Platform Patient Webinar

Trehalose EAP

An Expanded Access Protocol of Intravenous Trehalose Injection 90.5 mg/mL Treatment of Patients with Amyotrophic Lateral Sclerosis

July 27, 2023
5:00 – 5:30pm EST

NIH National Institute of Neurological Disorders and Stroke

SEELOS THERAPEUTICS

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

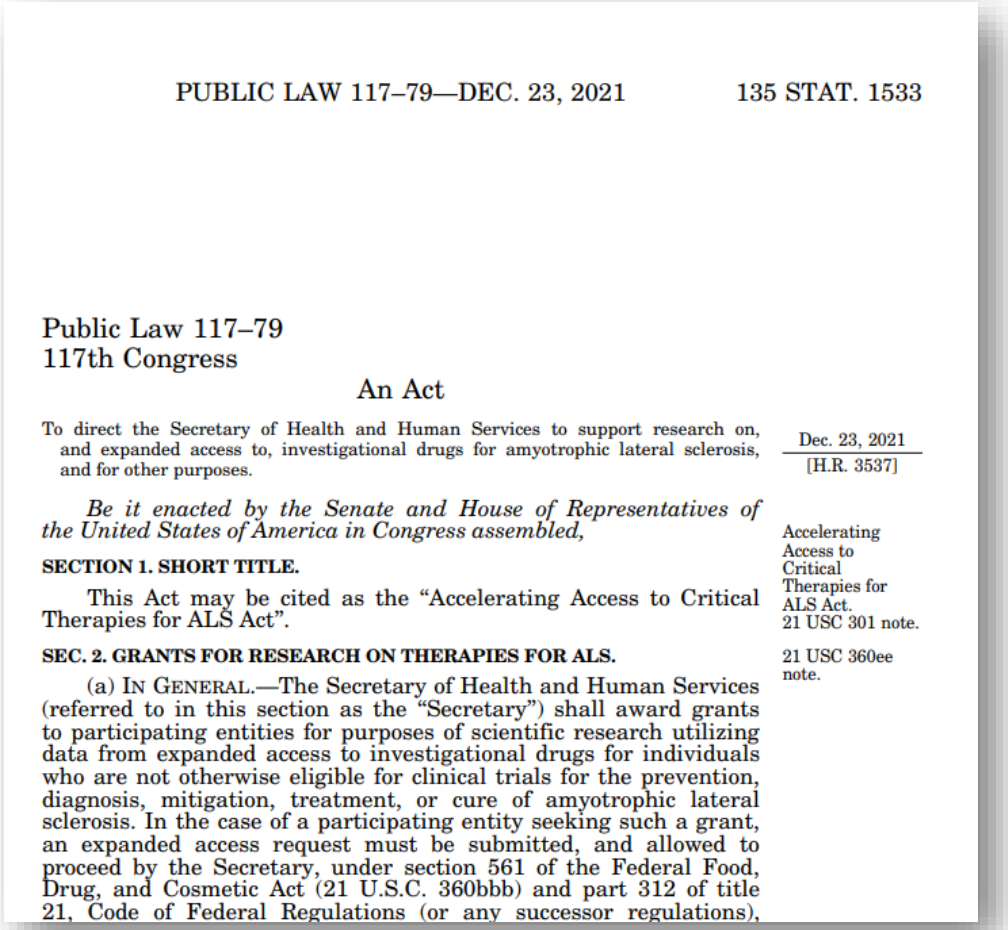
MGH 1811 MASSACHUSETTS GENERAL HOSPITAL
NEUROLOGICAL CLINICAL RESEARCH INSTITUTE

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 receives the first grant
- Trehalose companion EAP will occur in parallel to Regimen E of the HEALEY ALS Platform Trial
- PIs: Babu, Berry, Paganoni
- 25 sites
- 70 ALS participants
- FPFV in Q1 2023



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,

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

SECTION 1. SHORT TITLE.

