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Withdrawal of application for the marketing authorisation of Nugalviq (*govorestat*)

Advanz Pharma Limited withdrew its application for a marketing authorisation of Nugalviq for the treatment of classic galactosaemia, a condition where the body cannot break down a sugar called galactose.

The company withdrew the application on 10 December 2024.

What is Nugalviq and what was it intended to be used for?

Nugalviq was developed as a medicine for use in adults and children aged 2 years and above with a confirmed diagnosis of classic galactosemia (also known as galactose-1-phosphate uridylyltransferase deficiency). The medicine was to be used in addition to a galactose-restricted diet.

Nugalviq contains the active substance govorestat and was to be available as a liquid suspension to be taken by mouth.

Nugalviq was designated an 'orphan medicine' (a medicine used in rare diseases) on 21 June 2022 for galactosaemia. Further information on the orphan designation can be found on the Agency's website: ema.europa.eu/medicines/human/orphan-designations/eu3222642.

How does Nugalviq work?

Galactose is a type of sugar that is found in certain foods and is also made by the body. People with classic galactosaemia cannot break down galactose properly, causing it to build up in their body. An enzyme called aldose reductase converts this excess galactose to a substance called galactitol, which is harmful to the body and causes problems with development and damage to certain organs. The active substance in Nugalviq was expected to block the activity of aldose reductase, thereby reducing the formation of galactitol. Nugalviq was expected to improve the symptoms in people with classic galactosaemia.

What did the company present to support its application?

The main study submitted by the company included data from 47 children aged 2 years and older with classic galactosaemia. The study compared Nugalviq with placebo (a dummy treatment). The main

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measure of effectiveness was a combined measurement of the change in speech and language skills, behaviour and ability to carry out daily activities. In addition, the company submitted supportive data from a study involving 33 adults that measured a change in galactitol levels, as well as data on the long-term follow up of 7 adults.

How far into the evaluation was the application when it was withdrawn?

The application was withdrawn after the European Medicines Agency had evaluated the initial information from the company and had prepared questions for the company. The Agency was assessing the company's responses to the questions at the time of the withdrawal.

What did the Agency recommend at that time?

Based on the review of the data and the company's response to the Agency's questions, at the time of the withdrawal, the Agency had had some concerns and its provisional opinion was that Nugalviq could not have been authorised for the treatment of classic galactosaemia.

The Agency had a number of concerns about the results of the main study, as well as how the study was carried out and how the data had been collected and processed. The Agency further considered that the study could only be viewed as exploratory due to late changes to its design. There was also not enough information on how the medicine behaves in the body and how it would be affected by food, nor was its potential cancer risk adequately addressed. In addition, there were questions about the proposed dosing and about the quality of the medicine itself.

As the company was seeking a conditional marketing authorisation, the Agency noted that the requirements for such an authorisation were not met.

Therefore, at the time of the withdrawal, the Agency's opinion was that the results of the study were not reliable and concluded that the medicine could not have been authorised based on the data provided.

What were the reasons given by the company for withdrawing the application?

In its letter notifying the Agency of the withdrawal of the application, the company stated that it needed more time to collect further data to support the assessment of Nugalviq in the proposed indication.

Does this withdrawal affect patients in clinical trials or compassionate use programmes?

The company informed the Agency that there are no consequences for patients in clinical trials or in compassionate use programmes using govorestat.

If you are in a clinical trial or compassionate use programme and need more information about your treatment, speak with your clinical trial doctor.