FABHALTA® (iptacopan) 200 mg capsules

STRONG EVIDENCE, CONFIDENT DECISIONS

FOR ADULTS WITH PNH

- *Results from the APPLY, APPOINT, and APPULSE trials*
- *2-year efficacy & safety data in adults from APPLY or APPOINT who entered the rollover extension program (REP)*

INDICATION

FABHALTA is indicated for the treatment of adults with paroxysmal nocturnal hemoglobinuria (PNH).

IMPORTANT SAFETY INFORMATION

WARNING: SERIOUS INFECTIONS CAUSED BY ENCAPSULATED BACTERIA

FABHALTA, a complement inhibitor, increases the risk of serious infections, especially those caused by encapsulated bacteria, such as *Streptococcus pneumoniae, Neisseria meningitidis,* and *Haemophilus influenzae* type b. Life-threatening and fatal infections with encapsulated bacteria have occurred in patients treated with complement inhibitors. These infections may become rapidly life-threatening or fatal if not recognized and treated early.

- **Complete or update vaccinations for encapsulated bacteria at least 2 weeks prior to the first dose of FABHALTA, unless the risks of delaying therapy with FABHALTA outweigh the risk of developing a serious infection. Comply with the most current Advisory Committee on Immunization Practices (ACIP) recommendations for vaccinations against encapsulated bacteria in patients receiving a complement inhibitor.**
- **Patients receiving FABHALTA are at increased risk for invasive disease caused by encapsulated bacteria, even if they develop antibodies following vaccination. Monitor patients for early signs and symptoms of serious infections and evaluate immediately if infection is suspected.**

Because of the risk of serious infections caused by encapsulated bacteria, FABHALTA is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS) called the FABHALTA REMS.

Please click here for additional Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

DISCOVER DATA FOR YOUR PATIENTS' TREATMENT NEEDS

Explore data to guide your treatment approach for patients with PNH who are ready to start or switch their treatment[1-3]

Patients with **Hb <10 g/dL who switched from their C5i regimen to FABHALTA**[1,*]

APPLY *TRIAL*

Complement inhibitor-naive patients who **started treatment with FABHALTA**[1]

APPOINT *TRIAL*

Patients with **Hb ≥10 g/dL who switched from their stable C5i regimen to FABHALTA**[2,*]

APPULSE *TRIAL*

Patients from APPLY and APPOINT who entered the rollover extension program (REP)[3,4]

2-YEAR LONG-TERM SAFETY AND EFFICACY

*Including either eculizumab or ravulizumab.[1]
C5i, complement 5 inhibitor; Hb, hemoglobin.

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Superior and substantial Hb increases were achieved with FABHALTA vs C5is through the 24-week randomized treatment period

PRIMA

The APPLY study was a head-to-head trial of FABHALTA vs C5is in C5i-experienced adults with PNH[1]

APPLY was a 24-week, randomized,* open-label, active comparator–controlled, phase 3 trial to assess the efficacy and safety of switching to FABHALTA 200 mg twice daily compared with continuing on intravenous C5i therapy (US-approved and non–US-approved eculizumab or ravulizumab). The study enrolled patients with PNH and residual anemia (mean Hb <10 g/dL) despite previous treatment with a stable regimen of C5i treatment for at least 6 months; 97 patients were randomized. Patients were randomized in an 8:5 ratio to either switch to FABHALTA 200 mg taken orally twice daily (n=62) or continue their C5i regimen (n=35: eculizumab, n=23; ravulizumab, n=12).

PRIMARY END POINTS[1,5]

- Proportion of patients achieving sustained Hb increase of ≥2 g/dL† from baseline without a need for RBC transfusions‡ after 24 weeks
- Proportion of patients achieving sustained Hb level of ≥12 g/dL† without a need for RBC transfusions after 24 weeks‡

ADDITIONAL END POINTS[1,5]

- RBC transfusion avoidance§
- Change from baseline† in:
  - Hb levels g/dL‖
  - FACIT-Fatigue scores
  - Absolute reticulocyte count (ARC) (10^9/L)
  - Lactate dehydrogenase (LDH) levels
- Occurrence of major adverse vascular events¶
- Occurrence of clinical breakthrough hemolysis¶,#
- Safety¶

In the APPLY study analysis[6]:

- All end points were based on central laboratory data
- 95% CIs were based on the Sato variance estimator

*Randomization was stratified based on prior C5i treatment and transfusion history within the last 6 months.[1]
†Assessed between Days 126 and 168.[1]
‡Assessed between Days 14 and 168. Requiring RBC transfusions refers to any patient receiving transfusions or meeting protocol-defined criteria.[5]
§Transfusion avoidance is defined as absence of administration of packed-RBC transfusions between Days 14 and 168.[1]
‖Excludes values within 30 days post-transfusion in the randomized period.[1]
¶Throughout the study.[5]
#As per protocol definition.[5]

*Assessed between Days 126 and 168.
†Assessed between Days 14 and 168. Requiring RBC transfusions refers to any patient receiving transfusions or meeting protocol-defined criteria.[5]
‡Adjusted difference in proportion.[1]

vary but generally are 12-16 g/dL for women and 13-18 g/dL for men.[7]
RBC, red blood cell.

Superior and substantial Hb increases were achieved with FABHALTA vs C5is through the 24-week randomized treatment period

PRIMARY END POINTS[1,5]

Patients with **Hb increase of ≥2 g/dL***

from baseline in the absence of RBC transfusions† after 24 weeks

RESPONSE RATES

82.3% FABHALTA (n=51/62; 95% CI, 70.5-90.8)

vs

0% C5is (eculizumab or ravulizumab) (n=0/35; 95% CI, 0-10.0)

(Difference‡: 81.5%; 95% CI, 71.6-91.4; *P*<0.0001)

Patients with **normalized§ Hb of ≥12 g/dL***

in the absence of RBC transfusions† after 24 weeks

RESPONSE RATES

67.7% FABHALTA (n=42/62; 95% CI, 54.7-79.1)

vs

0% C5is (eculizumab or ravulizumab) (n=0/35; 95% CI, 0-10.0)

(Difference‡: 66.6%; 95% CI, 54.6-78.6; *P*<0.0001)

Significantly more patients achieved Hb improvements in the absence of RBC transfusions with FABHALTA vs C5is

VIEW STUDY DESIGN DETAILS

VIEW SELECT BASELINE CHARACTERISTICS

*Assessed between Days 126 and 168.[1]
†Assessed between Days 14 and 168. Requiring RBC transfusions refers to any patient receiving transfusions or meeting protocol-defined criteria.[5]
‡Adjusted difference in proportion.[1]

§Normalization defined as meeting the primary end point of Hb ≥12 g/dL.[5] Normal Hb levels vary but generally are 12-16 g/dL for women and 13-18 g/dL for men.[7]
RBC, red blood cell.

IMPORTANT SAFETY INFORMATION (continued)
CONTRAINDICATIONS

- Patients with serious hypersensitivity to FABHALTA or any of the excipients.

Please click here for additional Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

Superior and substantial Hb increases were achieved with FABHALTA vs C5is through the 24-week randomized treatment period

PRIMA

Select baseline characteristics[1,5]

(Baseline disease characteristics were generally well balanced between treatment groups)

Group	Mean (SD) age (years)	Mean (SD) Hb (g/dL)	Mean (SD) LDH (U/L)	Mean (SD) ARC (x 10^9/L)	Mean (SD) disease duration (years)	Required ≥1 transfusion in last 6 months (% of patients)	Mean time on prior C5i (years)	Prior C5i treatment with eculizumab (% of patients)	Prior C5i treatment with ravulizumab (% of patients)
FABHALTA	51.7 (16.9)	8.9 (0.7)	269 (70) (<1.5 x ULN)	193 (84)	11.9 (9.8)	56.5%	3.8	64.5%	35.5%
Continued C5is	49.8 (16.7)	8.9 (0.9)	273 (85) (<1.5 x ULN)	191 (81)	13.5 (10.9)	60.0%	4.2	65.7%	34.3%

ULN, upper limit of normal.

*Assessed between Days 168 and 182.
†Assessed between Days 14 and 168. Requiring RBC transfusions refers to any patient receiving transfusions or meeting protocol-defined criteria.[5]
‡Adjusted difference in proportion.[1]

Normalization defined as meeting the primary endpoint of Hb ≥12 g/dL. Normal Hb levels vary but generally are 12-16 g/dL for women and 13-18 g/dL for men.[7]
RBC, red blood cell.

