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EMA/COMP/10142/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pentosan polysulfate sodium for the treatment of interstitial cystitis

On 15 January 2015, orphan designation (EU/3/14/1411) was granted by the European Commission to Dr Ulrich Granzer, Germany, for pentosan polysulfate sodium for the treatment of interstitial cystitis.

What is interstitial cystitis?

Interstitial cystitis (sometimes referred to as 'painful bladder syndrome') is a long-term inflammatory condition of the bladder. The cause is unknown and a diagnosis is normally made after excluding other likely causes such as infection. Symptoms include pain in the pelvic area, increased frequency of urination, and pain while urinating.

Interstitial cystitis is debilitating in the long term due to the associated pain and discomfort and the increased urinary frequency.

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

At the time of designation, interstitial cystitis affected not more than 2 in 10,000 people in the European Union (EU). This was equivalent to fewer than 102,000 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, no satisfactory methods for the treating interstitial cystitis were authorised in the EU. Painkillers were used to manage the pain. Pentosan polysulfate sodium was authorised in some countries around the world to relieve pain and discomfort in patients with interstitial cystitis.

How is this medicine expected to work?

The exact cause of interstitial cystitis is not known, however it is thought that interstitial cystitis is caused by defects in the layer of sugar molecules called glycosaminoglycans (or GAGs) in the lining of

*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).

the bladder. This medicine, pentosan polysulfate sodium, is very similar to GAGs and it is expected to work by replenishing the defective GAG layer. This is expected to improve the symptoms of the disease.

What is the stage of development of this medicine?

As pentosan polysulfate is a well-known substance, the sponsor provided data from the published literature to support its application for orphan designation.

At the time of submission, the medicine was authorised in Canada for treating interstitial cystitis and in the United States for the relief of pain and discomfort associated with interstitial cystitis.

The medicine was not authorised anywhere in the EU for interstitial cystitis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 11 December 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

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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	Pentosan polysulfate sodium	Treatment of interstitial cystitis
Bulgarian	Пентозан полисулфат натрий	Лечение на интерстициален цистит
Croatian	Natrijev pentozanpolisulfat	Liječenje intersticijskog cistitisa
Czech	Pentosan polysulfát sodný	Léčba intersticiální cystitida
Danish	Pentosan polysulfat natrium	Behandling af interstitiel cystitis
Dutch	Pentosanpolysulfaatnatrium	Behandeling van Interstitiële cystitis
Estonian	Pentosaanpolüsulfaatnatrium	Interstitsiaalse tsüstiidi ravi
Finnish	Pentosaani polysulfaattinatrium	Interstitiaalisen kystiitin hoito
French	Polysulfate de pentosan sodique	Traitement de la cystite interstitielle
German	Pentosan Natrium Polysulfat	Behandlung der interstitiellen Zystitis
Greek	Νατριουχος πολυθειικη πεντοσανη	Θεραπεία της διάμεσης κυστίτιδας
Hungarian	Pentozan polisulfát nátrium	Interstitialis cystitis kezelése
Italian	Polisolfato di pentosano sodico	Trattamento del cistite interstiziale
Latvian	Pentozāna polisulfāta nātrija sāls	Intersticiāla cistīta ārstēšana
Lithuanian	Natrio pentozano polisulfatas	Intersticinio cistito gydymas
Maltese	Pentosan polysulfate sodium	Kura taċ-ċistite tal-interstizju
Polish	Polisiarczan pentozanu sodu	Leczenie śródmiąższowego zapalenia pęcherza moczowego
Portuguese	Pentosano polissulfato de sódio	Tratamento do cistite intersticial
Romanian	Polisulfat de pentosan sodic	Tratamentul cistitei interstiziale
Slovak	Pentózan polysulfát sodný	Liečba intersticiálna cystitída
Slovenian	Natrijev pentosan polisulfat	Zdravljenje intersticijskega cistitisa
Spanish	pentosano polisulfato sódico	Tratamiento del cistitis intersticial
Swedish	Pentosan polysulfat natrium	Behandling av interstitiell cystit
Norwegian	Pentosanpolysulfatnatrium	Behandling av interstitiell cystitt
Icelandic	Pentósan pólýsúlfat natrium	Meðferð á millivefsblöðrubólgu

¹ At the time of designation