Stock exchange announcement
For media and investors only

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# GSK's momelotinib granted Orphan Drug Designations in the US and EU for VEXAS syndrome

- VEXAS syndrome is a rare, life-threatening hemato-inflammatory condition with no approved treatments
- ATLAS phase II/III trial in VEXAS underway, advancing momelotinib's broader development program
- Designations support development efforts and regulatory evaluations for medicines with potential to treat or prevent rare disorders

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GSK plc (LSE/NYSE: GSK) today announced that momelotinib, a JAK inhibitor with a differentiated mechanism of action, has received Orphan Drug Designation (ODD) from the US Food and Drug Administration (FDA) and European Medicines Agency (EMA) for the treatment of VEXAS (Vacuoles, E1 enzyme, X-linked, Autoinflammatory, Somatic) syndrome. VEXAS syndrome is a clonal myeloid disorder with rheumatologic and hematologic clinical features. It is a highly symptomatic progressive condition with poor prognosis and a 30-40% five-year mortality rate.[1] There are currently no approved treatment options.

The ODDs were supported by retrospective case studies demonstrating that JAK inhibitors may be an effective therapeutic option for VEXAS syndrome as well as evidence from a case report that indicated potential clinical benefit from treatment with momelotinib, including improvements in symptoms and VEXAS-related inflammation and hematological manifestations.[2] ODDs are granted by regulators to support the development efforts and regulatory evaluations of new medicines that have the potential to treat or prevent rare disorders.

The planned phase II/III ATLAS trial will evaluate momelotinib's efficacy and safety in VEXAS syndrome and will support planned global regulatory submissions.[3] The study design will be presented at the 2026 European Hematology Association (EHA) Congress taking place June 11-14. The trial is part of momelotinib's ongoing development program evaluating its potential across multiple hematological conditions.

Momelotinib (*Ojjaara/Omjjara*) is currently approved in the US for the treatment of intermediate- or high-risk myelofibrosis in adults with anemia. It is also approved in the EU and UK for the treatment of myelofibrosis with disease-related splenomegaly or symptoms in adults with moderate to severe anemia, and in Japan for the treatment of myelofibrosis.

**About momelotinib**

Momelotinib 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).[4,5,6,7] Inhibition of JAK1 and JAK2 may improve constitutional symptoms and splenomegaly.[4,5,7] Additionally, inhibition of ACVR1 leads to a decrease in circulating hepcidin levels, potentially contributing to anemia-related benefit.[4,5,6,7]

**About VEXAS syndrome**

VEXAS syndrome is a recently classified clonal myeloid disorder with rheumatologic and hematologic clinical features. It is a highly symptomatic, severe, progressive condition with a poor prognosis and a 5-year mortality rate of 30-40%.[1] The syndrome is characterized by a broad spectrum of inflammatory manifestations such as prolonged fever, weight loss, uveitis, relapsing chondritis, neutrophilic dermatosis, vasculitis and lung involvement.[8,9,10,11,12,13] Additionally, patients often present with hematologic complications, including macrocytic anemia, thrombocytopenia

and progressive bone marrow failure, which can evolve to hematologic malignancy.[8] Diagnosis is confirmed by genetic testing for the *UBA1* gene mutation.[14] As *UBA1* is located on chromosome X and the mutation is somatic, VEXAS syndrome predominantly affects men aged over 50 years. There are currently no approved treatments for VEXAS syndrome.[14]

**INDICATION AND IMPORTANT SAFETY INFORMATION for OJJAARA (momelotinib)**

**INDICATION**
OJJAARA is indicated for the treatment of intermediate or high-risk myelofibrosis (MF), including primary MF or secondary MF [post-polycythemia vera (PV) and post-essential thrombocythemia (ET)], in adults with anemia.

**IMPORTANT SAFETY INFORMATION**
**Risk of Infections**

- Serious (including fatal) infections (e.g., bacterial and viral, including COVID-19) occurred in 13% of patients treated with OJJAARA. Infections regardless of grade occurred in 38% of patients. Delay starting therapy until active infections have resolved. Monitor patients for signs and symptoms of infection and initiate appropriate treatment promptly.

