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 haemato-inflammatory condition with no approved treatments
- ATLAS phase II/III trial in VEXAS underway, advancing momelotinib's broader development programme
- 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 haematologic 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 haematological 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 11-14 June. The trial is part of momelotinib's ongoing development programme evaluating its potential across multiple haematological conditions.

Momelotinib (*Ojjaara*/*Omjjara*) is currently approved in the US for the treatment of intermediate- or high-risk myelofibrosis in adults with anaemia. 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 anaemia, 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 anaemia-related benefit.[4,5,6,7]

The EU product information is available at: OMJJARA-EPAR-PRODUCT-INFORMATION_EN.PDF

The US product information is available at: OJJAARA-PI-PIL.PDF

About VEXAS syndrome

VEXAS syndrome is a recently classified clonal myeloid disorder with rheumatologic and haematologic 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 characterised 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 haematologic complications, including macrocytic anaemia, thrombocytopenia and progressive bone marrow failure, which can evolve to haematologic 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]

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.gsk.com.

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