

HEALEY ALS Platform Trial

Weekly Q&A – Sept 1, 2022

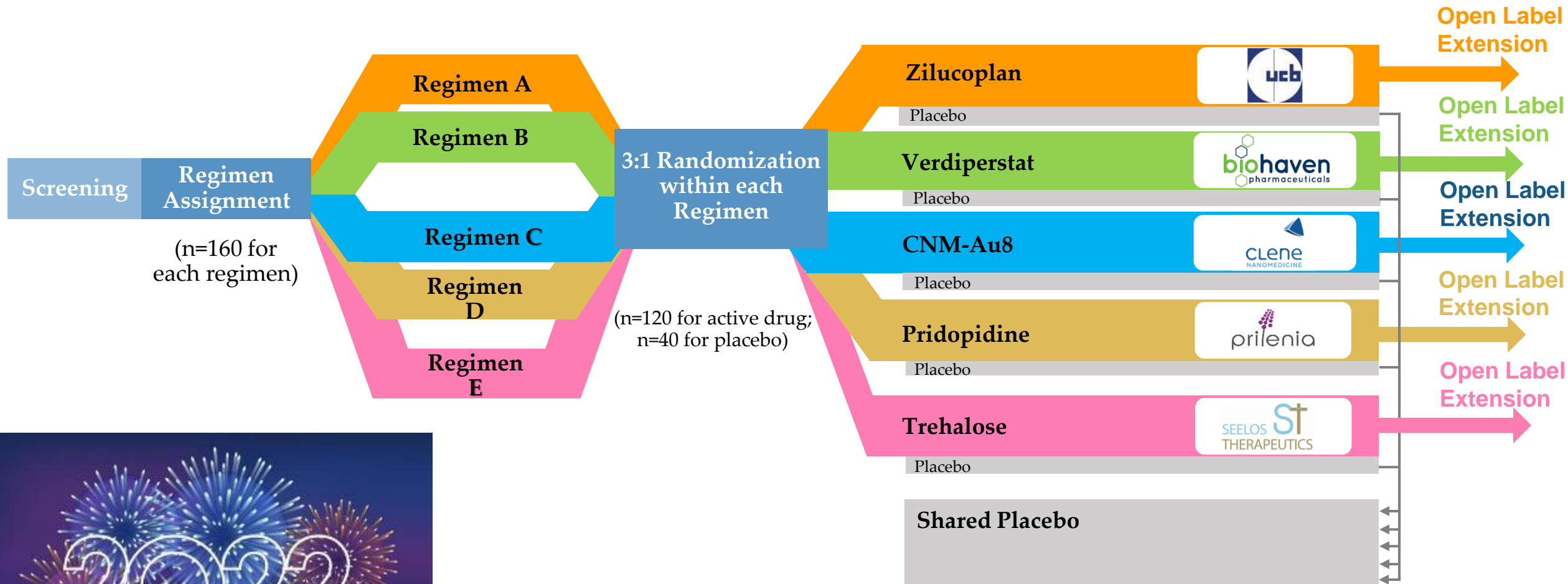


Guest Speaker

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

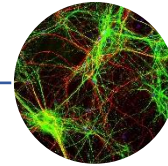
Safety Endpoints

Exploratory Endpoints

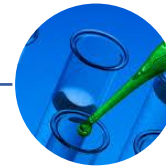
**Endpoint
Development
Engine**



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

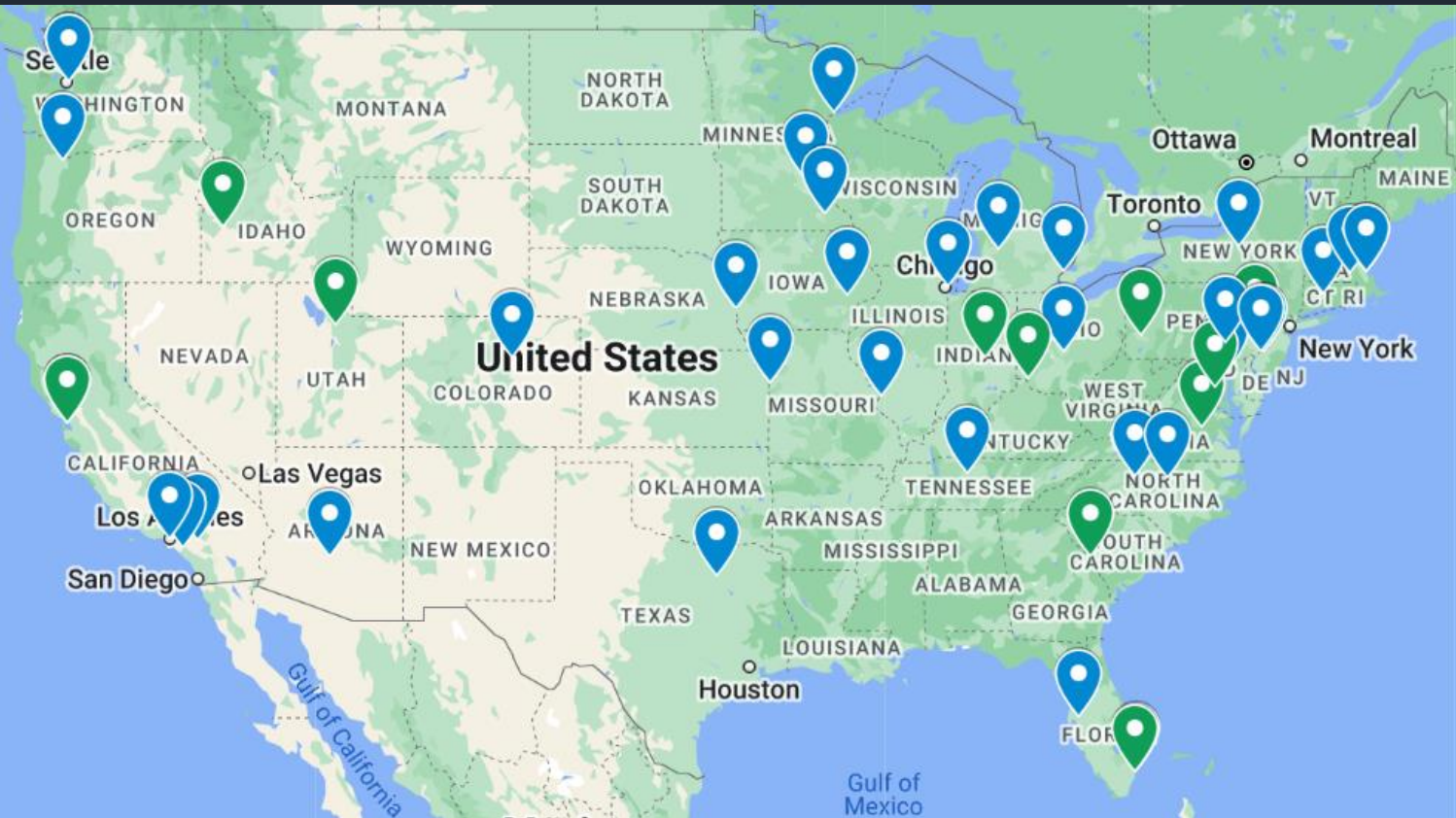
Thank You

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

Weekly webinar
registration:



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

ALS Link sign-up:



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

Upcoming Guest Speakers:

September 8th- HEALEY ALS Platform Trial Update

September 15th- Tawfiq Al-Lahham, MD (Site Investigator at UPMC in PA)

September 22nd- Mariah Connolly and Meghan Hall (Site Monitors for the Platform Trial)



Allison Bulat