

Thank you for joining the weekly webinar!

We are admitting audience members from the waiting room.

Please allow a few moments for the webinar to begin.



HEALEY ALS Platform Trial

Weekly Q&A – September 28, 2023



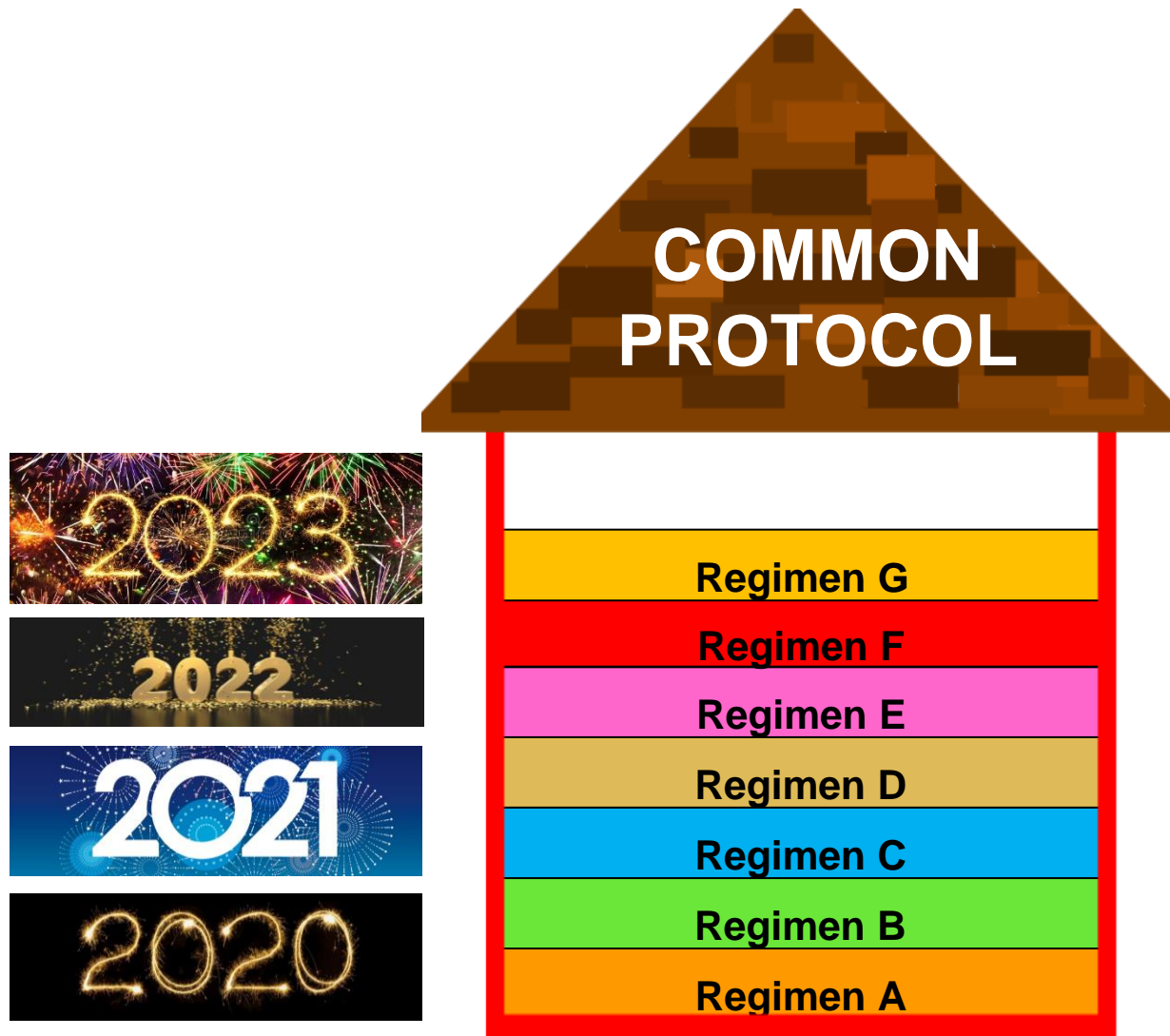
Healey & AMG Center

Sean M. Healey & AMG Center for ALS
at Massachusetts General Hospital



The AMG Foundation

Common Protocol and Shared Infrastructure Allow for Operational and Scientific Efficiencies

