



March 12, 2024

Dear Huntington's Disease Community,

We recently have had meetings with regulatory authorities in the European Union about next steps for our HD program. We are pleased to let you know that after positive and constructive feedback, we plan to submit a Marketing Authorization Application (MAA) for pridopidine for the treatment of Huntington's disease (HD) to the European Medicines Agency's (EMA) Committee for Medicinal Products for Human Use (CHMP). An MAA is submitted for approval to market a medicine in European Union member countries. Submission is planned for mid-2024.

In our PROOF-HD study, pridopidine demonstrated consistent treatment benefits across independent measures that are most important to patients and families, including day to day function, cognition, motor, and clinical progression of HD. These benefits are especially compelling in patients not taking antidopaminergics (medicines to treat chorea and/or neuroleptics). In clinical studies to date, pridopidine was well-tolerated with no serious treatment-related adverse events, with a safety and tolerability profile similar to placebo.

The support from regulators to submit an MAA is an important step towards making new treatments accessible for patients with Huntington's disease. Prilenia will have discussions with the U.S. Food and Drug Administration (FDA) about a potential path forward for pridopidine as a treatment option for those living with HD in the United States.

To learn more about this news and pridopidine, please read our press release [here](#).

We will continue moving with urgency on behalf of the many families affected by HD around the world. We thank you for your commitment and collaboration as we move one step closer to providing a potential treatment option to the HD community.

Sincerely,

Seth Rotberg

Seth Rotberg, Senior Manager of Patient Advocacy and Engagement
On behalf of the team at Prilenia

Pridopidine is an investigational drug that is not approved for the treatment of HD by health authorities.



Q&A

Who can I reach out to for help or if I have any questions?

- *For study participants and their family members, we encourage you to reach out to your study physician for more information.*
- *For members of the larger HD community, please reach out to your local HD care center or patient advocacy organization.*
- *You can also reach out to info@prilenia.com.*

What is pridopidine?

- *Pridopidine (45 mg twice daily) is an oral, highly selective and potent investigational S1R agonist that has exhibited a safety and tolerability profile similar to placebo in clinical studies to date. The S1R protein is highly expressed in the brain and spinal cord where it regulates several key processes that are commonly impaired in various neurodegenerative diseases. Activation of the S1R by pridopidine may lead to neuroprotective effects.*
- *It is an investigational drug, and its safety and efficacy have not been determined by regulatory authorities including FDA or CHMP.*

What is the European Medicines Agency (EMA) and Committee for Medicinal Products for Human Use (CHMP)?

- *EMA is responsible for the scientific evaluation, supervision, and safety monitoring of medicines. EMA protects public and animal health in EU Member States, as well as the countries of the European Economic Area, by ensuring that all medicines available on the EU market are safe, effective and of high quality.*
- *CHMP at EMA is responsible for the scientific assessment of the application dossier on the quality, safety and efficacy, including environmental safety, of a new medicinal product for human use as part of the centralized marketing*

Does submitting a Marketing Authorization Application (MAA) mean the drug will automatically get approved in Europe?

- *No, submitting a marketing authorization means that it will be submitted for the scientific assessment by CHMP. CHMP will assess the application for quality, safety and efficacy aspects and provide their opinion. Upon a positive CHMP opinion, the European Commission then will issue a legal approval.*
- *Regulatory approval is important but at country level there may still be additional requirements regarding health technology assessment and reimbursement that may need to be resolved prior to patients getting access to a new treatment.*

What are neuroleptics?

- *Neuroleptic medications are also known as antipsychotics. These are different from antidepressant medications.*
- *According to "HDSA's A Physician Guide to the Management of Huntington's Disease (Third Edition)," neuroleptics are used for psychosis and sometimes for irritability or for chorea suppression.*

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What is the takeaway for patients who are currently taking both pridopidine and antidopaminergics (neuroleptics and chorea medicine)? What should they do?

- *Study participants and community members should consult with their treating physician regarding any medical decisions, including prescription medications.*
- *Pridopidine is an investigational drug, and Prilenia is not making any recommendations to alter treatment plans.*

What are Prilenia's plans for juvenile onset HD (JHD)?

- *As part of the regulatory process in Europe, companies need to agree on a pediatric investigation plan (PIP) before submitting a Marketing Application. Prilenia has an agreed pediatric investigation plan with the European authorities, and we are planning for a future small clinical study.*

What are the next steps for Prilenia in the United States and Canada?

- *In the US, Prilenia is still having discussions with the FDA to determine next steps.*
- *In Canada, we are planning interactions with the Canadian authorities.*
- *We will also consider submissions for approval in additional countries and regions following the regulatory review process in Europe.*