

MSAC application 1834

**Efanesoctocog alfa for routine
prophylaxis in patients with
haemophilia A**

Application or referral for other medical service or health technology

HPP Application number:

HPP200445

Application title:

Efanesoctocog alfa for haemophilia A patients without inhibitors

Submitting organisation:

SANOFI-AVENTIS AUSTRALIA PTY LTD

Submitting organisation ABN:

31008558807

Application description

Succinct description of the medical condition/s:

Haemophilia A (HMA) is a rare, lifelong inherited bleeding disorder that prevents blood from clotting properly due to a deficiency in clotting factor VIII (FVIII). People with HMA experience excessive, painful and prolonged bleeding. This can occur spontaneously (without obvious cause), after minor injuries, or after major trauma or surgery. Most bleeding episodes occur in joints (70-80% of bleeds), and muscles (10-20% of bleeds).

Succinct description of the service or health technology:

Efanesoctocog alfa is a high-sustained FVIII therapy. It is an engineered fusion protein that combines recombinant FVIII with a region of von Willebrand factor (VWF) and XTEN polypeptides. This design allows efanesoctocog alfa to break through the 'von Willebrand factor ceiling', a biological limitation that has historically restricted how long FVIII replacement therapies remain active in the bloodstream.

By bypassing the natural half-life limitation imposed by von Willebrand factor, efanesoctocog alfa remains active in the body significantly longer than standard (SHL) or extended half-life (EHL) FVIII products, approximately 3 to 4 times longer. This allows FVIII activity levels to be maintained within the normal to near-normal range (above 40%) for most of the week following a single dose.

Efanesoctocog alfa is administered as a once-weekly injection at a dose of 50 IU/kg.

Application contact details

Are you applying on behalf of an organisation, or as an individual?

Organisation

Is the applicant organisation the organisation you are representing in the HPP today?

Yes

Applicant organisation name:

SANOFI-AVENTIS AUSTRALIA PTY LTD

Application details

Please select the program through which the health technology would be funded:

National Blood Agreement

Please provide justification for selecting the above program:

Haemophilia treatments are funded on the national product price list (NPPL). Efanesoctocog alfa is a high sustained factor VIII replacement product that provides high bleed protection in patients with congenital haemophilia A without inhibitors. We are seeking NPPL for routine prophylaxis and perioperative management.

What is the type of service or health technology?

Therapeutic

PICO sets

Application PICO sets:

PICO set name
Efanesoctocog alfa for routine prophylaxis and perioperative management for HMA

Population

Describe the population in which the proposed health technology is intended to be used:

This application seeks to include efanesoctocog alfa on the National Product Price List (NPPL) for routine prophylaxis to prevent or reduce the frequency of bleeding episodes in adults, adolescents, and children with haemophilia A (HMA) without factor VIII (FVIII) inhibitors. Funding is also requested for the perioperative management of bleeding (surgical prophylaxis) of all HMA patients without inhibitors.

Select the most applicable Medical condition terminology (SNOMED CT):

Hereditary factor VIII deficiency disease without inhibitor

Intervention

Name of the proposed health technology:

Efanesoctocog alfa

Comparator

Nominate the appropriate comparator(s) for the proposed medical service (i.e. how is the proposed population currently managed in the absence of the proposed medical service being available in the Australian health care system). This includes identifying health care resources that are needed to be delivered at the same time as the comparator service:

Prophylaxis

The nominated comparators in patients with moderate or severe HMA without inhibitors requiring prophylaxis are:

- Emicizumab prophylaxis plus EHL FVIII replacement therapy for breakthrough bleeds
- EHL FVIII replacement therapy for prophylaxis and additional doses to treat breakthrough bleeds.

Perioperative treatment

The nominated comparator in patients with HMA, of any severity, without inhibitors requiring perioperative management is:

- EHL FVIII replacement therapy.

Outcomes

Outcome description – please include information about whether a change in patient management, or prognosis, occurs as a result of the test information:

Prophylaxis

The outcomes of XTEND 1 (von Drygalski et al., 2023) and XTEND Kids (Malec et al., 2024), relevant to the assessment of prophylaxis are:

Health benefits:

- Annualised bleeding rate
 - Any bleed (treated or untreated)
 - Treated bleeds
 - Treated joint bleeds
 - Treated spontaneous bleeds
- Joint health (Haemophilia Joint Health Score).

Health harms:

- Adverse events
- FVIII inhibitor development.

Perioperative management

The outcomes of the analysis of patients undergoing major and minor surgery in XTEND 1 and XTEND Kids relevant to the assessment of perioperative management are (Klamroth et al., 2025b):

Health benefits:

- Surgeon/investigator's assessment of haemostatic response.

Health harms:

- Estimated blood loss during surgery and in the post operative period
- Safety in the surgical/rehabilitation period.

