Ojjaara (momelotinib) approved in the US as the first and only treatment indicated for myelofibrosis patients with anaemia

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For media and investors only

- Approval is for use in myelofibrosis patients with anaemia regardless of prior myelofibrosis therapy

- Nearly all myelofibrosis patients are estimated to develop anaemia over the course of the disease, and over 30% will discontinue treatment due to anaemia,,

- Ojjaara addresses key manifestations of myelofibrosis, namely anaemia, constitutional symptoms and splenomegaly

GSK plc (LSE/NYSE: GSK) today announced that the US Food and Drug Administration (FDA) has approved Ojjaara (momelotinib) for the treatment of intermediate or high-risk myelofibrosis, including primary myelofibrosis or secondary myelofibrosis (post-polycythaemia vera and post-essential thrombocythaemia), in adults with anaemia. Ojjaara is a once-a-day, oral JAK1/JAK2 and activin A receptor type 1 (ACVR1) inhibitor. To date, it is the only approved medicine for both newly diagnosed and previously treated myelofibrosis patients with anaemia that addresses the key manifestations of the disease, namely anaemia, constitutional symptoms, and splenomegaly (enlarged spleen).

Nina Mojas, Senior Vice President, Oncology Global Product Strategy, GSK, said:

> The vast majority of myelofibrosis patients eventually develop anaemia, causing them to discontinue treatments and require

> transfusions. Given this high unmet need, we are proud to add Ojjaara to our oncology portfolio and address a significant medical need in the community. We look forward to helping improve outcomes in this difficult-to-treat blood cancer.

Myelofibrosis is a blood cancer affecting approximately 25,000 patients in the US.,, Myelofibrosis can lead to severely low blood counts, including anaemia and thrombocytopaenia; constitutional symptoms such as fatigue, night sweats, and bone pain; and splenomegaly. About 40% of patients have moderate to severe anaemia at the time of diagnosis, and nearly all patients are estimated to develop anaemia over the course of the disease.,,, Physicians have had limited treatment options to treat myelofibrosis patients with anaemia. These patients often require transfusions and more than 30% will discontinue treatment due to anaemia. Patients who are transfusion dependent have a poor prognosis and shortened survival.,,,,,,,,

Ruben A. Mesa, MD, FACP, President and Executive Director, Atrium Health Levine Cancer Center and Atrium Health Wake Forest Baptist Comprehensive Cancer Center, said:

> With momelotinib we have the potential to establish a new standard of care for myelofibrosis patients with anaemia. Addressing key manifestations of myelofibrosis, including anaemia, constitutional symptoms and splenomegaly, makes a significant difference in the treatment regimen for these patients who have limited options to address these aspects of the disease.

The FDA approval of momelotinib is supported by data from the pivotal MOMENTUM study and a subpopulation of adult patients with anaemia from the SIMPLIFY-1 phase III trial. MOMENTUM was designed to evaluate the safety and efficacy of momelotinib versus danazol for the treatment and reduction of key manifestations of myelofibrosis in an anaemic, symptomatic, JAK inhibitor-experienced population. The MOMENTUM trial met all its primary and key secondary endpoints, demonstrating statistically significant response with respect to constitutional symptoms, splenic response and transfusion independence, in patients treated with momelotinib versus danazol. SIMPLIFY-1 was designed to evaluate the efficacy and safety of momelotinib versus ruxolitinib in myelofibrosis patients who had not

received a prior JAK-inhibitor therapy. Safety and efficacy results for SIMPLIFY-1 were based upon a subset of patients with anaemia.

In these clinical trials, the most common adverse reactions were thrombocytopaenia, haemorrhage, bacterial infection, fatigue, dizziness, diarrhoea, and nausea.

Kapila Viges, Chief Executive Officer, MPN (Myeloproliferative Neoplasms) Research Foundation, said:

> We are thrilled to see momelotinib reach the clinic, giving patients and their physicians another option to help manage myelofibrosis. Any new treatment that takes steps toward unlocking the mysteries of this complex and chronic blood cancer represents great progress for the field.

Momelotinib is currently not approved in any other market.

About Ojjaara (momelotinib)

Ojjaara has a differentiated mechanism of action, with inhibitory ability along three key signalling pathways: Janus kinase (JAK) 1, JAK2, and activin A receptor, type I (ACVR1).,, , Inhibition of JAK1 and JAK2 may improve constitutional symptoms and splenomegaly., Additionally, inhibition of ACVR1 leads to a decrease in circulating hepcidin, which is elevated in myelofibrosis and contributes to anaemia.,,,

Please see accompanying US Prescribing Information (PDF - 618KB).

Myelofibrosis is a rare blood cancer that results from dysregulated JAK-signal transducer and activator of transcription protein signalling and is characterised by constitutional symptoms, splenomegaly, and progressive anaemia. Myelofibrosis affects approximately 25,000 patients in the US.,,

About the pivotal MOMENTUM clinical trial

MOMENTUM was a phase III, global, multicentre, randomised, double-blind study investigating momelotinib versus danazol in patients with myelofibrosis who were symptomatic and anaemic and had been previously treated with an approved JAK inhibitor. The trial was designed to evaluate the safety and efficacy of momelotinib for treating and reducing key hallmarks of the disease: symptoms, blood

transfusions (due to anaemia) and splenomegaly. Results from the 24-week treatment period were presented at the 2022 American Society of Clinical Oncology (ASCO) Annual Meeting and subsequently published in The Lancet.,

About the SIMPLIFY-1 clinical trial

SIMPLIFY-1 was a multicentre, randomised, double-blind, phase III study that compared the safety and efficacy of momelotinib to ruxolitinib in patients with myelofibrosis who had not received prior treatment with a JAK inhibitor. Safety and efficacy results for SIMPLIFY-1 were based upon a subset of patients with anaemia (haemoglobin

GSK is committed to maximising patient survival through transformational medicines, with a current focus on breakthroughs in immuno-oncology and tumour-cell targeting therapies, and development in haematologic malignancies, gynaecologic cancers and other solid tumours.

