

# Pridopidine

## Expanded Access Protocol

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This **Pridopidine Expanded Access Protocol (EAP)** will be made available to 200 people with ALS at up to 45 US research centers. This EAP is a unique opportunity for people with ALS to gain access to an investigational product (IP) and contribute to clinical research. This EAP is for those ALS individuals who are otherwise ineligible for ongoing clinical trials.

**Pridopidine** was studied in Regimen D of the HEALEY ALS Platform Trial. The Pridopidine EAP will provide data to supplement the pridopidine clinical development program by gathering longer-term safety, biological, and clinical efficacy data.

This EAP will be conducted with the same scientific rigor as a traditional clinical trial, and the data collected will be reported as per NIH requirements.

## About Pridopidine

**What impact could Pridopidine have on ALS?** Pridopidine is an investigational drug recently in a clinical trial for ALS, so its effects are not yet known. Pridopidine activates the Sigma-1 Receptor (S1R) in motor neurons, which may help promote neuron health and reduce cell death. Pridopidine may help regulate pathways that are commonly impaired in neurological diseases like ALS.

View the Pridopidine  
Drug Science Webinar  
<https://bit.ly/3QZxU8I>



### How is Pridopidine administered?

Pridopidine is a capsule that can be taken by mouth or opened and placed through a feeding tube. The study drug is intended to be taken twice daily.

### How many in-person visits are there and how long could I participate in this EAP?

There are 3-4 in-person visits, and some additional visits may be done remotely. Participation is designed to last up to two years.

### How do I learn more about this EAP?

Follow the links below or reach out to a participating research center near you to learn what is involved in participation and whether you may be eligible for this EAP.

View the Pridopidine  
EAP on ClinicalTrials.gov  
<https://bit.ly/3MLZDrc>



View the Pridopidine  
EAP on the MGH website  
<https://bit.ly/3uni3lc>



# Information About Expanded Access

## What is Expanded Access (EA)?

EA is a pathway for people with a serious and life-threatening disease to access an investigational product (IP) that is not yet approved by the Federal Drug Administration (FDA). EA is an option for people who do not qualify for ongoing clinical trials. An IP offered through EA is experimental, so its effectiveness as a treatment for ALS is not yet confirmed. At this time, EA protocols have limited availability due to funding and staffing constraints.

## What are the benefits of participating in Expanded Access?

EA provides people living with ALS who are not eligible for clinical trials the opportunity to access an IP while the IP is being formally tested. Participation in EA may also contribute to research by providing safety and biomarker data (indicators of disease) that may benefit the greater ALS research community.

## How is Expanded Access different from clinical trials?

The primary purposes of clinical trials are to formally evaluate IPs and gather data that may lead to FDA approval. Clinical trials have strict eligibility criteria, frequent in-person visits, and often include a placebo group. Participation in clinical trials is optional and separate from clinical care, and clinical trials may be conducted by a research specialist.

EA protocols have broader and more inclusive eligibility criteria, fewer in-person visits, and do not have a placebo group. Participation in EA is considered an extension of clinical care and requires oversight by the treating clinician in addition to FDA and ethics board compliance requirements.

**General questions?** Email: [mghalsresearch@mgh.harvard.edu](mailto:mghalsresearch@mgh.harvard.edu)

## More Info About Expanded Access

Visit the FDA website



<https://bit.ly/3Jx32rj>

Visit NEALS.org



<https://bit.ly/402uapg>

Register for Weekly Platform Trial Webinars



<https://bit.ly/3TwAt1D>