



Healey Center

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



MASSACHUSETTS
GENERAL HOSPITAL

NEUROLOGICAL CLINICAL RESEARCH INSTITUTE

HEALEY ALS Platform Trial

The HEALEY ALS Platform Trial is designed to accelerate the development of breakthrough treatments for persons with ALS by testing multiple drugs at the same time. Data derived from the trial, if positive, could be used to support the potential approval of an investigational medication.

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

The drugs included in the Platform Trial were selected by a team of experts after careful review of the company and the science behind the medication. Each regimen has an equal chance of success for all forms of ALS based on available scientific evidence.

A regimen is an experimental treatment that specifies the dosage, schedule, and duration of treatment. Each regimen in the Platform Trial was designed to test whether certain drugs are safe and effective in people diagnosed with ALS.

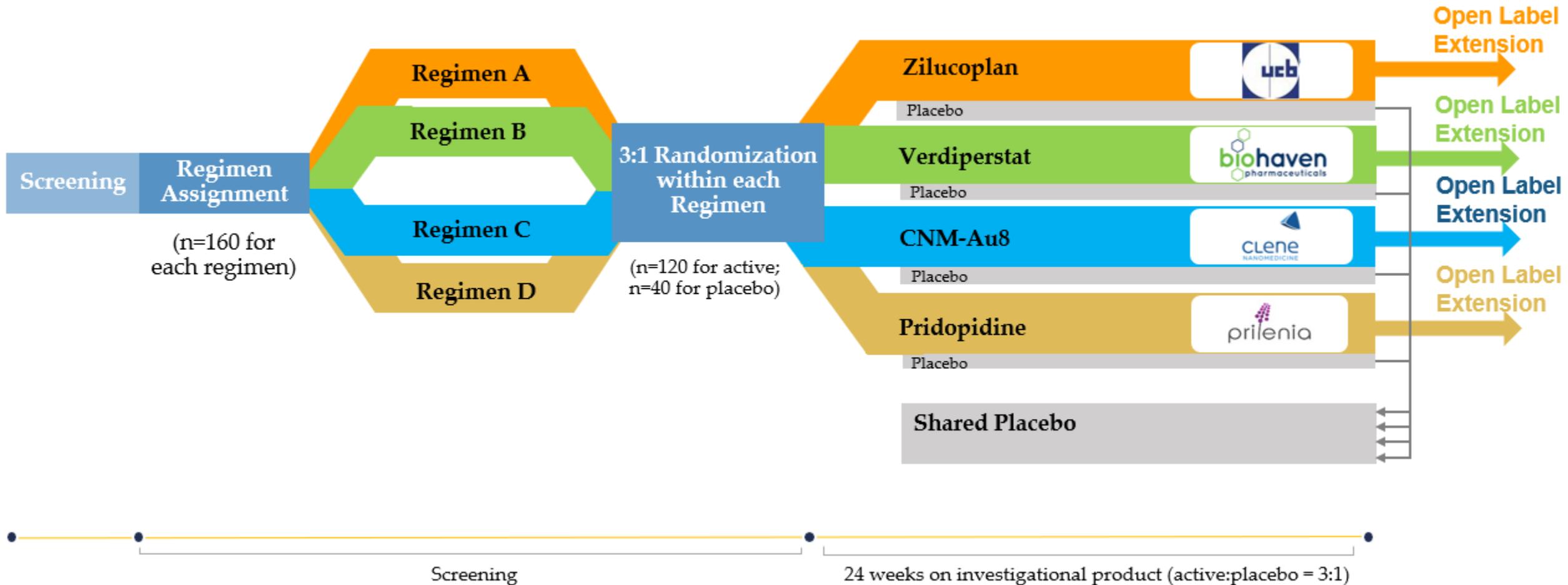
After providing informed consent to the master protocol, participants are randomly assigned to one of the available regimens. Neither the research team nor participants choose the regimen.

Each regimen consists of about 7 in-person visits over the course of approximately 6 months. There is a 3:1 active drug to placebo ratio, so participants have a 3 in 4 chance of being assigned to the active treatment group and a 1 in 4 chance of being assigned to the placebo group.

Upon completion of the 24-week trial, all regimens in the Platform Trial will offer an Open Label Extension (OLE), which allows participants to receive the active drug for the regimen to which they were assigned. The duration of OLE may vary by regimen.

More investigational products are anticipated to be added to the Platform Trial, through support by pharma, foundation partners, philanthropy, federal and other fundraising initiatives. To learn more about current and future regimens, [click here](#).

HEALEY ALS Platform Trial Design



To learn more about clinical trials in general, and to view a list of frequently asked questions, visit [introduction to clinical trials](#).

Trial of Zilucoplan

Developed By: UCB

Administration: Subcutaneous injection (prefilled syringe, self-administered once daily).

Learn More:

[What does this drug do?](#)

[Has this drug been studied before?](#)



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HEALEY ALS

Platform Trial



Study
Drugs



Trial of Verdiperstat

Developed By: Biohaven Pharmaceuticals

Administration: Oral (pill, 4 tablets daily).