Explore the efficacy of FABHALTA vs C5is in patients with Hb <10 g/dL after the 24-week randomized treatment period

ADDITIONAL END POINTS[1]	FABHALTA (n=62)	C5is (eculizumab or ravulizumab) (n=35)
Adjusted mean*,† change from baseline in Hb levels	**+3.6 g/dL** (n=62; 95% CI, 3.3-3.9)	**-0.1 g/dL** (n=35; 95% CI, -0.5-0.3)
	Adjusted mean difference: 3.7 g/dL; 95% CI, 3.2-4.1; *P*<0.0001	
Transfusion avoidance rate‡,§	**95.2%** (n=59; 95% CI, 86.5-99.0)	**45.7%** (n=16; 95% CI, 28.8-63.4)
	Adjusted difference in proportion: 49.5%; 95% CI, 32.5-66.6; *P*<0.0001	
Adjusted mean change from baseline in ARC‖	**-116 x 10^9/L** (n=62; 95% CI, -127 to -105) Baseline mean: 193 x10^9/L	**0 x 10^9/L** (n=35; 95% CI, -13 to 14) Baseline mean: 191 x10^9/L
	Adjusted mean difference: -116 x 109/L; 95% CI, -132 to -100; *P*<0.0001	

*Mean assessed between Days 126 and 168.[1]
†Excludes values within 30 days post-transfusion.[1]
‡Assessed between Days 14 and 168. Transfusion avoidance is defined as absence of administration of packed-RBC transfusions between Days 14 and 168.[1]
§35/62 patients in the FABHALTA arm and 21/35 in the C5i arm had at least 1 RBC transfusion in the 6 months prior to trial enrollment.[5]
‖Mean change from baseline in ARC (10^9/L) assessed between Days 126 and 168.[1] Values include post-transfusion data.[6]
ARC, absolute reticulocyte count.

IMPORTANT SAFETY INFORMATION (continued)
CONTRAINDICATIONS (continued)

- For initiation in patients with unresolved serious infection caused by encapsulated bacteria, including *Streptococcus pneumoniae, Neisseria meningitidis,* or *Haemophilus influenzae* type b.

Please click here for additional Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

Explore the data of FABHALTA vs C5is in patients with Hb <10 g/dL after the 24-week randomized treatment period

ADDITIONAL END POINTS[1,5,6]	FABHALTA (n=62)	C5is (eculizumab or ravulizumab) (n=35)
The data from this additional analysis are descriptive in nature, presented for observation only. No formal conclusions or comparisons between the 2 treatment arms can be made.		
Adjusted geometric mean ratio to baseline in LDH*	**0.96** (n=62) Baseline mean: 269 U/L	**0.97** (n=35) Baseline mean: 273 U/L
	No statistically significant difference in LDH was seen between FABHALTA and C5is	
Patient-reported FACIT-Fatigue scores may be an underestimation or overestimation because patients were not blinded to treatment. The data from this additional analysis are descriptive in nature, presented for observation only. At baseline, ~50% of participants reported the least severe response categories ("not at all" and "a little bit") for the 10/13 questions in the FACIT-Fatigue scale. Due to the small sample size, open-label design, and the low level of fatigue reported at baseline, no formal conclusions or comparisons between the 2 treatment arms can be made.		
Adjusted mean change from baseline in FACIT-Fatigue scores†,‡	**+8.6** (n§=62; 95% CI, 6.70-10.53) Baseline: 34.7; Final: 43.2	**+0.3** (n§=31; 95% CI, -2.28-2.84) Baseline: 30.8; Final: 31.1
	Difference: 8.34; 95% CI, 5.26-11.41	

*Mean ratio to baseline in LDH assessed between Days 126 and 168.[6]
†The Functional Assessment of Chronic Illness Therapy-Fatigue Scale (FACIT-Fatigue) is a 13-item questionnaire that assesses self-reported fatigue and its impact upon daily activities and function. The level of fatigue is measured on a 5-point Likert scale (in the study, 4=not at all fatigued to 0=very much fatigued), with 0 being the worst possible score and 52 the best.[5,8]
‡Mean assessed between Days 126 and 168.[5]
§Baseline mean FACIT-Fatigue scores were reported for 62 patients in the FABHALTA arm and 33 patients in the C5is arm. At Day 168, the adjusted mean change in FACIT-Fatigue score was reported for 62 patients in the FABHALTA arm and 31 patients in the C5is arm.[5,6]
LDH, lactate dehydrogenase.

APPLY Efficacy
Primary End Points
Additional End Points
LDH and FACIT-Fatigue
APPLY Safety

IMPORTANT SAFETY INFORMATION (continued)
WARNINGS AND PRECAUTIONS
Serious Infections Caused by Encapsulated Bacteria

- FABHALTA, a complement inhibitor, increases a patient's susceptibility to serious, life-threatening, or fatal infections caused by encapsulated bacteria, including *Streptococcus pneumoniae, Neisseria meningitidis* (caused by any serogroup, including nongroupable strains), and *Haemophilus influenzae* type b. Life-threatening and fatal infections with encapsulated bacteria have occurred in both vaccinated and unvaccinated patients treated with complement inhibitors. The initiation of FABHALTA is contraindicated in patients with unresolved serious infections caused by encapsulated bacteria.

Please click here for additional Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

Safety profile of FABHALTA

ADVERSE REACTIONS WITH FABHALTA (N=62) vs C5is (N=35)[1]

The adverse reactions reported in >5% of adults with PNH treated with FABHALTA vs C5is in APPLY (24-week randomized treatment period) were:

- Headache (19% vs 3%)
- Nasopharyngitis (16% vs 17%)
- Diarrhea (15% vs 6%)
- Abdominal pain (15% vs 3%)
- Bacterial infection (11% vs 11%)
- Nausea (10% vs 3%)
- Viral infection (10% vs 31%)
- Arthralgia (8% vs 3%)
- Thrombocytopenia (6% vs 0%)
- Dizziness (6% vs 0%)
- Systemic hypertension (6% vs 0%)
- Lipid disorder (6% vs 0%)

- Serious adverse reactions were reported in 2 (3%) patients with PNH who received FABHALTA. They included pyelonephritis, urinary tract infection, and COVID-19
- Rash was reported in 2 patients (3%)
- Of the 37 FABHALTA-treated patients who had normal platelet counts at baseline, 43% experienced any grade thrombocytopenia during the randomized treatment period
- Three FABHALTA-treated patients experienced decreased platelets that worsened to grade ≥3 from baseline (1 patient with normal platelets that worsened to grade 4; 1 patient with baseline grade 1 that worsened to grade 4; and 1 patient with baseline grade 3 that worsened to grade 4)

No patient discontinued FABHALTA or C5is due to an adverse reaction during the 24-week randomized treatment period.
One patient discontinued FABHALTA due to pregnancy[5]

Because of the risk of serious infections caused by encapsulated bacteria, FABHALTA is only available through a REMS program that requires vaccinations.

Click here for more details.

BTH and MAVEs in the 24-week randomized treatment period

ADDITIONAL END POINTS[5]	FABHALTA (n=62)	C5is (eculizumab or ravulizumab) (n=35)
The data from this additional analysis are descriptive in nature, presented for observation only. No formal conclusions or comparisons between the 2 treatment arms can be made. The planned power for the hypothesis testing was low.		
Annualized rate of clinical BTH*,†	0.07 (2 patients)	0.67 (6 patients)
	(95% CI, 0.02-0.61)	
The data from this additional analysis are descriptive in nature, presented for observation only. No formal conclusions or comparisons between the 2 treatment arms can be made.		
Annualized rate of MAVEs†,‡	0.03 (1 patient)	0
	(95% CI, -0.03-0.10) No significant statistical difference was seen between arms	

MAVEs:

- During the 24-week randomized period, 1 patient experienced transient ischemic attack in the FABHALTA arm, which was deemed unrelated to treatment by the investigator vs 0/35 with C5is (eculizumab or ravulizumab)[5]

*Clinical BTH was defined as meeting clinical criteria (either decrease of Hb level ≥2 g/dL compared with the last assessment or within 15 days; or signs or symptoms of gross hemoglobinuria, painful crisis, dysphagia, or any other significant clinical PNH-related signs and symptoms) and laboratory criteria (LDH >1.5 x ULN and increased as compared with the last 2 assessments).[5]
†Assessed between Days 1 and 168.[5]
‡There was no significant statistical difference in the annualized rates of MAVEs between FABHALTA and C5is. The definition of MAVEs included: acute peripheral vascular occlusion, amputation, cerebrovascular accident, cerebral venous occlusion, dermal thrombosis, gangrene, hepatic/portal vein thrombosis, mesenteric/visceral arterial or vein thrombosis or infarction, myocardial infarction, pulmonary embolus, renal arterial or vein thrombosis, thrombophlebitis/deep vein thrombosis, transient ischemic attack, and unstable angina.[5]
BTH, breakthrough hemolysis; MAVEs, major adverse vascular events; ULN, upper limit of normal.

