Proposal for assessment of new health technologies

Important information – read this first!

- Submitted proposals for national health technologies (HTAs) will be published in full. If the proposer thinks there is information necessary for filling out the form, that should not be made public, please contact the secretariat (Nye Metoder) before submission.

  The proposer is aware that the form will be published in its entirety (tick): ☒

- Proposer has filled out point 19 below «Interests and, if any, conflicts of interest» (tick): ☒
- This form serves the purpose to submit proposals for health technology assessment (HTA) at the national level in Nye Metoder - the national system for managed introduction of new health technologies within the specialist health service in Norway. The form does not apply to proposals for research projects. A health technology assessment is a type of evidence review, and for this to be possible, documentation is required, e.g. from completed clinical trials. Lack of documentation may be one of the reasons why the Commissioning Forum (Bestillerforum RHF) does not assign a health technology assessment.
- If the proposal concerns a medical device, the proposer is familiar with the document «Guidance criteria for management of medical devices in the National System for Managed Introduction of New Health Technologies within the Specialist Health Service in Norway» (link) (tick): ☐

Contact information:

Name of the proposer (organization / institution / company / manufacturer)**:**

CSL Behring

Name of proposal contact:

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Date and locality:

March 6, Danderyd, Sweden

1. Proposer's title on the proposal: *
   *This may be changed during the course of the process"

Hizentra pre-filles syringes

2. Brief description of the health technology proposed to be considered:

Hizentra is a replacement therapy in adults, children and adolescents with primary or secondary immunodeficiency syndromes (PID or SID), or patients with chronic inflammatory demyelinating polyneuropathy (CIDP). Hizentra contains immunoglobulins that have been prepared from plasma donors.

Hizentra is currently available in Norway in vials with solution for subcutaneous injection. A new formulation has now been developed, where the product is presented in pre-filled syringes (PFS). The PFS formulation has benefits for the patients and health care system, e.g. reduced time and effort for administration, easier to learn at start-up hence less time needed hospital and less nursing time needed at start up, less risk for errors etc. This is particularly important as the treatment often is self-administrated at home. It can also provide value by the ability to infuse without using infusion pumps, using so called rapid push, which could open up for home treatment for some additional patients earlier refusing subcutaneous injection.

As Hizentra is produced based on plasma donation, the manufacturing cost is a substantial part of the product cost/price and the production cost for the PFS formulation is higher than for the currently available vial formulation. Because of this and because there are patient and health system benefits with the PFS formulation, it is reasonable that the cost for the PFS formulation can be higher than the vial formulation.

3. Brief description of current standard of care (SOC) (Which health technology (ies) are currently used. What is the status of the technology (ies)? Whether it provides curative treatment, life extension, etc.)
Will the proposed technology replace or be a supplement to today's SOC?

Current standard of care for the patients expected use Hizentra PFS are those currently using Hizentra vial formulation

4. This proposal concerns:	Yes	No
A brand new and innovative health technology	☒	☐
A new application, or a new indication for an established method	☐	☒
A comparison between several methods	☐	☒
A technology that is already in use	☒	☐
If yes – technology used in clinical practice	☒	☐
If yes – technology used in research/clinical trials	☐	☒
A re-evaluation of technology used in clinical practice	☐	☒

The technology is relevant for disinvestment ☐ ☒

As described above, the treatment is already in use, but not in the same formulation/presentation. This is the same pharmaceutical as currently available Hizentra, but with a different formulation based on pre-filled syringes.

5. This health technology involves (Multiple ticks are possible)

Pharmaceutical ☒

Medical device/IVD medical device that is CE-marked* ☐

Medical device/IVD medical device that is not CE-marked ☐

Procedure ☐

Screening ☐

Highly specialized services / national offers ☐

Organization of the health services ☐

Other (describe) ☐

6. Application of the technology:

Prevention	☐
Assessment and diagnostics	☐
Treatment	☒
Rehabilitation	☐
Specialist health care	☐
Primary health care	☐

Hizentra is a replacement for patients with primary or secondary immunodeficiency syndromes (PID or SID), or patients with chronic inflammatory demyelinating polyneuropathy (CIDP).

7. Responsibility for funding

	Yes	No
Is the specialized health service responsible for financing the technology today?	☒	☐
May the specialized health service become responsible for funding the health technology?	☒	☐

Hizentra is prescribed through H-prescription

8. Is the technology mentioned in the national guidelines or action programs prepared by the Norwegian Directorate of Health?

Yes	No
☐	☒

There are no Norwegian guidelines in PID, SID and CIDP

9. Does the technology involve the use of radiation (ionizing/ non- ionizing)?

Yes	No
☐	☒

10. Which discipline(s) does the health technology apply to, and which patients are affected? (Could the health technology also affect other groups (e.g. health personnel or relatives)?)

