20 April 2015
EMA/COMP/682970/2012 Rev.2
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Ixazomib for the treatment of systemic light chain amyloidosis

First publication	11 December 2012
Rev.1: sponsor's name change	20 November 2013
Rev.2: transfer of sponsorship	20 April 2015
Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 8 November 2012, orphan designation (EU/3/12/1060) was granted by the European Commission to Takeda Global Research and Development Centre (Europe) Ltd, United Kingdom, for ixazomib for the treatment of systemic light chain amyloidosis.

In November 2013, Takeda Global Research and Development Centre (Europe) Ltd changed name to Takeda Development Centre Europe Ltd.

The sponsorship was transferred to Takeda Pharma A/S, Denmark, in March 2015.

What is systemic light chain amyloidosis?

Amyloidosis is a group of diseases in which deposits of proteins (called amyloids) accumulate in organs of the body, such as the heart and kidneys, damaging them and preventing them from working properly. In systemic light chain amyloidosis, the deposits are made of a blood protein called 'immunoglobulin light chain' produced by abnormal blood cells. This condition often occurs in patients with other blood disorders, such as multiple myeloma.

Systemic light chain amyloidosis is a long-term debilitating and life-threatening condition mainly due to damage to the heart.

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

At the time of designation, systemic light chain amyloidosis affected approximately 0.7 in 10,000 people in the European Union (EU). This was equivalent to a total of around 36,000 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, there were no treatments authorised in the EU for systemic light chain amyloidosis. Patients were treated with anticancer medicines to kill the abnormal blood cells responsible for the production of immunoglobulin light chain. Some patients also underwent stem cell transplantation. This is a complex procedure where the patient receives stem cells to help restore the bone marrow (the spongy tissue inside the large bones where blood cells are produced).

How is this medicine expected to work?

Ixazomib is expected to work by blocking the proteasome, which is a system within the cells that breaks down proteins when they are no longer needed. By blocking the proteasome of the abnormal blood cells in systemic light chain amyloidosis, the proteins in these cells are not broken down. As a result, the cells eventually die. This is expected to help improve the symptoms of the disease.

What is the stage of development of this medicine?

The effects of ixazomib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with ixazomib in patients with systemic light chain amyloidosis were ongoing.

At the time of submission, ixazomib was not authorised anywhere in the EU for systemic light chain amyloidosis. Orphan designation of ixazomib had been granted in the United States of America for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 October 2012 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.

*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 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 509,000,000 (Eurostat 2012).

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:

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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 languages[1], Norwegian and Icelandic

Language	Active substance	Indication
English	Ixazomib	Treatment of systemic light chain amyloidosis
Bulgarian	Иксазомиб	Лечение на системна леко-верижна (AL) амилоидоза
Croatian	Iksazomib	Liječenje sistemne amiloidoze lakih lanaca
Czech	Ixazomib	Léčba systémové amyloidózy z lehkýchřetězců (AL)
Danish	Ixazomib	Behandling af letkæde (AL) amyloidose
Dutch	Ixazomib	Behandeling van systemische lichte keten amyloïdose
Estonian	Ixazomib	Süsteemse kergete ahelatega (AL) amüloidoosi ravi
Finnish	Iksatsomibi	Systeemisen AL-amyloidoosin hoito
French	Ixazomib	Traitement de l'amylose systémique à chaine légère
German	Ixazomib	Behandlung der systemischen Leichtketten- Amyloidose
Greek	Ιξαζομίμπη	Θεραπεία της συστημικής αμυλοείδωσης ελαφριάς αλυσίδας
Hungarian	Ixazomib	Szisztémás könnyű láncú amyloidosis kezelése
Italian	Ixazomib	Trattamento dell'amiloidosi sistemica da catene leggere (AL)
Latvian	Iksazomibs	Sistēmiskās vieglās ķēdes amiloidozes ārstēšana
Lithuanian	Iksazomibas	Sisteminės lengvosios grandinės (AL) amiloidozės gydymas
Maltese	Ixazomib	Kura tal-amilojdosi sistemika ta' katina ħafifa
Polish	Iksazomib	Leczenie układowej amyloidozy łańcuchów lekkich (AL)
Portuguese	Ixazomib	Tratamento da amiloidose sistémica de cadeias leves (AL)
Romanian	Ixazomib	Tratamentul amiloidozei (AL) sistemice cu lanţ uşor
Slovak	Ixazomib	Liečba systémovej AL-amyloidózy ľahkých reťazcov (AL)
Slovenian	Iksazomib	Zdravljenje sistemske amiloidoze lahkih verig
Spanish	Ixazomib	Tratamiento de la amiloidosis sistémica de cadenas ligeras (AL)
Swedish	Ixazomib	Behandling av systemisk AL-amyloidos med lätt kedja
Norwegian	Iksazomib	Behandling av systemisk lettkjedeamyloidose
Icelandic	Ixazomib	Meðferð við léttkeðju (AL) mýlildi (e. systemic light chain (AL) amyloidosis)

[1] At the time of transfer of sponsorship