APPLY Efficacy
APPLY Safety
Adverse Reactions
BTH and MAVEs

With FABHALTA oral monotherapy, substantial Hb improvements without the need for RBC transfusions are within reach

The APPOINT study was designed to evaluate the efficacy and safety of an oral monotherapy for complement inhibitor–naive patients[1,9]

APPOINT was a 24-week, phase 3, single-arm, open-label, uncontrolled study of FABHALTA 200 mg twice daily in adults (N=40) with PNH who were complement inhibitor–naive and had an RBC clone size ≥10%, a mean Hb <10 g/dL,* and an LDH level >1.5 x ULN.†

PRIMARY END POINT

- Proportion of patients achieving sustained Hb increase of ≥2 g/dL‡ from baseline without a need for RBC transfusions§ after 24 weeks

In the APPOINT study analysis[1,6,9]:

- All additional end points were exploratory
- Unless otherwise noted, all end points were based on central laboratory data
- 95% CIs were based on the Clopper-Pearson method

ADDITIONAL END POINTS

- Proportion of patients with sustained Hb level of ≥12 g/dL‡ without a need for RBC transfusions§
- RBC transfusion avoidance‖
- Change from baseline‡ in:
  - Hb levels g/dL¶
  - FACIT-Fatigue scores
  - Absolute reticulocyte count (ARC) (10^9/L)
  - Lactate dehydrogenase (LDH) levels
- Occurrence of major adverse vascular events#
- Occurrence of clinical breakthrough hemolysis#,**
- Safety#

*Confirmed by 2 measurements 2 to 8 weeks apart for patients not receiving an RBC transfusion during screening, or by 1 measurement during the first screening visit for patients receiving an RBC transfusion.[9]
†Confirmed by at least 2 measurements 2 to 8 weeks apart during the screening period.[9] ‡Assessed between Days 126 and 168.[1]
§Assessed between Days 14 and 168. Requiring RBC transfusions refers to any patient receiving transfusions or meeting protocol-defined criteria.[9]
‖Transfusion avoidance is defined as absence of administration of packed-RBC transfusions between Days 14 and end of study period.[6,9]
¶Excludes values within 30 days post-transfusion in the randomized period.[9]
#Throughout the study.[9]
**As per protocol definition.[9]

- Complete or update vaccination against encapsulated bacteria at least 2 weeks prior to the start of FABHALTA, according to the current ACIP recommendations for patients receiving a complement inhibitor. Revaccinate patients in accordance with ACIP recommendations considering the duration of therapy with FABHALTA. Note that ACIP recommends an administration schedule in patients receiving complement inhibitors that differs from the administration schedule in the vaccine prescribing information. If urgent FABHALTA therapy is indicated in a patient who is not up to date with vaccines against encapsulated bacteria according to ACIP recommendations, provide the patient with antibacterial drug prophylaxis and administer these vaccines as soon as possible. The benefits and risks of treatment with FABHALTA, as well as the benefits and risks of antibacterial drug prophylaxis in unvaccinated or vaccinated patients, must be considered against the known risks for serious infections caused by encapsulated bacteria.

Please click here for additional Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

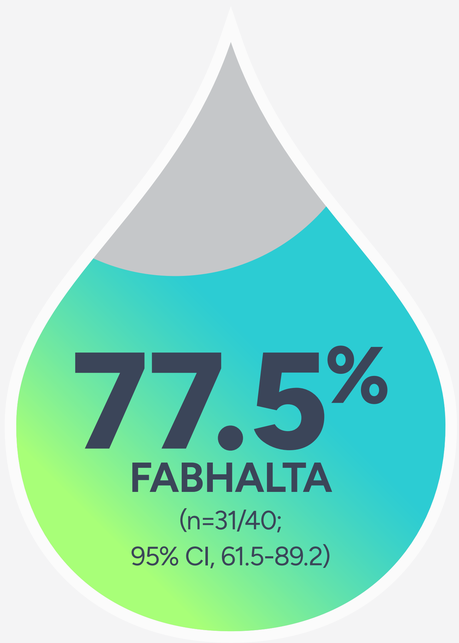
With FABHALTA oral monotherapy, substantial Hb improvements without the need for RBC transfusions are within reach

PRIMARY END POINT

Patients with sustained Hb increase of ≥2 g/dL* from baseline in the absence of RBC transfusions† after the 24-week core treatment period[1]

RESPONSE RATE

Based on central laboratory Hb values.



SENSITIVITY ANALYSIS

87.5%
(n=35/40; 95% CI, 73.2-95.8)

Response rate based on the inclusion of local laboratory values when central laboratory values were not available.

*Assessed between Days 126 and 168.[1]
†Assessed between Days 14 and 168. Requiring RBC transfusions refers to any patient receiving transfusions or meeting protocol-defined criteria.[9]

VIEW STUDY DESIGN DETAILS

VIEW SELECT BASELINE CHARACTERISTICS

APPOINT Efficacy
- Primary End Points
- Additional End Points
- LDH and FACIT-Fatigue
- APPOINT Safety

IMPORTANT SAFETY INFORMATION (continued)
WARNINGS AND PRECAUTIONS (continued)
Serious Infections Caused by Encapsulated Bacteria (continued)

- Complete or update vaccination against encapsulated bacteria at least 2 weeks prior to the start of FABHALTA, according to the current ACIP recommendations for patients receiving a complement inhibitor. Revaccinate patients in accordance with ACIP recommendations considering the duration of therapy with FABHALTA. Note that ACIP recommends an administration schedule in patients receiving complement inhibitors that differs from the administration schedule in the vaccine prescribing information. If urgent FABHALTA therapy is indicated in a patient who is not up to date with vaccines against encapsulated bacteria according to ACIP recommendations, provide the patient with antibacterial drug prophylaxis and administer these vaccines as soon as possible. The benefits and risks of treatment with FABHALTA, as well as the benefits and risks of antibacterial drug prophylaxis in unvaccinated or vaccinated patients, must be considered against the known risks for serious infections caused by encapsulated bacteria.

Please click here for additional Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

With FABHALTA oral monotherapy, substantial Hb improvements without the need for RBC transfusions are within reach

PRIMA

Pati
core

Select baseline characteristics[1,9]

Group	Mean (SD) age (years)	Mean (SD) Hb (g/dL)	Mean (SD) LDH (U/L)	Mean (SD) ARC (x 10^9/L)	Mean (SD) disease duration (years)	Required ≥1 transfusion in last 6 months (% of patients)
FABHALTA	42.1 (15.9)	8.2 (1.1)	1699 (683) (6.8 x ULN)	154 (64)	4.7 (5.5)	~70

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WARN
Serious Infections Caused by Encapsulated Bacteria (continued)

- Complete or update vaccination against encapsulated bacteria at least 2 weeks prior to the start of FABHALTA, according to the current ACIP recommendations for patients receiving a complement inhibitor. Revaccinate patients in accordance with ACIP recommendations considering the duration of therapy with FABHALTA. Note that ACIP recommends an administration schedule in patients receiving complement inhibitors that differs from the administration schedule in the vaccine prescribing information. If urgent FABHALTA therapy is indicated in a patient who is not up to date with vaccines against encapsulated bacteria according to ACIP recommendations, provide the patient with antibacterial drug prophylaxis and administer these vaccines as soon as possible. The benefits and risks of treatment with FABHALTA, as well as the benefits and risks of antibacterial drug prophylaxis in unvaccinated or vaccinated patients, must be considered against the known risks for serious infections caused by encapsulated bacteria.

Please click here for additional Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

Explore the data of FABHALTA in complement inhibitor–naive patients after the 24-week treatment period

The data from these additional analyses are exploratory; therefore, not subject to family-wise Type 1 error control, and presented for observation only. No formal conclusions can be made.

ADDITIONAL END POINTS[6]	FABHALTA (N=40)
Adjusted mean change in Hb from baseline*	**+4.29 g/dL** (n=40; 95% CI, 3.86-4.72)
Transfusion avoidance rate†	**100%** (n=40; 95% CI, 91.2-100.0)
Adjusted mean change from baseline in ARC‡	**-80.75 x 10^9/L** (n=40; 95% CI, -87.78 to -73.71) Baseline mean: 154.3 x 10^9/L

*Assessed between Days 126 and 168; excludes values within 30 days post-transfusion.[6]
†Transfusion avoidance in APPOINT was defined as absence of administration of packed-RBC transfusions between Days 14 and 168.[6,9]
‡Mean of visits between Days 126 and 168.[9]

APPOINT Efficacy
Primary End Points
Additional End Points
LDH and FACIT-Fatigue
APPOINT Safety

IMPORTANT SAFETY INFORMATION (continued)
WARNINGS AND PRECAUTIONS (continued)
Serious Infections Caused by Encapsulated Bacteria (continued)

- Vaccination does not eliminate the risk of serious encapsulated bacterial infections, despite development of antibodies following vaccination. Closely monitor patients for early signs and symptoms of serious infection and evaluate patients immediately if an infection is suspected. Inform patients of these signs and symptoms and instruct patients to seek immediate medical care if they occur. Promptly treat known infections. Serious infection may become rapidly life-threatening or fatal if not recognized and treated early. Consider interruption of FABHALTA in patients who are undergoing treatment for serious infections, depending on the risks of interrupting treatment in the disease being treated.

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Explore the data of FABHALTA in complement inhibitor–naive patients after the 24-week treatment period

ADDITIONAL END POINTS[6,9]

FABHALTA
(N=40)

The data from this additional analysis are exploratory; therefore, not subject to family-wise Type 1 error control, and presented for observation only. No formal conclusions can be made.

