NATIONAL INSTITUTE FOR HEALTH AND CARE EXCELLENCE

Health Technology Evaluation

Efgartigimod with recombinant human hyaluronidase PH20 for treating chronic inflammatory demyelinating polyneuropathy ID6409

Draft scope

Draft remit/evaluation objective

To appraise the clinical and cost effectiveness of efgartigimod with recombinant human hyaluronidase PH20 within its marketing authorisation for treating chronic inflammatory demyelinating polyneuropathy.

Background

Chronic inflammatory demyelinating polyneuropathy (CIDP) is a neurological disorder in which there is inflammation of nerve roots and peripheral nerves and destruction of the fatty protective covering (myelin sheath) of the nerve fibres. This causes weakness, paralysis and/or impairment in motor function, especially of the arms and legs. There are usually some alterations of sensation causing incoordination, numbness, tingling, or prickling sensations. The motor and sensory impairments associated with CIDP usually affect both sides of the body (symmetrical), and limb weakness typically starts in the legs. The condition may also impact everyday physical activities, such as getting out of a chair, walking, climbing stairs, and may also cause falling. Problems with gripping objects, tying shoelaces, and using utensils can also be brought on by upper limb involvement. CIDP shares many symptoms with Guillain–Barré syndrome but is a chronic rather than an acute disease.

CIDP is a rare disorder that can affect any age group and the onset of the disorder may begin during any decade of life. CIDP affects males twice as often as females and the average age of onset is 50.[1] Up to 650 people are diagnosed with CIDP each year in the UK.[2] The prevalence of CIDP is estimated to be around 5-7 cases per 100,000 individuals.[3]

CIDP is usually treated first with corticosteroids. In many cases, CIDP may respond to corticosteroids alone. However, individuals requiring high doses of corticosteroid drugs may experience side effects. If there is insufficient response to first-line treatment or the individual experiences significant side effects from corticosteroids, people may be offered intravenous immunoglobulin (IVIg). Subcutaneous delivery of immunoglobulin (SCIg) is also available as an alternative to IVIg. Plasma exchange (PLEx) may provide an alternative therapy for patients who do not respond to IVIg/SCIg and corticosteroids. Corticosteroids, IVIg/SCIg and PLEx may also be used in conjunction with immunosuppressive drugs (such as azathioprine, mycophenolate mofetil, methotrexate, cyclosporine and cyclophosphamide). IVIg and PLEx are often only effective for a few weeks and people may require chronic intermittent treatments. Access to IVIg and its use is variable. Long-term immunosuppressive therapy may be needed to prevent relapse.

The technology

Efgartigimod with recombinant human hyaluronidase PH20 (brand name unknown, Argenx) does not currently have a marketing authorisation in the UK for treating CIDP. It has been studied in clinical trials compared against placebo in adults with CIDP.

Intervention(s)	Efgartigimod with recombinant human hyaluronidase PH20
Population(s)	Adults with chronic inflammatory demyelinating polyneuropathy
Subgroups	If the evidence allows, the following subgroups will be taken into consideration: • untreated CIDP • previously treated CIDP
Comparators	• standard of care without efgartigimod with recombinant human hyaluronidase PH20 (including corticosteroids and immunosuppressive therapies, with or without intravenous immunoglobulin or plasma exchange)
Outcomes	The outcome measures to be considered include: • physical function change from baseline • disease progression • hospitalisations • adverse effects of treatment • health-related quality of life
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.
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 technology appraisals:** Vutrisiran for treating hereditary transthyretin-related amyloidosis (2023) NICE technology appraisal guidance 868. Review date not stated Tafamidis for treating transthyretin amyloidosis with cardiomyopathy (2021) NICE technology appraisal guidance 696. Review date 2024 Patisiran for treating hereditary transthyretin amyloidosis (2019) NICE highly specialised technology guidance 10. Review date 2022 Inotersen for treating hereditary transthyretin amyloidosis (2019) NICE highly specialised technology guidance 9. Review date 2022 Suspected neurological conditions: recognition and referral (2019) NICE guideline NG127. Last updated October 2023 **Related technology appraisals in development:** Eplontersen for treating polyneuropathy caused by hereditary transthyretin amyloidosis. NICE technology appraisal guidance ID6337. Publication expected August 2024 **Related NICE guidelines:** Suspected neurological conditions: recognition and referral (2019) NICE guideline NG127. Last updated October 2023
Related National Policy	The NHS Long Term Plan (2019) NHS Long Term Plan NHS England (2023) Manual for prescribed specialist services (2023/2024) Chapter 11

Questions for consultation

What is the existing treatment pathway for CIDP?

Where do you consider efgartigimod with recombinant human hyaluronidase PH20 will fit into the existing care pathway for CIDP?

How often are intravenous immunoglobulin or plasma exchange used for CIDP? If used, in which populations are they used and for how long (short-term v chronic longer-term use)?

Please select from the following, will efgartigimod with recombinant human hyaluronidase PH20 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 efgartigimod with recombinant human hyaluronidase PH20 be a candidate for managed access?

Do you consider that the use of efgartigimod with recombinant human hyaluronidase PH20 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 efgartigimod with recombinant human hyaluronidase PH20 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. National Institute of Neurological Disorders and Stroke. Chronic Inflammatory Demyelinating Polyneuropathy (CIDP) [accessed 16/10/24]
2. Gain charity. CIDP & the associated chronic variants [accessed 16/10/24]
3. NORD. Chronic Inflammatory Demyelinating Polyneuropathy [accessed 16/10/24]