

# Novartis presents new 48-week results from Phase III APPLY-PNH trial showing sustained efficacy and long-term safety of Fabhalta® (iptacopan) in adults with paroxysmal nocturnal hemoglobinuria (PNH)

Dec 11, 2023

- APPLY-PNH extension data show that continuous Fabhalta® (iptacopan) treatment in adults with paroxysmal nocturnal hemoglobinuria (PNH) enabled sustained hemoglobin-level increases to near-normal ( $\geq 12$  g/dL), blood transfusion avoidance, and improved patient-reported fatigue in the majority of patients, with a safety profile consistent with previously reported data<sup>1-5</sup>
- Patients switching from anti-C5s to Fabhalta in the extension period achieved outcomes comparable to the Fabhalta arm in the 24-week randomized controlled period, including transfusion avoidance and near-normal hemoglobin-levels ( $\geq 12$  g/dL) in the majority of patients<sup>1</sup>
- Fabhalta was recently approved by the FDA for adults with PNH, including for both previously treated and treatment-naïve patients<sup>6</sup>

EAST HANOVER, N.J., Dec. 10, 2023 -- Novartis today announced results from the extension period of the pivotal Phase III APPLY-PNH trial of oral monotherapy Fabhalta® (iptacopan) in adults with paroxysmal nocturnal hemoglobinuria (PNH) who had residual anemia (hemoglobin  $< 10$  g/dL) despite previous anti-C5 therapy<sup>1,2</sup>. Continuous Fabhalta treatment (200 mg twice daily) for 48 weeks enabled sustained hemoglobin-level increases to near-normal (12 g/dL or more), blood transfusion avoidance, and reduced patient-reported fatigue in the majority of patients; comparable benefits emerged in those patients switching from anti-C5 therapy to Fabhalta in the extension<sup>1</sup>. Data will be presented at the 65<sup>th</sup> American Society of Hematology Annual Meeting & Exposition (ASH).

"The new APPLY-PNH data are an expansion of the robust outcomes we saw in the randomized phase and demonstrate that patients with PNH who took Fabhalta experienced meaningful hemoglobin improvement over the longer term – nearly a year," said principal co-investigator Antonio Risitano, M.D., Ph.D., President of the International PNH Interest Group and Head of the Hematology and Hematopoietic Transplant Unit, Reference Center for Aplastic Anemia and Paroxysmal Nocturnal Hemoglobinuria at the AORN San Giuseppe Moscati, Avellino, Italy. "Additionally, the new data confirm that these benefits may occur within weeks after switching from anti-C5s. The APPLY-PNH findings continue to confirm Fabhalta as a promising therapeutic option for people living with PNH."

Patients completing the 24-week randomized treatment period of APPLY-PNH could elect to enter the extension, continuing Fabhalta (61/62 patients; one patient discontinued due to pregnancy) or switching from anti-C5s to Fabhalta (34/35 patients; one patient discontinued based on investigator decision) through week 48<sup>1,2</sup>.

In the continuous Fabhalta group, outcomes achieved in the randomized period were sustained at 48 weeks: mean hemoglobin level continued to be near-normal (12.2 g/dL), nearly all patients (91.9%) remained free of transfusions (Weeks 2-48), and improvements in patient-reported fatigue were observed (adjusted mean change from baseline: 9.80-point increase in Functional Assessment of Chronic Illness Therapy – Fatigue [FACIT-F] score)<sup>1</sup>.

In the anti-C5-to-Fabhalta group, similar benefits emerged after switch: mean hemoglobin levels increased to near-normal (from 9.1 g/dL at 24 weeks to 12.1 g/dL at 48 weeks), transfusion avoidance was achieved for almost all patients (94.1%, Weeks 26-48), and improvements in patient-reported fatigue were observed after switching to Fabhalta (adjusted mean change from baseline between Week 48 and Week 24: 10.79-point increase in FACIT-F score)<sup>1</sup>.

"Coming on the heels of Fabhalta's recent approval in PNH, these extended data from the APPLY-PNH phase III trial reinforce Fabhalta's utility as an important new oral monotherapy for people living with PNH," said David Soergel, M.D., Global Head, Cardiovascular, Renal and Metabolism Development Unit, Novartis. "We are eager to bring this novel treatment to even more people living with rare complement-driven disorders as we pursue several additional indications for Fabhalta."

