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EMA/747281/2018

Public summary of opinion on orphan designation

Apraglutide for the treatment of short bowel syndrome

On 19 November 2018, orphan designation (EU/3/18/2102) was granted by the European Commission to IQVIA RDS Ireland Limited, Ireland, for apraglutide for the treatment of short bowel syndrome.

What is short bowel syndrome?

Short bowel syndrome is a condition in which the body cannot absorb enough fluids and nutrients because much of the intestine is missing due to surgical removal, injury or an inborn defect. As a result, patients may have symptoms such as malnutrition, diarrhoea, dehydration and disturbances of the normal balance of fluids and salts.

In addition, oxalate, a substance that is produced by the breakdown of amino acids or absorbed from the diet, cannot be removed normally from the body in patients with short bowel syndrome, and can build up and cause damage to the kidneys.

Short bowel syndrome is a long-term debilitating and life-threatening condition due to the malnutrition, liver and kidney damage.

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

At the time of designation, short bowel syndrome affected approximately 0.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 31,000 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, the medicine Revestive (teduglutide) was authorised in the EU for treatment of short bowel syndrome. In addition, patients were treated with parenteral nutrition (feeding by a drip into a vein), vitamin and mineral supplements, and medicines to manage symptoms. In severe cases intestinal transplantation was used.

*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 517,400,000 (Eurostat 2018).

The sponsor has provided sufficient information to show that apraglutide might be of significant benefit for patients with short bowel syndrome. Early data show that patients receiving the medicine once weekly could absorb more food and gained weight. It was noted that the currently authorised medicine is given daily.

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?

Apraglutide is similar to human glucagon-like peptide 2 (GLP-2), a hormone made in the gut that increases absorption of nutrients from the intestine. Like GLP-2, apraglutide increases intestinal absorption by increasing blood flow to and from the gut, reducing the speed at which food passes through the gut and reducing acid secretions in the stomach which can interfere with absorption in the intestine.

What is the stage of development of this medicine?

The effects of apraglutide have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with apraglutide in patients with short bowel syndrome were ongoing.

At the time of submission, apraglutide was not authorised anywhere in the EU for short bowel syndrome 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 18 October 2018 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	Apraglutide	Treatment of short bowel syndrome
Bulgarian	Апраглутид	Лечение на синдром на късото черво
Croatian	Apraglutid	Liječenje sindroma kratkog crijeva
Czech	Apraglutid	Léčba syndromu krátkého střeva
Danish	Apraglutid	Behandling af korttarmssyndrom
Dutch	Apraglutide	Behandeling van kortedarm syndroom
Estonian	Apraglutiid	Lühikese soole sündroomi ravi
Finnish	Apraglutidi	Lyhytsuolioireyhtymän hoito
French	Apraglutide	Traitement du syndrome de l'intestin court
German	Apraglutide	Behandlung des Kurzdarmsyndroms
Greek	Απραγλουτιδη	Θεραπεία του συνδρόμου βραχέος εντέρου
Hungarian	Apraglutide	Rövid bél szindróma kezelése
Italian	Apraglutide	Trattamento della sindrome dell'intestino breve
Latvian	Apraglutide	Īsās zarnas sindroma ārstēšana
Lithuanian	Apragliutidas	Trumposios žarnos sindromui gydyti
Maltese	Apraglutid	Kura tas-sindromu tal-musrana qasira
Polish	Apraglutyd	Leczenie zespołu krótkiego jelita
Portuguese	Apraglutido	Tratamento do síndrome do intestino curto
Romanian	Apraglutid	Tratamentul sindromului de intestin scurt
Slovak	Apraglutid	Liečba syndrómu krátkeho čreva
Slovenian	Apraglutid	Zdravljenje sindroma kratkega črevesja
Spanish	Apraglutide	Tratamiento del síndrome del intestino corto
Swedish	Apraglutid	Behandling av korttarmssyndrom
Norwegian	Apraglutid	Behandling av korttarmsyndrom
Icelandic	Apraglutið	Meðferð heilkennis stuttra þarma

¹ At the time of designation