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# Public summary of opinion on orphan designation

Calcium oxybate, magnesium oxybate, potassium oxybate, sodium oxybate for the treatment of idiopathic hypersomnia

On 6 January 2021, orphan designation EU/3/20/2386 was granted by the European Commission to Jazz Pharmaceuticals Ireland Limited, Ireland, for calcium oxybate, magnesium oxybate, potassium oxybate, sodium oxybate (also known as JZP-258) for the treatment of idiopathic hypersomnia.

## What is idiopathic hypersomnia?

Idiopathic hypersomnia is a sleep disorder characterised by excessive daytime sleepiness. Idiopathic means that the cause of the disease is unknown.

People with this condition struggle to stay awake during the day and feel the need to take frequent long naps. These may be prolonged or at inappropriate times, such as during a meal or a conversation, and generally don't provide any relief from the sleepiness. Most people with idiopathic hypersomnia also sleep for more than 10 hours a night and struggle to wake in the morning, because they feel very drowsy and confused upon waking.

Idiopathic hypersomnia is a long-term debilitating disease because the need for sleep during the day interferes with normal life.

## What is the estimated number of patients affected by the condition?

At the time of designation, idiopathic hypersomnia affected approximately 3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 156,000 people*, and is below the ceiling for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and the knowledge of the Committee for Orphan Medicinal Products (COMP).

* For the purpose of the designation, the number of patients affected by the condition is estimated and assessed on the basis of data from the European Union, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

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## What treatments are available?

At the time of designation, no satisfactory methods were authorised in the EU for the treatment of idiopathic hypersomnia. Patients with idiopathic hypersomnia usually received advice on lifestyle changes to help regulate their sleeping pattern.

## How is this medicine expected to work?

The active substance in this medicine is a mixture of oxybate salts. Oxybate (gamma-hydroxybutyrate) is a substance that occurs naturally in the brain. Although the precise way this medicine works in the treatment of idiopathic hypersomnia is not fully understood, it works on targets in the brain called GABA receptors allowing the build-up of substances such as dopamine and noradrenaline, that are needed for alertness and activity by day. When taken at night this is expected to affect the way sleep and wakefulness is regulated by the brain and, in people with idiopathic hypersomnia, result in reduced sleepiness in the daytime.

## What is the stage of development of this medicine?

The effects of calcium oxybate, magnesium oxybate, potassium oxybate, sodium oxybate have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with idiopathic hypersomnia were ongoing.

At the time of submission, this medicine had been authorised in the United States for the treatment of narcolepsy, another condition associated with disordered sleeping, and an oxybate salt, sodium oxybate, was authorised in the EU for that condition.

At the time of submission, calcium oxybate, magnesium oxybate, potassium oxybate, sodium oxybate was not authorised anywhere in the EU for the treatment of idiopathic hypersomnia. Orphan designation of the medicine had been granted in the United States for idiopathic hypersomnia.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 3 December 2020, recommending the granting of this designation.

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Opinions on orphan medicinal product designations are based on the following three criteria:

the seriousness of the condition;

the existence of alternative methods of diagnosis, prevention or treatment;

either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

Designated orphan medicinal products are products that are still under investigation and are considered for orphan designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of quality, safety and efficacy is necessary before a product can be granted a marketing authorisation.

## For more information

Contact details of the current sponsor for this orphan designation can be found on EMA website.

For contact details of patients' organisations whose activities are targeted at rare diseases see:

- Orphanet, a database containing information on rare diseases, which includes a directory of patients' organisations registered in Europe;
- European Organisation for Rare Diseases (EURORDIS), a non-governmental alliance of patient organisations and individuals active in the field of rare diseases.