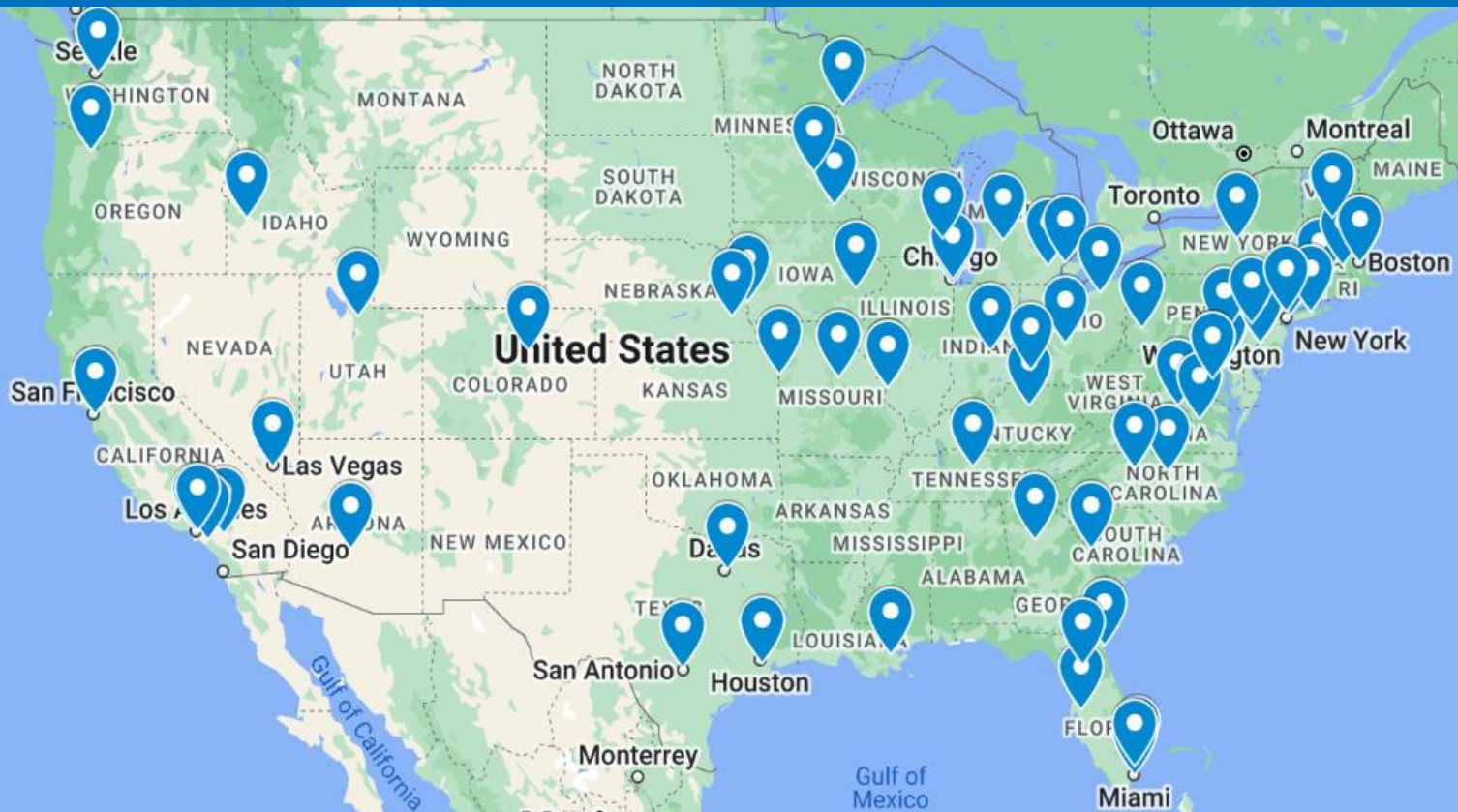


**1 Protocol
(Phase 2/3)
1 single IRB
Central Governance**

**7 Regimens
70+ Enrolling Sites
~1300 Participants**

Regimen: Active Study Drug + Matching Placebo

Providing research access across a diverse network of 70+ NEALS sites



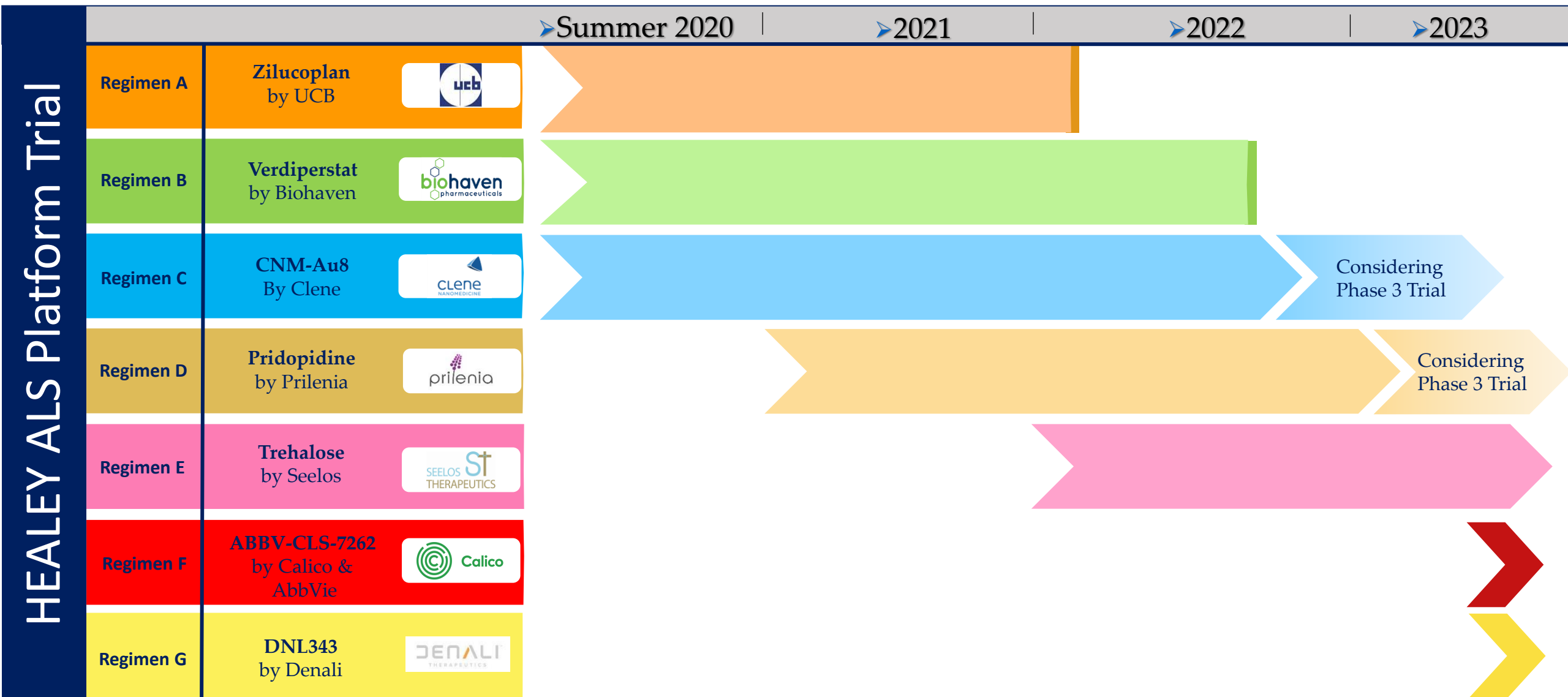
- ✓ Texas Neurology
- ✓ Mass General Hospital
- ✓ UTHSCSA
- ✓ Hospital for Special Care
- ✓ Holy Cross Hospital
- ✓ Thomas Jefferson
- ✓ Houston Methodist
- ✓ Henry Ford Health System
- ✓ Barrow Neurological Institute
- ✓ Ohio State University
- ✓ Northwestern University
- ✓ University of Chicago
- ✓ Wake Forest
- ✓ University of Nebraska
- ✓ Loma Linda University
- ✓ University of Washington
- ✓ University of Iowa
- ✓ Washington University
- ✓ University of Pennsylvania
- ✓ University of Michigan
- ✓ California Pacific Medical Center
- ✓ Penn State Hershey
- ✓ UMass Worcester
- ✓ University of Miami
- ✓ University of Colorado
- ✓ Cedars-Sinai
- ✓ University of Florida
- ✓ University of South Florida
- ✓ Columbia University
- ✓ University of Virginia
- ✓ Emory University
- ✓ University of Maryland
- ✓ SUNY Upstate
- ✓ Beth Israel Deaconess
- ✓ Temple University
- ✓ Dartmouth-Hitchcock
- ✓ Medical College of Wisconsin
- ✓ Spectrum Health
- ✓ University of Missouri
- ✓ University of Minnesota
- ✓ Johns Hopkins University
- ✓ University of CA Irvine
- ✓ University of Kansas
- ✓ Vanderbilt University
- ✓ University of Kentucky
- ✓ Mayo Rochester
- ✓ Duke University
- ✓ Neurology Associates
- ✓ Ochsner Health System
- ✓ Mayo Clinic Florida
- ✓ St. Louis University
- ✓ Providence Brain and Spine
- ✓ Georgetown University
- ✓ University of Southern California
- ✓ Cleveland Clinic
- ✓ George Washington University
- ✓ University of California, San Francisco
- ✓ Indiana University
- ✓ Stony Brook University
- ✓ University of Pittsburgh
- ✓ 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
- ✓ Nova Southeastern University



