

# HEALEY ALS Platform Trial

Weekly Q&A – Sept 1, 2022



## Healey Center

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



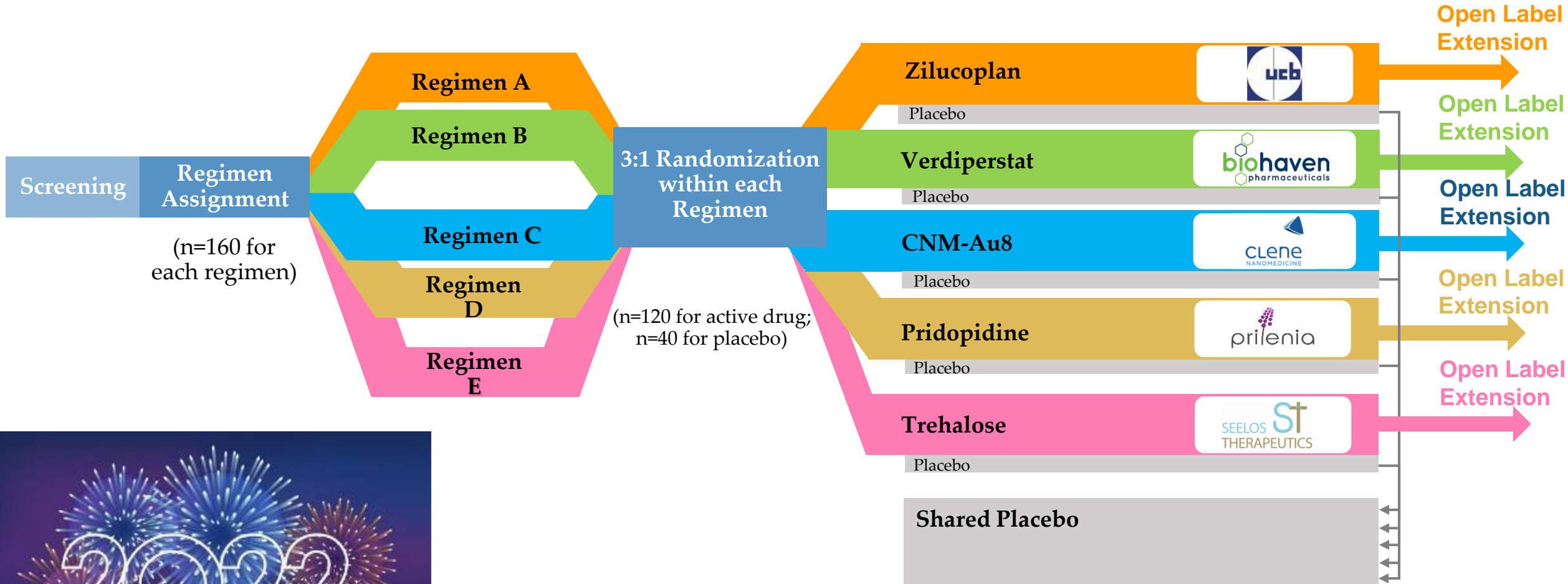
The AMG Foundation

# Guest Speaker

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



# The HEALEY ALS Platform Trial is a Perpetual Adaptive Trial



# 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

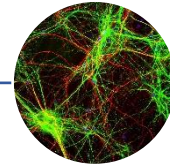
## Safety Endpoints

# Exploratory Endpoints

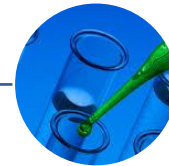
**Endpoint  
Development  
Engine**



**DNA**



**Neurofilaments**



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



**Speech Analysis**



**Home Spirometry**

# Enrollment Updates (as of Sept 1, 2022)

- **131** individuals have signed informed consent
- **88** individuals have been randomized within Regimen E