Adjusted mean percent change from baseline in LDH*

-83.5%
(n=40; 95% CI, -84.9 to -82.0)
Baseline mean: 1698.8 U/L

Patient-reported FACIT-Fatigue scores may be an underestimation or overestimation because patients were not blinded to treatment. The data from this additional analysis are exploratory; therefore, not subject to family-wise Type 1 error control, and presented for observation only. Due to the exploratory nature, small sample size, single-arm and open-label design, no formal conclusions can be made.

Mean change from baseline in FACIT-Fatigue scores†,‡

+10.8
(n§=40; 95% CI, 8.63-12.95)
Baseline: 32.8; Final: 43.9

Mean LDH values decreased by Day 7, reached <1.5 x ULN by Day 14, and stayed <1.5 x ULN through Day 168.[9]

*Mean of visits between Days 126 and 168.[9]
†Assessed between Days 126 and 168.[9]
‡The Functional Assessment of Chronic Illness Therapy-Fatigue Scale (FACIT-Fatigue) is a 13-item questionnaire that assesses self-reported fatigue and its impact upon daily activities and function. The level of fatigue is measured on a 5-point Likert scale (in the study, 4=not at all fatigued to 0=very much fatigued), with 0 being the worst possible score and 52 the best.[8,9]
§Baseline mean FACIT-Fatigue scores and adjusted mean change in FACIT-Fatigue scores at Day 168 were reported for 40 patients.[9]

APPOINT Efficacy
Primary End Points
Additional End Points
LDH and FACIT-Fatigue
APPOINT Safety

IMPORTANT SAFETY INFORMATION (continued)
WARNINGS AND PRECAUTIONS (continued).

FABHALTA REMS

- FABHALTA is available only through a restricted program under a REMS called FABHALTA REMS, because of the risk of serious infections caused by encapsulated bacteria.

Please click here for additional Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

Safety profile of FABHALTA

ADVERSE REACTIONS WITH FABHALTA[1]

The adverse reactions reported in >5% of adults with PNH treated with FABHALTA in APPOINT (24-week core treatment period; N=40) were:

- Headache (28%)
- Viral infection (18%)
- Nasopharyngitis (15%)
- Rash (10%)
- Abdominal pain (8%)
- Diarrhea (8%)
- Lipid disorder (8%)

Serious adverse reactions were reported in 2 (5%) patients with PNH who received FABHALTA. They included COVID-19 and bacterial pneumonia. Bacterial infection and nausea were reported in 2 patients each (5%). Dizziness and urticaria were reported in 1 patient each (3%).

ADDITIONAL END POINTS

The data from this additional analysis are exploratory; therefore, not subject to family-wise Type 1 error control, and presented for observation only. No formal conclusions can be made.

During the 24-week core treatment period, no clinical breakthrough hemolysis* or major adverse vascular events (MAVEs)† were observed in patients on FABHALTA[9]

No patient discontinued FABHALTA due to an adverse reaction in the 24-week core treatment period[9]

Because of the risk of serious infections caused by encapsulated bacteria, FABHALTA is only available through a REMS program that requires vaccinations.

Click here for more details.

APPOINT Efficacy

APPOINT Safety

*Clinical BTH was defined as meeting clinical criteria (either decrease of Hb level ≥2 g/dL compared with the latest assessment or within 15 days; or signs or symptoms of gross hemoglobinuria, painful crisis, dysphagia, or any other significant clinical PNH-related signs and symptoms) and laboratory criteria (LDH >1.5 x ULN and increased as compared with the last 2 assessments).[9]

†The definition of MAVEs included: acute peripheral vascular occlusion, amputation, cerebrovascular accident, cerebral venous occlusion, dermal thrombosis, gangrene, hepatic/portal vein thrombosis, mesenteric/visceral arterial or vein thrombosis or infarction, myocardial infarction, pulmonary embolus, renal arterial or vein thrombosis, thrombophlebitis/deep vein thrombosis, transient ischemic attack, and unstable angina.[9]

IMPORTANT SAFETY INFORMATION (continued)

WARNINGS AND PRECAUTIONS (continued).

FABHALTA REMS (continued)

- Under the FABHALTA REMS, prescribers must enroll in the program. Prescribers must counsel patients about the risks, signs, and symptoms of serious infections caused by encapsulated bacteria, provide patients with the REMS educational materials, ensure patients are vaccinated against encapsulated bacteria, prescribe antibacterial drug prophylaxis if patients' vaccine status is not up to date and treatment must be started urgently, and provide instructions to always carry the Patient Safety Card during treatment and for 2 weeks following last dose of FABHALTA.

Please click here for additional Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

The APPULSE study was designed to evaluate a switch to FABHALTA in patients with Hb ≥10 g/dL who are ready to switch from their stable C5i regimen

APPULSE was a 24-week, single-arm, open-label, multicenter, phase 3b trial to evaluate the efficacy and safety of switching to iptacopan 200 mg twice daily in adults with PNH who had achieved Hb levels ≥10 g/dL* in response to a stable regimen of anti-C5 antibody treatment (eculizumab or ravulizumab) for at least 6 months and had remained transfusion-free during that period. Patients (N=52) were enrolled following an 8-week screening to confirm eligibility, including transfusion history and vaccination status. All received oral iptacopan 200 mg twice daily for 24 weeks.[2]

PRIMARY END POINT[2]

- Change from baseline in Hb levels after switching from a C5i†

All end points in the APPULSE study analysis were based on central laboratory data

ADDITIONAL END POINTS[2]

- Proportion of patients with sustained Hb level of ≥12 g/dL‡ without a need for RBC transfusions§
- RBC transfusion avoidance§
- Change from baseline in:
  - FACIT-Fatigue scores‖
  - Absolute reticulocyte count (ARC) (10^9/L)¶
  - Lactate dehydrogenase (LDH) levels#
- Occurrence of major adverse vascular events**
- Occurrence of clinical breakthrough hemolysis**
- Safety††

VIEW SELECT BASELINE CHARACTERISTICS

*Mean Hb ≥10 g/dL over a period of 6 months before screening visit and confirmed by 2 different samples during the screening period.[2]
†Assessed as mean of visits between Days 126 and 168 compared with baseline, defined as mean of Hb collected at screening (2 samples) and Day 1.[2]
‡Assessed between Days 126 and 168 in the absence of RBC transfusions between Days 1 and 168, on 3 of 4 measurements taken at the visits occurring in the last 6 weeks. 95% CIs were based on the Clopper-Pearson method.[2,3]
§Transfusion avoidance is defined as absence of administration of packed-RBC transfusions between Days 1 and 168. 95% CIs were based on the Clopper-Pearson method.[2,3]
‖Assessed at Days 84 and 168.[2]
¶Change from baseline as mean of visits between Days 126 and 168.[2]
#Percentage change from baseline as mean of visits between Days 126 and 168.[2]
**Rate of occurrence through Day 168. Summary measure was occurrences per year.[2]
††Throughout the study.[2]

Study Design
APPULSE Efficacy
APPULSE Safety

IMPORTANT SAFETY INFORMATION (continued)
WARNINGS AND PRECAUTIONS (continued).
FABHALTA REMS (continued)

- Further information is available by telephone: 1-833-993-2242 or online at www.FABHALTA-REMS.com

Please click here for additional Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

Patients with baseline Hb ≥10 g/dL experienced an Hb increase after switching from a stable C5i regimen to FABHALTA

Select baseline characteristics[2]

Group	Mean (SD) age (years)	Mean (SD) Hb (g/dL)	Mean (SD) LDH (U/L)	Mean (SD) ARC (x 10^9/L)	Mean (SD) disease duration (years)	Mean time on prior C5i (years)	Prior C5i treatment with eculizumab (% of patients)	Prior C5i treatment with ravulizumab (% of patients)
FABHALTA	46.0 (13.7)	11.87 (1.3)	226.8 (69)	154.84 (76.2)	10.80 (7.5)	3.5	11.5%	88.5%

*Mean of
post-tra
†The 'n' values reflect patients with non-missing values.

IMPORTANT SAFETY INFORMATION (continued)
WARNINGS AND PRECAUTIONS (continued).
FABHALTA REMS (continued)

- Further information is available by telephone: 1-833-993-2242 or online at www.FABHALTA-REMS.com

Please click here for additional Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

Patients with baseline Hb ≥10 g/dL experienced an Hb increase after switching from a stable C5i regimen to FABHALTA

PRIMARY END POINT

The data are for observation only. No formal conclusions can be made.

Adjusted mean change from baseline in Hb[10,*]

+2.07 g/dL

(n†=51; 95% CI, 1.80-2.33)

Mean (SD) baseline Hb levels (g/dL): 11.87 (1.32)

*Mean of visits between Days 126 and 168 compared with baseline, defined as mean of Hb collected at screening (2 samples) and Day 1. Excludes values within 30 days post-transfusion.[10]
†The 'n' values reflect patients with non-missing values.[10]

IMPORTANT SAFETY INFORMATION (continued)
WARNINGS AND PRECAUTIONS (continued).
FABHALTA REMS (continued)

- Further information is available by telephone: 1-833-993-2242 or online at www.FABHALTA-REMS.com

Please click here for additional Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

Explore the data of FABHALTA in patients with Hb ≥10 g/dL after the 24-week treatment period

The data are for observation only. No formal conclusions can be made.

