



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pioglitazone hydrochloride for the treatment of sudden sensorineural hearing loss

On 12 January 2017, orphan designation (EU/3/16/1823) was granted by the European Commission to Regiomedica GmbH, Germany, for pioglitazone hydrochloride 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, surgery and infection or it may occur as a side effect of certain medicines. Symptoms develop over a few days, and can include hearing loss, 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 as well as tinnitus and vertigo.

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 pioglitazone hydrochloride might be of significant benefit for patients with SSNHL because laboratory studies suggest that it might help

^{*}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).



hearing to come back. 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?

The way pioglitazone hydrochloride works in SSNHL is not fully understood. Pioglitazone hydrochloride activates a receptor called PPAR γ in cells, and this is thought to reduce cell death, cell damage known as oxidative stress and inflammation, which are underlying causes of SSNHL. These effects are therefore expected to improve symptoms of SSNHL.

What is the stage of development of this medicine?

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

At the time of submission of the application for orphan designation, no clinical trials with pioglitazone hydrochloride in patients with SSNHL had been started.

At the time of submission, pioglitazone hydrochloride was authorised in the EU for type 2 diabetes.

At the time of submission, pioglitazone hydrochloride 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 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	Pioglitazone hydrochloride	Treatment of sudden sensorineural hearing loss
Bulgarian	Πιογλιταζон хидрохлорид	Лечение на внезапна сензориневрална загуба на слух
Croatian	Pioglitazon klorid	Liječenje iznenadnog sensorineuralnog gubitka sluha
Czech	Pioglitazon hydrochlorid	Léčba náhlé percepční nedoslýchavosti
Danish	Pioglitazonhydrochlorid	Behandling af akut sensorineuralt høretab
Dutch	Pioglitazonhydrochloride	Behandeling van plotse sensorineurale doofheid
Estonian	Pioglitason hüdrokloriid	Äkilise sensorineuraalse kuulmiskaotuse ravi
Finnish	Pioglitatsoni hydrokloridi	Äkillisen sensorineuraalisen huonokuuloisuuden hoito
French	Chlorhydrate de pioglitazone	Traitement contre la perte auditive neurosensorielle soudaine
German	Pioglitazonhydrochlorid	Behandlung von plötzlichem sensorineuralem Hörverlust
Greek	Πιογλιταζόνη υδροχλωρική	Θεραπεία αιφνίδιας νευροαισθητήριας απώλειας ακοής
Hungarian	Pioglitazon hidroklorid	Hirtelen bekövetkező szenzorineurális halláscsökkenés kezelése
Italian	Pioglitazone cloridrato	Trattamento dell'ipoacusia neurosensoriale improvvisa
Latvian	Pioglitazona hidrohlorīds	Pēkšņa sensorineirāla dzirdes zuduma ārstēšana
Lithuanian	Pioglitazono hidrochloridas	Ūminio neurosensorinio klausos sutrikimo gydymas
Maltese	Pioglitazon kloridrat	Kura għal telf fis-smiġħ sensornewrali f'daqqa
Polish	Chlorowodorek pioglitazonu	Leczenie nagłego niedosłuchu czuciowo-nerwowego
Portuguese	Cloridrato de pioglitazona	Tratamento para a perda auditiva neurosensorial súbita
Romanian	Clorhidrat de pioglitazonă	Tratamentul pierderii subite senzorieurale a auzului
Slovak	Pioglitazón hydrochlorid	Liečba náhlejš percepčnej poruchy sluchu
Slovenian	Pioglitazonijev hidroklorid	Zdravljenje nenadne senzorievralne izgube sluha
Spanish	Clorhidrato de pioglitazona	Tratamiento de la pérdida de audición neurosensorial súbita
Swedish	Pioglitazonhydroklorid	Behandling av plötslig sensorineural hörselnedsättning
Norwegian	Pioglitazonhydroklorid	Behandling av plutselig sensorinevralt hørselstap
Icelandic	Píóglítazón hýdróklóríð	Meðferð við skyndilegu skyntaugaheyrnartapi

¹ At the time of designation