NICE recommends Novartis Jakavi® (ruxolitinib) for patients living with polycythaemia vera (PV)

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- *Ruxolitinib, a JAK 1/2 inhibitor, has received final draft guidance from the National Institute for Health and Care Excellence (NICE) for eligible adult patients in England and Wales with PV resistant to or intolerant of hydroxycarbamide/ hydroxyurea (HC/HU).*[1,2]
- *Ruxolitinib will help address the unmet treatment need for eligible patients, as approximately 24% of patients treated with hydroxycarbamide/hydroxyurea (HC/HU) will develop resistance or intolerance.*[3]
- *PV, a rare and incurable blood cancer,* is associated *with an overproduction of blood cells that can lead to stroke or heart attack if left untreated.*[4,5] *An estimated 1,130 people are newly diagnosed with PV in the UK every year.*[6]

London, 14 September 2023 — Novartis is pleased to announce that eligible patients in England and Wales will soon have access to Jakavi® (ruxolitinib), a JAK 1/2 inhibitor authorised to treat polycythaemia vera (PV). The news comes as the National Institute for Health and Care Excellence (NICE) published its Final Draft Guidance (FDG) recommending ruxolitinib for eligible adults in England and Wales with PV that are resistant to or intolerant of the chemotherapy treatment hydroxycarbamide/hydroxyurea (HC/HU).[2]

Common treatment approaches for PV currently include blood withdrawal (venesection), and HC/HU.[7,8,9] However, approximately 24% of PV patients treated with HC/HU will develop resistance or intolerance, resulting in inadequate disease control and an increased risk of progression.[3]

“There is a significant unmet need for people with polycythaemia vera in England and Wales, who live with a large symptom burden as a result of their condition,’’ said Dr. Claire Harrison, Consultant Haematologist, Guy's and St Thomas' NHS Foundation Trust, London. “Today’s decision is a step in the right direction for providing additional treatment options that reduce the burden of these symptoms and improve disease progression, in this under-represented patient population.”

In PV, the overproduction of red blood cells causes the blood to thicken which can cause stroke or heart attack.[4,10] Other symptoms include headaches, fatigue, weakness, dizziness or itchy skin.[5,11] Uncontrolled PC usually involves haematocrit levels (proportion of red blood cells in the blood) greater than 45%, elevated white blood cell count and/or platelet count which may be accompanied by debillitating symptoms and/or an enlarged spleen.[3,9,12]

Findings from the Novartis international MPN LANDMARK survey, of patients with rare blood cancers including PV, demonstrated that 72% of PV patients who experienced symptoms suffered a reduction in quality of life.[13] In addition to physical symptoms, approximately one-third of PV patients in the study felt anxious or worried about their disease.[13]

Jon Mathias, Co-Chair of MPN Voice, a charity that supports and advocates on behalf of PV patients, said "We welcome this recommendation from NICE, as polycythaemia vera can be an extremely debilitating illness that has a significant impact on patients’ lives in terms of day-to-day symptoms. It affects not only patients but also

their families and carers and turns many everyday tasks into major hurdles. Ruxolitinib addresses a significant unmet need in patients who cannot tolerate or no longer respond to HC/HU."

"Today's NICE recommendation in PV is an example of how we are reimagining medicine to transform the treatment of people with various blood cancers. The availability of ruxolitinib for eligible patients with PV in England and Wales will give them and their healthcare professionals more options in the management of this debilitating condition," said Marie-Andree Gamache, President & Managing Director of Novartis UK and Ireland.

About polycythaemia vera

PV is a rare and incurable blood cancer associated with an overproduction of blood cells in the bone marrow, and an estimated 1,130 people are newly diagnosed with PV in the UK every year.[4,5,6] The disease is driven by the dysregulation of the JAK-STAT pathway.[14] It is typically characterised by elevated haematocrit, the volume percentage of red blood cells in whole blood, which can lead to a thickening of the blood and an increased risk of blood clots, as well as an elevated white blood cell and platelet count.[4,10] This can cause serious cardiovascular complications, such as stroke and heart attack, resulting in increased morbidity and mortality.[4,7] Additionally, patients with PV may have an enlarged spleen and symptoms that are frequent and burdensome, with an overall impact on quality of life similar to that seen with myelofibrosis.[12,13,15]

A common PV treatment includes venesection, a procedure to remove blood from the body to reduce the concentration of red blood cells, which is used to help maintain a haematocrit level below 45%.[7,8,9] Cytoreductive agents, such as HC/HU, may be used.[7,8,9] Unfortunately, it is estimated that approximately 24% of patients treated with HC/HU will develop resistance or intolerance, resulting in inadequate disease control and an increased risk of progression.[3]

About Ruxolitinib

Ruxolitinib is an oral inhibitor of the JAK 1 and JAK 2 tyrosine kinases. Ruxolitinib is approved in the United Kingdom for the treatment of adult patients with polycythaemia vera (PV) who are resistant to or intolerant of HC/HU and for the treatment of disease-related splenomegaly or symptoms in adult patients with primary myelofibrosis (also known as chronic idiopathic myelofibrosis), post-polycythaemia vera myelofibrosis or post-essential thrombocythemia myelofibrosis.

About Novartis

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In the UK, we employ approximately 1,300 people to serve healthcare needs across the whole of the UK, as well as supporting the global operations of Novartis. Since 2014, Novartis has invested over £200 million in R&D and is a leading sponsor of clinical trials, in the UK. For more information, please visit www.novartis.com/uk-en/

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