FABHALTA® (iptacopan)

Product Information

A comparison of select agents in PNH and their non-clinical product characteristics†

FABHALTA® (iptacopan) is indicated as monotherapy in the treatment of adult patients with paroxysmal nocturnal hemoglobinuria (PNH) who have hemolytic anemia.

Register on the Novartis Pro Portal to learn about FABHALTA®

† Comparative clinical significance has not been established.

A comparison of select agents in PNH†

Indication, route of administration and dosing overview†

	FABHALTA® (iptacopan)	Empaveli™ (pegcetacoplan)	Voydeya™ (danicopan)	Soliris® (eculizumab)	Ultomiris® (ravulizumab)
Indication in PNH	FABHALTA® (iptacopan) is indicated as monotherapy in the treatment of adult patients with paroxysmal nocturnal hemoglobinuria (PNH) who have hemolytic anemia.[1]	Empaveli™ (pegcetacoplan) is a complement inhibitor indicated for the treatment of adult patients with PNH who have an inadequate response to, or are intolerant of, a C5 inhibitor.[2]	Voydeya™ (danicopan) is indicated as an add-on to ravulizumab or eculizumab for the treatment of adult patients with PNH who have residual hemolytic anemia due to extravascular hemolysis (EVH).[3]	Soliris® (eculizumab) is indicated for the treatment of patients with PNH to reduce hemolysis. Soliris® was studied in clinical trials in patients with a history of at least one transfusion during the past two years.[4]	Ultomiris® (ravulizumab) is indicated for the treatment of adult and pediatric patients one month of age and older with PNH.[5]
Route of administration	Oral[1]	Subcutaneous (SC) administration using a syringe system infusion pump[2]	Oral (plus intravenous [IV] infusion for ravulizumab or eculizumab)[3]	IV infusion[4]	IV infusion[5]
Dosing	200 mg twice daily[1]	1080 mg twice weekly or every 3 days[2]	150 mg to 200 mg three times a day (plus dose of ravulizumab or eculizumab)[3]	600 mg every 7 days for the first 4 weeks, followed by 900 mg for the fifth dose 1 week later, then 900 mg every 2 weeks thereafter[4]	**≥5kg to <10kg:** 600 mg loading dose; 300 mg maintenance dose every 4 weeks **≥10kg to <20kg:** 600 mg loading dose; 600 mg maintenance dose every 4 weeks **≥20kg to <30kg:** 900 mg loading dose; 2,100 mg maintenance dose every 8 weeks **≥30kg to <40kg:** 1,200 mg loading dose; 2,700 mg maintenance dose every 8 weeks **≥40kg to <60kg:** 2,400 mg loading dose; 3,000 mg maintenance dose every 8 weeks **≥60kg to <100kg:** 2,700 mg loading dose; 3,300 mg maintenance dose every 8 weeks **≥100kg:** 3,000 mg loading dose; 3,600 mg maintenance dose every 8 weeks[5]

Please see the respective product monographs for complete instructions regarding dosing and administration.

† Comparative clinical significance has not been established.

A comparison of select agents in PNH†

Product storage†

	FABHALTA® (iptacopan)	Empaveli™ (pegcetacoplan)	Voydeya™ (danicopan)	Soliris® (eculizumab)	Ultomiris® (ravulizumab)
Storage	Store below 30°C. This medicinal product does not require any special storage conditions. FABHALTA® must be kept out of the reach and sight of children.[1]	Store in the refrigerator (2°C to 8°C). Keep Empaveli™ in its original package to protect from light.[2]	Store in the original container at room temperature between 15°C and 30°C. Keep in a safe place out of reach and sight of children.[3]	Soliris® vials must be stored in the original carton until time of use under refrigerated conditions at 2-8°C and protected from light. Soliris® vials may be held in the original carton at room temperature (not more than 25°C) for a single period of up to 3 days.[4]	Ultomiris® vials must be stored under refrigerated conditions at 2 to 8°C. Keep the vial in the outer carton to protect from light.[5]

Please see the respective product monographs for complete instructions regarding dosing and administration.
† Comparative clinical significance has not been established.

