Formulary Listing Recommendation Report:

Vonvendi® (recombinant von Willebrand factor)

Canadian Blood Services' Plasma Protein and Related Products Formulary

Canadian Blood Services is an independent not-for-profit organization, operating independently of government. Operating within a broader national network of health-care systems, we are responsible for providing blood and blood products, as well as transfusion and stem cell registry services, on behalf of all provincial and territorial governments except Quebec.

As part of our work, Canadian Blood Services manage a pan-Canadian formulary of plasma protein and related products (PPRP) which are accessible to clinicians in Canada for use in caring for their patients. The formulary is fully integrated with the national blood system, as we also manage the procurement, inventory management, and distribution of PPRP.

For more information about the PPRP program and formulary, please visit Plasma protein and related products (blood.ca).

CADTH-Canadian Blood Services Interim Plasma Protein and Related Product Review Process

In November 2019, CADTH and Canadian Blood Services established a new interim process for the review of PPRP.

Applications from manufacturers for PPRP which are new in Canada are submitted to the Canadian Agency for Drugs and Technologies in Health (CADTH) and Canadian Blood Services for consideration.

Provincial and Territorial Ministries of Health make an initial decision on whether the new product will be assessed through the interim PPRP review process.

Once confirmed, CADTH and Canadian Blood Services conduct assessments on the product to incorporate clinical, pharmacoeconomic, and ethical considerations before a final recommendation is submitted to Provincial and Territorial Ministries of Health for a decision on whether the product will be carried under Canadian Blood Services' formulary.

*In the case of procurement of new brands of existing products on Canadian Blood Services' formulary, Canadian Blood Services makes the final decision.

This formulary listing recommendation report provides details on CADTH's and Canadian Blood Services' recommendations for Vonvendi®, supporting notes, and key milestones of the review.

Submission Summary

Brand name: Vonvendi®

Chemical name: Recombinant von Willebrand Factor (rVWF)

Dosage form: IV infusion

Supplier: Takeda Canada

Health Canada indication:

- Treatment and Control of bleeding episodes in adults (age ≥18) diagnosed with von Willebrand Disease (VWD).
- Perioperative management of bleeding in adults (age ≥18) diagnosed with VWD.

Reimbursement request (from supplier):

- Adults (age ≥18) diagnosed with severe VWD.
- Adults (age ≥18) diagnosed with mild or moderate VWD who do not respond or are intolerant to DDAVP (desmopressin).

Review type: Interim Plasma Protein and Related Products Review

Final listing decision: Eligible for listing

CADTH recommendation

Date recommendation issued: March 22, 2021

Recommendation: CADTH CPEC recommended to list with criteria. Visit the CADTH website for more details.

ST0639 Vonvendi® - Final CPEC Recommendation March 22, 2021 for posting.pdf (cadth.ca)

CADTH recommendation □ List ☒ List with conditions. □ Do not list.	Initiation criteria 1. Adult patients with severe non-type 3 and type 3 VWD defined as any of the following: 1.1. Type 1 (VWF:RCo < 20 IU/dL) 1.2. Type 2A (VWF:RCo < 20 IU/dL), Type 2B (diagnosed by genotype), Type 2N (FVIII:C < 10% and historically documented genetics), or Type 2M 1.3. Type 3 (VWF:Ag ≤ 3 IU/dL) Prescribing conditions 1. The patients must be under the care of a hematologist with experience in the diagnosis and management of VWD. Pricing conditions 1. The public payer cost for rVWF with concomitant rFVIII should not exceed the public payer cost of treatment with the least costly alternative treatment regimen for the management of VWD.

Canadian Blood Services recommendation

Date of recommendation: Sep 15, 2022

Recommendation:

Canadian Blood Services recommends that Vonvendi® be eligible to be listed for treatment and control of bleeding episodes or for perioperative management of bleeding in adults (age ≥ 18) diagnosed with VWD on a condition of a lower price that demonstrates its value compared to plasma-derived products already on Canadian Blood Services' PPRP formulary.

Notes

- CADTH recommendation report noted that no direct or indirect evidence was available comparing the efficacy or safety of Vonvendi® with plasma-derived VWF/FVIII products (the current standard of care). In the absence of a control group or any comparative data, there is no evidence to support a price premium over currently available plasma-derived products for the control of bleeding episodes or perioperative management in patients with VWD.
- There is no indication that Vonvendi is a safer product than currently available plasma derived therapies. Several aspects of safety were considered, including the risk of blood-borne infection with plasma-derived products which is extremely low and managed by modern risk mitigation strategies.
- Exceptional access will be assessed on a case-by-case basis.
- Negotiations with the vendor for Vonvendi® through the interim PPRP review were not able to justify a price premium of Vonvendi® over current plasma-derived VWF replacement products.
- The manufacturer can submit a proposal for Vonvendi® through a future Canadian Blood Services' RFP to be evaluated alongside comparable products with the following criteria:
  - Eligibility criteria: for the treatment and control of bleeding episodes or perioperative management of bleeding in adults (aged ≥ 18) diagnosed with
    - severe VWD, or
    - mild or moderate VWD who do not respond or are intolerant to DDAVP.
  - Prescribing criteria: patients should be under the care of a hematologist with experience in the diagnosis and management of VWD.