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Public summary of opinion on orphan designation

Oxymetazoline hydrochloride for the treatment of spinal cord injury

On 17 July 2017, orphan designation (EU/3/17/1892) was granted by the European Commission to RDD Pharma Limited, United Kingdom, for oxymetazoline hydrochloride for the treatment of spinal cord injury.

What is spinal cord injury?

The spinal cord can be injured through accidents or by internal causes such as tumours or bleeding within the spine putting pressure on the spinal cord. Injury to the spinal cord can damage the nerves that run through the cord and that branch out from it. This can stop the flow of nerve impulses between the brain and the body, resulting in loss of feeling, paralysis and even death, depending upon the severity of the injury and where it is located. Patients may also experience loss of control of bladder or bowels, breathing difficulties, blood clots in veins and lungs, and recurring infections.

Spinal cord injury is a debilitating and life-threatening disease, because it can cause paralysis of the arms and legs and reduces life expectancy.

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

At the time of designation, spinal cord injury affected approximately 4.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 22,000 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, methylprednisolone (a steroid) was authorised for the treatment of spinal cord injury in some countries in the EU. Methylprednisolone reduces the inflammation and pressure on the spinal cord that can occur after it is damaged. Patients with spinal cord injury could also have decompression surgery to reduce the pressure on the spine. Other medicines or procedures were used to manage various symptoms such as spasticity (painful muscle spasm) and incontinence.

*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 515,700,000 (Eurostat 2017).

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The sponsor has provided sufficient information to show that oxymetazoline hydrochloride might be of significant benefit for patients with spinal cord injury because early studies indicate that it can reduce episodes of faecal incontinence in patients already receiving standard care. 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?

Some patients with spinal cord injury lose control of the anal sphincter (the ring of muscles that hold the anus closed) and so suffer from faecal incontinence. Oxymetazoline hydrochloride is a medicine that has been approved in the EU for many years for the treatment of nasal congestion (blocked nose). It has a relatively long-lasting action on targets called alpha adrenoceptors, which are found in various locations including the muscles of the anal sphincter. When oxymetazoline is applied locally to the area of the anus, its action is expected to stimulate these muscles to contract more tightly, reducing the risk of faecal incontinence.

What is the stage of development of this medicine?

The effects of oxymetazoline hydrochloride have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with spinal cord injury were ongoing.

At the time of submission, oxymetazoline hydrochloride was not authorised anywhere in the EU for spinal cord injury 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 15 June 2017 recommending the granting of this designation.

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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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Oxymetazoline hydrochloride	Treatment of spinal cord injury
Bulgarian	Оксиметазолин хидрохлорид	Лечение на гръбначно-мозъчни травми
Croatian	Oksimetazolin hidroklorid	Liječenje ozljede kralježnične moždine
Czech	Oxymetazolin hydrochlorid	Léčba míšního traumatu
Danish	Oxymetazolinhydrochlorid	Behandling af rygmarvslæsion
Dutch	Oxymetazolinehydrochloride	Behandeling van ruggenmergletsel
Estonian	Oksümetasoliinvesinikkloriid	Seljaaju vigastuse ravi
Finnish	Oksimetatsoliinihydrokloridi	Selkäydinvamman hoito
French	Chlorhydrate d'oxymétazoline	Traitement des lesions de la moëlle épinière
German	Oxymetazolinhydrochlorid	Behandlung der Rückenmarkverletzung
Greek	Υδροχλωρική οξυμεταζολίνη	Θεραπεία τραύματος της σπονδυλικής στήλης
Hungarian	Oximetazolin-hidroklorid	Gerincvelő sérülés kezelése
Italian	Ossimetazolina cloridrato	Trattamento delle lesioni del midollo spinale
Latvian	Oksimetazolīna hidrohlorīds	Muguras smadzeņu traumu ārstēšana
Lithuanian	Oksimetazolino hidrochloridas	Nugaros smegenų sužalojimo gydymas
Maltese	Idroklorur tal-ossimetazolin	Kura ta' korriment tan-nerv qawwi li jgħaddi minn ġos-sinsla
Polish	Chlorowodorek oksymetazoliny	Leczenie uszkodzenia rdzenia kręgowego
Portuguese	Cloridrato de oximetazolina	Tratamento da lesão da medula espinal
Romanian	Clorhidrat de oximetazolină	Tratamentul leziunilor măduvei spinării
Slovak	Oxymetazolín hydrochlorid	Liečba poškodenia miechy
Slovenian	Oksimetazolinijev hidroklorid	Zdravljenje poškodbe hrbtenjače
Spanish	Clorhidrato de oximetazolina	Tratamiento de las lesiones de la médula espinal
Swedish	Oximetazolinhydroklorid	Behandling av ryggmärgsskada
Norwegian	Oksymetazolinhydrolorid	Behandling av ryggmargsskade
Icelandic	Oxýmetazólín hýdróklóríð	Meðferð mænuskaða vegna slyss

[1] At the time of designation