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

21 USC 360ee 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),

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
  - Co-Director, MGH Neurological Clinical Research Institute (NCRI)

MASSACHUSETTS GENERAL HOSPITAL
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Study Design

- Planned enrollment: 70 pALS at up to 25 sites
- Weekly IV infusions of trehalose, 90.5 mg/mL, at a dose of 0.75 g/kg
- Infusions may take place at the study center or at home

Cohort 1 (Trehalose Naïve)

- Patients who do not qualify for any reasonably accessible ongoing clinical trial.

Cohort 2 (RGE OLE Rollover)

- Patients who have completed Regimen E of the HEALEY ALS Platform Trial and are not eligible for enrollment in another treatment regimen of the platform study.

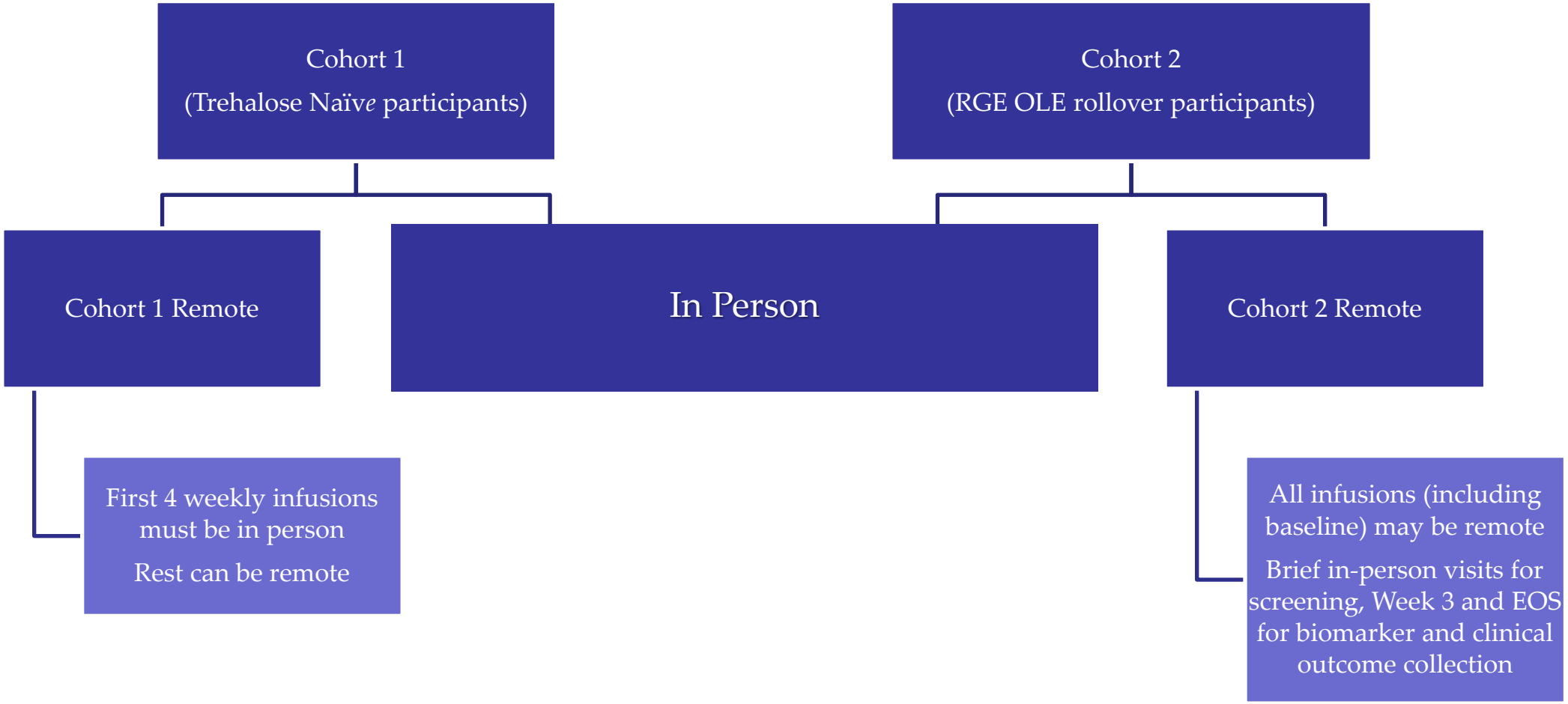
Inclusion Criteria

1. Sporadic or familial ALS.
2. Age 18 years or older.
3. Cohort 1: Patients who do not qualify for any reasonably accessible ongoing clinical trial.
4. Cohort 2: Patients who have completed Regimen E and the open label extension (OLE) period of the HEALEY ALS Platform Trial, or completed Regimen E of the HEALEY ALS Platform Trial if the OLE is not available at the site, and are not eligible for enrollment in another treatment regimen of the platform study.
5. Capable of providing informed consent and complying with study procedures, in the Site Investigator's (SI's) opinion.
6. Participants have established care with a physician at a specialized ALS center involved in the study and will maintain this clinical care throughout the duration of the EAP.
7. Participants must have a life expectancy of at least 6 months in SI's opinion.