A comparison of select agents in PNH†

Recommended dose in PNH†

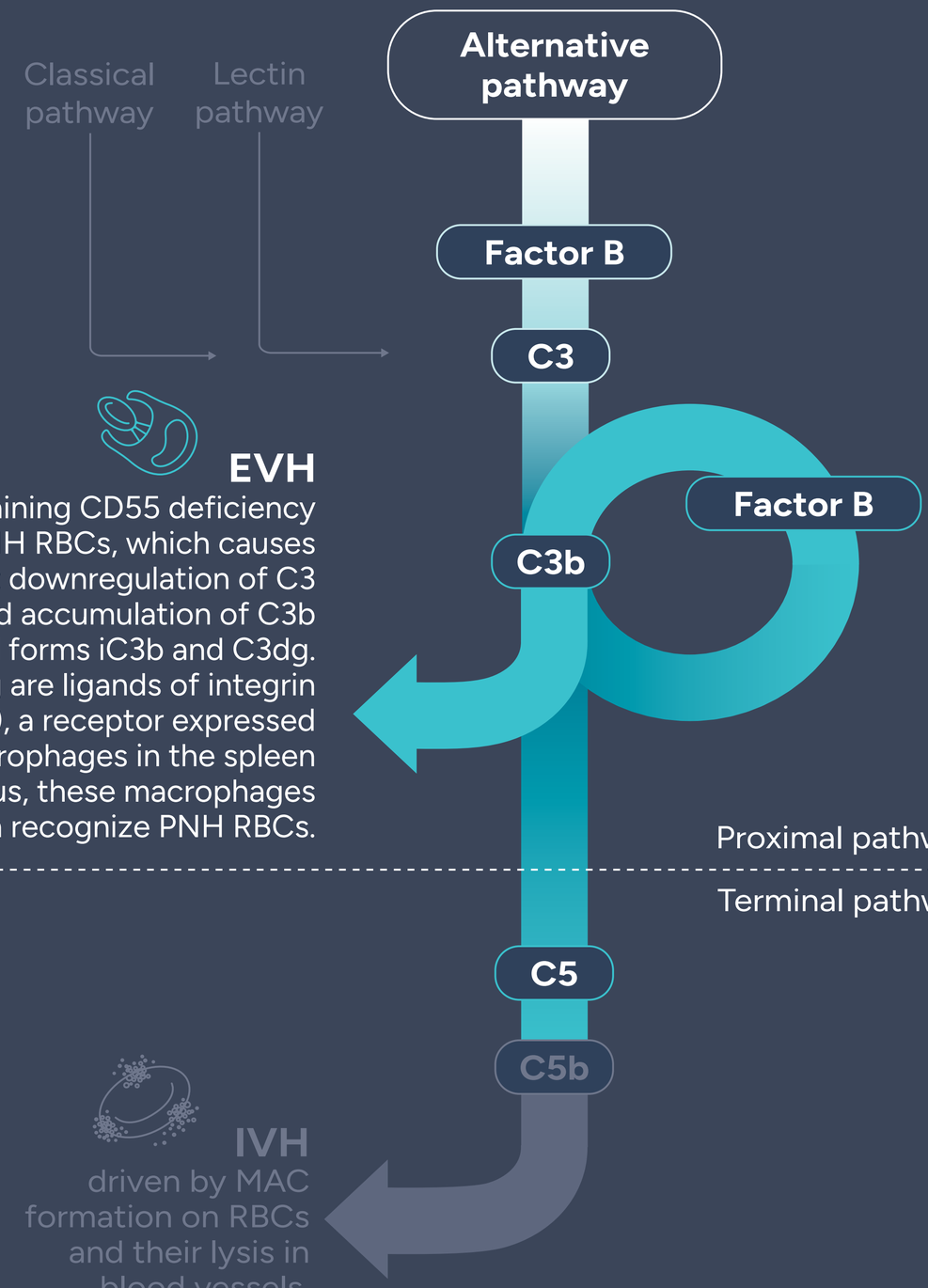
	FABHALTA® (iptacopan)	Empaveli™ (pegcetacoplan)	Voydeya™ (danicopan)	Soliris® (eculizumab)	Ultomiris® (ravulizumab)
Recommended dose	200 mg taken orally twice daily. PNH is a disease that requires chronic treatment. Discontinuation of this medicinal product is not recommended unless clinically indicated.[1]	Empaveli™ is administered twice weekly as a 1080 mg SC infusion with a commercially available syringe system infusion pump that can deliver doses up to 20 mL. The twice weekly dose should be administered on Day 1 and Day 4 of each treatment week. Empaveli™ can be given by a healthcare professional or administered by the patient or caregiver following proper instruction. Infuse Empaveli™ in the abdomen, thighs, hips, or upper arms. PNH is a chronic disease and treatment with Empaveli™ is recommended to continue for the patient's lifetime unless the discontinuation is clinically indicated.[2]	The recommended starting dose of Voydeya™ is 150 mg three times a day administered orally, approximately 8 hours apart (± 2 hours). The dose of Voydeya™ can be increased to 200 mg three times a day if a patient's hemoglobin level has not increased by at least 2 g/dL after 4 weeks of therapy, if a patient required a transfusion within the previous 4 weeks, or to achieve an appropriate hemoglobin response based on clinical judgement.