Treatment is used for patients with primary or secondary immunodeficiency syndromes (PID or SID), or patients with chronic inflammatory demyelinating polyneuropathy (CIDP). They may be treated by different health care disciplines.

The treatment is often administrated at home and patients and their relatives will mainly benefit from an improved administration form. If more patients can be treated at home because of the new PFS formulation, the hospital will benefit from reduced resources need for administration at hospital. Also less time needed for start-ups of new patients.

11. Which aspects are relevant to the assessment? (Multiple ticks are possible)

Clinical efficacy	☐
Safety/adverse effects	☐
Costs/resource use	☒
Cost-effectiveness	☐
Organizational consequences	☐
Ethical	☐
Legal	☐

12. Please suggest the main scope/objective for the health technology assessment, as well as secondary scopes/objectives (in compliance with question 10). For those familiar with "PICO" (Patient, Intervention, Comparator, Outcome) – please include tentative suggestions for PICO.

This assessment is suggested to focus on the consequences of PFS formulation and to assess a reasonable additional cost for this formulation. The main benefits can be translated into cost differences, although there may be patient convenience/utility benefits as well, which are difficult to quantify as cost.

13. Please give a brief explanation of why it is important that the health technology assessment proposed should be conducted.

New formulations like this PFS are not always evaluated through HTAs. However, the situation for Hizentra is different because Hizentra is produced based on blood donation and the manufacturing cost therefore is a substantial part of the product cost/price. The production cost for the PFS formulation is higher than for the currently available vial formulation and there has been R&D investments made to develop the PFS. To ensure there are incentives to invest in developing new improved formulations, it is important that there is a chance for the manufacturer to recover and benefit from these investments and increased production costs.

Because of this and because there are potential patient, relatives and health care benefits with the PFS formulation, it is reasonable that the cost for the PFS formulation can be higher than the vial formulation. To be able to have a different price for this PFS formulation, there need to be a HTA assessment through NyeMetoder.

14. Please comment on the technology that is proposed to be assessed with regard to the following points:

The severity of the disease/condition the health technology targets

PID, SID or CIDP are severe diseases with high risk of complications without proper treatment

Expected effect

There is no expected difference in treatment effect between the new PFS formulation and the currently available formulation. Benefits mainly relates to administration costs and patient convenience.

Safety

There is no expected difference in treatment safety between the new PFS formulation and the currently available formulation, but the new PFS formulation may reduce the risk of administration errors

Total number of patients in Norway the health technology is applicable to

According to Norwegian prescription register, about 900 patients per year in Norway receive prescription in ATC group J06BA01. There are a few different products within that group and only a part of these 900 receives Hizentra.

Consequences for resource use in the public health service

Reduction in administration-related burden

Need for revision of existing national guidelines or preparation of new guidelines

No

15. Please provide references to documentation of the health technology's effect and safety (i.e. previous technology assessments). (Up to 10 key references can be provided, please do not send attachments in this step of the process):

There are no clinical studies published of the administration of Hizentra with PFS. The long-term efficacy and safety of Hizentra vials in general administered with pump is evaluated in a phase 3 study (Jolles 2011, Jolles 2014). The tolerability and safety of manual push administration (using vials instead of PFS) is evaluated in a study (Cowan 2021). Jolles S et al. Efficacy and safety of Hizentra(®) in patients with primary immunodeficiency after a dose-equivalent switch from intravenous or subcutaneous replacement therapy. Clin Immunol. 2011 Oct;141(1):90-102 Jolles S et al. Long-term efficacy, safety, and tolerability of Hizentra® for treatment of primary immunodeficiency disease. Clin Immunol. 2014 Feb;150(2):161-9 Cowan J et al. Safety and Tolerability of Manual Push Administration of Subcutaneous IgPro20 at High Infusion Rates in Patients with Primary Immunodeficiency: Findings from the Manual Push Administration Cohort of the HILO Study. J Clin Immunol. 2021 Jan;41(1):66-75

16. Please provide the name of the marketing authorization holder/manufacturer/supplier of the health technology (if applicable/available):

CSL Behring GmbH

17. Marketing Authorization Status (MA) or CE-marking: When is MA or CE- marking expected? If possible, provide the time of planned marketing:

MA is available

18. Additional relevant information (up to 300 words.)

19. Interests and potential conflicts of interests

Please describe the proposer's relationships or activities that may affect, be influenced by, or be perceived by others to be important for further management of the health technology that is proposed assessed. (E.g. proposer has financial interests in the matter. Proposer has or has had assignments in connection with the technology or to other actors with interest in the technology)

CSL Behring is the manufacturer of Hizentra PFS