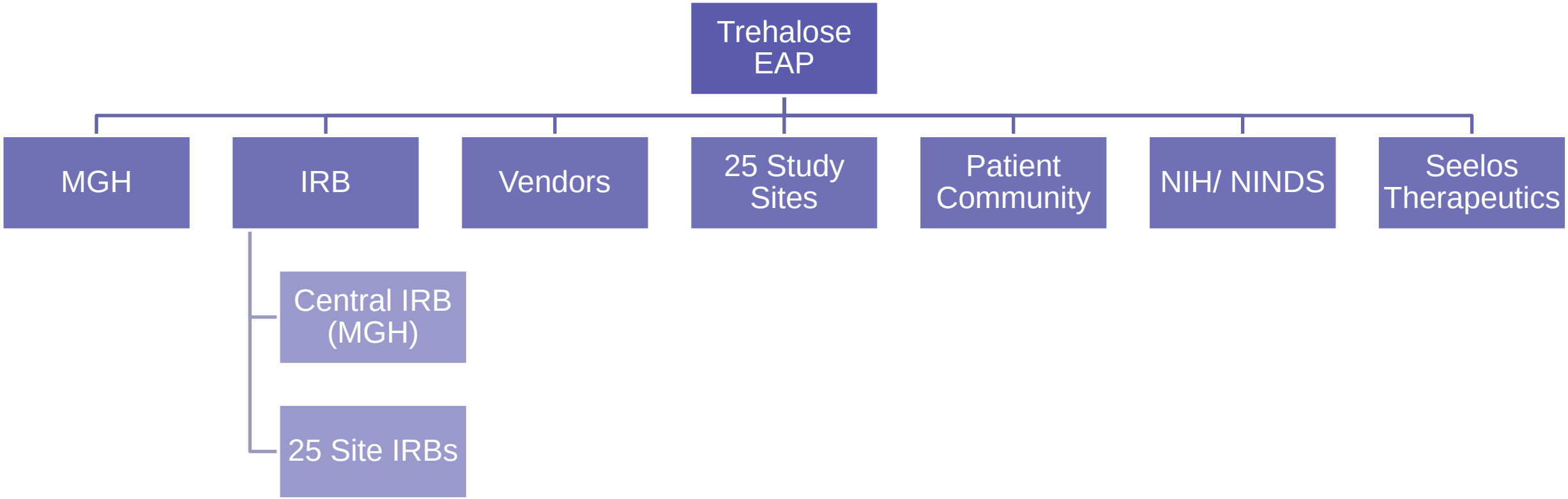
Exclusion Criteria

1. Current diagnosis or healthcare professional-recommended treatment (medication, exercise or diet) of diabetes mellitus.
2. Screening glucose >=140 mg/dl.
3. Known hypersensitivity to trehalose.
4. Current use of oral trehalose.
5. Inability for participant to return to site for weekly drug administration, until approved for home infusions.
6. Screening body weight >144 kilograms.
7. Participant with a history of any clinically significant or unstable medical condition or lab abnormality based on the SI's judgment that may interfere with assessment of the study objectives, with safety or full participation.
8. Females who are pregnant or nursing or who plan to get pregnant during the course of the EAP.
9. Females of child-bearing potential, or males, who are unwilling or unable to use highly-effective methods of birth control.
10. Use of investigational treatments for ALS (as part of participation in a clinical trial or another EAP) within 5 half-lives (if known) or 30 days (whichever is longer) prior to the Screening Visit.
11. Permanent assisted ventilation (PAV), defined as more than 22 hours per day of noninvasive or invasive mechanical ventilation for more than seven consecutive days. The date of onset of PAV is the first day of the seven days.
12. Active cancer or history of cancer, except for the following: basal cell carcinoma or successfully treated squamous cell carcinoma of the skin, cervical carcinoma in situ, prostatic carcinoma in situ, or other malignancies curatively treated and with no evidence of disease recurrence for at least 3 years.
13. Presence of unstable psychiatric disease, cognitive impairment, dementia, or substance abuse that would impair ability of the participant to provide informed consent, in the SI's opinion.
14. Patients who chose to take experimental medications and/or supplements, and that is the only reason they are not eligible for trials, won't be eligible for the EAP.