ADDITIONAL END POINTS[2,10]	FABHALTA (N=52)
Hb ≥12 g/dL*	**92.3%** (n=48†; 95% CI, 81.5-97.9)
Transfusion avoidance rate‡,§	**100%** (n=52; 95% CI, 93.2-100.0)
Adjusted mean change from baseline‖ in ARC	**-90.77 x 10^9/L** (n¶=51; 95% CI, -95.75 to -85.79) Baseline mean: 154.84 x 10^9/L

*Assessed between visits Days 126 and 168 in the absence of RBC transfusions between Days 1 and 168, on 3 of 4 measurements taken at the visits occurring in the last 6 weeks.[10]
†Percentages are based on the number of patients with Hb results at that time point.[10]
‡From 6 months to screening, no transfusions received. In the 12 to 6 months prior to the trial screening period, 3.8% (n=2/52) of patients received a transfusion.[2]
§Transfusion avoidance response rate in APPULSE was defined as absence of administration of packed-RBC transfusions between Days 1 and 168.[2]
‖Change from baseline as mean of visits between Days 126 and 168.[10]
¶The 'n' values reflect patients with non-missing values.[10]

IMPORTANT SAFETY INFORMATION (continued)
WARNINGS AND PRECAUTIONS (continued)

Monitoring of PNH Manifestations After FABHALTA Discontinuation

- After discontinuing FABHALTA, closely monitor patients for at least 2 weeks after the last dose for signs and symptoms of hemolysis. These signs include elevated lactate dehydrogenase (LDH) levels along with sudden decrease in hemoglobin or PNH clone size, fatigue, hemoglobinuria, abdominal pain, dyspnea, major adverse vascular events (such as thrombosis, stroke, and myocardial infarction), dysphagia, or erectile dysfunction. If discontinuation of FABHALTA is necessary, consider alternative therapy.

Please click here for additional Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

Explore the data of FABHALTA in patients with Hb ≥10 g/dL after the 24-week treatment period

ADDITIONAL END POINTS[2,10]	FABHALTA (N=52)
The data are for observation only. No formal conclusions can be made.	
Adjusted mean percent change from baseline* in LDH	**-0.81%** ($n^{\dagger}$=51; 95% CI, -6.27 to 4.96) Baseline mean: 226.6 U/L
Patient-reported FACIT-Fatigue scores may be an underestimation or overestimation because patients were not blinded to treatment. The data from this additional analysis are descriptive in nature, presented for observation only. At baseline, on average, 70% of participants reported the least severe response categories ("not at all" and "a little bit") for the 11/13 questions in the FACIT-Fatigue scale. Due to the small sample size, open-label design, and the low level of fatigue reported at baseline, no formal conclusions can be made.	
Mean change from baseline in FACIT-Fatigue scores‡ at Day 84	**+4.88** ($n^{\dagger,\S}$=50; 95% CI, 3.23-6.53) Baseline: 38.9; Final: 43.9
Mean change from baseline in FACIT-Fatigue scores‡ at Day 168	**+4.29** ($n^{\dagger,\S}$=47; 95% CI, 1.74-6.85) Baseline: 38.9; Final: 43.1

*Percentage change from baseline as mean of visits between Days 126 and 168.[10]
†The 'n' values reflect patients with non-missing values.[10]
‡The Functional Assessment of Chronic Illness Therapy-Fatigue Scale (FACIT-Fatigue) is a 13-item questionnaire that assesses self-reported fatigue and its impact upon daily activities and function. The level of fatigue is measured on a 5-point Likert scale (in the study, 4=not at all fatigued to 0=very much fatigued), with 0 being the worst possible score and 52 the best.[2,8]
§Baseline mean FACIT-Fatigue scores and adjusted mean change in FACIT-Fatigue scores at Day 84 and 168 were reported for 50 and 47 patients, respectively.[2]

IMPORTANT SAFETY INFORMATION (continued)
WARNINGS AND PRECAUTIONS (continued)

Monitoring of PNH Manifestations After FABHALTA Discontinuation (continued)

- If hemolysis occurs after discontinuation of FABHALTA, consider restarting treatment with FABHALTA, if appropriate, or initiating another treatment for PNH.

Please click here for additional Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

Safety profile of FABHALTA

ADVERSE REACTIONS WITH FABHALTA[11]

The adverse reactions reported in >5% of adults with PNH treated with FABHALTA in APPULSE (24-week treatment period) were:

- Headache (17.3%)
- Nasopharyngitis (17.3%)
- Viral infection (13.5%)
- Diarrhea (11.5%)
- Nausea (11.5%)
- Bacterial infection (9.6%)
- Lipid disorder (7.7%)
- Thrombocytopenia (5.8%)

A serious adverse reaction (bacterial pneumonia) was reported in 1 patient (2%).

ADDITIONAL END POINTS

The data are for observation only. No formal conclusions can be made.

During the 24-week treatment period, no clinical breakthrough hemolysis* or major adverse vascular events (MAVEs)† were observed in patients on FABHALTA[2]

One patient discontinued FABHALTA due to an adverse reaction (palpitations)[11]

*Rate of occurrence through Day 168. Summary measure was occurrences per year.[2]
†The definition of MAVEs included: acute peripheral vascular occlusion, amputation, cerebrovascular accident, cerebral venous occlusion, dermal thrombosis, gangrene, hepatic/portal vein thrombosis, mesenteric/visceral arterial or vein thrombosis or infarction, myocardial infarction, pulmonary embolus, renal arterial or vein thrombosis, thrombophlebitis/deep vein thrombosis, transient ischemic attack, and unstable angina.[2]

Study Design
APPULSE Efficacy
APPULSE Safety

IMPORTANT SAFETY INFORMATION (continued)

WARNINGS AND PRECAUTIONS (continued)

Hyperlipidemia

- FABHALTA may increase total cholesterol, LDL cholesterol, and serum triglycerides.
- Of 88 FABHALTA-treated patients who had normal total cholesterol at baseline, 31 developed grade 1 hypercholesterolemia during the randomization or core treatment period and 1 patient worsened from baseline grade 1 to grade 2.
- Of 96 FABHALTA-treated patients with LDL cholesterol ≤ 130 mg/dL at baseline during the randomization or core treatment period, 14 patients developed LDL cholesterol > 130-160 mg/dL, 6 patients developed LDL cholesterol > 160-190 mg/dL and 4 patients developed LDL cholesterol > 190 mg/dL.
- Of 89 FABHALTA-treated patients with normal triglycerides during the randomization or core treatment period, 22 patients developed grade 1 elevated triglycerides. Three patients experienced an increase in triglycerides from grade 1 to grade 2.

Please click here for additional Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

The long-term efficacy and safety of FABHALTA were evaluated through 2 years in patients from APPLY and APPOINT who entered the REP

The REP is an ongoing open-label, single-arm, multicenter, phase 3b rollover extension study to assess the long-term safety, tolerability, and efficacy of iptacopan 200 mg taken orally twice daily in adults with PNH who completed the treatment extension periods of a Novartis-sponsored phase 2 study or any phase 3 clinical study (without tapering), including the APPLY and APPOINT studies. The data presented is the 2-year long term APPLY and APPOINT safety and efficacy from REP.[12,13]

The following assessments were included[3,4]**:**

- Hematological responses, defined as the percentage of patients with an increase of ≥2 g/dL in Hb level from baseline and percentage of patients achieving a Hb level of ≥12 g/dL, without RBC transfusion in the prior 12 months or irrespective of RBC transfusions received during the treatment period
- Proportion of patients achieving transfusion avoidance
- Changes from baseline in Hb levels, FACIT-Fatigue scores, and LDH levels
- Occurrences of clinical breakthrough hemolysis (BTH) and major adverse vascular events (MAVEs)
- Safety

The first day of iptacopan treatment in the parent studies is considered as the baseline.[12]

Study Design

Hb Response Rates

Additional End Points

REP Safety

Explore the long-term data of FABHALTA through 2 years

The data are for observation only. No formal conclusions can be made.

END POINTS[3,*]	APPLY patients in REP: 2 years N=96	APPOINT patients in REP: 2 years N=40	Both APPLY and APPOINT patients in REP: 2 years N=136
Without RBC transfusions within the prior 12 months			
Hb ≥12 g/dL	**69.0%** (n=58/84[†])	**75.0%** (n=30/40[†])	**71.0%** (n=88/124[†])
Hb increase ≥2 g/dL	**79.8%** (n=67/84[†])	**85.0%** (n=34/40[†])	**81.5%** (n=101/124[†])
Irrespective of RBC transfusions			
Hb ≥12 g/dL	**69.0%** (n=58/84[†])	**77.5%** (n=31/40[†])	**71.8%** (n=89/124[†])
Hb increase ≥2 g/dL	**82.1%** (n=69/84[†])	**87.5%** (n=35/40[†])	**83.9%** (n=104/124[†])

*Data from the REP are summarized descriptively.[3]
[†]Percentages at 2 years are based on the number of patients with Hb results at that time point.[3]

SEE ADDITIONAL DATA

IMPORTANT SAFETY INFORMATION (continued)
WARNINGS AND PRECAUTIONS (continued)
Hyperlipidemia (continued)

- Of the 102 FABHALTA-treated patients in APPLY-PNH and APPOINT-PNH, 2 patients required cholesterol-lowering medications.

