BRINGING TOGETHER A COMMUNITY
to launch the first platform trial for ALS
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Research brings results. Be part of our solution.

Our sole mission is to fund ALS research to find a cure.

• Updates on the HEALEY ALS Platform Trial
  - Study Treatments
  - Enrollment
  - Sites
• How to stay in touch and find a site near you
HEALEY ALS Platform Trial
Offers Several Benefits Over Traditional Trials

- Regimen were selected by ALS scientists based on a competitive process.

Traditional Clinical Trial vs. HEALEY ALS Platform Trial

- Cuts costs by 1/3
- Cuts time in 1/2

Randomization

- Increases Active by 3X

Shared infrastructure and common protocol, allowing sharing of placebo participants
We want to keep adding great therapies

• Submissions accepted on a rolling basis

• We opened with 3 regimens (zilucoplan, verdiperstat, CNM-Au8)

• The 4\textsuperscript{th} regimen will be added in 2020 (pridopidine)

• We are in discussions with 3 additional companies for 2021!

https://www.massgeneral.org/neurology/als/research/platform-trial
Enrollment Updates

• 152 individuals with ALS signed informed consent
• 111 are currently on study drug

We will continue to update the ALS community on enrollment (website, webinars)

Questions about enrollment? Please contact the Patient Navigator
  Phone: 833-425-8257 (HALT ALS)
How to Find a Center Near You

- 28 sites are actively enrolling
- A total of 45 sites are already IRB approved
- Goal is to activate the other sites soon and add more in early 2021!

Contact Info of Participating Sites by State

https://www.massgeneral.org/neurology/als/research/platform-trial-sites
List of enrolling sites as of Oct 29, 2020
28 sites are enrolling

- Texas Neurology
- MGH
- 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 Medical Center
- Loma Linda
- University of Washington
- University of Iowa
- Washington University
- University of Pennsylvania
- University of Michigan
- Forbes Norris MDA/ALS Research Center, CPMC
- Penn State Hershey
- UMass
- University of Miami
- University of Colorado
- Cedars-Sinai
- University of Florida
- Columbia University
For More Updates

• **Weekly informal Q&A + monthly webinars**
  The idea of came from our Patient Advisory Committee: we are excited to be talking with you on a weekly basis and take any questions you might have

• **Find the schedule and registration links on our website**
  https://www.massgeneral.org/neurology/als/news/webinars
QUESTIONS

• To submit text questions to today’s presenters, please type your questions into the ‘Q&A’ box

• Today’s presentation is being recorded and will be posted on the Healey & AMG Center for ALS website

https://www.massgeneral.org/neurology/als/research/platform-trial