[3]	Soliris® therapy consists of: • 600 mg every 7 days for the first 4 weeks, followed by • 900 mg for the fifth dose 1 week later, then • 900 mg every 2 weeks thereafter. Soliris® should be administered at the recommended dosage regimen time points, or within two days of these time points.[4]	The recommended Ultomiris® IV maintenance dosing in adult and pediatric patients with PNH with a body weight ≥5 kg or adult patients (≥18 years of age) is based on the patient's body weight, with maintenance doses administered every 4 or 8 weeks, starting 2 weeks after loading dose. **≥5 kg to <10 kg:** 600 mg loading dose; 300 mg maintenance dose every 4 weeks **≥10 kg to <20 kg:** 600 mg loading dose; 600 mg maintenance dose every 4 weeks **≥20 kg to <30 kg:** 900 mg loading dose; 2,100 mg maintenance dose every 8 weeks **≥30 kg to <40 kg:** 1,200 mg loading dose; 2,700 mg maintenance dose every 8 weeks **≥40 kg to <60 kg:** 2,400 mg loading dose; 3,000 mg maintenance dose every 8 weeks **≥60 kg to <100 kg:** 2,700 mg loading dose; 3,300 mg maintenance dose every 8 weeks **≥100 kg:** 3,000 mg loading dose; 3,600 mg maintenance dose every 8 weeks[5] Dosing schedule is allowed to occasionally vary by ± 7 days of the scheduled infusion day (except for the first maintenance dose of Ultomiris®) but the subsequent dose should be administered according to the original schedule.[5]

