts General Hospital**  
**Regimen co-Lead (Regimen C)**



# Perpetual Adaptive Trial

## Shared Placebo; Randomization Ratio 3:1

### Open Label Extension (OLE) offered



# Endpoints

## Primary Endpoint

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

## 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

## Exploratory Endpoints

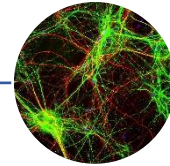
## Safety Endpoints

# Exploratory Endpoints

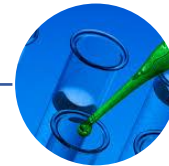
**Endpoint  
Development  
Engine**



**DNA**



**Neurofilaments**



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



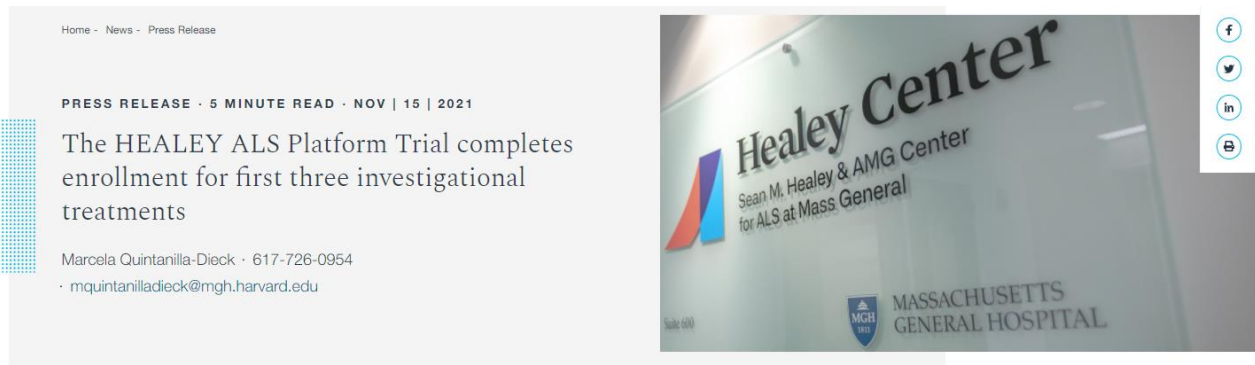
**Speech Analysis**



**Home Spirometry**

# Regimens A, B and C completed enrollment!

- ✓ **162** individuals were randomized within Regimen A
- ✓ **167** individuals were randomized within Regimen B
- ✓ **161** individuals were randomized within Regimen C
- 146** individuals were randomized within Regimen D
- 282** have entered the Open Label Extension (OLE)



*Thank You*

**This breakthrough trial would not be possible without your partnership**

“This is a critical milestone, guiding the timeline for trial completion and result reporting, ultimately putting us on the path forward towards finding treatments for people with ALS.”

Type  
Press Release  
Centers and Departments  
Healey Center for ALS, Neurology  
Topics  
Research

Merit Cudkowicz, MD  
Director, Sean M. Healey & AMG Center for ALS at MGH

# Plans for Expansion: New Sites Coming Soon



- ✓ Atrium Health
- ✓ Cleveland Clinic
- ✓ George Washington University
- ✓ University of California, San Francisco
- ✓ Yale University
- ✓ Indiana University
- ✓ Stony Brook University
- ✓ **Georgetown University**
- ✓ University of Arkansas
- ✓ University of Pittsburgh
- ✓ **University of Southern California**
- ✓ University of Utah
- ✓ Augusta University
- ✓ University of Cincinnati
- ✓ Virginia Commonwealth University
- ✓ Swedish Medical Center
- ✓ Las Vegas Clinic
- ✓ Kaiser, Los Angeles
- ✓ Lehigh Valley Health Network
- ✓ St. Alphonsus Regional Medical Center
- ✓ Hackensack University
- ✓ Essentia Health

Note: Only those sites highlighted in blue are active at this time.

Additional sites will be activated on a rolling basis.

# Patient Navigator



**Catherine Small**

**Allison Bulat**

Phone: 833-425-8257 (HALT ALS)

E-mail: [healeyalsplatform@mgh.harvard.edu](mailto:healeyalsplatform@mgh.harvard.edu)



Eligibility criteria:



Enrolling sites:





# Finding Other Clinical Trial Opportunities

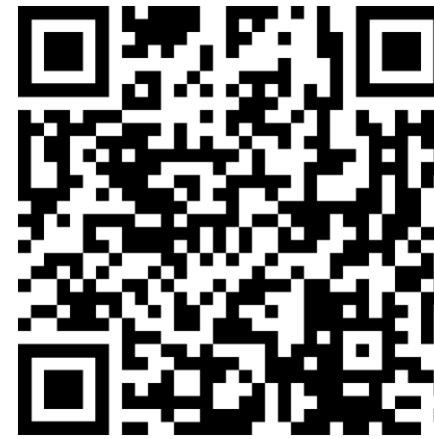
[clinicaltrials.gov](https://clinicaltrials.gov)



[CDC National ALS Registry](#)



[NEALS Website](#)



ALS Clinical Trial  
Liaison contact info  
**(855) 437- 4823**  
**alstrials@neals.org**

[ALS Signal  
from I AM ALS](#)



# Send us webinar ideas!

- Biostatistics
- Get to know our sites



## Upcoming Guest Speakers:

**Nov 25<sup>th</sup>- No Webinar, Happy Thanksgiving!**

**Dec 2<sup>nd</sup>- Neil Thakur, PhD (The ALS Association)**

**Dec 9<sup>th</sup>- Nicholas Olney, MD (Providence ALS Clinic, OR)**

**Dec 16<sup>th</sup>- Lori Chibnik, PhD, MPH (Biostatistics)**

**Dec 23<sup>rd</sup>- No Webinar, Happy Holidays!**

**Dec 30<sup>th</sup>- No Webinar, Happy Holidays!**

# Join the ALS Link!

- Sign up to receive news and updates via email from the Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital
- Get the latest information about ALS clinical trials, clinical care, and webinar opportunities

Home - Neurology - ALS



At the Healey Center for ALS, we are on a quest to discover life-saving therapies for approximately 500,000 people worldwide who are affected by amyotrophic lateral sclerosis (ALS).

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