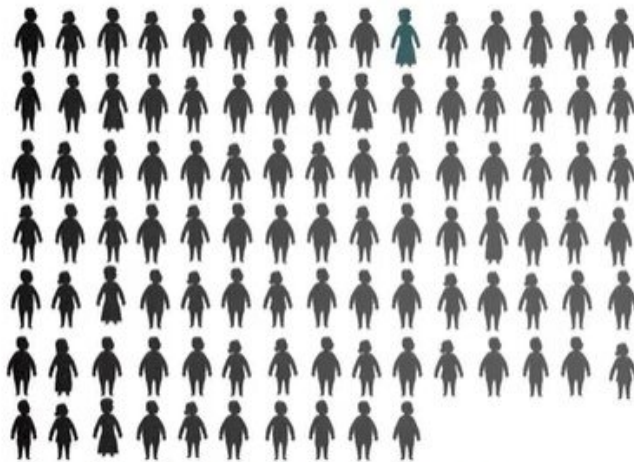


Trial to investigate multiple products simultaneously to accelerate development of new therapies for ALS

July 12 2022

ALS affects:



10% of cases are hereditary

approximately
30,000
in the U.S.

approximately
500,000
worldwide

 = 5000

Sean M. Healey & AMG Center for ALS.
"About the Sean M. Healey & AMG Center for ALS,"
Massachusetts General Hospital. Accessed May 20, 2022.

Credit: Massachusetts General Hospital

The HEALEY ALS Platform Trial led by the Sean M. Healey & AMG Center for ALS at Massachusetts General Hospital in collaboration with the Northeast ALS Consortium (NEALS) is a trial in which multiple investigational products are tested and evaluated simultaneously to

accelerate the development of potential new therapies for people with Amyotrophic Lateral Sclerosis (ALS). Drug candidates that enter the platform trial are chosen by a group of expert ALS scientists and members of the Healey & AMG Center Science Advisory Committee. The Healey & AMG Center for ALS at Mass General and Calico Life Sciences LLC recently signed an agreement to work together to test ABBV-CLS-7262 in the HEALEY ALS Platform Trial.

ABBV-CLS-7262 targets eIF2B, a key regulator of the integrated stress response (ISR), a pathway activated in people with ALS. In neurons exposed to cellular stressors, inhibition of the ISR by ABBV-CLS-7262 restores [protein synthesis](#) and dissolves pre-formed TDP-43 containing stress granules. This effect of ABBV-CLS-7262 is of clinical interest because TDP-43 containing stress granules are thought to lead to TDP-43 inclusions, a hallmark of ALS pathology. ABBV-CLS-7262 is currently being investigated in a Phase 1b study in ALS patients (NCT04948645).

ABBV-CLS-7262 is an [investigational drug](#) being developed by Calico in collaboration with AbbVie and not yet approved for use in any country. Calico is an Alphabet-founded research and development company focused on understanding the biology that controls human aging. Together with AbbVie, they are collaborating to discover and develop potential new therapies for patients with age-related diseases, including neurodegenerative disorders.

"We look forward to testing ABBV-CLS-7262 as another ALS investigational product in the HEALEY ALS Platform Trial. It's exciting to be able to test multiple products at once in hopes of accelerating the path for more treatments for ALS," says Merit Cudkowicz, MD, MSc, principal investigator and sponsor of the HEALEY ALS Platform Trial, director of the Sean M. Healey & AMG Center for ALS, chief of the Department of Neurology at MGH, and the Julieanne Dorn Professor of

Neurology at Harvard Medical School.

"We are excited to add ABBV-CLS-7262 to the HEALEY ALS Platform Trial. The Therapy Evaluation Committee has evaluated ABBV-CLS-7262 for inclusion in the platform trial based on the quality of Calico and AbbVie's science and readiness for the platform and we look forward to having them on board," says Sabrina Paganoni, MD, Ph.D., a physician-scientist at the Healey & AMG Center and co-principal investigator.

The [platform](#) trial is testing several other products including: a) zilucoplan, a small macrocyclic peptide inhibitor of complement component 5 [C5], b) verdiperstat, a brain-penetrant myeloperoxidase enzyme inhibitor, c) CNM-Au8, a cellular energetic nanocatalyst, d) pridopidine, an oral, highly selective Sigma-1 receptor (S1R) agonist, and e) trehalose, a low molecular weight disaccharide that affects autophagy and lysosomal pathways.

For updates on the trial, please join our weekly [Healey ALS Platform webinars](#).

Provided by Massachusetts General Hospital

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