Resource use:

- Number of doses, total consumption of FVIII
- Number and type of blood transfusions.

Specified restrictions for funding

Please add one or more items, with specified restrictions for funding, for each Population / Intervention: (repeat the fields highlighted below for each proposed item provided)

Proposed item:

AAAAA

Is the proposed item restricted?

Yes - restricted

Provide a short description of the restriction:

Routine prophylaxis and perioperative management of haemophilia A patients without inhibitors

Please draft a proposed restriction to define the population and health technology usage characteristics that would define eligibility for funding:

Prophylaxis

Routine prophylaxis to prevent or reduce bleeding episodes in patients with haemophilia A (congenital factor VIII) without inhibitors.

Perioperative management

Perioperative management of bleeding (surgical prophylaxis) in patients with haemophilia A (congenital factor VIII) without inhibitors.

Proposed price of supply:

To be determined

Indicate the overall cost per patient of providing the proposed health technology:

To be determined

Provide details and explain:

A weighted price based on prophylaxis and perioperative management is to be determined.

Prophylaxis

The data for efanesoctocog alfa as prophylaxis supports a clinical claim of superior efficacy and non-inferior safety claim versus emicizumab and EHL FVIII. Cost equivalence is sought for efanesoctocog alfa prophylaxis and breakthrough bleeds versus:

- Emicizumab prophylaxis plus FVIII replacement for breakthrough bleeds
- EHL FVIII prophylaxis and breakthrough bleeds.

This is consistent with approach take for emicizumab (MSAC PSD for 1579, p12).

Perioperative management

For perioperative management, the evidence supports a clinical claim of superiority for efanesoctocog alfa versus EHL FVIII. The total cost of efanesoctocog alfa is expected to be lower than EHL FVIII, as fewer IU/kg are required to achieve haemostasis (Table 17), resulting in reduced total FVIII consumption and cost per surgical episode (Staber et al., 2026). Together with reduced postoperative bleeding episodes, blood transfusions required, and length of hospital stay, efanesoctocog alfa represents less costly option.

How is the technology / service funded at present? (For example: research funding; State-based funding; self-funded by patients; no funding or payments):

No funding or payments

Claims

In terms of health outcomes (comparative benefits and harms), is the proposed technology claimed to be superior, non-inferior or inferior to the comparator(s)?

Superior

Please state what the overall claim is, and provide a rationale:

Prophylaxis:

1. Efanesoctocog alfa is superior versus emicizumab as prophylaxis in adult and adolescent HMA patients aged ≥ 12 years without inhibitors based on a matching adjusted indirect comparison (MAIC) informed by XTEND-1 and the pivotal emicizumab trial, HAVEN 3.
2. Efanesoctocog alfa is superior versus EHL FVIII as prophylaxis in adults and adolescents with HMA without inhibitors based on meta-analyses of MAICs.

Perioperative management

Efanesoctocog alfa is superior versus EHL FVIII for perioperative management.

Estimated utilisation

Estimate the prevalence and/or incidence of the proposed population:

Prophylaxis

The prevalence of adult, adolescent and paediatric patients with moderate to severe HMA without inhibitors for prophylaxis and treatment of breakthrough bleeds is estimated from the ABDR Annual Report 2024-25. These data indicate that there are 785 patients with moderate to severe HMA without inhibitors receiving prophylaxis in Australia. This cohort comprises 590 adults and adolescent (75.1%) and 195 paediatric patients (24.9%).

Perioperative management

The ABDR does not report data on the prevalence and/or incidence of the adult, adolescent and paediatric patients with HMA who require perioperative management of bleeding (surgical prophylaxis). The number of patients who may be eligible for perioperative treatment with efanesoctocog alfa will, therefore, be estimated from data that Sanofi aims to collect from Haemophilia Treatment Centres. Hence, an estimate of the prevalent and/or incident population cannot be provided at this time.

Provide the percentage uptake of the proposed health technology by the proposed population:

Year 1 estimated uptake (%):

10

Year 2 estimated uptake (%):

15

Year 3 estimated uptake (%):

20

Year 4 estimated uptake (%):

22.5

Estimate the number of patients who will utilise the proposed technology for the first full year:

115 for prophylaxis; perioperative management to be determined

Optionally, provide details:

Prophylaxis

Informed by the eligible adult and adolescent, and paediatric populations as well as the anticipated percentage uptake in the first year, 115 patients are expected to receive efanesoctocog alfa for prophylaxis in the first year. This comprises 87 adults and adolescent patients and 29 paediatric patients based on the age distribution from an analysis of the ABDR registry data.

Perioperative management

The number and percentage uptake of efanesoctocog alfa for perioperative management has not yet been determined and will be estimated as soon as the total eligible population is known. While an estimate cannot be provided at this time, with advances in haemophilia treatment that offer higher levels of protection in preventative management it could be expected that surgical interventions may decline over time.

