FABHALTA® is the first and only oral monotherapy for patients with PNH who have hemolytic anemia[1,2]*

FABHALTA® (iptacopan)

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

Starting or switching to FABHALTA® in patients with PNH

Healthcare Professional Guide

* Comparative clinical significance has not been established.

Register on the Novartis Pro Portal to learn about FABHALTA®

Prior to starting with FABHALTA®

Required vaccinations to reduce risk of serious infections[1]

The use of complement inhibitors like FABHALTA® may predispose patients to serious, life-threatening or fatal infections caused by encapsulated bacteria, such as *Streptococcus pneumoniae* and *Neisseria meningitidis*.

To reduce the risk of infection:

Patients must be vaccinated against encapsulated bacteria:

N. meningitidis
S. pneumoniae

Patients are recommended to be vaccinated against:

H. influenzae

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)

Vaccination reduces, but does not eliminate, the risk of serious infection. Serious infection may rapidly become life-threatening or fatal if not recognized and treated early. Patients should be informed of and monitored for early signs and symptoms of serious infection and should be immediately evaluated and treated if infection is suspected. The use of FABHALTA® during treatment of serious infection may be considered following an assessment of the risks and benefits. However, it is contraindicated to initiate FABHALTA® in patients with unresolved serious infections caused by encapsulated bacteria.[1]

The FABHALTA® ASSIST Patient Support Program for patients receiving FABHALTA® offers patients vaccination appointment coordination/reminders for the required and recommended vaccinations in regard to FABHALTA® therapy.

FABHALTA®: a convenient oral monotherapy[1]

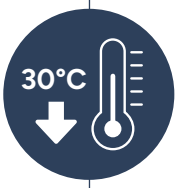
Dosing and storage considerations

Twice daily capsule

The recommended dose is 200 mg taken orally twice daily.

Oral monotherapy option

May be taken with or without food



Store below 30°C

No special storage conditions required

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.

Patient counselling tip: Advise your patients to take FABHALTA® once in the morning and once in the evening. Remind them that taking FABHALTA® at the same time each day will help them to remember when to take their medicine.

If a dose or doses are missed, the patient should be advised to take one dose of FABHALTA® as soon as possible (even if it is soon before the next scheduled dose) and then to resume the regular dosing schedule.

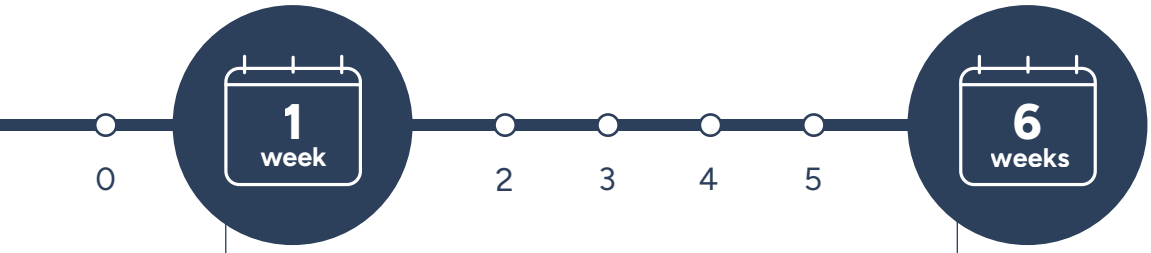
Please refer to the Product Monograph for complete dosing and administration instructions.

FABHALTA® must be kept out of the reach and sight of children.

Switching to FABHALTA®

From anti-C5 (eculizumab, ravulizumab) or other PNH therapies

To reduce the potential risk of hemolysis with abrupt complement inhibitor treatment discontinuation:[1]



For patients switching from eculizumab, **FABHALTA® should be initiated no later than 1 week** after the last dose of eculizumab.

For patients switching from ravulizumab, **FABHALTA® should be initiated no later than 6 weeks** after the last dose of ravulizumab.

Switching from other complement inhibitors to FABHALTA® has not been studied. When switching from other PNH therapies to FABHALTA®, the dosing interval and mode of action of the previous medicinal products should be considered.[1]

If necessary, patients should be revaccinated in accordance with local vaccination guideline recommendations.

See page 2 for more information on vaccination requirements to reduce risk of serious infections prior to starting treatment with FABHALTA®.

FABHALTA ASSIST PATIENT SUPPORT PROGRAM

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.

FABHALTA®: Proven safety profile

The safety of FABHALTA® in patients with PNH was evaluated in two trials[1]

≥5% The most common adverse reactions as assessed by investigators (≥5%) with FABHALTA® were headache and vomiting.

There were no discontinuations due to adverse events or serious adverse events in either of these studies.

A total of 8 serious adverse events (irrespective of causality) were reported in 6 (9.7%) patients who received FABHALTA® during the 24-week randomized treatment period in APPLY-PNH. These included sinus node dysfunction, COVID-19, pyelonephritis, urinary tract infection, blood creatine phosphokinase increased, basal cell carcinoma, myelodysplastic syndrome, transient ischemic attack.