NEALS

Northeast Amyotrophic
Lateral Sclerosis
Consortium®

The HEALEY ALS Platform Trial is a perpetual trial to provide decisive answers and direction with efficient execution



Many people living with ALS cannot participate in clinical trials

Expanded Access

FDA Definition:

- Sometimes called “compassionate use”
- Potential pathway for a patient with an immediately life-threatening or serious disease or condition
- Allows access to an investigational medical product outside of clinical trials
- Used when no comparable or satisfactory alternative therapy available

<https://www.fda.gov/news-events/public-health-focus/expanded-access>

Categories of Expanded Access Protocols (EAPs)

Individual Patient, including emergency use

Intermediate-size Patient Populations

Treatment Protocol (large patient population)

Requirements for All EAPs

- Patient has a **serious** or **immediately life-threatening** disease or condition; no comparable or satisfactory alternative therapy
- **Potential benefit justifies potential risk**; potential risk not unreasonable in context of disease or condition
- Providing drug will **not interfere with clinical trials** that could support marketing approval of the expanded access use or otherwise compromise the potential development of the expanded access use

Community Support to EAPs

EAP COMPANION PROGRAM SUPPORTERS

- Healey Center for ALS
- Clearing Corporation Charitable Foundation
- Eddie and Jo Allison Smith Family Foundation
- Richard Stravitz Foundation
- I AM ALS
- Biohaven
- Clene
- Prilenia
- Elliott & Frantz, Inc.
- **Community Fundraisers**
 - Tackle ALS – Team Change ALS
 - Ellen Corindia’s Fundraiser
 - 2019 Olson Cornhole Tournament
 - 2019 Worthington Fore ALS
 - 2020 and 2021 Fishing for ALS Warriors
 - 2020 and 2021 sALSa For a Cure Pick Your Own Path Walk

- 2020 and 2021 Lori’s Shoes "Hope Is In the Bag"
- 2021 and 2022 MLB Lou Gehrig Day 4-ALS
- 2021 Russ Pallesen Fundraiser for EAP
- 2021 Voices for ALS Golf Tournament
- 2021 The Martha Olson-Fernandez Foundation Golf Tournament
- 2021 Gwendolyn Strong Walk
- TechVs.ALS

• **Big thanks to countless individual contributors**



Healey Center

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

Never Surrender Inc.
Funding the Fight Against ALS



TECH Vs ALS

Benefiting the Sean M. Healey and AMG Center for ALS

ACT for ALS- A new opportunity to expand access and collect real world data in parallel to clinical trials via EAP

Signed into law on Dec 23, 2021

Grants for Research on Therapies via Intermediate-Size EAPs for ALS

- NIH U01 grant mechanism
- MGH Healey Center received the first grant
- Trehalose companion EAP to occur in parallel to Regimen E of the HEALEY ALS Platform Trial

PUBLIC LAW 117-79—DEC. 23, 2021

135 STAT. 1533

Public Law 117-79
117th Congress

An Act

To direct the Secretary of Health and Human Services to support research on, and expanded access to, investigational drugs for amyotrophic lateral sclerosis, and for other purposes.

