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Media and Public Relations

Press release

First treatment for rare disease characterised by high levels of triglycerides in blood

The European Medicines Agency has recommended granting a conditional marketing authorisation (CMA) for Waylivra (volanesorsen), the first medicine for the treatment of the familial chylomicronaemia syndrome (FCS).

FCS is a rare genetic disease that prevents the body from breaking down fats (lipids). Patients with this condition have extremely high levels of triglycerides in their blood. This causes a range of symptoms including for instance severe abdominal pain, potentially fatal attacks of acute pancreatitis, hepatosplenomegaly, diabetes, lack of concentration, memory loss and fat-filled spots on the skin (called xanthomas).

There is currently no authorised medicine available to treat this rare disease. Patients need to strictly limit their fat intake through diet, but this is not always feasible and sufficiently effective to reduce the level of triglycerides and prevent pancreatitis. Existing lipid-lowering medications are only minimally effective to reduce triglyceride levels in patients with FCS and there is an urgent unmet medical need for new treatments to help patients to manage this disease.

The benefits and safety of Waylivra were investigated in a phase III clinical study involving 66 patients with FCS. Data from this study showed that levels of triglycerides in the blood of patients treated with Waylivra decreased on average by 77% after three months' treatment, compared to an increase of 18% in the placebo-receiving control group. The observed substantial reduction in levels of triglycerides is expected to lead to a reduction in the incidence of potentially life-threatening pancreatitis. The most common side effects are reduced platelet counts and injection site reactions. A number of cases of severe platelet reduction were observed in the Waylivra trials, which may result in an increased risk of bleeding. To manage this risk, a number of additional risk minimisation measures will be implemented including strict dosing guidance based on regular platelet monitoring and specific information to patients and their carers on this potential risk. As part of the CMA, the applicant is also required to conduct a study that further investigates the safety and efficacy of the medicine and the feasibility of implemented risk minimisation measures.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 8427 **Facsimile** +44 (0)20 3660 5555
E-mail press@ema.europa.eu **Website** www.ema.europa.eu

CMA is one of the EU's regulatory mechanisms to facilitate early access to medicines that address an unmet medical need. Conditional approval allows the Agency to recommend a medicine for marketing authorisation in the interest of public health where the benefit to patients of its immediate availability outweighs the risk inherent in the fact that additional data are still required.

FCS is a rare disease which was granted an orphan designation in the EU in February 2014. At the time of orphan designation, it was considered that the condition affected less than 1 in 100,000 persons. As always at the time of approval, this orphan designation will now be reviewed by EMA's Committee for Orphan Medicinal Products (COMP) to determine whether the information available to date allows maintaining Waylivra's orphan status and granting this medicine ten years of market exclusivity.

The opinion adopted by the CHMP is an intermediary step on Waylivra's path to patient access. The opinion will now be sent to the European Commission for the adoption of a decision on an EU-wide marketing authorisation. Once the marketing authorisation has been granted, decisions about price and reimbursement will take place at the level of each Member State, taking into account the potential role/use of this medicine in the context of the national health system of that country.

Notes

1. This press release, together with all related documents, is available on the Agency's website.
2. The applicant for Waylivra is Akcea Therapeutics Ireland Ltd.
3. More information on the work of the European Medicines Agency can be found on its website: www.ema.europa.eu

Contact our press officers

Tel. +44 (0)20 3660 8427
E-mail: press@ema.europa.eu
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