NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Pridopidine for treating Huntington's disease ID6525

Draft scope

Draft remit/evaluation objective

To appraise the clinical and cost effectiveness of pridopidine within its marketing authorisation for treating Huntington's disease.

Background

Huntington's disease is an inherited, progressive neurodegenerative condition. It is caused by a fault in the huntingtin gene, known as HTT, which leads to too many repeated genetic sequences in the huntingtin protein. This altered protein goes on to damage neurones in the brain. If one parent has the faulty gene, there is a 50% chance of each of their children developing the condition. Everyone with the faulty gene will develop the condition. Symptoms usually begin when people are aged between 30 and 50 years old. In the early stages of Huntington's disease, the main symptoms often include making uncontrolled muscle movements, difficulty concentrating, memory lapses, depression, stumbling or clumsiness and personality changes. People with Huntington's disease over time may also experience more frequent involuntary movement of the limbs and body (known as chorea), difficulty walking and may lose the ability to walk or sit up independently, difficulty speaking and swallowing, breathing problems and personality changes. Advanced Huntington's disease has a severe impact on daily life, for people living with the condition, their families and carers, and people living with Huntington's disease lose the ability to walk, talk and feed themselves.

There are around 7,000 people living with Huntington's disease in the UK.[1] It is identical in incidence for male and female. The disease is usually fatal after a period of up to 20 years from symptoms appearing.

There is no cure for Huntington's disease and there are currently no licensed treatments approved for use in the UK to stop disease progression. The condition is managed through multidisciplinary supportive care. Supportive care aims to relieve the symptoms caused by Huntington's disease and minimise the impact of disability, address complications and improve quality of life. These may involve input from neurology, nutritional support, gastroenterology, physiotherapy, psychiatry, speech and language therapy, occupational therapy, palliative care and social care. To treat involuntary muscle movements tetrabenazine might be prescribed.

The technology

Pridopidine (brand name unknown, Prilenia) does not currently have a marketing authorisation in the UK. It has been studied in clinical trials compared with placebo in people with stage I and stage II Huntington's disease, defined as scoring 7 or higher on the unified Huntington's disease rating scale-total functional capacity.

Intervention(s)	Pridopidine
Population(s)	Adults with Huntington's disease
Subgroups	If evidence allows, results by disease stage may be considered
Comparators	Best supportive care (including tetrabenazine)
Outcomes	The outcome measures to be considered include: • total functional capacity • non-cognitive symptoms (e.g behavioural and psychiatric symptoms) • ability to remain independent • admission to full-time care • hospitalisations • health-related quality of life • pain • adverse effects of treatment • mortality
Economic analysis	The reference case stipulates that the cost effectiveness of treatments should be expressed in terms of incremental cost per quality-adjusted life year. The reference case stipulates that the time horizon for estimating clinical and cost effectiveness should be sufficiently long to reflect any differences in costs or outcomes between the technologies being compared. Costs will be considered from an NHS and Personal Social Services perspective. The availability of any commercial arrangements for the intervention, comparator and subsequent treatment technologies will be taken into account. The availability and cost of biosimilar and generic products should be taken into account.
Other considerations	Guidance will only be issued in accordance with the marketing authorisation. Where the wording of the therapeutic indication does not include specific treatment combinations, guidance will be issued only in the context of the evidence that has underpinned the marketing authorisation granted by the regulator.

Related NICE recommendations	**Related NICE guidelines:** Suspected neurological conditions: recognition and referral (2023) NICE guideline NG127 **Related quality standards:** Suspected neurological conditions: recognition and referral (2021) NICE quality standard 198.

Questions for consultation

Population

- Is pridopidine only to be considered for people with early-stage Huntington's disease?
    - How is early-stage Huntington's disease stage defined in NHS clinical practice?
    - Or would pridopidine be used in other populations with Huntington's disease?
    - Would this treatment be used in pre-symptomatic disease?

Clinical management

- How is Huntington's diagnosed in NHS clinical practice?
    - What proportion of cases are diagnosed pre-symptomatic?
- What is established clinical management without pridopidine for people with Huntington's?
    - Does this vary by stage of disease, and if so, how?

Outcomes

- Are the outcomes listed appropriate?

Subgroups

- Are there any subgroups of people in whom pridopidine is expected to be more clinically effective and cost effective or other groups that should be examined separately?

Where do you consider pridopidine will fit into the existing care pathway for Huntington's disease?

Please select from the following, will pridopidine be:

A. Prescribed in primary care with routine follow-up in primary care
B. Prescribed in secondary care with routine follow-up in primary care
C. Prescribed in secondary care with routine follow-up in secondary care
D. Other (please give details):

For comparators and subsequent treatments, please detail if the setting for prescribing and routine follow-up differs from the intervention.

Would pridopidine be a candidate for managed access?

Do you consider that the use of pridopidine can result in any potential substantial health-related benefits that are unlikely to be included in the QALY calculation?

Please identify the nature of the data which you understand to be available to enable the committee to take account of these benefits.

NICE is committed to promoting equality of opportunity, eliminating unlawful discrimination and fostering good relations between people with particular protected characteristics and others. Please let us know if you think that the proposed remit and scope may need changing in order to meet these aims. In particular, please tell us if the proposed remit and scope:

- could exclude from full consideration any people protected by the equality legislation who fall within the patient population for which pridopidine will be licensed;

- could lead to recommendations that have a different impact on people protected by the equality legislation than on the wider population, e.g. by making it more difficult in practice for a specific group to access the technology;

- could have any adverse impact on people with a particular disability or disabilities.

Please tell us what evidence should be obtained to enable the committee to identify and consider such impacts.

NICE intends to evaluate this technology through its Single Technology Appraisal process. (Information on NICE's health technology evaluation processes is available at https://www.nice.org.uk/about/what-we-do/our-programmes/nice-guidance/nice-technology-appraisal-guidance/changes-to-health-technology-evaluation).

References

1. Brain Research UK (2022) Huntington's disease key information Accessed March 2025