Dec. 23, 2021
[H.R. 3537]

Be it enacted by the Senate and House of Representatives of the United States of America in Congress assembled,

SECTION 1. SHORT TITLE.

This Act may be cited as the “Accelerating Access to Critical Therapies for ALS Act”.

Accelerating
Access to
Critical
Therapies for
ALS Act.
21 USC 301 note.

SEC. 2. GRANTS FOR RESEARCH ON THERAPIES FOR ALS.

(a) IN GENERAL.—The Secretary of Health and Human Services (referred to in this section as the “Secretary”) shall award grants to participating entities for purposes of scientific research utilizing data from expanded access to investigational drugs for individuals who are not otherwise eligible for clinical trials for the prevention, diagnosis, mitigation, treatment, or cure of amyotrophic lateral sclerosis. In the case of a participating entity seeking such a grant, an expanded access request must be submitted, and allowed to proceed by the Secretary, under section 561 of the Federal Food, Drug, and Cosmetic Act (21 U.S.C. 360bbb) and part 312 of title 21, Code of Federal Regulations (or any successor regulations),

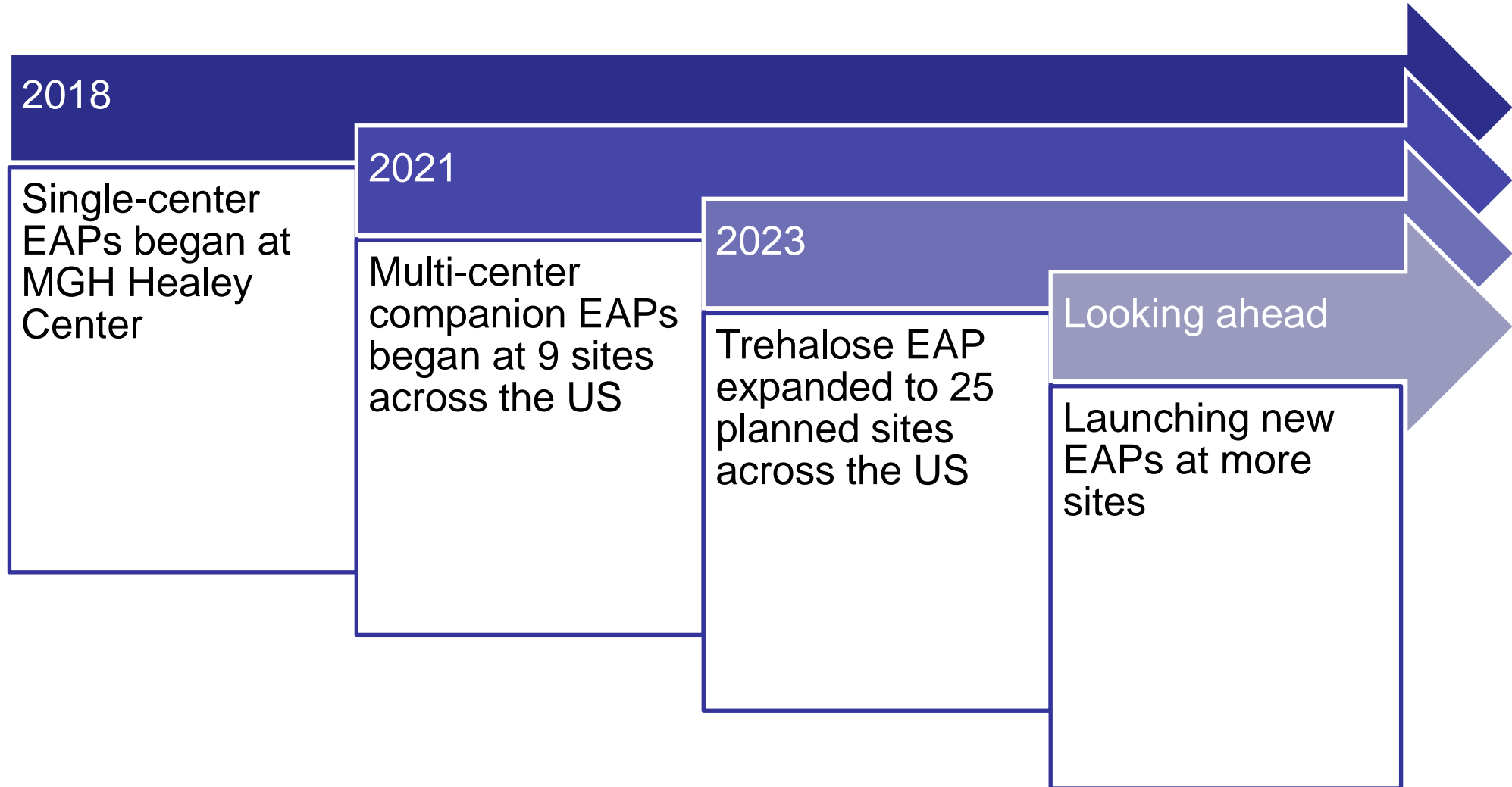
21 USC 360ee
note.

