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Public summary of opinion on orphan designation

Diazoxide choline for the treatment of Prader-Willi syndrome

On 12 October 2017, orphan designation (EU/3/17/1941) was granted by the European Commission to Capnia (UK) Ltd, United Kingdom, for diazoxide choline for the treatment of Prader-Willi syndrome.

In December 2017, Capnia (UK) Ltd changed name to Soleno Therapeutics UK Ltd.

What is Prader-Willi syndrome?

Prader-Willi syndrome is an inherited condition caused by defects in specific regions of chromosome 15. This causes a wide range of symptoms, some of which can appear at birth, such as feeding problems, small size and reduced muscle strength. During childhood further symptoms develop, including increased appetite leading to constant eating and severe obesity, short stature, incomplete sexual development, learning difficulties and behavioural problems, such as aggression and stubbornness.

Prader-Willi syndrome is a life-long debilitating and life-threatening disease because of its serious symptoms, particularly learning difficulties, behavioural problems and obesity.

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

At the time of designation, Prader-Willi syndrome affected approximately 0.3 in 10,000 people in the European Union (EU). This was equivalent to a total of around 15,000 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).

What treatments are available?

At the time of designation, growth hormone was authorised in the EU for treating Prader-Willi syndrome. In addition, symptoms were treated or managed in various ways, including supervised access to food to prevent obesity.

*Disclaimer: 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 (EU 28), Norway, Iceland and Liechtenstein. This represents a population of 515,700,000 (Eurostat 2017).

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The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with Prader-Willi syndrome, with data showing that it may reduce the excessive food intake – benefit not seen with current therapy. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

The way diazoxide choline works in Prader-Willi syndrome is unclear, but it is thought to stimulate potassium channels in the brain, which in turn reduces production of a substance called neuropeptide Y that nerve cells use to communicate with neighbouring cells. Neuropeptide Y is responsible for stimulating appetite. By reducing its production, diazoxide choline is expected to reduce the excessive food intake seen in patients with Prader-Willi syndrome.

What is the stage of development of this medicine?

The effects of diazoxide choline 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 Prader-Willi syndrome were ongoing.

At the time of submission, diazoxide was authorised in some EU countries for the treatment of hypoglycaemia (low blood glucose levels) and hypertensive crises (sudden, dangerously high blood pressure).

At the time of submission, diazoxide choline was not authorised anywhere in the EU for Prader-Willi syndrome. Orphan designation of diazoxide choline had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 October 2017 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

Sponsor's contact details:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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.

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Diazoxide choline	Treatment of Prader-Willi syndrome
Bulgarian	Диазоксид холин	Лечение на синдром на Прадер-Вили
Croatian	Diazoksid kolin	Liječenje Prader-Willijevog sindroma
Czech	Diazoxid cholin	Léčba Prader-Williho syndromu
Danish	Diazoxidcholin	Behandling af Prader-Willis syndrom
Dutch	Diazoxide choline	Behandeling van Prader-Willi syndroom
Estonian	Diasoksiidkoliin	Prader-Willi sündroomi ravi
Finnish	Diatsoksidi koliini	Prader-Willin oireyhtymän hoito
French	Diazoxide choline	Traitement du syndrome de Prader-Willi
German	Diazoxid-Cholin	Behandlung des Prader-Willi-Syndroms
Greek	Διαζοξείδη χολική	Θεραπεία του συνδρόμου Prader-Willi
Hungarian	Diazoxid-kolin	Prader-Willi szindróma kezelése
Italian	Diazossido colina	Trattamento della sindrome di Prader-Willi
Latvian	Diazoksīda holīns	Prader-Wili sindroma ārstēšana
Lithuanian	Diazoksido cholinas	*Prader-Willi* sindromo gydymas
Maltese	Kolina tad-diażossidu	Kura għal Prader-Willi Syndrome
Polish	Diazoksyd choliny	Leczenie zespołu Pradera-Williego
Portuguese	Diazóxido colina	Tratamento da síndrome de Prader-Willi
Romanian	Diazoxid-colină	Tratamentul sindromului Prader-Willi
Slovak	Diazoxid cholín	Liečba Praderovho-Williho syndrómu
Slovenian	Diazoksid holin	Zdravljenje Prader-Willijevega sindroma
Spanish	Diazóxido colina	Tratamiento del Síndrome de Prader-Willi
Swedish	Diazoxidkolin	Behandling av Prader Willis syndrom
Norwegian	Diazoksidkolin	Behandling av Prader-Willis syndrom
Icelandic	Díazoxíðkólín	Meðhöndlun á Prader-Willi heilkenni

[1] At the time of designation