



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Glibenclamide for the treatment of neonatal diabetes

On 15 January 2016, orphan designation (EU/3/15/1589) was granted by the European Commission to AMMTeK, France, for glibenclamide for the treatment of neonatal diabetes.

What is neonatal diabetes?

Neonatal diabetes is a form of diabetes that is diagnosed in the first 6 months of life. This condition is distinct from the more common type 1 diabetes (which occurs later).

Signs and symptoms are those that typically occur with poor blood sugar control including thirst, frequent urination, dehydration and, in severe cases, a harmful build-up of acid (ketoacidosis). Infants with neonatal diabetes may also suffer nervous system disorders which may cause muscle weakness and convulsions (fits).

Neonatal diabetes is life threatening and debilitating in the long term because of the symptoms caused by high blood sugar levels and the risk of ketoacidosis.

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

At the time of designation, neonatal diabetes affected less than 0.01 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 500 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 the orphan designation, insulin was authorised in the EU for use in neonatal diabetes.

The sponsor has provided sufficient information to show that glibenclamide might be of significant benefit for patients with neonatal diabetes because early studies indicate that patients treated with glibenclamide may not need to be treated with insulin or may need less insulin. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

^{*}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 512,900,000 (Eurostat 2015).



How is this medicine expected to work?

Glibenclamide is authorised for treating type 2 diabetes. It works on insulin-producing cells in the pancreas by attaching to a KATP channel, which controls the release of insulin. In many newborn babies with neonatal diabetes, the cells in the pancreas produce insulin but they are not able to release it into the blood. The lack of insulin in the blood causes symptoms of diabetes. Glibenclamide's effect on the KATP channel restores the cells' ability to release insulin into the blood. Glibenclamide is also thought to make the rest of the body respond more effectively to insulin. These effects are expected to reduce the symptoms of neonatal diabetes.

What is the stage of development of this medicine?

The effects of glibenclamide 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 neonatal diabetes were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for neonatal diabetes or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 November 2015 recommending the granting of this designation.

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Glibenclamide	Treatment of neonatal diabetes
Bulgarian	Глибенкламид	Лечение на неонатален диабет
Croatian	Glibenklamid	Liječenje neonatalnog dijabetesa
Czech	Glibenklamid	Léčba neonatálního diabetu
Danish	Glibenclamid	Behandling af neonatal diabetes
Dutch	Glibenclamide	Behandeling van neonatale diabetes
Estonian	Glibenklamiid	Neonataalse diabeedi ravi
Finnish	Glibenklamidi	Neonataalisen diabeteksen hoito
French	Glibenclamide	Traitement du diabète néonatal
German	Glibenclamid	Behandlung von neonataler Diabetes
Greek	Γλιβενκλαμιδη	Θεραπεία του νεογνικού διαβήτη
Hungarian	Glibenklamid	Csecsemőkori diabetes kezelése
Italian	Glibenclamida	Trattamento del diabete neonatale
Latvian	Glibenklamīds	Neonatāla diabēta ārstēšana
Lithuanian	Glibenklamidas	Naujagimių diabeto gydymas
Maltese	Glibenclamide	Kura tad-dijabete fi trabi tat-twelid
Polish	Glibenklamid	Leczenie neonatalnej cukrzycy
Portuguese	Glibenclamida	Tratamento da diabetes neonatal
Romanian	Glibenclamidă	Tratamentul diabetului neonatal
Slovak	Glibenklamid	Liečba neonatálneho diabetu
Slovenian	Glibenklamid	Zdravljenje neonatalnega diabetesa
Spanish	Glibenclamida	Tratamiento de la diabetes neonatal
Swedish	Glibenklamid	Behandling av neonatal diabetes
Norwegian	Glibenklamid	Behandling av neonatal diabetes
Icelandic	Glíbenklamíð	Meðferð við nýbura sykursýki

¹ At the time of designation