Learn More:

[What does this drug do?](#)

[Has this drug been studied before?](#)



Trial of CNM-Au8

Developed By: Clene Nanomedicine

Administration: Oral (ingestible liquid, 2oz once daily). Can be taken through G-tube.

Learn More:

[What does this drug do?](#)

[Has this drug been studied before?](#)



Trial of Pridopidine

Developed By: Prilenia Therapeutics

Administration: Oral (pill, 2 capsules daily).

Learn More:

[What does this drug do?](#)

[Has this drug been studied before?](#)



Zilucoplan



What does this drug do?



Activation of the immune system, including a high expression of complement proteins, has been observed in the spinal cord and motor neurons of patients with ALS.



This medication is designed to prevent inflammation and tissue damage caused by the immune system in ALS by binding to and blocking a protein called complement component 5.

Zilucoplan

Has this drug been studied before?

Zilucoplan has previously been studied in people with a neuromuscular condition called generalized myasthenia gravis. The Phase 2 trial of Zilucoplan demonstrated rapid, clinically meaningful and statistically significant improvements for patients with generalized myasthenia gravis.

Additionally, in an animal model of ALS, inhibiting complement protein was found to improve disease-like characteristics.

[Click here](#) to watch a webinar about the science behind Zilucoplan.
Read more on the UCB [website](#).

To learn more about clinical trials in general, visit [introduction to clinical trials](#).

Verdiperstat



What does this drug do?

In neurodegenerative diseases like ALS, immune system over-activation leads to neuroinflammation, which can contribute to cellular injury. Microglia **cells** are key agents in the immune system, and people with ALS have been shown to exhibit increased microglial activation which corresponds to excessive levels of an enzyme called myeloperoxidase (MPO). MPO is thought to be responsible for generating toxic compounds that drive neuroinflammation.



This medication is designed to reduce neural inflammation and possibly slow neurodegeneration and disease progression in ALS by inhibiting myeloperoxidase. Verdiperstat binds to MPO right where the enzyme releases toxic compounds, rendering MPO inactive.



Verdiperstat

Has this drug been studied before?

Verdiperstat has previously been studied in about 490 people in Phase 1, Phase 2, and ongoing Phase 3 clinical trials. PET scans of patients with Parkinson's disease demonstrated reduced microglial activation, a type of neuroinflammation that is also relevant in ALS, following treatment with verdiperstat. Currently, verdiperstat is also being tested in a Phase 3 clinical trial for Multiple System Atrophy, a neurological disorder similar to ALS.

Additionally, data from animal models of neurodegenerative diseases showed that reducing microglial immune cell activation had anti-inflammatory and neuroprotective effects.

[Click here](#) to watch a webinar about the science behind verdiperstat.
Read more on the Biohaven Pharmaceuticals [website](#).

To learn more about clinical trials in general, visit [introduction to clinical trials](#).

CNM-Au8



What does this drug do?

CNM-Au8 catalyzes key energy reactions to provide the brain, spinal cord, and motor neurons with more access to energy metabolites. This may help to repair and improve motor neuron health and survival.



This drug is designed to work by providing an energetic assist to impaired motor neurons. CNM-Au8 nanocrystals (very small gold particles) travel through the body and enter the brain and motor neuron cells, where they enhance the ability of these cells to function more normally in ALS.



CNM-Au8

Has this drug been studied before?

This drug has previously been studied in a Phase 1 trial using healthy volunteers, which demonstrated its safety and tolerability. Currently, CNM-Au8 is being studied in multiple Phase 2 trials, including trials for patients with other neurodegenerative diseases such as Multiple Sclerosis and Parkinson's Disease.

In a mouse model of ALS, CNM-Au8 was shown to improve motor neuron survival and neuron connections.

[Click here](#) to watch a webinar about the science behind CNM-Au8.
Read more on the Clene Nanomedicine [website](#).

To learn more about clinical trials in general, visit [introduction to clinical trials](#).

Pridopidine



What does this drug do?



Pridopidine selectively activates the Sigma-1 receptor (S1R), which plays an important role in cellular responses to stress and is highly expressed in motor neurons in the brain and spinal cord.



Activating the S1R may help regulate signaling pathways that are commonly impaired in neurodegenerative disorders, such as ALS and Huntington Disease, and exert neuroprotective effects.

Pridopidine

Has this drug been studied before?

Pridopidine has previously been studied in >1300 people. In a Phase 2 trial in patients with Huntington Disease, this drug was shown to be the first drug ever to maintain functional capacity (i.e. activities of daily living), with the effects lasting up to 5 years (the longest time analyzed to date).

Additionally, these studies showed pridopidine, at the dose used in the platform (45mg, 2 capsules/day), to be safe and well-tolerated, with a side effect profile like that of placebo.

[Click here](#) to watch a webinar about the science behind Pridopidine.
Read more on the Prilenia Therapeutics [website](#).

To learn more about clinical trials in general, visit [introduction to clinical trials](#).