Novartis receives Health Canada approval for Fabhalta® oral treatment for adult patients with PNH

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- *Fabhalta® is the first oral monotherapy for previously treated and treatment-naïve adults with paroxysmal nocturnal hemoglobinuria (PNH)*[1,2]
- *Significant unmet need remains in PNH, a chronic, rare and potentially life-threatening blood disorder; a large proportion of patients can remain anemic and dependent on blood transfusions*[3,4]

MONTREAL, Feb. 13, 2025 /CNW/ - Novartis Canada is pleased to announce that Health Canada has granted a Notice of Compliance for Fabhalta® (iptacopan capsules) as the first oral monotherapy treatment for adult patients with paroxysmal nocturnal hemoglobinuria (PNH) who have hemolytic anemia.[1] In clinical trials, treatment with Fabhalta® offered superior hemoglobin improvement in the absence of red blood cell (RBC) transfusions.[1,5,6,7,8]

Complement Component C5 is a gene that encodes a component of the complement system, a part of the body's immune system that helps defend against pathogens and inflammation, and maintain homeostasis.[9] Existing C5 inhibitor treatments, administered as infusions, may leave PNH symptoms uncontrolled.[3,4] In fact, patients on anti-C5 treatment have reported persistent anemia, with some patients requiring blood transfusions at least once per year.[3,4] Fabhalta® is the only Factor B inhibitor – a small-molecule therapy that treats diseases caused by dysregulation of the alternative complement pathway – providing comprehensive control of RBC destruction within and outside the blood vessels (intra- and extravascular hemolysis [IVH and EVH]) approved in Canada.

"When I was diagnosed with PNH in 2003, very few doctors were aware of PNH, let alone knowledgeable about how to treat it. The availability of a new treatment was game-changing for me – my energy came back and I started to live again," said Barry Katsof, president, Canadian Association of PNH Patients. "Health Canada's approval of iptacopan now provides PNH patients options to assure that they get optimal treatment benefit."

The Health Canada approval is based on the Phase III APPLY-PNH trial in patients with residual anemia despite prior anti-C5 treatment who switched to Fabhalta®[1,5] and supported by the Phase III APPOINT-PNH study in complement inhibitor-naïve patients.[1,6]

"A safe and effective oral option[5,6,10,11] to treat PNH could be practice-changing and can relieve the treatment burden experienced by people with PNH receiving parenteral therapies," said Dr. Christopher Patriquin, Assistant Professor of Medicine and Clinician Investigator, University of Toronto. "In treatment-experienced patients, iptacopan was superior to C5 inhibition with respect to hemoglobin improvements and achievement of transfusion avoidance. It was also shown effective in treatment-naive patients, providing meaningful increases in hemoglobin without the need for transfusions."[5,10]

"We are proud that Fabhalta® has received approval to bring a new oral medicine to Canadians living with PNH, a chronic and life-altering blood disease," said Mark Vineis, Country President, Novartis Canada. "This new treatment option provides renewed hope for patients, their loved ones and the healthcare providers who care for them."

The clinical effectiveness, safety and cost-effectiveness of Fabhalta® is currently under review by Canada's Drug Agency (CDA) and Institut National d'Excellence en Santé et Services Sociaux (INESSS). We look forward to communicating their recommendations with the PNH community, when available.

About paroxysmal nocturnal hemoglobinuria (PNH)

PNH is a rare, chronic and potentially life-threatening complement-mediated blood disorder[2] (a disease that occurs when the complement system is activated inappropriately or in excess). 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.[2,4] 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) and other debilitating symptoms in varying combinations and levels of severity.[2-4]

It is estimated that approximately 10-20 people per million worldwide live with PNH.[2] Although PNH can develop at any age, it is often diagnosed in people between 30-40 years old.[12,13]

About Fabhalta® (iptacopan capsules)

Fabhalta® is an oral, Factor B inhibitor of the alternative complement pathway.[14,15,16] Fabhalta® is indicated as monotherapy in the treatment of adult patients with paroxysmal nocturnal hemoglobinuria (PNH) who have hemolytic anemia.

Fabhalta® is only available through a controlled distribution program under which prescribers must enroll patients and confirm vaccination against encapsulated bacteria.[1]

About Novartis

Novartis is a focused 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.

In Canada, Novartis Pharmaceuticals Canada Inc. employs approximately 600 people to serve the evolving needs of patients and the healthcare system and invests over $30 million in R&D yearly in the country. For more information visit **www.novartis.ca**.

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