Please see the respective product monographs for complete instructions regarding dosing and administration.

† Comparative clinical significance has not been established.

FABHALTA® (iptacopan)

Complement cascade overview[6]



Adapted from Hill *et al.* 2017.[6]

FABHALTA® (iptacopan)

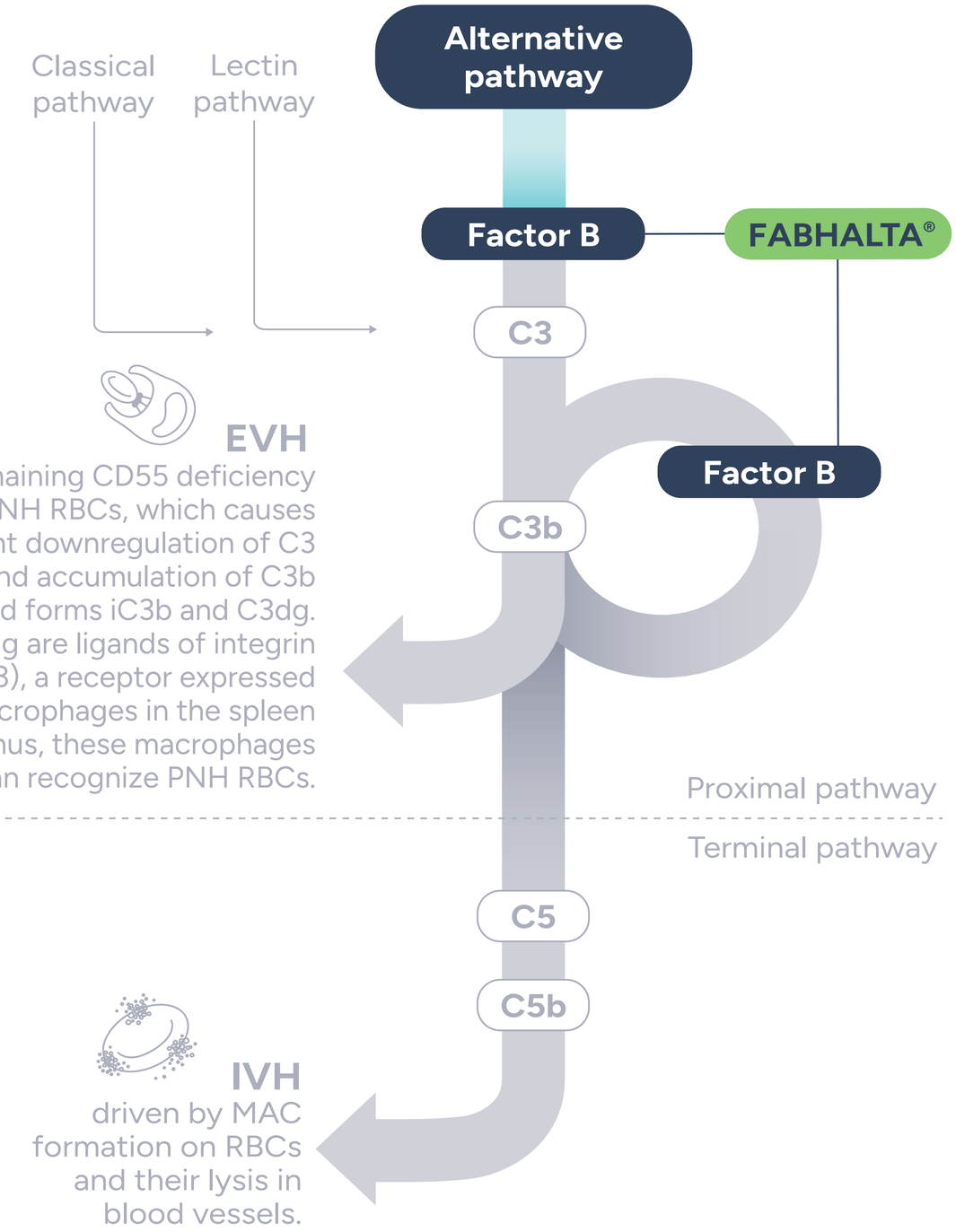
Mechanism of action

FABHALTA® binds to complement Factor B and inhibits the alternative complement pathway[1]

FABHALTA® inhibits the cleavage of the complement component C3, and the subsequent formation of the membrane attack complex (MAC).

In PNH, intravascular hemolysis (IVH) is mediated by the MAC, while extravascular hemolysis (EVH) is facilitated by C3 fragment opsonization. Iptacopan acts proximally in the alternative pathway of the complement cascade to control both C3 fragment-mediated EVH and MAC-mediated IVH.[1]

Adapted from Hill *et al.* 2017 and FABHALTA® Product Monograph.[1,6]



FABHALTA® is the first and only oral monotherapy for patients with PNH who have hemolytic anemia[1,8†]

FABHALTA® (iptacopan)

FABHALTA® (iptacopan) is indicated as monotherapy in the treatment of adult patients with paroxysmal nocturnal hemoglobinuria (PNH) who have hemolytic anemia.

FABHALTA®: a convenient oral monotherapy[1]

Twice daily capsule

The recommended dose is 200 mg taken orally twice daily.

Discontinuation of FABHALTA® is not recommended unless clinically indicated.

Advise your FABHALTA® patients about the importance of adherence to the dosing schedule in order to minimize the risk of hemolysis.

Please refer to the Product Monograph for complete dosing and administration instructions.

Before starting treatment with FABHALTA®, patients must receive vaccines to reduce the risk of serious infections with encapsulated bacteria. Vaccinate patients according to current NACI or regional practice guidelines to reduce the risk of serious infection. Vaccinations are recommended at least 2 weeks before starting FABHALTA®, or, if FABHALTA® must be initiated prior to vaccination, as soon as possible after starting FABHALTA® (with antibiotic prophylaxis until 2 weeks after vaccination).[1]

† Comparative clinical significance has not been established.

The FABHALTA® ASSIST Patient Support Program (PSP)

The FABHALTA® ASSIST PSP is personalized to meet the individual needs of your PNH patients who have been prescribed FABHALTA®

FABHALTA® ASSIST PSP offerings:

Dedicated Clinical Care Manager
You and your patients will have access to a dedicated point of contact, a nurse Clinical Care Manager. The nurse will provide your patients with tailored support including a welcome call and onboarding discussion, reminder services and follow-up calls, and education about their treatment and condition.