Please click here for additional Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

Explore the long-term data of FABHALTA through 2 years

The data are for observation only. No formal conclusions can be made.

The data are for observation only. No formal conclusions can be made.

END POINTS[3,*]	APPLY patients in REP: 2 years N=96	APPOINT patients in REP: 2 years N=40	Both APPLY and APPOINT patients in REP: 2 years N=136
	Without RBC transfusions within the prior 6 months		
Hb ≥12 g/dL	69.0% (n=58/84†)	77.5% (n=31/40†)	71.8% (n=89/124†)
Hb increase ≥2 g/dL	81.0% (n=68/84†)	87.5% (n=35/40†)	83.1% (n=103/124†)

*Data from the REP are summarized descriptively.[3]
†Percentages at 2 years are based on the number of patients with Hb results at that time point.[3]

SEE ADDITIONAL DATA

IMPORTANT SAFETY INFORMATION (continued)
WARNINGS AND PRECAUTIONS (continued)
Hyperlipidemia (continued)

- Of the 102 FABHALTA-treated patients in APPLY-PNH and APPOINT-PNH, 2 patients required cholesterol-lowering medications.

Please click here for additional Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

Explore the long-term data of FABHALTA through 2 years

The data are for observation only. No formal conclusions can be made.

END POINTS[3,*]	APPLY patients in REP: 2 years N=96	APPOINT patients in REP: 2 years N=40	Both APPLY and APPOINT patients in REP: 2 years N=136
Mean change from baseline in Hb levels g/dL*,†	**+3.20** (SD; 1.855)	**+5.37** (SD; 2.919)	**+3.90** (SD; 2.461)
Transfusion avoidance rate‡	**88.5%** (n=85)	**95%** (n=38)	**90.4%** (n=123)

*Data from the REP are summarized descriptively.[3]
†Excludes values within 30 days post-transfusion.[3]
‡The number of transfusions does not include the period Days 1-14 after starting LNP.[3]

Patient-reported FACIT-Fatigue scores may be an underestimation or overestimation because patients were not blinded to treatment.
In APPLY: The data from this additional analysis are descriptive in nature, presented for observation only. At baseline, ~50% of participants reported the least severe response categories ("not at all" and "a little bit") for the 10/13 questions in the FACIT-Fatigue scale. Due to the small sample size, open-label design, and the low level of fatigue reported at baseline, no formal conclusions or comparisons between the 2 treatment arms can be made.
In APPOINT: The data from this additional analysis are exploratory; therefore, not subject to family-wise Type 1 error control, and presented for observation only. Due to the exploratory nature, small sample size, single-arm and open-label design, no formal conclusions can be made.

Mean change from baseline in FACIT-Fatigue scores	**+8.4** (SD; 11.31)	**+13.8** (SD; 10.05)	**+10.0** (SD; 11.18)

Mean LDH* remained <1.5 ULN through 2 years on FABHALTA[3]

- **In patients from APPLY: 33.00 U/L change from baseline** (SD; 230.261); baseline: 273.84 (SD; 94.25)
- **In patients from APPOINT: -1399.18 U/L change from baseline** (SD; 652.417); baseline: 1698.78 (SD; 683.33)
- **In patients from APPLY and APPOINT: -428.99 U/L change from baseline** (SD; 789.05);† baseline: 692.94 (SD; 752.20)

Study Design
Hb Response Rates
Additional End Points
REP Safety

IMPORTANT SAFETY INFORMATION (continued)
WARNINGS AND PRECAUTIONS (continued)
Hyperlipidemia (continued)

- Monitor serum lipid parameters periodically during treatment with FABHALTA and initiate cholesterol-lowering medications, if indicated.

Please click here for additional Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

Safety profile of FABHALTA through 2 years

Most frequent AEs reported in ≥10% of patients[4]

Preferred terms	Adverse events from APPLY patients in REP: 2 years N=96		Adverse events from APPOINT patients in REP: 2 years N=40		Adverse events from both APPLY and APPOINT patients in REP: 2 years N=136	
	n (%)*	Events (occurrence rate†). Exposure= 183.6 PY.‡	n (%)*	Events (occurrence rate†). Exposure= 80 PY.‡	n (%)*	Events (occurrence rate†). Exposure= 263.6 PY.‡
Number of patients with >1 adverse event	93 (96.9%)	894 (486.9)	40 (100%)	303 (378.8)	133 (97.8%)	1197 (454.1)
COVID-19	47 (49.0%)	53 (28.9)	16 (40.0%)	19 (23.8)	63 (46.3%)	72 (27.3)
Headache	16 (16.7%)	35 (19.1)	13 (32.5%)	16 (20.0)	29 (21.3%)	51 (19.3)
Diarrhea	18 (18.8%)	26 (14.2)	8 (20.0%)	(9 (11.3)	26 (19.1%)	35 (13.3)
Upper respiratory tract infection	10 (10.4%)	18 (9.8)	14 (35.0%)	18 (22.5)	24 (17.6%)	36 (13.7)
Nasopharyngitis	22 (22.9%)	32 (17.4)	1 (2.5%)	1 (1.3)	23 (16.9%)	33 (12.5)
Abdominal pain	11 (11.5%)	16 (8.7)	6 (15.0%)	6 (7.5)	17 (12.5%)	22 (8.3)
Nausea	14 (14.6%)	19 (10.3)	2 (5.0%)	3 (3.8)	16 (11.8%)	22 (8.3)
Vomiting	13 (13.5%)	17 (9.3)	3 (7.5%)	3 (3.8)	16 (11.8%)	20 (7.6)
Pyrexia	10 (10.4%)	13 (7.1)	5 (12.5%)	6 (7.5)	15 (11.0%)	19 (7.2)
Breakthrough hemolysis (BTH)	11 (11.5%)	19 (10.3)	3 (7.5%)	3 (3.8)	14 (10.3%)	22 (8.3)

For TEAEs through 2 years, a frequency threshold of 10% or higher was selected. This is to account for the expected increase in frequency of TEAEs that is due to the longer observation period (2 years of exposure to FABHALTA in the REP; 24 weeks exposure in APPLY and APPOINT).[4]

8 patients discontinued FABHALTA in the APPLY and APPOINT 48-week studies as well as the 2-year REP:
2 patients in the 48-week studies (pregnancy), 4 patients during the REP (2 deaths, 1 adverse event, 1 physician decision), and 2 patients did not enter the REP program at all (eligibility, delayed IRB approval).[4,13]

*The 'n' values reflect the number of patients with at least 1 event.[4]
†The occurrence rate (number of episodes per 100 patient-years) was calculated as 100 times (the total number of AE episodes from all patients in the population divided by the total number of patient-years).[4]
‡Total exposure in patient-years is computed as (sum of the duration of on treatment periods over patients, in days) divided by 365.25.[4]
AE, adverse event; IRB, Institutional Review Board; PY, patient-years; TEAE, treatment-emergent adverse event.

Please click here for additional Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

Clinical breakthrough hemolysis and major adverse vascular events through 2 years

ADDITIONAL END POINTS

The data are for observation only. No formal conclusions can be made.

	APPLY patients in REP: 2 years[3] N=62		APPOINT patients in REP: 2 years[3] N=40		Both APPLY and APPOINT patients in REP: 2 years[3] N=136	
	% (n)*	Occurrence rate† (events per 100 PY,‡ PY=183.6)	% (n)*	Occurrence rate† (events per 100 PY,‡ PY=80)	% (n)*	Occurrence rate† (events per 100 PY,‡ PY=263.6)
Number of patients with BTH	11.5% (11)	10.3 (19 events) (95% CI; 5.0-21.4)	7.5% (3)	3.8 (3 events) (95% CI; 1.2-11.3)	10.3% (14)	8.3 (22 events) (95% CI; 4.4-15.9)
Number of patients with ≥1 MAVE§	3.1% (3)	2.2 (4 events) (95% CI; 0.7-7.2)	0% (0)	0.0 (0 events) (95% CI; 0.0-4.6)	2.2%(3)	1.5 (4 events) (95% CI; 0.5-5.0)

BTH:

- Adult patients with PNH on FABHALTA experienced 8.3 clinical BTH events per 100 PY (22 events, 95% CI 4.4-15.9)[4]

MAVEs:

- Adult patients with PNH on FABHALTA experienced 1.5 MAVEs§ per 100 PY (4 events, 95% CI 0.5-5.0)[3]

*The 'n' values reflect the number of patients with at least 1 event.[3]
†The occurrence rate (number of episodes per 100 patient-years) was calculated as 100 times (the total number of AE episodes from all patients in the population divided by the total number of patient-years).[3]
‡Total exposure in patient-years is computed as (sum of the duration of on treatment periods over patients, in days) divided by 365.25.[3]
§The definition of MAVEs included: acute peripheral vascular occlusion, amputation, cerebrovascular accident, cerebral venous occlusion, dermal thrombosis, gangrene, hepatic/portal vein thrombosis, mesenteric/visceral arterial or vein thrombosis or infarction, myocardial infarction, pulmonary embolus, renal arterial or vein thrombosis, thrombophlebitis/deep vein thrombosis, transient ischemic attack, and unstable angina.[2]

Study Design
Hb Response Rates
Additional End Points
REP Safety
Adverse Events
▸ BTH and MAVEs

IMPORTANT SAFETY INFORMATION (continued)

WARNINGS AND PRECAUTIONS (continued)

ADVERSE REACTIONS

- The most common adverse reactions (≥10%) in adults with PNH receiving FABHALTA were headache, nasopharyngitis, diarrhea, abdominal pain, bacterial infection, viral infection, nausea, and rash.