Will the technology be needed more than once per patient?

Yes, multiple times

Over what duration will the health technology or service be provided for a patient? (preferably a number of years):

Life long

Optionally, provide details:

Yes, efanesoctocog alfa for prophylaxis requires continuous treatment. While treatment of breakthrough bleeds and perioperative management are episodic, some patients may experience more than one breakthrough bleed and/or undergo surgery over their lifetime.

What frequency will the health technology or service be required by the patient over the duration? (range, preferably on an annual basis):

Once weekly; 52 intravenous infusions per year

Optionally, provide details:

Once weekly; 52 intravenous infusions per year

Consultation

List all entities that are relevant to the proposed service / health technology. The list can include professional bodies / organisations who provide, request, may be impacted by the service/health technology; sponsor(s) and / or manufacturer(s) who produce similar products; patient and consumer advocacy organisations or individuals relevant to the proposed service/health technology.

Entities who provide the health technology/service:

Australian and New Zealand Haemophilia Physiotherapy Group (ANZHPG)
AUSTRALIAN ASSOCIATION OF SOCIAL WORKERS LIMITED (AASW)
AUSTRALIAN HAEMOPHILIA CENTRE DIRECTORS ORGANISATION INC
Australian Haemophilia Nurses Group (AHNG)
National Blood Authority
THE ROYAL COLLEGE OF PATHOLOGISTS OF AUSTRALASIA
THROMBOSIS & HAEMOSTASIS SOCIETY OF AUSTRALIA & NEW ZEALAND LIMITED

Entities who may be impacted by the health technology/service

Australian and New Zealand Haemophilia Physiotherapy Group (ANZHPG)
AUSTRALIAN ASSOCIATION OF SOCIAL WORKERS LIMITED (AASW)
AUSTRALIAN HAEMOPHILIA CENTRE DIRECTORS ORGANISATION INC
Australian Haemophilia Nurses Group (AHNG)
National Blood Authority
THE ROYAL COLLEGE OF PATHOLOGISTS OF AUSTRALASIA
THROMBOSIS & HAEMOSTASIS SOCIETY OF AUSTRALIA & NEW ZEALAND LIMITED

Patient and consumer advocacy organisations relevant to the proposed service/health technology

HAEMOPHILIA FOUNDATION AUSTRALIA INC
World Federation of Hemophilia (WFH)

Entities who produce similar products

ROCHE PRODUCTS PTY LTD

TAKEDA PHARMACEUTICALS AUSTRALIA PTY. LTD.

PFIZER AUSTRALIA PTY LTD

CSL BEHRING (AUSTRALIA) PTY LTD

SANOFI-AVENTIS AUSTRALIA PTY LTD

Regulatory information

Would the proposed health technology involve the use of a medical device, in-vitro diagnostic test, radioactive tracer or any other type of therapeutic good?

Yes

Has it been listed or registered or included in the Australian Register of Therapeutic Goods (ARTG) by the Therapeutic Goods Administration (TGA)?

Yes

Is the therapeutic good classified by the TGA as either a Class III or Active Implantable Medical Device (AIMD) against the TGA regulatory scheme for devices?

No

Please enter all relevant ARTG IDs:

ARTG ID	ARTG name
492505	ALTUVIIIIO efanesoctocog alfa 1000 IU powder for injection vial
492506	ALTUVIIIIO efanesoctocog alfa 2000 IU powder for injection vial
492507	ALTUVIIIIO efanesoctocog alfa 250 IU powder for injection vial
492508	ALTUVIIIIO efanesoctocog alfa 3000 IU powder for injection vial
492509	ALTUVIIIIO efanesoctocog alfa 4000 IU powder for injection vial
492510	ALTUVIIIIO efanesoctocog alfa 500 IU powder for injection vial

Is the intended purpose in this application the same as the intended purpose of the ARTG listing(s)?

No

Provide details:

TGA-approved indication:

ALTUVIIIIO is a long-acting recombinant anti-haemophilic factor (coagulation FVIII) indicated in adults, adolescents and children with haemophilia A (congenital FVIII deficiency) for:

- Routine prophylaxis to prevent or reduce the frequency of bleeding episodes
- Treatment and control of bleeding episodes
- Perioperative management of bleeding (surgical prophylaxis).

ALTUVIIIIO does not contain von Willebrand factor, and therefore is not indicated in patients with von Willebrand's disease.

We are seeking NPPL funding for:

- Routine prophylaxis to prevent or reduce the frequency of bleeding episodes
- Perioperative management of bleeding (surgical prophylaxis).

There are already a number of factor replacement therapies for the on demand treatment and control of bleeding episodes.