Welcome call and onboarding discussion
Your patients will receive a welcome call and onboarding discussion to tailor PSP services to their individual needs.

Patient support materials
The PSP will provide patients with a pill fob and educational materials, including a patient safety card.

Reimbursement navigation and support
The PSP will support your patients with navigating reimbursement options and investigating financial options for costs not covered by insurance.

Vaccination support
The PSP can provide coordination of vaccination appointments and revaccination reminders for required and recommended vaccinations.

FABHALTA® emergency supply
The PSP can help coordinate an emergency supply of FABHALTA®. Emergency supply services can be reached by calling the PSP at 1-877-580-5303 and pressing 1.

Healthcare professional materials
Healthcare professionals will have access to informative materials about FABHALTA®.

Risk Management Program (RMP) and controlled access materials
The RMP materials provide patient information and support about required vaccinations. Controlled access materials allow patients access to FABHALTA®. You can access these materials at **fabhalta.ca**.

Questions?

The FABHALTA® ASSIST Patient Support Program (PSP) is here to answer any questions you may have about our support services. You can reach us at 1-877-580-5303, Monday to Friday, from 8:00 AM to 8:00 PM EST.

For all other inquiries, please contact the Novartis Canada Medical Information team by phone at 1-800-363-8883 or email at **medinfocanada@novartis.com**.

Important safety information

Consult the Product Monograph at **www.novartis.ca/fabhaltamonograph** for important information about:

- Contraindications in patients who are not currently vaccinated against *Neisseria meningitidis*, and *Streptococcus pneumoniae* unless the risk of delaying FABHALTA® treatment outweighs the risk of developing an infection from these encapsulated bacteria, have unresolved serious infection caused by encapsulated bacteria including *Streptococcus pneumoniae*, *Neisseria meningitidis*, or *Haemophilus influenzae* type B, or have hypersensitivity to the product or its excipients.
- The most serious warnings and precautions regarding complying to National Advisory Committee on Immunization (NACI) recommendations or regional practice guidelines for vaccination requirements against encapsulated bacteria, specifically *Neisseria meningitidis* and *Streptococcus pneumoniae*, at least 2 weeks prior to initiating FABHALTA®, unless the risks of delaying FABHALTA® therapy outweigh the risks of developing a serious infection; prophylactic antibiotic treatment until 2 weeks after vaccination for patients who initiate FABHALTA® less than 2 weeks after vaccination; monitoring for serious infections caused by encapsulated bacteria and monitoring of PNH manifestations after discontinuation; and FABHALTA® availability through a controlled distribution program under which prescribers must enroll patients and confirm vaccination, counsel patients about the serious risks of infection, and provide patients with the Patient Guide and Patient Card.
- Other relevant warnings and precautions regarding fertility, pregnancy, disease-associated maternal and/or embryo/fetal risk, breastfeeding and monitoring and lab tests such as blood pressure and cholesterol levels.
- Conditions of clinical use, adverse reactions, drug interactions and dosing/administration instructions.

Consult the Product Monograph at **www.novartis.ca/fabhaltamonograph** for important information relating to adverse drug reactions, drug interactions and dosing information which have not been discussed in this piece. The Product Monograph is also available by calling 1-800-363-8883 or emailing **medinfo.canada@novartis.com**.

References:

1. FABHALTA® Product Monograph. Novartis Pharmaceuticals Canada Inc.
2. Empaveli™ Product Monograph. Swedish Orphan Biovitrum. December 7, 2022.
3. Voydeya™ Product Monograph. Alexion Pharmaceuticals. July 19. 2024.
4. Soliris® Product Monograph. Alexion Pharmaceuticals. March 25, 2021.
5. Ultomiris® Product Monograph. Alexion Pharmaceuticals. October 30, 2023.
6. Hill A, DeZern AE, Kinoshita T, Brodsky RA. Paroxysmal nocturnal haemoglobinuria. *Nat Rev Dis Primers*. 2017 May 18;3:17028.
7. Data on file. Novartis Pharmaceuticals Canada Inc. February 2025.

NOVARTIS

Novartis Pharmaceuticals Canada Inc.
Montréal, Quebec H2Y 0C1
www.novartis.ca
514.631.6775 514.631.1867

FA-11356433E

MEMBER OF INNOVATIVE MEDICINES CANADA

REVIEWED BY PAAB

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