The safety of FABHALTA® in patients with PNH was evaluated in a randomized, active-comparator controlled, open-label trial (APPLY-PNH) for 24 weeks (FABHALTA® n=62). The safety profile is further supplemented by a supportive single-arm, open-label trial (APPOINT-PNH) for 24 weeks (FABHALTA® n=40).

Important safety information

Clinical use:

Pediatrics (<18 years of age): The safety and efficacy of FABHALTA® in pediatric patients below 18 years of age have not been established; therefore, Health Canada has not authorized an indication for pediatric use.

Geriatrics (≥65 years of age): FABHALTA® may be administered to patients aged 65 years and over. Evidence from clinical studies suggests that use in the geriatric population is not associated with differences in safety or effectiveness.

Contraindications:

- Hypersensitivity to iptacopan or to any of the other excipients.
- 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.
- Initiation in patients with unresolved serious infection caused by encapsulated bacteria, including *Streptococcus pneumoniae*, *Neisseria meningitidis*, or *Haemophilus influenzae* type B.

Most serious warnings and precautions:

Serious infections caused by encapsulated bacteria:

Due to its mechanism of action, the use of FABHALTA® may predispose individuals to serious infections caused by encapsulated bacteria, such as *Streptococcus pneumoniae* and *Neisseria meningitidis*.

- Comply with the most current National Advisory Committee on Immunization (NACI) recommendations or regional practice guidelines for vaccinations against encapsulated bacteria, deficiencies.
- Patients must be vaccinated 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.
- Patients who initiate treatment with FABHALTA® less than 2 weeks after vaccination must receive treatment with appropriate prophylactic antibiotics until 2 weeks after vaccination
- Vaccination reduces, but does not eliminate, the risk of serious infections. Monitor patients for early signs of serious infections and treat immediately if infection is suspected.
- FABHALTA® is only available through a controlled distribution program under which prescribers must enroll patients and confirm vaccination against encapsulated bacteria. Prescribers must also counsel patients about the risk of serious infection and provide them with the Patient Guide and Patient Card. Information about the FABHALTA® controlled distribution program is available at **www.fabhalta.ca**.

Other relevant warnings and precautions:

- **Monitoring and laboratory tests:** Patients with PNH receiving FABHALTA® should be monitored as per standard PNH management and regularly for signs and symptoms of hemolysis, including measuring lactate dehydrogenase levels. Patients should also be monitored for increased diastolic blood pressure and cholesterol. If treatment with FABHALTA® must be discontinued, patients must be closely monitored for signs and symptoms of hemolysis for at least 2 weeks after the last dose. If discontinuation of FABHALTA® is necessary, consider alternative therapy. If hemolysis occurs after discontinuation of FABHALTA®, restarting FABHALTA® treatment or initiating an alternative therapy should be considered.
- **Fertility:** There are no data on the effect of FABHALTA® on human fertility. In animal fertility studies, iptacopan did not impact fertility in male rats up to the highest dose tested, which corresponds to 4-fold the maximum recommended human dose (MRHD) based on AUC. Reversible effects on the male reproductive system were observed in repeated dose toxicity studies in dogs at doses 3-fold the MRHD based on AUC, with no apparent effects on sperm numbers, morphology or motility. In female rats, increased pre- and post-implantation losses and, consequently, decreased numbers of live embryos, were observed at 4-fold the MRHD based on AUC.
- **Pregnant women:** There are insufficient data on FABHALTA® use in pregnant women to inform a drug-associated risk of major birth defects, miscarriage, or other adverse maternal or fetal outcomes. There are risks to the mother and fetus associated with untreated PNH in pregnancy. The use of FABHALTA® in pregnant women or women planning to become pregnant may be considered following an assessment of the risks and benefits.
- **Disease-associated maternal and/or embryo/fetal risk:** PNH in pregnancy is associated with adverse maternal outcomes, including worsening cytopenia, thrombotic events, infections, bleeding, miscarriages and increased maternal mortality, as well as adverse fetal outcomes, including fetal death and premature delivery.
- **Breastfeeding:** It is not known if iptacopan is transferred into human milk after oral administration of FABHALTA®. There are no data on the effects of FABHALTA® on the breastfed child or on milk production. As potential serious adverse effects in breastfed infants cannot be ruled out, breastfeeding should be discontinued during treatment and for 5 days after the final dose.

For more information:

Consult the Product Monograph at **www.novartis.ca/fabhaltamonograph** for important information relating to contraindications, serious warnings and precautions, other warnings and precautions, adverse reactions, drug interactions, dosing, and conditions of clinical use 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**.

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References: 1. FABHALTA® Product Monograph. Novartis Pharmaceuticals Canada Inc. **2.** Data on file. Novartis Pharmaceuticals Canada Inc. February 2025.

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REVIEWED BY PAAB

FABHALTA® (iptacopan)