Multi-PIs – Healey Center for ALS at MGH

- **Suma Babu, MBBS, MPH**
 - Assistant Professor of Neurology, Harvard Medical School
- **James Berry, MD, MPH**
 - Winthrop Family Scholar in ALS Sciences
 - Averill Healey Endowed Chair in ALS
 - Director, MGH Neurological Clinical Research Institute (NCRI)
- **Sabrina Paganoni, MD, PhD**
 - Associate Professor of PM&R, Harvard Medical School
 - Co-Director, MGH Neurological Clinical Research Institute (NCRI)



Healey EAP Companion Program Growth

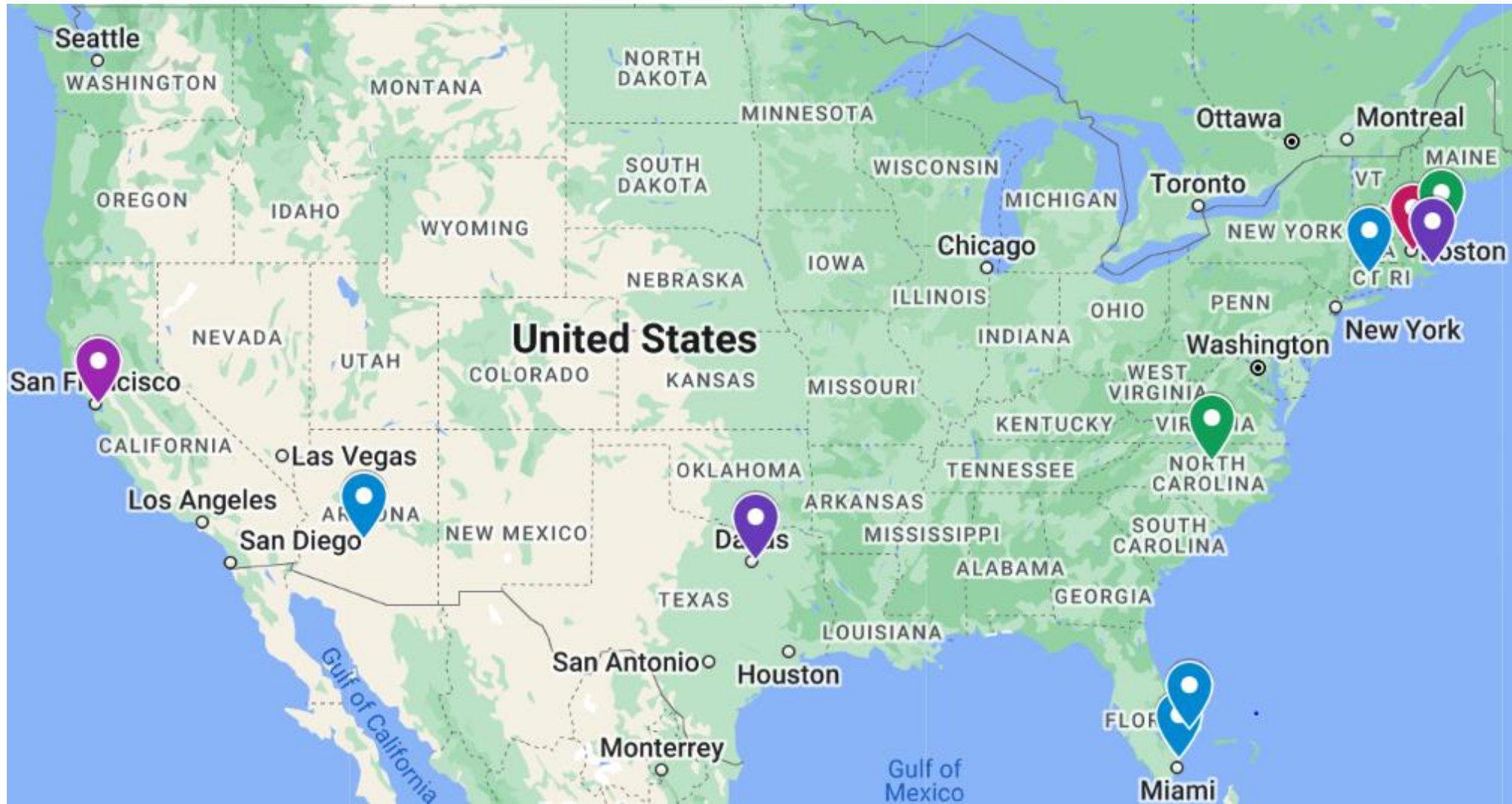


Healey EAP Companion Program – 2018



Key
MGH Healey Center EAPs