GSK is a global biopharma company with a purpose to unite science, technology, and talent to get ahead of disease together. Find out more at gsk.com.

Cautionary statement regarding forward-looking statements

GSK cautions investors that any forward-looking statements or projections made by GSK, including those made in this announcement, are subject to risks and uncertainties that may cause actual results to differ materially from those projected. Such factors include, but are not limited to, those described under Item 3.D 'Risk factors" in the company's Annual Report on Form 20-F for 2022, and Q2 Results for 2023 and any impacts of the COVID-19 pandemic.

Naymagon, L, Mascarenhas, J. Myelofibrosis-Related Anemia: Current and Emerging Therapeutic Strategies. HemaSphere. 2017;1(1):e1.

Verstovsek S, et al. MOMENTUM: momelotinib vs danazol in patients with myelofibrosis previously treated with JAKi who are symptomatic and anemic. Future Oncol. 2021;17(12):1449-1458.

Kuykendall AT, Shah S, Talati C, et al. Between a rux and a hard place: evaluating salvage treatment and outcomes in myelofibrosis

after ruxolitinib discontinuation. Ann Hematol. 2018;97(3):435-441.

Harrison C, et al. Presented at: European Hematology Association; June 2022. Poster EP1113.

Data on file. Sierra Oncology. 2021.

Chifotides, HT, Bose, P, Verstovsek, S. Momelotinib: an emerging treatment for myelofibrosis patients with anemia. J Hematol Oncol. 2022;15(7):1-18.

Tefferi A, Lasho TL, Jimma T, et al. One thousand patients with primary myelofibrosis: the mayo clinic experience. Mayo Clin Proc. 2012;87(1):25-33. doi:10.1016/j.mayocp.2011.11.001

Bose P, et al. Curr Hematol Malign Rep. 2018;13:164-172. doi: https://doi.org/10.3109/10428194.2013.813500

Scherber, R.M., Mesa, R. Management of challenging myelofibrosis after JAK inhibitor failure and/or progression. Blood Rev. 2020;42:100716. https://doi.org/10.1016/j.blre.2020.100716

Bassiony S, Harrison CN, McLornan DP. Evaluating the Safety, Efficacy, and Therapeutic Potential of Momelotinib in the Treatment of Intermediate/High-Risk Myelofibrosis: Evidence to Date. Ther Clin Risk Manag. 2020;16:889-901. Published 2020 Sep 25. doi:10.2147/TCRM.S258704

Tefferi A, et al. Use of the Functional Assessment of Cancer Therapy--anemia in persons with myeloproliferative neoplasm-associated myelofibrosis and anemia. Clin Ther. 2014;36(4):560-566. https://doi.org/10.1016/j.clinthera.2014.02.016

Tefferi A. Primary myelofibrosis: 2021 update on diagnosis, risk-stratification and management. Am J Hematol. 2021;96(1):145-162. https://doi.org/10.1002/ajh.26050

Rumi E, et al. The Genetic Basis of Primary Myelofibrosis and Its Clinical Relevance. Int J Mol Sci. 2020;21(23):8885. https://doi.org/10.3390/ijms21238885

How J, Hobbs GS. A Practical Guide for Using Myelofibrosis Prognostic Models in the Clinic. J Natl Compr Canc Netw. 2020;18(9):1271-1278. https://doi.org/10.6004/jnccn.2020.7557

QxMD. DIPSS prognosis in myelofibrosis. Accessed September 12, 2022. https://qxmd.com/calculate/calculator_187/dipss-prognosis-in-m....

QxMD. DIPSS plus score for prognosis of myelofibrosis. Accessed September 12, 2022.

Nicolosi M, et al. Sex and degree of severity influence the prognostic impact of anemia in primary myelofibrosis: analysis based on 1109 consecutive patients. Leukemia. 2018;32(5):1254-1258. https://doi.org/10.1038/s41375-018-0028-x

Elena C, et al. Red blood cell transfusion-dependency implies a poor survival in primary myelofibrosis irrespective of IPSS and DIPSS. Haematologica. 2011;96(1):167-170. https://doi.org/10.3324/haematol.2010.031831

Mesa RA, Kiladjian JJ, Catalano JV, et al. SIMPLIFY-1: A Phase III Randomized Trial of Momelotinib Versus Ruxolitinib in Janus Kinase Inhibitor-Naïve Patients With Myelofibrosis. J Clin Oncol. 2017;35(34):3844-3850.

Asshoff M, et al. Momelotinib inhibits ACVR1/ALK2, decreases hepcidin production, and ameliorates anemia of chronic disease in rodents. Blood. 2017;129(13):1823-1830.

Oh S, et al. ACVR1/JAK1/JAK2 inhibitor momelotinib reverses transfusion dependency and suppresses hepcidin in myelofibrosis phase 2 trial. Blood Adv. 2020;4(18):4282-4291.

Mesa R, et al. Presented at: American Society of Clinical Oncology; June 2022. Abstract 7002.

Verstovsek S, et al. Momelotinib versus danazol in symptomatic patients with anaemia and myelofibrosis (MOMENTUM): results from an international, double-blind, randomised, controlled, phase 3 study. The Lancet. 2023;401(10373):269-280.

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