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EMA/5068/2017
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pentosan polysulfate sodium for the treatment of interstitial cystitis

On 12 January 2017, orphan designation (EU/3/16/1822) was granted by the European Commission to Kyoto tech Limited, United Kingdom, for pentosan polysulfate sodium for the treatment of interstitial cystitis.

What is interstitial cystitis?

Interstitial cystitis is a long-term inflammatory condition of the bladder. The cause of the condition is unknown and a diagnosis is normally made after other likely causes such as infection have been ruled out. 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 approximately 2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 103,000 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 treating interstitial cystitis were authorised in the EU. Painkillers were used to manage the pain. Pentosan polysulfate sodium was authorised in some countries to relieve pain and discomfort in patients with interstitial cystitis.

*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 513,700,000 (Eurostat 2016).

How is this medicine expected to work?

It is thought that in interstitial cystitis defects may occur in the protective layer of molecules called glycosaminoglycans (or GAGs) in the lining of the bladder. This medicine, pentosan polysulfate sodium, is very similar to GAGs and it is expected to work by attaching to and repairing the defective GAG layer. This is expected to protect bladder cells from irritating substances in the urine, thereby improving the symptoms of the disease.

What is the stage of development of this medicine?

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

At the time of submission of the application for orphan designation, pentosan polysulfate sodium was authorised in several countries outside the EU for treating interstitial cystitis.

At the time of submission, the medicine was not authorised anywhere in the EU for interstitial cystitis. Orphan designation of the medicine had been granted in the EU and United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 December 2016 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	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	Pentosaanpolüsulfaatnaatrium	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	Νατριούχος πολυθειική πεντοσάνη	Θεραπεία της διάμεσης κυστίτιδας
Hungarian	Pentozan poliszulfát nátrium	Interstitialis cystitis kezelése
Italian	Polisolfato di pentosano sodico	Trattamento della 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 interstiale
Slovak	Pentózan polysulfát sodný	Liečba intersticiálnej cystitídy
Slovenian	Natrijev pentosan polisulfat	Zdravljenje intersticijskega cistitisa
Spanish	pentosano polisulfato sódico	Tratamiento del cistitis intersticial
Swedish	Pentosanpolysulfat natrium	Behandling av interstitiell cystit
Norwegian	Pentosanpolysulfat natrium	Behandling av interstitiell cystitt
Icelandic	Pentósan pólýsúlfat natríum	Meðferð á millivefsblöðrubólgu

¹ At the time of designation