Hepatitis B Reactivation

- Hepatitis B viral load (HBV-DNA titer) increases, with or without associated elevations in alanine transaminase (ALT) or aspartate transaminase (AST), have been reported in patients with chronic hepatitis B virus (HBV) infection taking Janus Kinase (JAK) inhibitors, including OJJAARA. The effect of OJJAARA on viral replication in patients with chronic HBV infection is unknown. In patients with HBV infections, check hepatitis B serologies prior to starting OJJAARA. If HBsAg and/or anti-HBc antibody is positive, consider consultation with a hepatologist regarding monitoring for reactivation versus prophylactic hepatitis B therapy. Patients with chronic HBV infection who receive OJJAARA should have their chronic HBV infection treated and monitored according to clinical HBV guidelines.

**Thrombocytopenia and Neutropenia**

- New or worsening thrombocytopenia, with platelet count less than 50 × $10^9$/L, was observed in 20% of patients treated with OJJAARA. Eight percent of patients had baseline platelet counts less than 50 × $10^9$/L.
- Severe neutropenia, absolute neutrophil count (ANC) less than 0.5 × $10^9$/L, was observed in 2% of patients treated with OJJAARA.
- Assess complete blood counts (CBC), including platelet and neutrophil counts, before initiating treatment and periodically during treatment as clinically indicated. Interrupt dosing or reduce the dose for thrombocytopenia or neutropenia.

**Hepatotoxicity**

- Two of the 993 patients with MF who received at least one dose of OJJAARA in clinical trials experienced reversible drug-induced liver injury. Overall, new or worsening elevations of ALT and AST (all grades) occurred in 23% and 24%, respectively, of patients treated with OJJAARA; Grade 3 and 4 transaminase elevations occurred in 1% and 0.5% of patients, respectively. New or worsening elevations of total bilirubin occurred in 16% of patients treated with OJJAARA. All total bilirubin elevations were Grades 1-2. The median time to onset of any grade transaminase elevation was 2 months, with 75% of cases occurring within 4 months.
- Delay starting therapy in patients presenting with uncontrolled acute and chronic liver disease until apparent causes have been investigated and treated as clinically indicated. When initiating OJJAARA, refer to dosing in patients with hepatic impairment.
- Monitor liver tests at baseline, every month for 6 months during treatment, then periodically as clinically indicated. If increases in ALT, AST or bilirubin related to treatment are suspected, modify OJJAARA dosage based upon Table 1 within the Prescribing Information.

**Severe Cutaneous Adverse Reactions (SCARs)**

- Severe cutaneous adverse reactions (SCARs), including toxic epidermal necrolysis (TEN), have been observed in some patients treated with OJJAARA.
- If signs or symptoms of SCARs occur, interrupt OJJAARA until the etiology of the reaction has been determined. Consider early consultation with a dermatologist for evaluation and management.
- If etiology is considered to be associated with OJJAARA, permanently discontinue OJJAARA and do not reintroduce OJJAARA in patients who have experienced SCARs or other life-threatening cutaneous reactions during treatment with OJJAARA.

**Major Adverse Cardiovascular Events (MACE)**

- Another JAK inhibitor increased the risk of MACE, including cardiovascular death, myocardial infarction, and stroke [compared with those treated with tumor necrosis factor (TNF) blockers] in patients with rheumatoid arthritis, a condition for which OJJAARA is not indicated.
- Consider the benefits and risks for the individual patient prior to initiating or continuing therapy with OJJAARA, particularly in patients who are current or past smokers and patients with other cardiovascular risk factors. Inform patients receiving OJJAARA of the symptoms of serious cardiovascular events and the steps to take if they occur.

**Thrombosis**

- Another JAK inhibitor increased the risk of thrombosis, including deep venous thrombosis, pulmonary embolism, and arterial thrombosis (compared with those treated with TNF blockers) in patients with rheumatoid arthritis, a condition for which OJJAARA is not indicated. Evaluate patients with symptoms of thrombosis and treat appropriately.