Healey EAP Companion Program – 2021



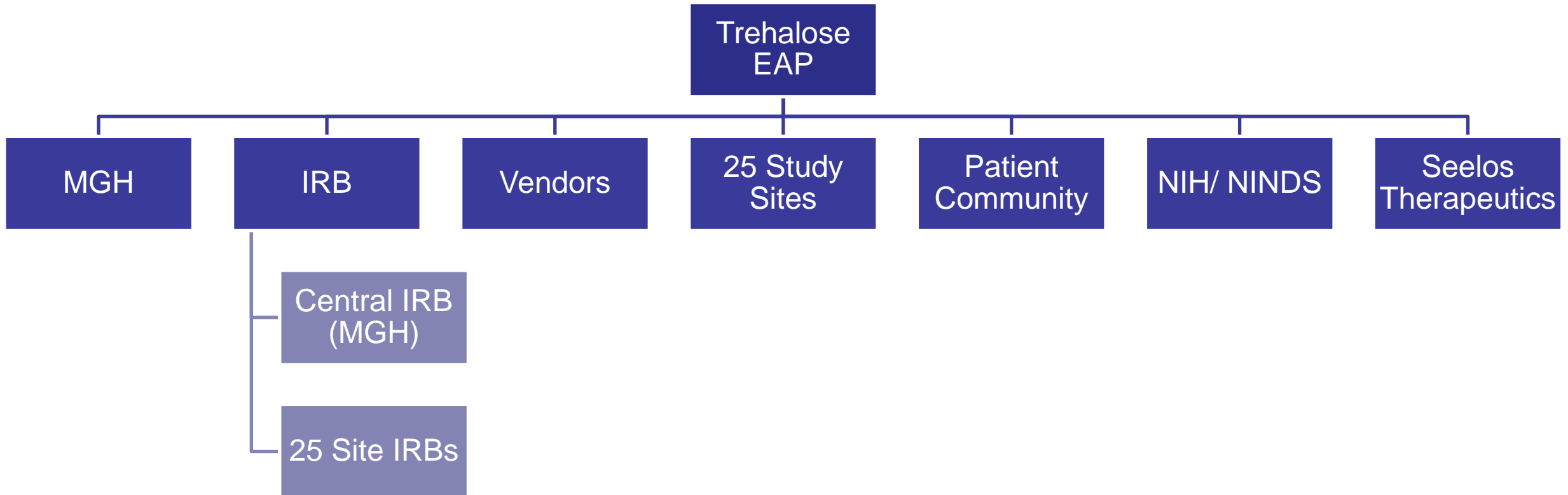
Key
MGH Healey Center EAPs
Verdiperstat EAP
CNM-Au8 EAP
Pridopidine EAP

Healey EAP Companion Program – 2023



Key
MGH Healey Center EAPs
Verdiperstat EAP
CNM-Au8 EAP
Pridopidine EAP
Trehalose EAP - Activated
Trehalose EAP - In start up

Trehalose EAP Overview



Trehalose EAP Overview



This EAP may be impacted by results of the Healey ALS Platform Trial RGE

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 Webinars:

October 5th- canceled for NEALS conference

October 12th- weekly Q&A



Allison Bulat