Status on US FDA Advisory Committee for roxadustat

16 July 2021 07:00 BST

Status on FDA Advisory Committee vote on

roxadustat in anaemia of chronic kidney disease

The Food and Drug Administration's (FDA) Cardiovascular and Renal Drugs Advisory Committee (CRDAC) has voted 13 to 1 that the benefit-risk profile of roxadustat does not support approval for the treatment of anaemia in chronic kidney disease (CKD) in non-dialysis dependent (NDD) adult patients, and 12 to 2 that the benefit-risk profile of roxadustat does not support approval for the treatment of anaemia in CKD in dialysis-dependent (DD) adult patients.

The FDA will consider the vote, independent opinions and recommendations from experts as it reviews the new drug application (NDA) and is not bound by the Committee's recommendation.

The safety and efficacy of roxadustat, an oral hypoxia-inducible factor prolyl hydroxylase (HIF-PH) inhibitor, have been demonstrated in the Phase III programme including more than 8,000 patients and published in five peer-reviewed journal articles.

Mene Pangalos, Executive Vice President, BioPharmaceuticals R&D, said: "New solutions are needed for the six million people in the US affected by anaemia of chronic kidney disease Although we are disappointed by today's outcome, we will continue to work closely with our partner FibroGen and the FDA to determine the path forward for roxadustat."

Earlier this year, the FDA confirmed it would convene a meeting of the CRDAC to review the NDA for roxadustat and requested further clarifying analyses of clinical data to support the assessment. The FDA has not announced when it will make its final decision for the roxadustat NDA.

The CRDAC provides the FDA with independent, expert advice and reviews and evaluates available data concerning the safety and efficacy of marketed and potential new medicines for use in the treatment of cardiovascular (CV) and renal disorders.[1]

Roxadustat is approved in a number of countries, including China, Japan, Chile and South Korea for the treatment of anaemia in CKD in NDD and DD adult patients. It is under regulatory review in other jurisdictions, including in the European Union, where it has recently received a positive CHMP opinion.

Anaemia

Anaemia can be a serious medical condition in which patients have insufficient RBCs and low levels of haemoglobin, a protein in RBCs that carries oxygen to cells throughout the body.[1] Anaemia of CKD frequently causes significant fatigue, cognitive dysfunction and decreased quality of life, and is associated with increased risk of hospitalisation, CV complications and death.[1,2] Severe anaemia is common in patients with CKD, cancer, myelodysplastic syndrome (MDS), inflammatory diseases and other serious illnesses. Anaemia is particularly prevalent in patients with CKD.[2,3,4] CKD affects 840 million patients worldwide and is generally progressive, characterised by gradual loss of kidney function that may eventually lead to kidney failure.[5,6]

Phase III programme

The Phase III programme included more than 8,000 patients and was conducted by AstraZeneca, FibroGen and Astellas Pharma Inc. (Astellas).
The OLYMPUS, ALPS and ANDES trials evaluated roxadustat compared to placebo in NDD-CKD patients. ROCKIES, SIERRAS and HIMALAYAS evaluated roxadustat compared to epoetin alfa in DD-CKD and incident dialysis (ID) patients. HIMALAYAS evaluated roxadustat compared to epoetin alfa in ID patients; ROCKIES and SIERRAS included ID and prevalent dialysis patients.

Roxadustat

Roxadustat, an oral medicine, could be the first in a new class of treatments called oral HIF-PH inhibitors that promotes erythropoiesis, or RBC production, through increased endogenous production of erythropoietin, improved iron absorption and mobilisation, and reduction of hepcidin. Roxadustat is also in clinical development for anaemia associated with MDS and for chemotherapy-induced anaemia.

Roxadustat is approved in China, Japan, Chile and South Korea (under the name *Evrenzo*), for the treatment of anaemia in CKD in NDD and DD adult patients. In Europe, the Marketing Authorisation Application for *Evrenzo* for the treatment of anaemia in CKD in NDD and DD patients was submitted by Astellas and accepted by the European Medicines Agency for review in May 2020 and is under final regulatory review following a positive EU CHMP opinion in June 2021.

AstraZeneca and FibroGen are collaborating on the development and commercialisation of roxadustat for the potential treatment of anaemia in the US, China and other countries in the Americas, Australia and New Zealand, as well as Southeast Asia. Astellas and FibroGen are collaborating on the development and commercialisation of roxadustat for the potential treatment of anaemia in Japan, Europe, Turkey, Russia and the Commonwealth of Independent States, the Middle East and South Africa.

AstraZeneca in CVRM

Cardiovascular, Renal and Metabolism (CVRM), part of BioPharmaceuticals, forms one of AstraZeneca's three disease areas and is a key growth driver for the Company. By following the science to understand more clearly the underlying links between the heart, kidneys and pancreas, AstraZeneca is investing in a portfolio of medicines for organ protection and improve outcomes by slowing disease progression, reducing risks and tackling co-morbidities. The Company's ambition is to modify or halt the natural course of CVRM diseases and potentially regenerate organs and restore function, by continuing to deliver transformative science that improves treatment practices and CV health for millions of patients worldwide.

AstraZeneca
AstraZeneca (LSE/STO/Nasdaq: AZN) is a global, science-led biopharmaceutical company that focuses on the discovery, development, and commercialisation of prescription medicines in Oncology and BioPharmaceuticals, including Cardiovascular, Renal & Metabolism, and Respiratory & Immunology. Based in Cambridge, UK, AstraZeneca operates in over 100 countries, and its innovative medicines are used by millions of patients worldwide. Please visit astrazeneca.com and follow the Company on Twitter @AstraZeneca.

Contacts
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