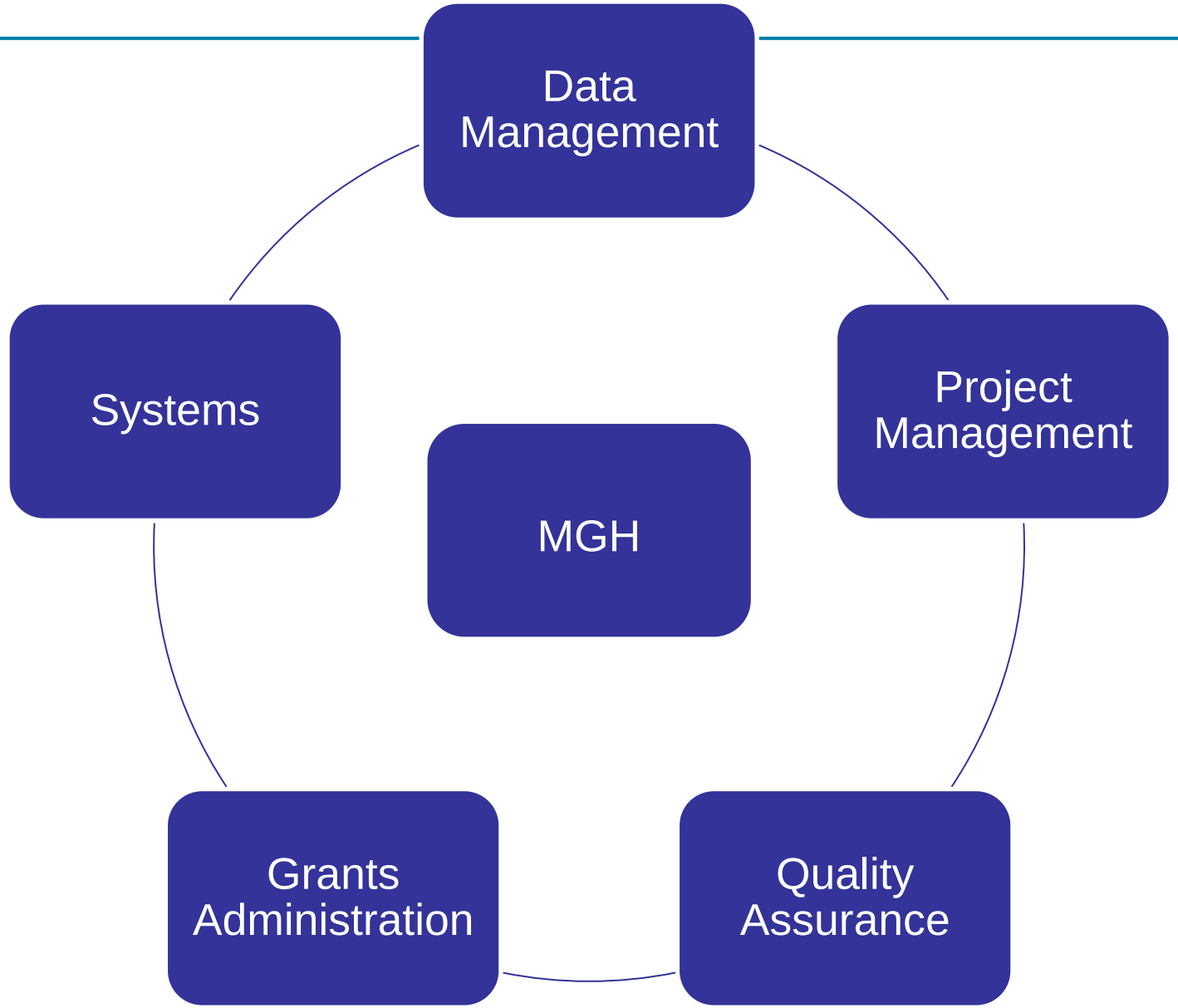
Workflow for sites



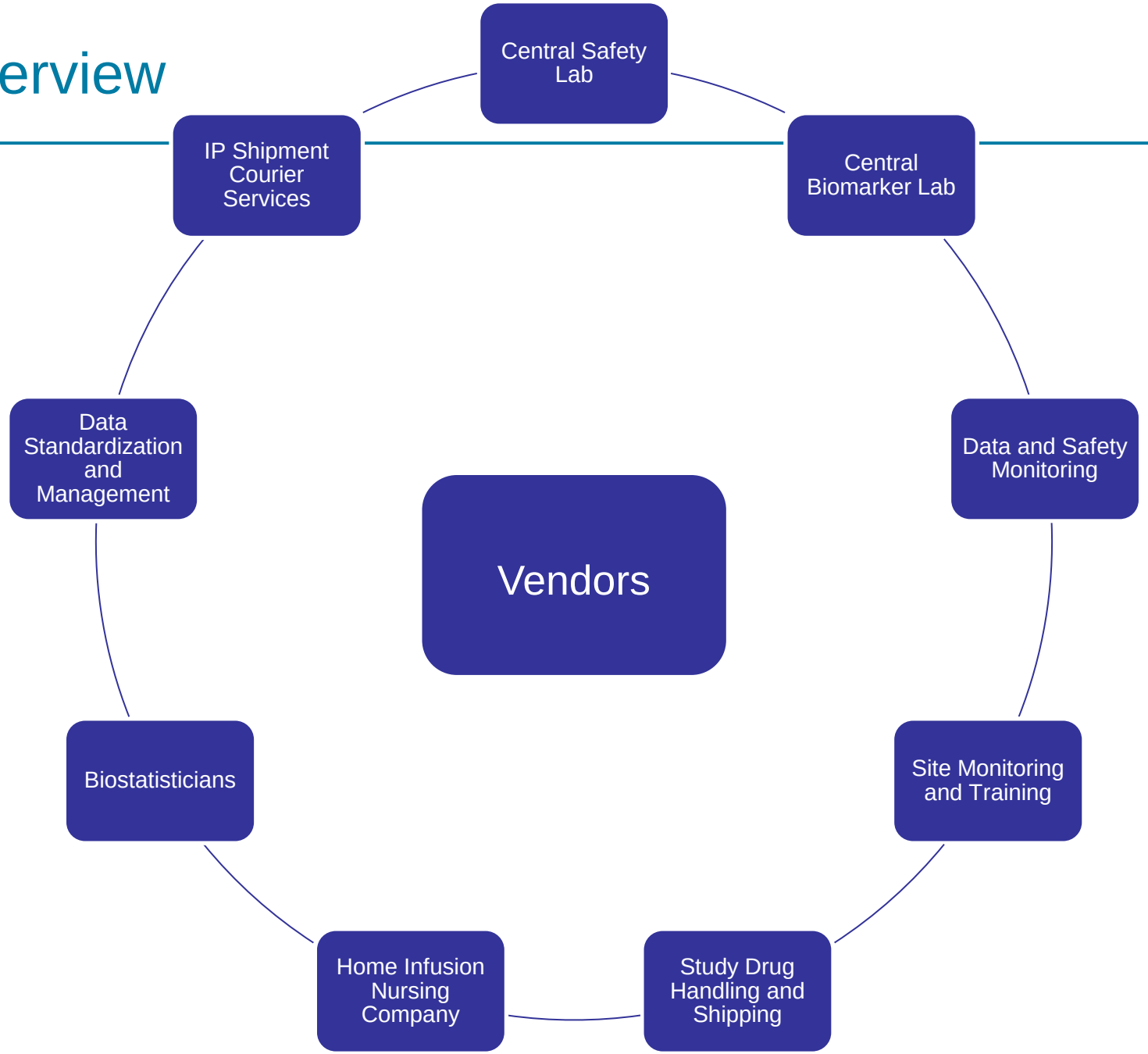
Project Overview



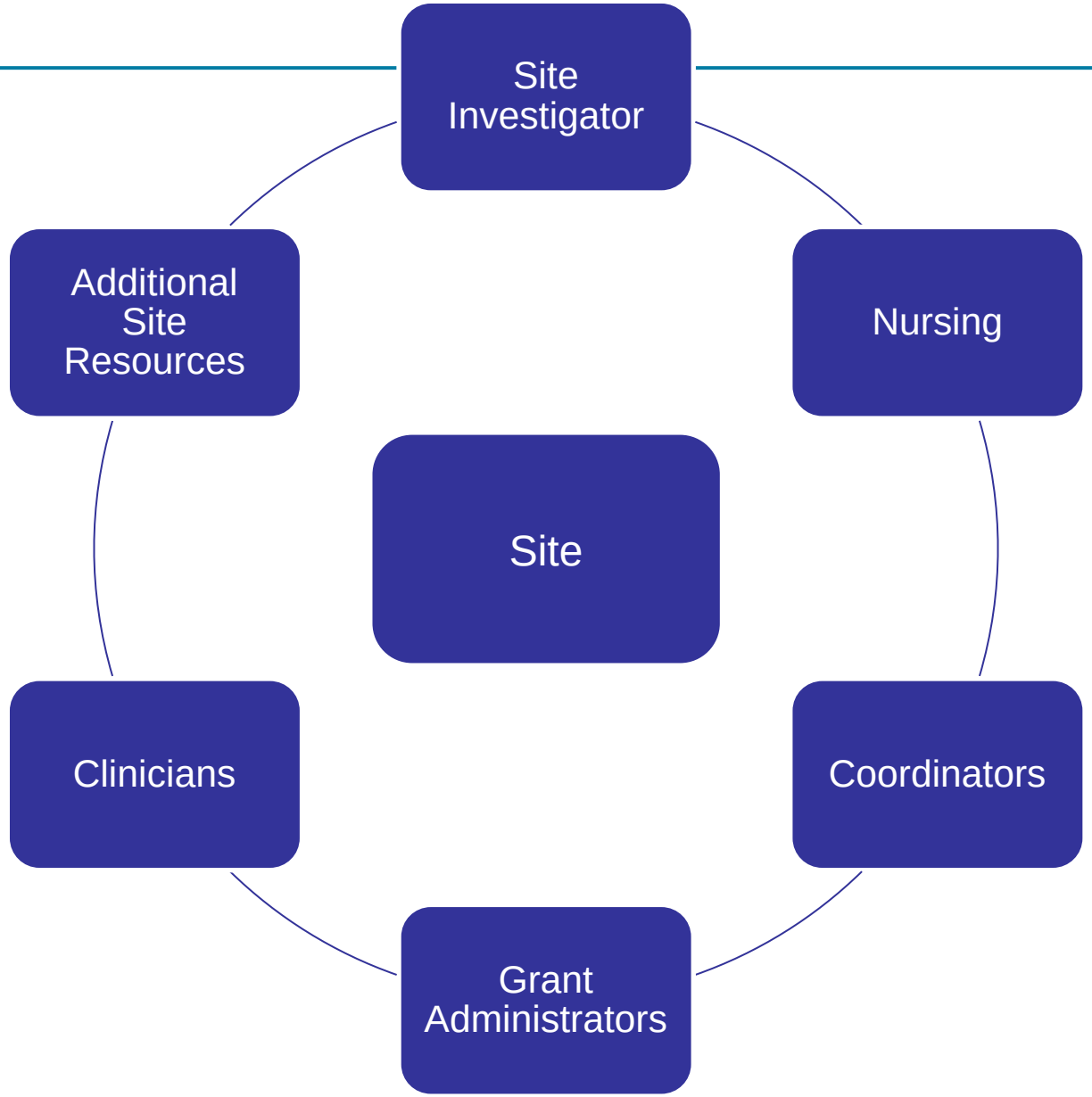
MGH Overview



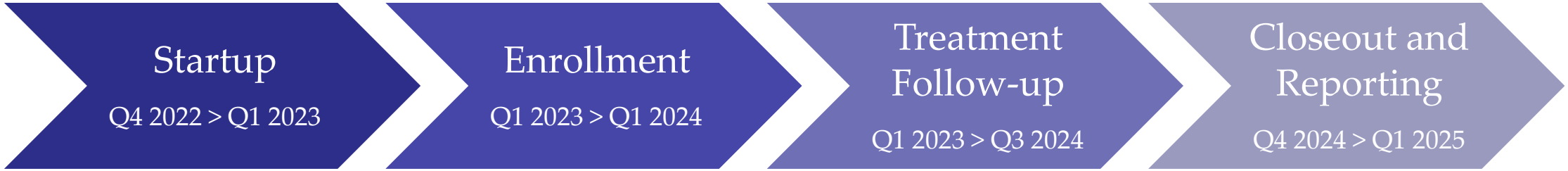
Vendors Overview



Site Overview