Fabhalta had a similar safety profile at 48 weeks vs. 24 weeks<sup>1,2</sup>. Three patients had major adverse vascular events (MAVEs), all considered unrelated to Fabhalta (one serious transient ischemic attack [TIA] occurred in the randomized period and was reported previously)<sup>1,2</sup>. In the extension, there was one non-serious TIA and one serious portal vein thrombosis (PVT; this patient had a history of PVT and discontinued heparin prior to the MAVE)<sup>1</sup>. Six patients of 62 receiving continuous Fabhalta for 48 weeks had clinical breakthrough hemolysis (BTH); one patient in the anti-C5-to-Fabhalta extension arm had clinical BTH after switching (compared to six of 35 patients while on anti-C5s prior to switch)<sup>1,2</sup>. All cases of clinical BTH resolved without changing Fabhalta dosing<sup>1</sup>. During the 48-week study period, the most frequently reported treatment-emergent adverse events (TEAEs) in the Fabhalta arm were COVID-19 (29.0% of patients), headache (19.4%), and diarrhea (16.1%)<sup>1</sup>. Throughout the full 48 weeks on Fabhalta, 9.7% of patients experienced any severe TEAE, and 14.5% experienced any serious TEAE, none of which was suspected to be related to Fabhalta treatment; there were no serious hemolysis TEAEs on Fabhalta<sup>1,2</sup>. There were no serious infections caused by *N. meningitidis*, *S. pneumoniae*, or *H. influenzae* and no treatment discontinuations because of TEAEs<sup>1,2</sup>.

PNH is a rare, chronic, and serious complement-mediated blood disorder in which a large proportion of patients can remain anemic and some dependent on blood transfusions despite currently available standard of care, anti-C5 treatments<sup>7-10</sup>.

Full 48-week results from the Phase III APPOINT-PNH trial in treatment-naïve PNH patients will be presented at a congress in 2024.

## Indication

FABHALTA is a prescription medicine used to treat adults with paroxysmal nocturnal hemoglobinuria (PNH).

It is not known if FABHALTA is safe and effective in children.

## Important Safety Information

FABHALTA is a medicine that affects part of the immune system and may lower one's ability to fight infections. FABHALTA increases the chance of getting serious infections caused by encapsulated bacteria, including *Streptococcus pneumoniae*, *Neisseria meningitidis*, and *Haemophilus influenzae* type B. These serious infections may quickly become life-threatening or fatal if not recognized and treated early. Patients must complete or update vaccinations against these bacteria at least 2 weeks before starting FABHALTA. If patients have not completed these vaccinations and FABHALTA therapy must be started right away, they should receive the required vaccinations as soon as possible. If patients have not been vaccinated and FABHALTA must be started right away, they should also receive antibiotics to take for as long as their doctor tells them. If patients have been vaccinated against these bacteria in the past, they might need additional vaccinations before starting FABHALTA. Their doctor will decide if they need additional vaccinations. Vaccines do not prevent all infections caused by encapsulated bacteria. Patients should call their doctor or get emergency medical care right away if they have any of these signs and symptoms of a serious infection: fever with or without shivers or chills; fever with chest pain and cough; fever with high heart rate; headache and fever; confusion; clammy skin; fever and a rash; fever with breathlessness/fast breathing; headache with nausea or vomiting; headache with stiff neck or stiff back; body aches with flu-like symptoms; or eyes sensitive to light. Doctors will give their patients a Patient Safety Card about the risk of serious infections. Patients must carry it with them at all times during treatment and for 2 weeks after their last dose of FABHALTA. The risk of serious infections may continue for a few weeks after their last dose of FABHALTA. It is important for patients to show

this card to any doctor who treats them. This will help doctors diagnose and treat patients quickly.

FABHALTA is only available through a program called the FABHALTA Risk Evaluation and Mitigation Strategy (REMS). Before patients can take FABHALTA, their doctor must enroll in the FABHALTA REMS program, counsel patients about the risk of serious infections caused by certain bacteria, give patients information about the symptoms of serious infections, make sure that patients are vaccinated against serious infections caused by encapsulated bacteria and that they receive antibiotics if they need to start FABHALTA right away and are not up to date on vaccinations, as well as give patients a Patient Safety Card about the risk of serious infections.

Since FABHALTA may increase patients' cholesterol and triglycerides, their doctor will do blood tests to check their levels periodically.