Please click here for additional Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

With twice-daily oral dosing, FABHALTA gives your adult patients an option without the need for infusions

Patients take 1 capsule twice daily[1]

- 200-mg capsule
- FABHALTA can be taken without regard to food
- No refrigeration requirement*
- Patients should swallow capsules whole. Do not open, break, or chew capsules

Show your patients a world without infusions[1]

- No need to schedule infusion appointments or travel to infusion centers
- No potential for injection-site reactions
- No loading dose or dose adjustment required

What to do if your patient misses a dose[1]

If a dose or doses are missed, advise your patient to take 1 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.

Switching from C5is (eculizumab or ravulizumab)[1]

To reduce the potential risk of hemolysis with abrupt discontinuation of other PNH therapies:

- For patients switching from eculizumab, initiate FABHALTA no later than 1 week after the last dose of eculizumab
- For patients switching from ravulizumab, initiate FABHALTA no later than 6 weeks after the last dose of ravulizumab

There is no available information regarding time frame for initiation of FABHALTA after other PNH therapies.

Drug interactions with FABHALTA[1]

- Concomitant use of CYP2C8 inducers (eg, rifampin) may decrease iptacopan exposure, which may result in loss of or reduced efficacy of FABHALTA. Monitor the clinical response and discontinue use of the CYP2C8 inducer if loss of efficacy of FABHALTA is evident
- Concomitant use of strong CYP2C8 inhibitors (eg, gemfibrozil) may increase iptacopan exposure, which may result in an increased risk for adverse reactions with FABHALTA. Coadministration with a strong CYP2C8 inhibitor is not recommended

Use of FABHALTA in specific populations[1]

Those who are breastfeeding:

- Because of the potential for serious adverse reactions in a breastfed child, breastfeeding should be discontinued during treatment with FABHALTA and for 5 days after the final dose

Those with hepatic impairment:

- FABHALTA is not recommended in patients with severe hepatic impairment (Child-Pugh class C). No dose adjustment is required for patients with mild (Child-Pugh class A) or moderate (Child-Pugh class B) hepatic impairment

*Store at 20 °C to 25 °C (68 °F to 77 F); excursions permitted between 15 °C and 30 °C (59 °F and 86 °F).[1]

IMPORTANT SAFETY INFORMATION (continued)
WARNINGS AND PRECAUTIONS (continued)
DRUG INTERACTIONS

- Concomitant use of CYP2C8 inducers (eg, rifampin) may decrease iptacopan exposure, which may result in loss of or reduced efficacy of FABHALTA. Monitor the clinical response and discontinue use of the CYP2C8 inducer if loss of efficacy of FABHALTA is evident.

Please click here for additional Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

Help your patients start their journey with FABHALTA

Get your patients ready with these 3 steps[1]:

1 GET REMS certified to prescribe FABHALTA

Because of the risk of serious infections caused by encapsulated bacteria, you will need to become certified in the FABHALTA REMS and fulfill its requirements.

To enroll in the REMS:

- ✓ **Review** the FABHALTA Prescribing Information and REMS materials
- ✓ **Submit** the completed Prescriber Enrollment form to the FABHALTA REMS at **www.FABHALTA-REMS.com**, or by fax to 1-877-206-3255

After enrollment:

- ✓ **Counsel** patients about the risk of serious infections caused by encapsulated bacteria, the need for vaccinations, and the early signs and symptoms of serious infections
- ✓ **Provide** patients with REMS educational materials and the Patient Safety Card
- ✓ **Instruct** patients to always carry this card with them during treatment and for 2 weeks following the last dose of FABHALTA

FABHALTA, a complement inhibitor, increases the risk of serious infections, especially those caused by encapsulated bacteria, such as *Streptococcus pneumoniae*, *Neisseria meningitidis*, and *Haemophilus influenzae* type b. Life-threatening and fatal infections with encapsulated bacteria have occurred in patients treated with complement inhibitors. These infections may become rapidly life-threatening or fatal if not recognized and treated early.

Additional information is available by telephone at 1-833-99FABHA or online at **www.FABHALTA-REMS.com**.

2 COMPLETE or update vaccinations before starting treatment with FABHALTA

- ✓ **Comply** with the most current ACIP recommendations for vaccinations against encapsulated bacteria in patients receiving a complement inhibitor
- ✓ **Required** vaccinations: *Streptococcus pneumoniae* and *Neisseria meningitidis* (serogroups A, C, W, Y, and B)
- ✓ **Complete or update** vaccinations for encapsulated bacteria at least 2 weeks prior to starting FABHALTA, unless the risks of delaying FABHALTA outweigh the risk of developing a serious infection
  - If urgent FABHALTA therapy is indicated in a patient who is not up to date with vaccines against encapsulated bacteria, provide the patient with antibacterial drug prophylaxis and administer these vaccines as soon as possible. For additional details on antibacterial drug prophylaxis, please see the FABHALTA Prescribing Information, Warnings and Precautions (Section 5.1)

During treatment with FABHALTA:
As vaccination does not eliminate the risk of serious encapsulated bacterial infections, closely monitor patients for early signs and symptoms. Inform patients of these signs and symptoms, and instruct patients to seek immediate medical care if they occur.

- Evaluate and treat immediately if infection is suspected, as serious infection may rapidly become life-threatening or fatal if not recognized and treated early. Promptly treat known infections
- Consider interruption of FABHALTA in patients who are receiving treatment for serious infections
- While on therapy, patients are required to be revaccinated as needed

3 PRESCRIBE FABHALTA through a limited network of specialty pharmacies

- ✓ **Inform** your patient which specialty pharmacy will be dispensing their FABHALTA prescription, and tell them to expect a phone call to arrange delivery of their prescription. Pharmacies that dispense FABHALTA must be certified in the FABHALTA REMS and must verify that prescribers are certified

Onco360®

- Website: **onco360.com**
- Phone: 1 (877) 662-6633; Fax: 1 (877) 662-6355

Biologics by McKesson

- Website: **biologics.mckesson.com**
- Phone: 1 (800) 850-4306; Fax: 1 (800) 823-4506

Getting Started

Novartis Patient Support

For more information on how to get your patients started on FABHALTA, please visit **fabhalta-hcp.com/pnh.**

Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

A dedicated team for you and your patients

Novartis Patient Support is a comprehensive program that can help your eligible patients start, stay and save on FABHALTA® (iptacopan)

We support you and your patients' journey with:

Insurance support

Help navigating the insurance process, including benefits verification and support with the prior authorization and appeals processes.

Financial support

Assistance with relevant savings options for your eligible patients, including $0 Co-Pay Plus* offer and affordability programs.

Eligible† privately insured patients can receive up to 12 months of FABHALTA for free through the Bridge Program while coverage is pursued.

Vaccination support‡

For eligible patients, our dedicated Novartis Patient Support team can help schedule in-home administration appointments, find local vaccination locations, and offer guidance on accessing existing vaccination records.

Ongoing support

Dedicated assistance from our team which includes:

- A welcome kit with resources to help them get started and stay on treatment
- A dose reminder program to help them remember to take their medication
- A choice of texts, calls, virtual calls, and emails to get ongoing resources

Novartis Patient Assistance Foundation

The Novartis Patient Assistance Foundation, Inc. (NPAF) is a non-profit, 501(c)3 charitable organization that supports access to prescribed Novartis medications for patients facing financial hardship with limited or no prescription or medical coverage. More information can be found on the NPAF website **www.PAP.Novartis.com** or by calling **1-800-277-2254**.

Access for FABHALTA‡

- **84%** of initiated Prior Authorizations (PAs) were approved for adults with PNH who were prescribed FABHALTA
- Adults with PNH who were prescribed FABHALTA had their PA approved in less than **13** days on average
- Adults with PNH who were prescribed FABHALTA started treatment **20** days from time of prescription on average

Download the FABHALTA Start Form

Questions?

Call Novartis Patient Support at **833-99FABHA (833-993-2242)**, Monday-Friday, 8:00 AM-8:00 PM ET, excluding holidays.

*__Limitations apply.__ Up to a $20,000 annual limit. Offer not valid under Medicare, Medicaid, or any other federal or state programs. Novartis reserves the right to rescind, revoke, or amend this program without notice. Additional limitations may apply. See complete Terms & Conditions at www.fabhalta.com for details.