*Thank You*

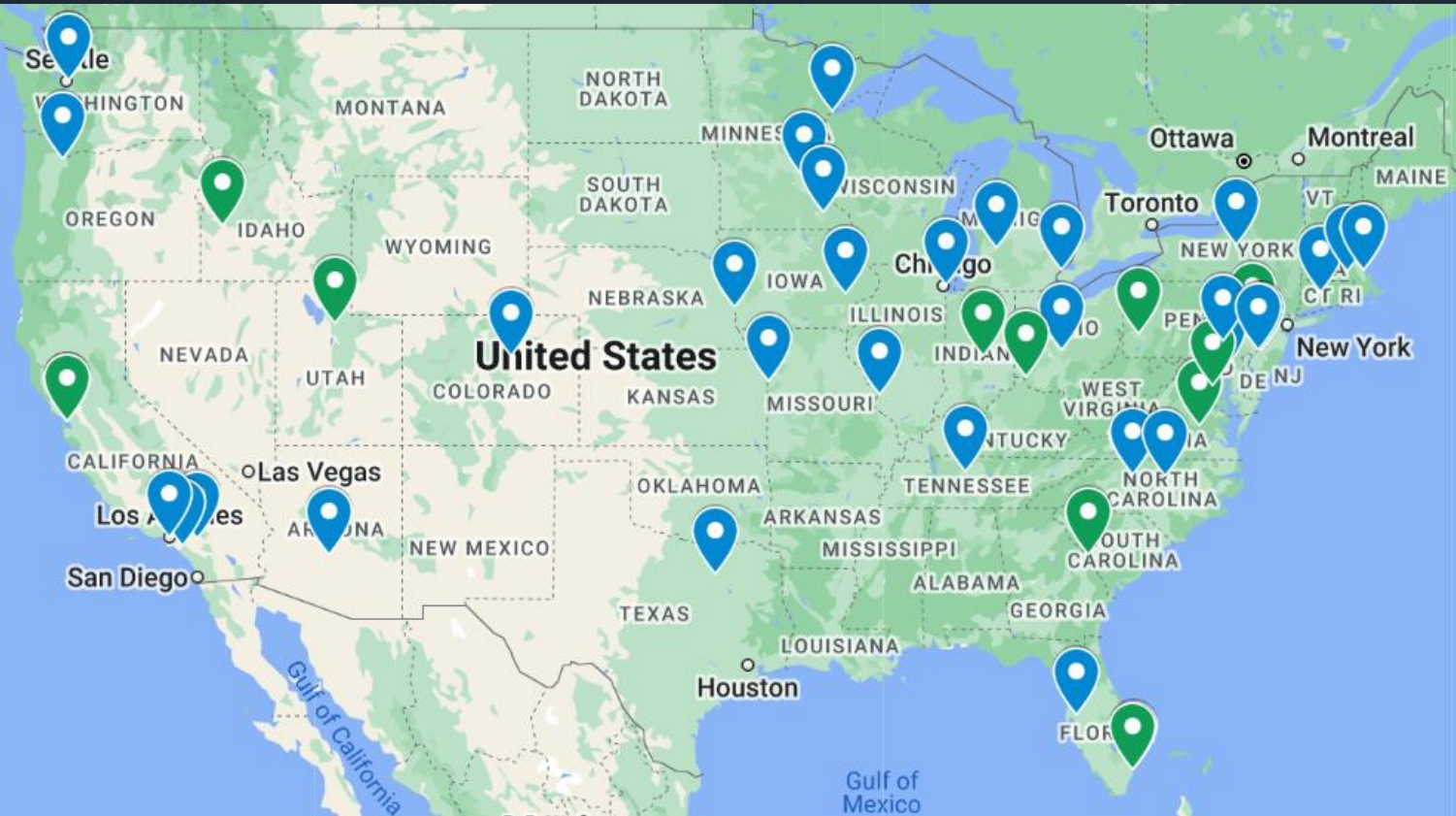
**This breakthrough trial would not be possible without your participation**

Your **partnership** in research is what keeps us filled with passion, dedication, and the commitment to uncover new promising treatments for ALS

**Every research participant, whether on the active drug or placebo, plays a critical role in making the hope of finding a cure for ALS a reality**



# 47 Sites Currently Activated for Regimen E



(as of 9/1/22)

*Sites in blue participated in previous regimens. Sites in green (underlined to the side) are new additions to the Platform Trial!*

- Lehigh Valley Health Network
- Mass General Hospital
- University of Kansas
- University of Maryland
- California Pacific Medical Center
- Northwestern University
- Virginia Commonwealth University
- University of Nebraska
- Washington University
- Wake Forest University
- Hospital for Special Care
- Saint Alphonsus Regional
- University of Massachusetts
- Duke University
- Barrow Neurological Institute
- Georgetown University
- Texas Neurology
- Beth Israel Deaconess Medical Center
- SUNY Upstate
- Spectrum Health
- Henry Ford Hospital
- Essentia Health
- University of Southern California
- University of South Florida
- University of Colorado
- Providence Brain and Spine
- University of Minnesota
- Loma Linda University
- University of Iowa
- Swedish Medical Center
- Ohio State University
- University of Cincinnati
- Thomas Jefferson University
- UC San Francisco
- Mayo Rochester
- University of Washington
- Vanderbilt University
- UPMC
- Indiana University
- Augusta University
- University of Utah
- Holy Cross Hospital
- Penn State Hershey
- University of CA, Irvine
- Cedars Sinai Medical Center
- University of Pennsylvania
- Nova Southeastern University

Site Map & Contacts:



<https://bit.ly/3g2NZr5>

# Patient Navigation

## Central resource for people living with ALS



Catherine Small

Phone: 833-425-8257 (HALT ALS)

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

Weekly webinar  
registration:



<https://bit.ly/3r6Nd2L>

ALS Link sign-up:



<https://bit.ly/3o2Ds3m>



Allison Bulat

### Upcoming Guest Speakers:

**September 8<sup>th</sup>**- HEALEY ALS Platform Trial Update

**September 15<sup>th</sup>**- Tawfiq Al-Lahham, MD (Site Investigator at UPMC in PA)

**September 22<sup>nd</sup>**- Mariah Connolly and Meghan Hall (Site Monitors for the Platform Trial)