Patients should not take FABHALTA if they are allergic to FABHALTA or any of the ingredients in FABHALTA. Patients should not take FABHALTA if they have a serious infection caused by encapsulated bacteria, including *Streptococcus pneumoniae*, *Neisseria meningitidis*, or *Haemophilus influenzae* type B when starting FABHALTA.

Before taking FABHALTA, patients should tell their doctor about all their medical conditions, including if they have an infection or fever, have kidney or liver problems, are pregnant or plan to become pregnant (it is not known if FABHALTA will harm an unborn baby), or are breastfeeding or plan to breastfeed as it is not known if FABHALTA passes into breast milk. Patients should not breastfeed during treatment and for 5 days after the last dose of FABHALTA.

Patients should tell their doctor about all the medicines they take, including prescription and over-the-counter medicines, vitamins, and herbal supplements. Taking FABHALTA with certain other medicines may affect the way FABHALTA works and may cause side effects. Patients should know the medicines they take and the vaccines they receive. Patients should keep a list of them to show their doctor and pharmacist when they get a new medicine.

If patients have PNH and stop taking FABHALTA, their doctor will need to monitor them closely for at least 2 weeks after stopping FABHALTA. Stopping treatment with FABHALTA may cause a breakdown of red blood cells due to PNH. Symptoms or problems that can happen due to breakdown of red blood cells include decreased hemoglobin level in the blood; blood in the urine; shortness of breath; trouble swallowing; tiredness; pain in the stomach (abdomen); blood clots, stroke, and heart attack; and erectile dysfunction (ED). It is important that patients take FABHALTA exactly as their doctor tells them to lower the possibility of breakdown of red blood cells due to PNH.

The most common side effects of FABHALTA include headache; nasal congestion, runny nose, cough, sneezing, and sore throat (nasopharyngitis); diarrhea; pain in the stomach (abdomen); infections (viral and bacterial); nausea; and rash.

Please see full [Prescribing Information](#), including Boxed WARNING and [Medication Guide](#).

#### About APPLY-PNH

APPLY-PNH (NCT04558918) was a Phase III, randomized, multinational, multicenter, active-comparator controlled, open-label trial to evaluate the efficacy and safety of twice-daily, oral Fabhalta monotherapy (200 mg) for the treatment of PNH by assessing if switching to Fabhalta was superior to continuing on anti-C5 therapies (US-approved and non-US-approved eculizumab or ravulizumab) in adult patients presenting with residual anemia (Hb <10 g/dL) despite a stable regimen of anti-C5 treatment in the last six months prior to randomization<sup>4,11</sup>. The trial enrolled 97 patients who were randomized in an 8:5 ratio to either twice-daily, oral Fabhalta monotherapy, or intravenous anti-C5 therapies (continuing with the same regimen as they were on prior to randomization)<sup>4,11</sup>.

#### About paroxysmal nocturnal hemoglobinuria (PNH)

PNH is a rare, chronic and serious complement-mediated blood disorder<sup>7</sup>. People with PNH have an acquired mutation in some of their hematopoietic stem cells (which are located in the bone marrow and can grow and develop into RBCs, white blood cells and platelets) that causes them to produce RBCs that are susceptible to premature destruction by the complement system<sup>7,8</sup>. This leads to intravascular hemolysis (destruction of RBCs within blood vessels) and extravascular hemolysis (destruction of RBCs mostly in the spleen and liver), which cause anemia (low levels of circulating RBCs), thrombosis (formation of blood clots), fatigue and other debilitating symptoms<sup>7,8</sup>.

It is estimated that approximately 10-20 people per million worldwide live with PNH<sup>7</sup>. Although PNH can develop at any age, it is often diagnosed in people between 30-40 years old<sup>12-14</sup>.

PNH has a significant unmet need not addressed by anti-C5 therapies (eculizumab or ravulizumab): despite treatment with anti-C5s, a large proportion of people with PNH remain anemic, and some dependent on blood transfusions<sup>7-10,15</sup>.

#### About Fabhalta® (iptacopan)

Fabhalta (iptacopan) is an oral, Factor B inhibitor of the alternative complement pathway<sup>14,16,17</sup>. Fabhalta is FDA-approved for the treatment of adults with paroxysmal nocturnal hemoglobinuria (PNH).

