



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Oxalobacter formigenes strain HC-1 for the treatment of short bowel syndrome

On 15 October 2014, orphan designation (EU/3/14/1346) was granted by the European Commission to OxThera AB, Sweden, for *Oxalobacter formigenes* strain HC-1 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 suffer from 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 caused by high levels of oxalate.

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

At the time of designation, short bowel syndrome affected not more than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of not more than 51,000 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).

*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 511,100,000 (Eurostat 2014).



What treatments are available?

At the time of application patients with short bowel syndrome were normally treated with parenteral nutrition, vitamin and mineral supplements, and medicines to manage symptoms. In severe cases intestinal transplantation might be used. In addition, in the EU the medicine Revestive (teduglutide) was authorised for treatment of the condition by improving absorption from the intestine.

The sponsor has provided sufficient information to show that *Oxalobacter formigenes* strain HC-1 might be of significant benefit for patients with short bowel syndrome because results of preliminary studies suggest that it can reduce the level of oxalate in the body. 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?

Oxalobacter formigenes strain HC-1 is a bacterium that breaks down oxalate. This bacterium lives naturally in the gut but is lacking or only present in small amounts in patients with short bowel syndrome. When given as regular supplements to patients, it is expected to replace the missing bacterium. This will increase breakdown of oxalate in the intestines and encourage its removal from the body, thus reducing the high levels found in patients with short bowel syndrome, and lowering the risk of damage to the kidneys.

What is the stage of development of this medicine?

The effects of *Oxalobacter formigenes* strain HC-1 have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with the medicine in patients with short bowel syndrome had been started.

At the time of submission, *Oxalobacter formigenes* strain HC-1 was not authorised anywhere in the EU for short bowel syndrome or designated as an orphan medicinal product elsewhere for this condition. Orphan designation of the medicine had been granted in the EU and the United States for primary hyperoxaluria, another condition resulting in high levels of oxalate in the body.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 4 September 2014 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:

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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	<i>Oxalobacter formigenes</i> strain HC-1	Treatment of short bowel syndrome
Bulgarian	<i>Oxalobacter formigenes</i> щам HC-1	Лечение на синдром на късото черво
Croatian	<i>Oxalobacter formigenes</i> soj HC-1	Liječenje sindroma kratkog crijeva
Czech	Bakterie <i>Oxalobacter formigenes</i> kmen HC-1	Léčba syndromu krátkého střeva
Danish	<i>Oxalobacter formigenes</i> HC-1 <i>Oxalobacter</i>	Behandling af korttarmssyndrom
Dutch	<i>Oxalobacter formigenes</i> stam HC-1	Behandeling van kortedarm syndroom
Estonian	<i>Oxalobacter formigenes</i> 'e tüvi HC-1	Lühikese soole sündroomi ravi
Finnish	<i>Oxalobacter formigenes</i> kanta HC-1	Lyhytsuolioireyhtymän hoito
French	<i>Oxalobacter formigenes</i> souche HC-1	Traitement du syndrome de l'intestin court
German	<i>Oxalobacter formigenes</i> Stamm HC-1	Behandlung des Kurzdarmsyndroms
Greek	<i>Oxalobacter formigenes</i> – στέλεχος HC-1	Θεραπεία του συνδρόμου βραχείας ελίκας σε ενήλικες και παιδιά
Hungarian	<i>Oxalobacter formigenes</i> HC-1 törzs	Rövid bél szindróma kezelése
Italian	<i>Oxalobacter formigenes</i> ceppo HC-1	Trattamento della sindrome dell'intestino breve
Latvian	<i>Oxalobacter formigenes</i> celms HC-1	Īsās zarnas sindroma ārstēšana
Lithuanian	<i>Oxalobacter formigenes</i> , HC-1 štamai	Trumposios žarnos sindromui gydyti
Maltese	Razza HC-1 ta' <i>Oxalobacter formigenes</i>	Kura tas-sindromu tal-musrana qasira
Polish	<i>Oxalobacter formigenes</i> szczep HC-1	Leczenie zespołu krótkiego jelita
Portuguese	<i>Oxalobacter formigenes</i> estirpe HC-1	Tratamento do síndrome do intestino curto
Romanian	<i>Oxalobacter formigenes</i> tulpina HC-1	Tratamentul sindromului de intestin scurt
Slovak	<i>Oxalobacter formigenes</i> , kmeň HC-1	Liečba syndrómu krátkeho čreva
Slovenian	<i>Oxalobacter formigenes</i> sev HC-1	Zdravljenje sindroma kratkega črevesja
Spanish	<i>Oxalobacter formigenes</i> cepa HC-1	Tratamiento del síndrome del intestino corto
Swedish	<i>Oxalobacter formigenes</i> stam HC-1	Behandling av korttarmssyndrom
Norwegian	<i>Oxalobacter formigenes</i> stamme HC-1	Behandling av korttarmsyndrom
Icelandic	<i>Oxalobacter formigenes</i> stofn HC-1	Meðferð heilkennis stuttra þarma

¹ At the time of designation