†__Limitations apply.__ Patients with commercial insurance, a valid prescription for FABHALTA, and a denial of insurance coverage based on a prior authorization requirement may receive a monthly maintenance dose for up to 12 months or until insurance coverage approval, whichever occurs first. Not available to patients whose medications are reimbursed in whole or in part by Medicare, Medicaid, TRICARE, VA, DoD or any other federal or state program, or where prohibited by law. A prior authorization and/or appeal of coverage denial must be submitted within 90 days to remain in the program. No purchase necessary. Program is not health insurance, nor is participation a guarantee of insurance coverage. Additional restrictions may apply. Novartis reserves the right to rescind, revoke or amend this Program without notice.

‡__Vaccination Support: Limitations Apply.__ Please contact Novartis Patient Support at 833-99FABHA (833-993-2242) for more information.

Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

Indication and Important Safety Information

INDICATION

FABHALTA is indicated for the treatment of adults with paroxysmal nocturnal hemoglobinuria (PNH).

IMPORTANT SAFETY INFORMATION

WARNING: SERIOUS INFECTIONS CAUSED BY ENCAPSULATED BACTERIA

FABHALTA, a complement inhibitor, increases the risk of serious infections, especially those caused by encapsulated bacteria, such as *Streptococcus pneumoniae, Neisseria meningitidis,* and *Haemophilus influenzae* type b. Life-threatening and fatal infections with encapsulated bacteria have occurred in patients treated with complement inhibitors. These infections may become rapidly life-threatening or fatal if not recognized and treated early.

- **Complete or update vaccinations for encapsulated bacteria at least 2 weeks prior to the first dose of FABHALTA, unless the risks of delaying therapy with FABHALTA outweigh the risk of developing a serious infection. Comply with the most current Advisory Committee on Immunization Practices (ACIP) recommendations for vaccinations against encapsulated bacteria in patients receiving a complement inhibitor.**
- **Patients receiving FABHALTA are at increased risk for invasive disease caused by encapsulated bacteria, even if they develop antibodies following vaccination. Monitor patients for early signs and symptoms of serious infections and evaluate immediately if infection is suspected.**

Because of the risk of serious infections caused by encapsulated bacteria, FABHALTA is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS) called the FABHALTA REMS.

CONTRAINDICATIONS

- Patients with serious hypersensitivity to FABHALTA or any of the excipients.
- For initiation in patients with unresolved serious infection caused by encapsulated bacteria, including *Streptococcus pneumoniae, Neisseria meningitidis,* or *Haemophilus influenzae* type b.

WARNINGS AND PRECAUTIONS

Serious Infections Caused by Encapsulated Bacteria

- FABHALTA, a complement inhibitor, increases a patient's susceptibility to serious, life-threatening, or fatal infections caused by encapsulated bacteria, including *Streptococcus pneumoniae, Neisseria meningitidis* (caused by any serogroup, including nongroupable strains), and *Haemophilus influenzae* type b. Life-threatening and fatal infections with encapsulated bacteria have occurred in both vaccinated and unvaccinated patients treated with complement inhibitors. The initiation of FABHALTA is contraindicated in patients with unresolved serious infections caused by encapsulated bacteria.
- Complete or update vaccination against encapsulated bacteria at least 2 weeks prior to the start of FABHALTA, according to the current ACIP recommendations for patients receiving a complement inhibitor. Revaccinate patients in accordance with ACIP recommendations considering the duration of therapy with FABHALTA. Note that ACIP recommends an administration schedule in patients receiving complement inhibitors that differs from the administration schedule in the vaccine prescribing information. If urgent FABHALTA therapy is indicated in a patient who is not up to date with vaccines against encapsulated bacteria according to ACIP recommendations, provide the patient with antibacterial drug prophylaxis and administer these vaccines as soon as possible. The benefits and risks of treatment with FABHALTA, as well as the benefits and risks of antibacterial drug prophylaxis in unvaccinated or vaccinated patients, must be considered against the known risks for serious infections caused by encapsulated bacteria.
- Vaccination does not eliminate the risk of serious encapsulated bacterial infections, despite development of antibodies following vaccination. Closely monitor patients for early signs and symptoms of serious infection and evaluate patients immediately if an infection is suspected. Inform patients of these signs and symptoms and instruct patients to seek immediate medical care if they occur. Promptly treat known infections. Serious infection may become rapidly life-threatening or fatal if not recognized and treated early. Consider interruption of FABHALTA in patients who are undergoing treatment for serious infections, depending on the risks of interrupting treatment in the disease being treated.

ADDITIONAL IMPORTANT SAFETY INFORMATION

Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

Important Safety Information (continued)

WARNINGS AND PRECAUTIONS (continued)

FABHALTA REMS

- FABHALTA is available only through a restricted program under a REMS called FABHALTA REMS, because of the risk of serious infections caused by encapsulated bacteria.
- Under the FABHALTA REMS, prescribers must enroll in the program. Prescribers must counsel patients about the risks, signs, and symptoms of serious infections caused by encapsulated bacteria, provide patients with the REMS educational materials, ensure patients are vaccinated against encapsulated bacteria, prescribe antibacterial drug prophylaxis if patients' vaccine status is not up to date and treatment must be started urgently, and provide instructions to always carry the Patient Safety Card during treatment and for 2 weeks following last dose of FABHALTA.
- Further information is available by telephone: 1-833-993-2242 or online at www.FABHALTA-REMS.com.

Monitoring of PNH Manifestations After FABHALTA Discontinuation

- After discontinuing FABHALTA, closely monitor patients for at least 2 weeks after the last dose for signs and symptoms of hemolysis. These signs include elevated lactate dehydrogenase (LDH) levels along with sudden decrease in hemoglobin or PNH clone size, fatigue, hemoglobinuria, abdominal pain, dyspnea, major adverse vascular events (such as thrombosis, stroke, and myocardial infarction), dysphagia, or erectile dysfunction. If discontinuation of FABHALTA is necessary, consider alternative therapy.
- If hemolysis occurs after discontinuation of FABHALTA, consider restarting treatment with FABHALTA, if appropriate, or initiating another treatment for PNH.

Hyperlipidemia

- FABHALTA may increase total cholesterol, LDL cholesterol, and serum triglycerides.
- Of 88 FABHALTA-treated patients who had normal total cholesterol at baseline, 31 developed grade 1 hypercholesterolemia during the randomization or core treatment period and 1 patient worsened from baseline grade 1 to grade 2.
- Of 96 FABHALTA-treated patients with LDL cholesterol ≤ 130 mg/dL at baseline during the randomization or core treatment period, 14 patients developed LDL cholesterol > 130-160 mg/dL, 6 patients developed LDL cholesterol > 160-190 mg/dL and 4 patients developed LDL cholesterol > 190 mg/dL.
- Of 89 FABHALTA-treated patients with normal triglycerides during the randomization or core treatment period, 22 patients developed grade 1 elevated triglycerides. Three patients experienced an increase in triglycerides from grade 1 to grade 2.
- Of the 102 FABHALTA-treated patients in APPLY-PNH and APPOINT-PNH, 2 patients required cholesterol-lowering medications.
- Monitor serum lipid parameters periodically during treatment with FABHALTA and initiate cholesterol-lowering medications, if indicated.

ADVERSE REACTIONS

- The most common adverse reactions (≥10%) in adults with PNH receiving FABHALTA were headache, nasopharyngitis, diarrhea, abdominal pain, bacterial infection, viral infection, nausea, and rash.

DRUG INTERACTIONS

- Concomitant use of CYP2C8 inducers (eg, rifampin) may decrease iptacopan exposure, which may result in loss of or reduced efficacy of FABHALTA. Monitor the clinical response and discontinue use of the CYP2C8 inducer if loss of efficacy of FABHALTA is evident.
- Concomitant use of strong CYP2C8 inhibitors (eg, gemfibrozil) may increase iptacopan exposure, which may result in increased risk for adverse reactions with FABHALTA. Coadministration with a strong CYP2C8 inhibitor is not recommended.

USE IN SPECIFIC POPULATIONS

- Because of the potential for serious adverse reactions in a breastfed child, breastfeeding should be discontinued during treatment and for 5 days after the final dose.
- FABHALTA is not recommended in patients with severe hepatic impairment (Child-Pugh class C). No dose adjustment is required for patients with mild (Child-Pugh class A) or moderate (Child-Pugh class B) hepatic impairment.

Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

FABHALTA® (iptacopan) 200 mg capsules

HELP YOUR PATIENTS GET STARTED ON FABHALTA

LEARN MORE ABOUT FABHALTA
at www.fabhalta-hcp.com

ENROLL IN REMS
at www.fabhalta-rems.com

DOWNLOAD THE START FORM
at www.fabhalta-startform.com

Please click here for Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

REFERENCES

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East Hanover, New Jersey 07936-1080

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Please click here for Important Safety Information. Please click here for full Prescribing Information, including Boxed WARNING and Medication Guide.

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