Discovered at Novartis, Fabhalta is currently in development for a range of complement-mediated diseases including, immunoglobulin A nephropathy (IgA nephropathy), C3 glomerulopathy (C3G), immune complex membranoproliferative glomerulonephritis (IC-MPGN) and atypical hemolytic uremic syndrome (aHUS).

Based on disease prevalence, unmet needs and data from Phase II studies, Fabhalta has received FDA approval in PNH, FDA Breakthrough Therapy Designation in C3G, orphan drug designations from the FDA and EMA in PNH and C3G, EMA PRIME designation for C3G, and EMA orphan drug designation in IgAN<sup>6,18-22</sup>.

#### Disclaimer

This press release contains forward-looking statements within the meaning of the United States Private Securities Litigation Reform Act of 1995. Forward-looking statements can generally be identified by words such as "potential," "can," "will," "plan," "may," "could," "expectations," "investigational," "drives," "remains," "ongoing," "exploring," "goal," "expected," "estimated," or similar terms, or by express or implied discussions regarding potential marketing approvals, new indications or labeling for FABHALTA (iptacopan), or regarding potential future revenues from such products. You should not place undue reliance on these statements. Such forward-looking statements are based on our current beliefs and expectations regarding future events, and are subject to significant known and unknown risks and uncertainties. Should one or more of these risks or uncertainties materialize, or should underlying assumptions prove incorrect, actual results may vary materially from those set forth in the forward-looking statements. There can be no guarantee that FABHALTA (iptacopan) will be submitted or approved for sale or for any additional indications or labeling in any market, or at any particular time. Nor can there be any guarantee that such products will be commercially successful in the future. In particular, our expectations regarding FABHALTA (iptacopan) could be affected by, among other things, the uncertainties inherent in research and development, including clinical trial results and additional analysis of existing clinical data; regulatory actions or delays or government regulation generally; global trends toward health care cost containment, including government, payor and general public pricing and reimbursement pressures and requirements for increased pricing transparency; our ability to obtain or maintain proprietary intellectual property protection; the particular prescribing preferences of physicians and patients; general political, economic and business conditions, including the effects of and efforts to mitigate pandemic diseases; safety, quality, data integrity or manufacturing issues; potential or actual data security and data privacy breaches, or disruptions of our information technology systems, and other risks and factors referred to in Novartis AG's current Form 20-F on file with the US Securities and Exchange Commission. Novartis is providing the information in this press release as of this date and does not undertake any obligation to update any forward-looking statements contained in this press release as a result of new information, future events or otherwise.

#### About Novartis

Novartis is an innovative medicines company. Every day, we work to reimagine medicine to improve and extend people's lives so that patients, healthcare professionals and societies are empowered in the face of serious disease. Our medicines reach more than 250 million people worldwide.

Reimagine medicine with us: Visit us at <https://www.novartis.com> and <https://www.novartis.us> and connect with us on [LinkedIn](#), [LinkedIn US](#), [Facebook](#), [X/Twitter](#), [X/Twitter](#) [US](#) and [Instagram](#).

