



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

30 September 2015  
EMA/COMP/507070/2015  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Insulin human for the treatment of short bowel syndrome

On 10 August 2015, orphan designation (EU/3/15/1532) was granted by the European Commission to Sirius Regulatory Consulting Limited, United Kingdom, for insulin human 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 small bowel, the part of the intestines between the stomach and the large bowel (colon), 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 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. Patients usually require feeding by a drip into a vein (parenteral nutrition).

Short bowel syndrome is a chronically debilitating and life-threatening condition due to the complications of parenteral nutrition (which include liver failure and infection) and to kidney damage.

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

At the time of designation, short bowel syndrome affected approximately 0.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 10,000 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, patients with short bowel syndrome were normally treated with parenteral nutrition, vitamin and mineral supplements, and medicines to manage symptoms. In severe cases

---

\*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).



intestinal transplantation might be used. In addition, in the EU the medicine Revestive (teduglutide) was authorised for treatment of the condition.

The sponsor has provided sufficient information to show that insulin human might be of significant benefit for patients with short bowel syndrome. Data provided show that the medicine may improve the condition of the patient's intestine sufficiently so that the patient can absorb more food when fed by a tube. In addition, the medicine works in a different way to existing treatments. These assumptions 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?**

Insulin human is a copy of the hormone insulin, the hormone that regulates blood sugar levels. The hormone is also known to promote growth and development of intestinal cells. In short bowel syndrome, the insulin given by mouth reaches the intestines, where it is expected to stimulate intestinal cell growth, thus regenerating the area of intestine that can absorb nutrients and helping to improve the symptoms of the disease.

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

The effects of insulin human have been evaluated in experimental models.

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

At the time of submission, insulin human 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 16 July 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:

Sirius Regulatory Consulting Limited  
29 Hambling Meadow  
Eddington  
Hungerford  
Berkshire, RG17 0HJ  
United Kingdom  
Tel. +44 (0)1488 686 449  
Fax +44 (0)1488 686 449  
E-mail: [info@siriusregulatory.com](mailto:info@siriusregulatory.com)

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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Insulin human	Treatment of short bowel syndrome
Bulgarian	Човешки инсулин	Лечение на синдром на късото черво
Croatian	Inzulin, ljudski	Liječenje sindroma kratkog crijeva
Czech	Inzulin lidský	Léčba syndromu krátkého střeva
Danish	Insulin humant	Behandling af korttarmssyndrom
Dutch	Humane insuline	Behandeling van kortedarm syndroom
Estonian	Iniminsuliin	Lühikese soole sündroomi ravi
Finnish	Insuliini	Lyhytsuolioireyhtymän hoito
French	Insuline humaine	Traitement du syndrome de l'intestin court
German	Insulin human	Behandlung des Kurzdarmsyndroms
Greek	Ανθρώπινη ινσουλίνη	Θεραπεία του συνδρόμου βραχέος εντέρου
Hungarian	Humán inzulin	Rövid bél szindróma kezelése
Italian	Insulina umana	Trattamento della sindrome dell'intestino breve
Latvian	Cilvēka insulīns	Īsās zarnas sindroma ārstēšana
Lithuanian	Žmogaus insulinas	Trumposios žarnos sindromui gydyti
Maltese	Insulina umana	Kura tas-sindromu tal-musrana qasira
Polish	Insulina ludzka	Leczenie zespołu krótkiego jelita
Portuguese	Insulina humana	Tratamento do síndrome do intestino curto
Romanian	Insulină umană	Tratamentul sindromului de intestin scurt
Slovak	Inzulín ľudský	Liečba syndrómu krátkeho čreva
Slovenian	Humani insulin	Zdravljenje sindroma kratkega črevesja
Spanish	Insulina humana	Tratamiento del síndrome del intestino corto
Swedish	Humant insulin	Behandling av korttarmssyndrom
Norwegian	Humaninsulin	Behandling av korttarmsyndrom
Icelandic	Mannainsúlín	Meðferð heilkennis stuttra þarma

<sup>1</sup> At the time of designation