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Aqneursa (*levacetylleucine*)

An overview of Aqneursa and why it is authorised in the EU

What is Aqneursa and what is it used for?

Aqneursa is a medicine used to treat neurological symptoms (symptoms affecting the brain and nerves) of Niemann-Pick type C (NPC) disease in adults and children aged 6 years and older who weigh at least 20 kg.

NPC is an inherited disease that affects the body's ability to transport fats inside cells. Over time, these fats build up in cells of the brain and other parts of the nervous system, causing the cells to stop working properly and eventually die. This leads to symptoms such as problems with balance, movement and swallowing, unclear speech and shaking that cannot be controlled.

Aqneursa is used in combination with miglustat, another medicine for treating neurological symptoms of NPC. It can also be used on its own if treatment with miglustat has caused side effects that makes it unsuitable for the patient.

NPC is rare, and Aqneursa was designated an 'orphan medicine' (a medicine used in rare diseases) on 20 March 2017. Further information on the orphan designation can be found on the EMA website.

Aqneursa contains the active substance levacetylleucine.

How is Aqneursa used?

Aqneursa can only be obtained with a prescription. The medicine is available as sachets containing granules that are mixed with water to make a suspension, which is to be taken by mouth 2 to 3 times a day. The suspension can also be given by a feeding tube.

The dose depends on the patient's body weight. For more information about using Aqneursa, see the package leaflet or contact your doctor or pharmacist.

How does Aqneursa work?

NPC is caused by changes (mutations) in genes that provide instructions for making proteins called NPC1 and NPC2. These proteins are normally involved in transporting fats from lysosomes (small sacs in cells that break down large molecules such as fats) to other parts of the cell. In people with NPC, NPC1 or NPC2 do not function properly, leading to a build-up of fat in lysosomes which is harmful to cells.

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The exact way the active substance in Aqneursa, levacetylleucine, works is not known. However, studies in animals suggest that it may help correct the cells' ability to generate energy. This includes the cells' ability to make adenosine triphosphate, the main source of energy for cells in the brain that control movement and balance.

What benefits of Aqneursa have been shown in studies?

In a main study involving 60 adults and children aged 6 years and older who weighed at least 20 kg, treatment with Aqneursa resulted in improvements in their movement, coordination and ability to carry out everyday tasks compared with placebo (a dummy treatment).

In the study, patients were given either Aqneursa or placebo for the first 12 weeks; 51 of the 60 participants were also receiving treatment with miglustat. After this period, the groups switched; patients treated with Aqneursa were given placebo, and those who had been taking placebo were given Aqneursa for another 12 weeks. The main measure of effectiveness was the change in the scale for the assessment and rating of ataxia (SARA) score. The SARA score measures how much a person's movement, coordination and ability to carry out everyday tasks are affected by ataxia (a condition that causes a loss of muscle coordination). The scale ranges from 0 (no ataxia) to 40 (severe ataxia); a decrease in a person's score means their symptoms are improving.

Overall, treatment with Aqneursa resulted in an average reduction of around 2 points in the SARA score, compared with a reduction of 0.6 with placebo. Additionally, those who were given Aqneursa and then switched to placebo experienced a worsening of their SARA score after discontinuing treatment with the medicine.

What are the risks associated with Aqneursa?

For the full list of side effects and restrictions with Aqneursa, see the package leaflet.

The most common side effect with Aqneursa (which may affect up to 1 in 10 people) is flatulence (gas).

Why is Aqneursa authorised in the EU?

NPC is a rare, life-threatening condition with limited treatment options. At the time of approval of Aqneursa, the only medicine authorised for the treatment of neurological symptoms of NPC was miglustat.

Despite some uncertainties in the main study, such as the relatively short treatment duration for a medicine intended for long-term use, Aqneursa resulted in improvements in neurological symptoms of NPC, including those affecting movement and coordination. Furthermore, those who stopped treatment had a significant worsening of their symptoms. At the time of approval, the only side effect associated with Aqneursa was flatulence.

The European Medicines Agency therefore decided that Aqneursa's benefits are greater than its risks and that it can be authorised for use in the EU.

What measures are being taken to ensure the safe and effective use of Aqneursa?

Recommendations and precautions to be followed by healthcare professionals and patients for the safe and effective use of Aqneursa have been included in the summary of product characteristics and the package leaflet.

As for all medicines, data on the use of Aqneursa are continuously monitored. Suspected side effects reported with Aqneursa are carefully evaluated and any necessary action taken to protect patients.

Other information about Aqneursa

Aqneursa received a marketing authorisation valid throughout the EU on 19 January 2026.

Further information on Aqneursa can be found on the Agency's website: ema.europa.eu/medicines/human/EPAR/aqneursa.

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