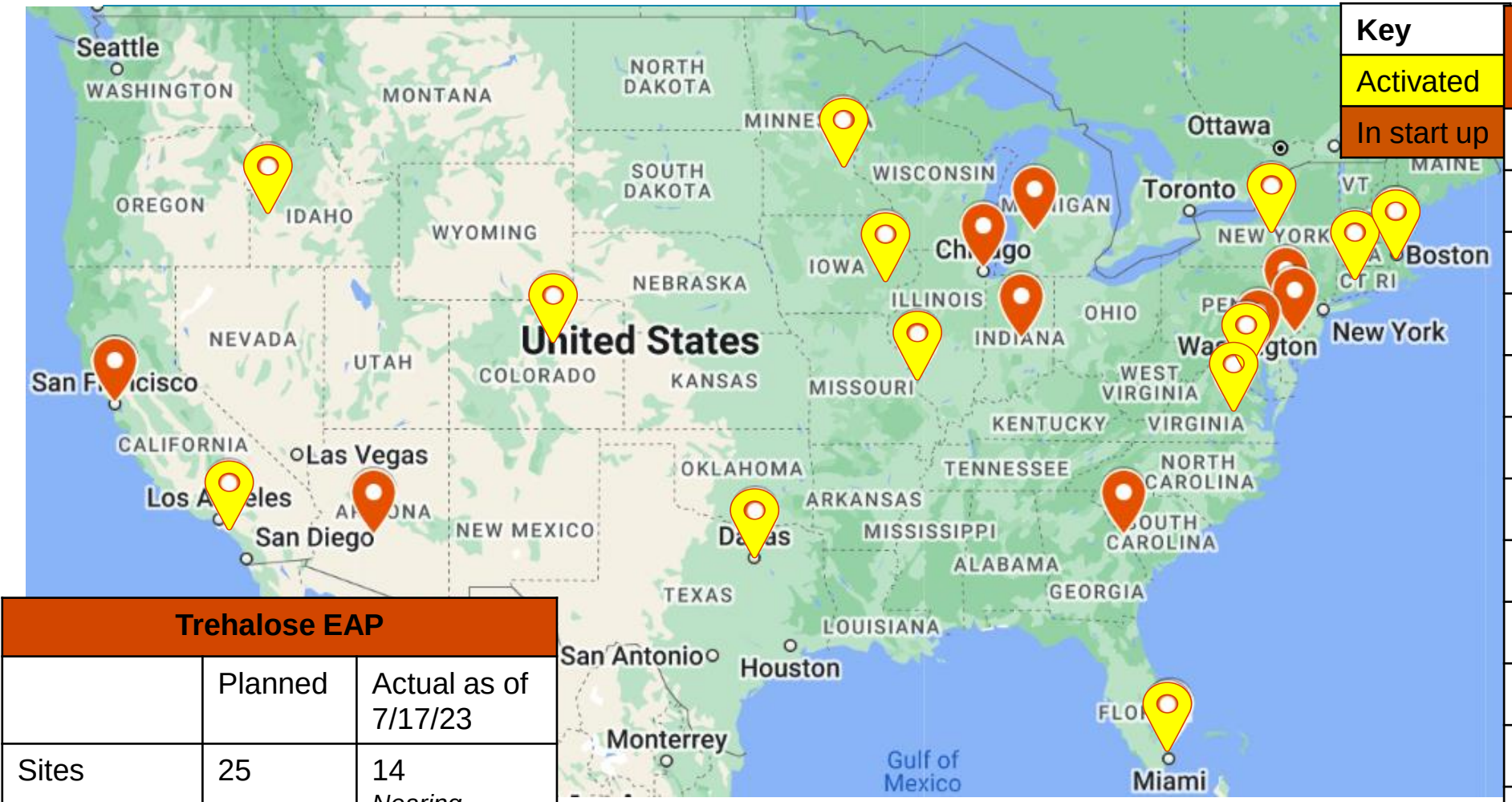


Site Startup Overview



- First patient first visit occurred at MGH in March 2023
- Key elements for site activation:
  - Completion of all local requirements (IRB, infusion center, pharmacy, etc.)
  - Fully executed Clinical Site Agreement (CSA)
  - sIRB approval
  - Regulatory document collection

Study Sites



Trehalose EAP		
	Planned	Actual as of 7/17/23
Sites	25	14 *Nearing activation: 2*
Participants	70	25 *In screening/ scheduled: 25*

Activated Sites As of 7/25/23
Mass General Hospital
Texas Neuro
St. Alphonsus
Holy Cross Hospital
Virginia Commonwealth University
Nova Southeastern University
University of Iowa
Washington University
University Colorado
Hospital for Special Care
George Washington University
SUNY Upstate Medical University
University of California, Irvine
University of Minnesota

Site Enrollment Progress

Overall Enrollment as of 7/25/23	
Total enrollment = 25	
Cohort 1 Total = 15	Cohort 2 Total = 10

Thank you!

Contact:

Suma Babu
SBABU@MGH.HARVARD.EDU

Healey Patient Navigators
mghalsresearch@mgh.harvard.edu