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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

R-azasetron besylate for the treatment of sudden sensorineural hearing loss

On 18 November 2016, orphan designation (EU/3/16/1785) was granted by the European Commission to Sensorion, France, for R-azasetron besylate for the treatment of sudden sensorineural hearing loss.

What is sudden sensorineural hearing loss?

Sudden sensorineural hearing loss (SSNHL) is a sudden loss of hearing in one or sometimes both ears, caused by damage to the cells in the inner ear that detect sound and to the nerves that supply them. The damage can have various causes including loud noise and infection or may occur as a side effect of certain medicines. Symptoms may develop over a period of up to 3 days, and can include tinnitus (buzzing or ringing in the ears), a sensation of blockage and vertigo (dizziness). It is a long-term debilitating condition because of the often irreversible deafness it produces.

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

At the time of designation, SSNHL affected approximately 4 in 10,000 people in the European Union (EU). This was equivalent to a total of around 205,000 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 application, various medicines were authorised in the EU to treat symptoms associated with SSNHL such as tinnitus and vertigo. No medicines were authorised to treat the hearing loss itself.

The sponsor has provided sufficient information to show that R-azasetron besylate might be of significant benefit for patients with SSNHL because laboratory studies suggest it may help to treat the underlying cause of the condition and improve recovery of hearing. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

*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?

The death of sound-detecting cells (hair cells) and nerve cells in patients with SSNHL is thought to be associated with increased activation of certain cellular pathways. R-azasetron besylate blocks the actions of these pathways and, if taken shortly after SSNHL begins, is expected to reduce damage to the hair cells and nerves and thereby help patients recover.

R-azasetron besylate is a form of another medicine, azasetron, that has been used in Japan since 1994 to treat nausea (feeling sick) and vomiting related to cancer treatment.

What is the stage of development of this medicine?

The effects of R-azasetron besylate have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with R-azasetron besylate in patients with SSNHL had been started.

At the time of submission, R-azasetron besylate was not authorised anywhere in the EU for SSNHL 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 6 October 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	R-azasetron besylate	Treatment of sudden sensorineural hearing loss
Bulgarian	R-азасетрон безилат	Лечение на внезапна сензориневрална загуба на слух
Croatian	R-azasetron bezilat	Liječenje iznenadnog senzorineuralnog gubitka sluha
Czech	R-azasetron besylát	Léčba náhlé percepční nedoslýchavosti
Danish	R-azasetron besylat	Behandling af akut sensorineuralt høretab
Dutch	R-azasetron besylaat	Behandeling van plotse sensorineurale doofheid
Estonian	R-azasetroonbesülaat	Äkilise sensorineuraalse kuulmiskaotuse ravi
Finnish	R-atsasetronibesylaatti	Äkillisen sensorineuraalisen huonokuuloisuuden hoito
French	R-azasétron bésylate	Traitement contre la perte auditive neurosensorielle soudaine
German	R-Azasetron Besilat	Zur Behandlung von plötzlichem sensorineuralem Hörverlust
Greek	R-Βεσυλική αζασετρόνη	Θεραπεία αιφνίδιας νευροαισθητήριας απώλειας ακοής
Hungarian	R-azaszetron bezilát	Hirtelen bekövetkező szenzorineurális halláscsökkenés kezelése
Italian	R-azasetron besilato	Trattamento dell'ipoacusia neurosensoriale improvvisa
Latvian	R-azasetrona besilāts	Pēkšņa sensorineirāla dzirdes zuduma ārstēšana
Lithuanian	R-azasetrono besilatas	Ūminio neurosensorinio klausos sutrikimo gydymas
Maltese	R-azasetron besilat	Kura għal telf fis-smiġħ sensonewrali f'daqqa
Polish	R-azasetron benzenosulfonian	Leczenie nagłego niedosłuchu czuciowo-nerwowego
Portuguese	R-Besilato de R-azasetrom	Tratamento para a perda auditiva neurosensorial súbita
Romanian	R-azasetron besilat	Tratamentul pierderii subite senzorineurale a auzului
Slovak	R-azasetron besylát	Liečba náhlej percepčnej poruchy sluchu
Slovenian	R-azasetron benzensulfonat	Zdravljenje nenadne senzorinevralne izgube sluha
Spanish	Besilato de R-azasetron	Tratamiento de la pérdida de audición neurosensorial súbita
Swedish	R-azasetronbensylat	Behandling av plötslig sensorineural hörselnedsättning
Norwegian	R-azasetronbesilat	Behandling av plutselig sensorinevralt hørselstap
Icelandic	R-azasetrón besýlat	Meðferð við skyndilegu skyntaugaheyrnartapi

¹ At the time of designation