

MSAC Application 1728.1

**Etranacogene dezaparvovec for the
treatment of congenital
haemophilia B**

Application for MBS eligible service or health technology

HPP Application number:

HPP200260

Application title:

Etranacogene dezaparovec for the treatment of congenital haemophilia B

Submitting organisation:

CSL BEHRING (AUSTRALIA) PTY LTD

Submitting organisation ABN:

48160734761

Application description

Succinct description of the medical condition/s:

Haemophilia is a rare congenital bleeding disorder caused by deficiencies in coagulation factors as a result of mutations in clotting factor genes. There are two main types of haemophilia, with type A (HMA) accounting for 80-85% and type B (HMB) around 15% of the total prevalent population.

HMB is characterised by congenital underproduction or dysfunction of coagulation factor IX (FIX), an essential protein involved in promoting clot formation. The FIX gene is found on the X chromosome and because the genetic defect is expressed in an X-linked recessive manner the vast majority of people living with the disease are male. A family history is present in about two-thirds of patients and the remaining cases are caused by spontaneous mutations. HMB is a lifelong condition typically causing bleeding tendency. Serious bleeds can result in disabling sequelae and may even be fatal in some circumstances.

Succinct description of the service or health technology:

Hemgenix® (also known as etranacogene dezaparovec, AMT-061 and CSL222) is a gene therapy designed to introduce a copy of the human FIX gene to address the lack of functional FIX protein expression in a haemophilia B patient. Hemgenix® is an infusion of recombinant adeno-associated virus 5 (AAV5) vector including a gene cassette containing the FIX Padua variant under the control of a liverspecific promoter. After infusion, EtranaDez preferentially targets liver cells, where vector DNA is released into the nucleus instructing the cell to produce FIX. Following

transduction, functional FIX is produced at near normal to normal levels and circulates in the body, reducing the risk of bleeding.

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:

CSL BEHRING (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:

Under the national blood arrangements, blood and blood-related products and services are jointly funded by the Australian Government and state and territory governments, in accordance with the National Blood Agreement (Agreement), which is administered by the National Blood Authority (NBA). Although Hemgenix does not consist of human blood or components of human blood, nor is it derived from human blood, it could be regarded as a blood-related product as defined by the Agreement, as it is proposed as an alternative therapy to the use of blood products currently funded under the national blood arrangements.

What is the type of service or health technology?

Therapeutic

Relevant MBS items

Please select any relevant MBS items.

MBS item number	Selected reason type
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What is the type of service or health technology?

Therapeutic

PICO sets

Application PICO sets:

PICO set name
Etranacogene dezaparovec for the treatment of haemophilia B

Population

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

Intervention (Hemgenix®): Adult patients (≥ 18 years) with severe or moderately severe ($\leq 2\%$) congenital haemophilia B (cHMB), currently receiving stable FIX prophylactic therapy, who also meet the following criteria:

- anti-AAV5 NAb titre $< 1:900$ using 9-point assay as determined by the AAV5 NAb assay
- No active infections, either acute or uncontrolled chronic
- No known advanced hepatic fibrosis, or cirrhosis

Intervention

Name of the proposed health technology:

Hemgenix®

Specified restrictions for funding

Please add one or more items, with specified restrictions for funding, for each Population / Intervention:

Proposed item:

AAAAA

Is the proposed item restricted?

Yes - restricted

Provide a short description of the restriction:

For treatment of haemophilia B

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

Adult patients (≥ 18 years) with severe or moderately severe ($\leq 2\%$) congenital haemophilia B (cHMB), currently receiving stable FIX prophylactic therapy, who also meet the following criteria:

- anti-AAV5 NAb titre $< 1:900$ using 9-point assay as determined by the AAV5 NAb assay
- No active infections, either acute or uncontrolled chronic
- No known advanced hepatic fibrosis, or cirrhosis

Proposed price of supply:

\$0.00

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

\$0.00

Provide details and explain:

The cost of Hemgenix® and cost-utility model will be provided in the Application Developed Assessment Report (ADAR).

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

Not currently funded

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:

Standard of care for cHMB with no gene therapy

Outcomes

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

For outcome description of health outcomes for Hemgenix® and assay please refer to the Ratified PICO for Application 1728 and the PICO set for this application. The AAV5 NAb test will inform eligibility for treatment with Hemgenix® (etranacogene dezaparvovec), and as such, patient management will change to include Hemgenix® as a treatment option.

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:

HOPE-B (pivotal phase 3 trial) demonstrates a significantly reduce annualised bleeding rate (ABR), sustained increases to FIX % activity level, significant reduction in FIX consumption and frequency and improvements in QoL metrics. The pivotal Phase III trial also shows that Hemgenix® is reasonably safe and well tolerated in the target population of patients with severe or moderately severe HMB receiving routine FIX prophylaxis, being associated with similar or even lower rates of adverse events than the pre-treatment standard of care (Pipe 2023).

Estimated utilisation

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

Based on information from the Australian Bleeding Disorders Registry (ABDR), there were 71 adults living with severe disease and 30 living with moderately severe disease enrolled in the ABDR on the 20th of June. Among these, **redacted** with

severe disease and **redacted** with moderately severe disease received prophylaxis therapy with FIX concentrates (total = **redacted**). It is expected that this cohort (severe and moderately severe HMB on prophylaxis) will be approximately **redacted** patients by CY 2026 based on historical growth rates from the ABDR. **Redacted.**

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

Year 1 estimated uptake (%):

TBC

Year 2 estimated uptake (%):

TBC

Year 3 estimated uptake (%):

TBC

Year 4 estimated uptake (%):

TBC

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

Estimates on uptake will be provided in the Applicant-Developed Assessment Report (ADAR).

Will the technology be needed more than once per patient?

No, once only

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 Haemophilia Centre Directors' Organisation (AHCDO)

Royal Australasian College of Physicians (RACP)

Royal College of Pathologists of Australasia (RCPA)

Entities who produces similar products

Australian Haemophilia Centre Directors' Organisation (AHCDO)

National Blood Authority (NBA)

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

Haemophilia Foundation Australia (HFA)

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)? *(if 'Yes' above)*

No

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

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

No

Is the therapeutic good to be used in the service exempt from the regulatory requirements of the Therapeutic Goods Act 1989?

No

Is the therapeutic good classified by the TGA as for Research Use Only (RUO)?

No

Is the therapeutic good in the process of being considered by the TGA? *(if 'Yes' above)*

No

Please provide details of when you intend to lodge an ARTG inclusion application, or provide a rationale if you do not intend to lodge an ARTG inclusion application: *(if 'No' above)*

There are no plans to lodge an ARTG inclusion application as the AAV5 NAb assay will not be conducted onshore.