## References

1. Risitano AM, Kulasekararaj A, Röth A, et al. Factor B Inhibition with Oral Iptacopan Monotherapy Demonstrates Sustained Long-Term Efficacy and Safety in Anti-C5-Treated Patients (pts) with Paroxysmal Nocturnal Hemoglobinuria (PNH) and Persistent Anemia: Final 48-Week Results from the Multicenter, Phase III APPLY-PNH Trial. Presented at: 65th American Society of Hematology Annual Meeting & Exposition (ASH); December 9-12, 2023; San Diego, CA
2. Risitano AM, Röth A, Kulasekararaj A, et al. Oral Iptacopan Monotherapy Has Superior Efficacy to Anti-C5 Therapy in Patients with Paroxysmal Nocturnal Hemoglobinuria and Residual Anemia: Results From the Phase III APPLY-PNH Study. Presented at: 49th Annual Meeting of the European Society for Blood and Marrow Transplantation (EBMT); April 23-36, 2023; Paris, France.
3. Risitano AM, Han B, Ueda Y, et al. Oral Complement Factor B Inhibitor Iptacopan Monotherapy Improves Hemoglobin to Normal/Near-Normal Levels in Paroxysmal Nocturnal Hemoglobinuria Patients Naïve to Complement Inhibitors: Phase III APPOINT-PNH Trial. Presented at: 49th Annual Meeting of the European Society for Blood and Marrow Transplantation (EBMT); April 23-36, 2023; Paris, France.
4. Risitano AM, Röth A, Soret J, et al. Addition of iptacopan, an oral factor B inhibitor, to eculizumab in patients with paroxysmal nocturnal haemoglobinuria and active haemolysis: an open-label, single-arm, phase 2, proof-of-concept trial. *Lancet Haematol.* 2021;8(5):e344-e354. doi:10.1016/S2352-3026(21)00028-4
5. Jang H, Wong L, Ko BS, et al. Iptacopan monotherapy in patients with paroxysmal nocturnal hemoglobinuria: a 2-cohort open-label proof-of-concept study. *Blood Adv.* 2022;6(15):4450-4460. doi:10.1182/bloodadvances.2022006960
6. Novartis receives FDA approval for Fabhalta® (iptacopan), offering superior hemoglobin improvement in the absence of transfusions as the first oral monotherapy for adults with PNH. Novartis. Accessed December 6, 2023. <https://www.novartis.com/news/media-releases/novartis-receives-fda-approval-fabhalta-iptacopan-offering-superior-hemoglobin-improvement-absence-transfusions-first-oral-monotherapy-adults-pnh>
7. Cançado RD, Araújo A da S, Sandes AF, et al. Consensus statement for diagnosis and treatment of paroxysmal nocturnal haemoglobinuria. *Hematol Transfus Cell Ther.* 2021;43(3):341-348. doi:10.1016/j.htct.2020.06.006
8. Dingli D, Matos JE, Lehrhaupt K, et al. The burden of illness in patients with paroxysmal nocturnal hemoglobinuria receiving treatment with the C5-inhibitors eculizumab or ravulizumab: results from a US patient survey. *Ann Hematol.* 2022;101(2):251-263. doi:10.1007/s00277-021-04715-5
9. Debureaux PE, Kulasekararaj AG, Cacace F, et al. Categorizing hematological response to eculizumab in paroxysmal nocturnal hemoglobinuria: a multicenter real-life study. *Bone Marrow Transplant.* 2021;56(10):2600-2602. doi:10.1038/s41409-021-01372-0
10. Debureaux PE, Cacace F, Silva BGP, et al. Hematological Response to Eculizumab in Paroxysmal Nocturnal Hemoglobinuria: Application of a Novel Classification to Identify Unmet Clinical Needs and Future Clinical Goals. *Blood.* 2019;134(Supplement\_1):3517-3517. doi:10.1182/blood-2019-125917
