Medicine: volanesorsen sodium (brand name: Waylivra®) for familial chylomicronaemia syndrome

Akcea Therapeutics UK Ltd

Volanesorsen sodium meets the SMC definition of an ultra-orphan medicine, which is a medicine to treat an extremely rare condition. This document summarises the initial SMC assessment of volanesorsen sodium.

**What does this mean for patients?**

From 16 November 2020, if your healthcare professional thinks that volanesorsen sodium is the right medicine for you, you should be able to have the treatment on the NHS in Scotland within the ultra-orphan pathway (see next page).

SMC will reassess the medicine and make a decision on routine availability after 3 years.

**What is volanesorsen sodium used for?**

Volanesorsen sodium is used to treat patients with familial chylomicronaemia syndrome (FCS). FCS is a rare genetic condition that leads to high levels of triglyceride fats in the blood. This leads to a build-up of fat in different parts of the body and causes abdominal pain and pancreatitis (inflammation of the pancreas which can be life threatening). People with FCS are also more likely to develop type 2 diabetes.

**How does volanesorsen sodium work?**

Volanesorsen blocks the production of a protein called apolipoprotein – CIII in the body. Apolipoprotein CIII slows the breakdown of fats. By blocking the formation of apolipoprotein CIII, volanesorsen helps to reduce the level of triglyceride fats in the blood.
How do we assess ultra-orphan medicines?

SMC uses a broad assessment framework for ultra-orphan medicines. This is part of the ultra-orphan pathway in NHSScotland which has **four stages**:

<table>
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<th>Stage</th>
<th>Description</th>
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<td>1 Validation</td>
<td>SMC reviews information from the company to decide if the ultra-orphan definition is met. The company must then agree to further requirements of the ultra-orphan pathway*.</td>
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| 2 Initial Assessment | SMC considers the:  
- nature of the condition  
- health benefits of the medicine  
- impact beyond direct health benefits and on specialist services  
- value for money, and  
- costs to the NHS. |
| 3 Evidence Generation | The medicine can be prescribed while the company gathers further data, including on the patient and carer lived experience. |
| 4 Reassessment | After 3 years the company provides an updated submission for reassessment. SMC considers all the evidence and makes a decision on routine use of the medicine in NHSScotland. |

* provide a confidential discount known as a Patient Access Scheme (PAS) to increase the cost-effectiveness of the medicine, and provide a data collection plan.

**What have we said in this assessment?**

- A study showed volanesorser reduces triglyceride levels. However, it can cause a reduced number of platelets in the blood, meaning that intense monitoring is needed. Long-term data on its safety and efficacy in treating this lifelong condition are limited.

- Volanesorser may bring improvements in quality of life but there are uncertainties over its benefits in reducing pancreatitis and the development of diabetes in the longer-term.

- Despite the PAS offered by the company, the cost in relation to the health benefits of volanesorser remains high.

For further information please see the SMC ultra-orphan medicine initial assessment report (SMC2299).

**More information**

The organisation below can provide more information and support for people with FCS and their families. SMC is not responsible for the content of any information provided by external organisations.

**Action FCS**

[http://www.actionfcs.org](http://www.actionfcs.org)  
07517 752168

You can find out more about volanesorser sodium (Waylivra®) in the European public assessment report (EPAR) summary for the public by searching for the medicine name on the European Medicines Agency (EMA) website.


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