**Malignancies**

- Another JAK inhibitor increased the risk of lymphoma and other malignancies excluding nonmelanoma skin cancer (NMSC) (compared with those treated with TNF blockers) in patients with rheumatoid arthritis, a condition for which OJJAARA is not indicated. Current or past smokers were at increased risk.
- Consider the benefits and risks for the individual patient prior to initiating or continuing therapy with OJJAARA, particularly in patients with a known malignancy (other than a successfully treated NMSC), patients who develop a malignancy, and patients who are current or past smokers.

**Symptom Exacerbation Following Interruption or Discontinuation of Treatment**

- Following discontinuation of JAK inhibitors, including OJJAARA, signs and symptoms from myeloproliferative neoplasms may flare. Some patients with MF have experienced one or more of the following after discontinuing JAK inhibitors: fever, respiratory distress, hypotension, disseminated intravascular coagulation, or multi-organ failure.
- If one or more of these signs and symptoms occur after discontinuation of OJJAARA, evaluate for and treat any intercurrent illness and consider restarting OJJAARA. Instruct patients not to interrupt or discontinue therapy without consulting their healthcare provider. When discontinuing or interrupting therapy for reasons other than potentially life-threatening toxicities, consider tapering the dose of OJJAARA gradually rather than discontinuing abruptly.

**Adverse Reactions**

- The most common adverse reactions (≥20% in either study) are thrombocytopenia, hemorrhage, bacterial infection, fatigue, dizziness, diarrhea, and nausea.

**Organic Anion Transporting Polypeptide (OATP)1B1/B3 Inhibitors**

- Momelotinib is an OATP1B1/B3 substrate. Concomitant use with an OATP1B1/B3 inhibitor increases momelotinib maximal concentrations ($C_{max}$) and area under the concentration-time curve (AUC), which may increase the risk of adverse reactions with OJJAARA. Monitor patients concomitantly receiving an OATP1B1/B3 inhibitor for adverse reactions and consider OJJAARA dose modifications.

**Breast Cancer Resistance Protein (BCRP) Substrates**

- Momelotinib is a BCRP inhibitor. OJJAARA may increase exposure of BCRP substrates, which may increase the risk of BCRP substrate adverse reactions. When administered concomitantly with OJJAARA, initiate rosuvastatin (BCRP substrate) at 5 mg and do not increase to more than 10 mg once daily. Dose adjustment of other BCRP substrates may also be needed. Follow approved product information recommendations for other BCRP substrates.

**Pregnancy**

- Available data in pregnant women are insufficient. OJJAARA should only be used during pregnancy if the expected benefits to the mother outweigh the potential risks to the fetus.

**Lactation**

- It is not known whether OJJAARA is excreted in human milk. Because of the potential for serious adverse reactions in a breastfed child, patients should not breastfeed during treatment with OJJAARA, and for at least 1 week after the last dose of OJJAARA.

**Females and Males of Reproductive Potential**

- Advise females of reproductive potential who are not pregnant to use highly effective contraception during therapy and for at least 1 week after the last dose of OJJAARA.

**Hepatic Impairment**

- Momelotinib exposure increased with severe hepatic impairment (Child-Pugh C). The recommended starting dose of OJJAARA in patients with severe hepatic impairment (Child-Pugh C) is 150 mg orally once daily. No dose modification is recommended for patients with mild hepatic impairment (Child-Pugh A) or moderate hepatic impairment (Child-Pugh B).

To report SUSPECTED ADVERSE REACTIONS, contact GSK at gsk.public.reportum.com or 1-888-825-5249, or the FDA at 1-800-FDA-1088 or www.fda.gov/medwatch.

**Please see full Prescribing Information, including Patient Information, for OJJAARA.**

**About GSK**

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 www.us.gsk.com.

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**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 in the "Risk Factors" section in GSK's Annual Report on Form 20-F for 2025, and GSK's Q1 Results for 2026.

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