11. Novartis Pharmaceuticals. A Randomized, Multicenter, Active-Comparator Controlled, Open-Label Trial to Evaluate Efficacy and Safety of Oral, Twice Daily LNP023 in Adult Patients With PNH and Residual Anemia, Despite Treatment With an Intravenous Anti-C5 Antibody. *clinicaltrials.gov*; 2022. Accessed September 21, 2022. <https://clinicaltrials.gov/ct2/show/NCT04558918>
12. Hill A, DeZern AE, Kinoshita T, Brodsky RA. Paroxysmal nocturnal haemoglobinuria. *Nat Rev Dis Primer.* 2017;3(1):17028. doi:10.1038/nrdp.2017.28
13. Schrezenmeier H, Röth A, Araten DJ, et al. Baseline clinical characteristics and disease burden in patients with paroxysmal nocturnal hemoglobinuria (PNH): updated analysis from the International PNH Registry. *Ann Hematol.* 2020;99(7):1505-1514. doi:10.1007/s00277-020-04052-z
14. Schubart A, Anderson K, Mainolfi N, et al. Small-molecule factor B inhibitor for the treatment of complement-mediated diseases. *Proc Natl Acad Sci.* 2019;116(16):7926-7931. doi:10.1073/pnas.1820892116
15. McKinley CE, Richards SJ, Munir T, et al. Extravascular Hemolysis Due to C3-Loading in Patients with PNH Treated with Eculizumab: Defining the Clinical Syndrome. *Blood.* 2017;130(Supplement 1):3471. doi:10.1182/blood.V130.Suppl\_1.3471.3471
16. Barratt J, Rovin B, Zhang H, et al. POS-546 EFFICACY AND SAFETY OF IPTACOPAN IN IgA NEPHROPATHY: RESULTS OF A RANDOMIZED DOUBLE-BLIND PLACEBO-CONTROLLED PHASE 2 STUDY AT 6 MONTHS. *Kidney Int Rep.* 2022;7(2):S236. doi:10.1016/j.ekir.2022.01.577
17. Rizk DV, Rovin BH, Zhang H, et al. Targeting the Alternative Complement Pathway With Iptacopan to Treat IgA Nephropathy: Design and Rationale of the APPLAUSE-IgAN Study. *Kidney Int Rep.* 2023;8(5):968-979. doi:10.1016/j.ekir.2023.01.041
18. Novartis Pharmaceuticals. A Multicenter, Randomized, Double-Blind, Parallel Group, Placebo-Controlled Study to Evaluate the Efficacy and Safety of Iptacopan (LNP023) in Complement 3 Glomerulopathy. *clinicaltrials.gov*; 2022. Accessed September 20, 2022. <https://clinicaltrials.gov/ct2/show/NCT04817618>
19. Novartis Pharmaceuticals. A Multi-Center, Randomized, Double-Blind, Placebo-Controlled, Parallel Group, Phase III Study to Evaluate the Efficacy and Safety of LNP023 in Primary IgA Nephropathy Patients. *clinicaltrials.gov*; 2022. Accessed September 21, 2022. <https://clinicaltrials.gov/ct2/show/NCT04578834>
20. Novartis announces European Medicines Agency (EMA) has granted orphan drug designation for iptacopan (LNP023) in IgA nephropathy (IgAN). Novartis. Accessed September 22, 2022. <https://www.novartis.com/news/media-releases/novartis-announces-european-medicines-agency-ema-has-granted-orphan-drug-designation-iptacopan-lnp023-iga-nephropathy-igan>
21. Novartis received European Medicines Agency (EMA) PRIME designation for iptacopan (LNP) in C3 glomerulopathy (C3G). Novartis. Accessed September 22, 2022. <https://www.novartis.com/news/media-releases/novartis-received-european-medicines-agency-ema-prime-designation-iptacopan-lnp-c3-glomerulopathy-c3g>
22. Novartis investigational oral therapy iptacopan (LNP023) receives FDA Breakthrough Therapy Designation for PNH and Rare Pediatric Disease Designation for C3G. Novartis. Accessed September 22, 2022. <https://www.novartis.com/news/media-releases/novartis-investigational-oral-therapy-iptacopan-lnp023-receives-fda-breakthrough-therapy-designation-pnh-and-rare-pediatric-disease-designation-c3g>
23. Data on file. Novartis Pharmaceuticals Corp; 2023.

