**Highlights of the HEALEY ALS Platform Trial**

- This trial is being conducted at 50+ sites nationwide, with more US sites expected to join.
- Participation in the trial lasts approximately 6 months and involves about 7 in-person visits.
- Approximately 160 participants will be enrolled in each regimen. The trial is perpetual, so enrollment will continue as more investigational products are added over time.
- The active study drug to placebo ratio is 3:1 across all regimens (meaning there is a 75% chance of receiving active drug, 25% chance of placebo).
- Eligible participants who provide informed consent to the master protocol will be randomly assigned to one of the available regimens.
- After completing a regimen, participants may have the option to receive the investigational product through an open label extension (100% chance of receiving active drug).
- If you are interested in participating, the study team will review the inclusion/exclusion criteria with you in more detail.

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**Stay Connected to the Platform Trial**

More investigational products are anticipated to be added to the HEALEY ALS Platform Trial through support by pharma, foundation partners, philanthropy, federal, and other fundraising initiatives.

Visit our website to learn more about current and future regimens:
www.massgeneral.org/neurology/als/research/first-platform-trial-treatments

Register to attend weekly Platform Trial updates or view recordings of previous webinars:
https://www.massgeneral.org/neurology/als/research/platform-trial-news

Sign up for the ALS Link to receive news and updates about research and clinical care from the Healey Center for ALS at Mass General:
https://lp.constantcontactpages.com/su/saTzwIp/ALS

For general questions about the HEALEY ALS Platform Trial, contact the Patient Navigator:
email: healeyalsplatform@mgh.harvard.edu
phone: 833-425-8257 (HALT ALS)

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**Stay Connected to ALS Research Worldwide**

To learn about investigational drug trials or observational studies for ALS, please visit:
www.clinicaltrials.gov
www.neals.org
www.iamas.org
www.als.net

Register for the National ALS Registry:
www.cdc.gov/als/ALSJoinALSRegistry.html

**HEALEY ALS Platform Trial:**

Overview and Currently Enrolling Regimens

Updated April 2021
What is a Platform Trial?
A platform trial is a trial in which multiple investigational products are tested at the same time in different participants using a master protocol and specialized statistical tools. This results in a more efficient and expedited trial. More regimens can be added as new investigational products become available; thereby decreasing or eliminating the gap in time from identification of a potential therapy to trial in humans.

How does the Platform Trial work?
Participants in the Platform Trial will be randomly assigned to one of the regimens that are available at the time of their enrollment. Each participant will then be randomized within a regimen to receive active drug or placebo. All current regimens in the Platform Trial have a 3:1 active study drug to placebo ratio.

What is a Regimen?
A regimen is an arm of the trial that specifies the dosage, schedule, and duration of experimental treatment with an investigational product. After informed consent to the master protocol, each participant will be randomly assigned to one regimen. All regimens follow the Master Protocol but may include additional activities and inclusion/exclusion criteria.

Why Platform Trial?
Faster answers, More access, Less placebo, More learning about ALS.

Platform Trial Contact
Information at Mass General Hospital

If interested in participating in the Platform Trial at the Mass General site, please fill out our Patient Interest Form online at: https://redcap.partners.org/redcap/surveys/?s=DX8H3CWXTH
Our study staff will reach out to you with further details about the Platform Trial after receiving your completed Patient Interest Form.
Principal Investigator: Sabrina Paganoni, MD
Study Coordinator: Danny Hevert, Mia Resendes, Simon Shulman
Email: MGHsiteHealeyPlatform@mgh.harvard.edu
Phone: 617-643-3902

Current Platform Regimens
Each regimen in the trial is designed to test whether an investigational product is safe and effective in people diagnosed with ALS.

Regimen A: Trial of Zilucoplan
Developed by UCB
Zilucoplan is designed to work by blocking a protein called complement component 5 (C5), which may lead to a reduction in tissue damage caused by the immune system in ALS. This drug is administered as a daily injection under the skin.

Regimen B: Trial of Verdiperstat
Developed by Biohaven Pharmaceuticals
Verdiperstat is an investigational anti-inflammatory drug that inhibits myeloperoxidase and may work by reducing neural inflammation that occurs in ALS. This drug is administered as two pills taken by mouth twice a day.

Regimen C: Trial of CNM-Au8
Developed by Clene Nanomedicine
CNM-Au8 is a liquid suspension of pure gold nanocrystals that may work by providing an energetic assist to impaired motor neurons and improving their ability to function. This drug is taken daily as a 2oz drink.

Regimen D: Trial of Pridopidine
Developed by Prilenia Therapeutics
Pridopidine is a selective and potent activator of a protein called the Sigma-1 receptor (S1R). The S1R is highly expressed in the brain and spinal cord. Activation of the S1R promotes brain cell health, function and prevents neuronal damage in ALS. Pridopidine is a capsule taken by mouth twice daily.