###

## Novartis Media Relations

E-mail: [media.relations@novartis.com](mailto:media.relations@novartis.com)

## North America

Julie Masow +1 862 579 8456

Michael Meo +1 862 274 5414

Marlena Abdinoor +1 617 335 9525

## Novartis Investor Relations

E-mail: [investor.relations@novartis.com](mailto:investor.relations@novartis.com)

North America

Sloan Simpson +1 862 345 4440

Jonathan Graham +1 201 602 9921

Parag Mahanti +1 973 876 4912

---

**Source URL:** <https://qa1.novartis.us/news/media-releases/novartis-presents-new-48-week-results-from-phase-iii-apply-pnh-trial-showing-sustained-efficacy-and-long-term-safety-fabhalta-iptacopan-adults-paroxysmal-nocturnal-hemoglobinuria-pnh>

**List of links present in page**

1. <https://qa1.novartis.us/news/media-releases/novartis-presents-new-48-week-results-from-phase-iii-apply-pnh-trial-showing-sustained-efficacy-and-long-term-safety-fabhalta-iptacopan-adults-paroxysmal-nocturnal-hemoglobinuria-pnh>
2. [https://www.novartis.com/us-en/sites/novartis\\_us/files/fabhalta.pdf](https://www.novartis.com/us-en/sites/novartis_us/files/fabhalta.pdf)
3. [https://www.novartis.com/us-en/sites/novartis\\_us/files/fabhalta\\_pmg.pdf](https://www.novartis.com/us-en/sites/novartis_us/files/fabhalta_pmg.pdf)
4. <https://nam111.safelinks.protection.outlook.com/?url=https%3A/www.novartis.com/&data=05%7C02%7Cemailedpressreleases%40N0151C.onmicrosoft.com%7C946952dfee5b4b66a72708dbf9dfeb54%7C887bf9ee3c824b88t>
5. <https://nam111.safelinks.protection.outlook.com/?url=https%3A/www.novartis.us/&data=05%7C02%7Cemailedpressreleases%40N0151C.onmicrosoft.com%7C946952dfee5b4b66a72708dbf9dfeb54%7C887bf9ee3c824b88t>
6. <https://nam111.safelinks.protection.outlook.com/?url=https%3A/www.linkedin.com/company/novartis/&data=05%7C02%7Cemailedpressreleases%40N0151C.onmicrosoft.com%7C946952dfee5b4b66a72708dbf9dfeb54%7C887bf9ee3c824b88t>
7. <https://nam111.safelinks.protection.outlook.com/?url=https%3A/www.linkedin.com/showcase/novartis-us/&data=05%7C02%7Cemailedpressreleases%40N0151C.onmicrosoft.com%7C946952dfee5b4b66a72708dbf9dfeb54%7C887bf9ee3c824b88t>
8. <https://nam111.safelinks.protection.outlook.com/?url=https%3A/www.facebook.com/novartis/&data=05%7C02%7Cemailedpressreleases%40N0151C.onmicrosoft.com%7C946952dfee5b4b66a72708dbf9dfeb54%7C887bf9ee3c824b88t>
9. <https://nam111.safelinks.protection.outlook.com/?url=https%3A/twitter.com/Novartis&data=05%7C02%7Cemailedpressreleases%40N0151C.onmicrosoft.com%7C946952dfee5b4b66a72708dbf9dfeb54%7C887bf9ee3c824b88t>
10. <https://nam111.safelinks.protection.outlook.com/?url=https%3A/twitter.com/NovartisUS&data=05%7C02%7Cemailedpressreleases%40N0151C.onmicrosoft.com%7C946952dfee5b4b66a72708dbf9dfeb54%7C887bf9ee3c824b88t>
11. [https://nam111.safelinks.protection.outlook.com/?url=https%3A/instagram.com/novartis%3Ffigshid%3DMzRIODBiNWFIZA%3D%3D\\_\\_%3B%21%21N3hqHg43uw%21pjp8z253J5NjaOYrW65UbAAIHeHRdQ-w0m4ezZxEQEi0ptafXN2M99VRik39pf49PAc8NbK93Pxp3uaSBQkAf8oEnzWXG8Sk%24&data=05%7C02%7Cemailedpressreleases%40N0151C.onmicrosoft.com%7C946952dfee5b4b66a72708dbf9dfeb54%7C887bf9ee3c824b88t](https://nam111.safelinks.protection.outlook.com/?url=https%3A/instagram.com/novartis%3Ffigshid%3DMzRIODBiNWFIZA%3D%3D__%3B%21%21N3hqHg43uw%21pjp8z253J5NjaOYrW65UbAAIHeHRdQ-w0m4ezZxEQEi0ptafXN2M99VRik39pf49PAc8NbK93Pxp3uaSBQkAf8oEnzWXG8Sk%24&data=05%7C02%7Cemailedpressreleases%40N0151C.onmicrosoft.com%7C946952dfee5b4b66a72708dbf9dfeb54%7C887bf9ee3c824b88t)
12. <https://www.novartis.com/news/media-releases/novartis-receives-fda-approval-fabhalta-iptacopan-offering-superior-hemoglobin-improvement-absence-transfusions-first-oral-monotherapy-adults-pnh>
13. <https://clinicaltrials.gov/ct2/show/NCT04558918>
14. <https://clinicaltrials.gov/ct2/show/NCT04817618>
15. <https://clinicaltrials.gov/ct2/show/NCT04578834>
16. <https://www.novartis.com/news/media-releases/novartis-announces-european-medicines-agency-ema-has-granted-orphan-drug-designation-iptacopan-lnp023-iga-nephropathy-igan>
17. <https://www.novartis.com/news/media-releases/novartis-received-european-medicines-agency-ema-prime-designation-iptacopan-lnp-c3-glomerulopathy-c3g>
18. <https://www.novartis.com/news/media-releases/novartis-investigational-oral-therapy-iptacopan-lnp023-receives-fda-breakthrough-therapy-designation-pnh-and-rare-pediatric-disease-designation-c3g>
19. <mailto:media.relations@novartis.com>
20. <mailto:investor.relations@novartis.com>