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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

(E)-2,4,6-trimethoxystyryl-3-carboxymethylamino-4-methoxybenzyl-sulfone sodium salt for the treatment of myelodysplastic syndromes

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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 26 April 2012, orphan designation (EU/3/12/987) was granted by the European Commission to JJGConsultancy Ltd, United Kingdom, for (E)-2,4,6-trimethoxystyryl-3-carboxymethylamino-4-methoxybenzyl-sulfone sodium salt for the treatment of myelodysplastic syndromes.

The sponsorship was transferred to Onconova Europe GmbH, Germany, in February 2014.

What are myelodysplastic syndromes?

Myelodysplastic syndromes (MDSs) are a group of disorders in which the red blood cells, white blood cells and platelets produced by the bone marrow (the spongy tissue inside the large bones) do not grow and mature normally. Patients with MDSs can develop several symptoms including tiredness or weakness due to anaemia (low red blood cell counts), infections due to low white blood cells, and bruising or abnormal bleeding due to low platelet counts.

MDSs are long-term debilitating and life-threatening diseases because they can lead to severe anaemia, infections or bleeding, and can result in leukaemia (cancer of the white blood cells).

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

At the time of designation, MDSs affected not more 2 in 10,000 people in the European Union (EU)*. This is equivalent to a total of not more than 102,000 people, and is below the ceiling for orphan

*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).



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, some medicines were authorised in the EU for the treatment of MDSs. The choice of treatment for MDSs depended on a number of factors, including the type and the extent of the disease, whether it had been treated before, and the patient's age, symptoms and general state of health. The main treatments for MDSs included chemotherapy (medicines to treat cancer) and bone marrow transplantation. This is a complex procedure where the bone marrow of the patient is cleared of cells and replaced with healthy bone marrow cells from a matched donor.

The sponsor has provided sufficient information to show that (E)-2,4,6-trimethoxystyryl-3-carboxymethylamino-4-methoxybenzyl-sulfone sodium salt might be of significant benefit for patients with MDSs because early studies show that it might improve the treatment of patients who have not responded to previous treatment, when used alone or in combination with other authorised medicines. 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?

This medicine is thought to work by blocking different types of enzymes known as protein kinases, which are involved in the growth and division of cancer cells. By blocking these enzymes, this medicine is expected to block the division of the abnormal blood cells in MDSs, leading to cell death.

What is the stage of development of this medicine?

The effects of (E)-2,4,6-trimethoxystyryl-3-carboxymethylamino-4-methoxybenzyl-sulfone sodium salt have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with this medicine in patients with MDSs were ongoing.

At the time of submission, this medicine was not authorised anywhere in the EU for MDSs. Orphan designation of this medicine had been granted in the United States of America for MDSs.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 March 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.

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	(E)-2,4,6-trimethoxystyryl-3-carboxymethylamino-4-methoxybenzyl-sulfone sodium salt	Treatment of myelodysplastic syndromes
Bulgarian	(E)-2,4,6-триметоксистирил-3-карбоксиметиламино-4-метоксибензил-сулфон, натриева сол	Лечение на миелодиспластичен синдром
Croatian	(E)-2,4,6-trimetoksistiril-3-karboksimetilamino-4-metoksibenzil-sulfonnatrij	Liječenje mijelodisplastičnih sindroma
Czech	Sodná sůl (E)-2,4,6-trimethoxystyryl-3-carboxymethylamino-4-methoxybenzyl-sulfon	Léčba myelodysplastického syndromu
Danish	(E)-2,4,6-trimethoxystyryl-3-carboxymethylamino-4-methoxybenzyl-sulfon natriumsalt	Behandling af myelodysplastiske syndromer
Dutch	(E)-2,4,6-Trimethoxystyryl-3-carboxymethylamino-4-methoxybenzylsulfone, natriumzout	Behandeling van myelodysplastische syndromen
Estonian	(E)-2,4,6-trimetoksüstirüül-3-karboksümetüülamino-4-metoksübensüül-sulfoon naatriumi sool	Müelodüsplastiliste sündroomide ravi
Finnish	(E)-2,4,6-trimetoksistryyli-3-karboksimeetyliamino-4-metoksibentsyylisulfonin natriumsuola	Myelodysplastisten syndroomien hoito
French	(E)-2,4,6-trimethoxystyryl-3-carboxyméthylamino-4-methoxybenzylsulfone, sel de sodium	Traitement des syndromes myélodysplasiques
German	(E)-2,4,6-Trimethoxystyryl-3-carboxymethylamino-4-methoxybenzylsulfone, Natriumsalz	Behandlung der myelodysplastischen Syndrome
Greek	(E)-2,4,6-τριμεθοξυστυρυλ-3-καρβοξυμεθυλαμινο-4-μεθυξυβενζυλο-σουλφονικό άλας νατρίου	Θεραπεία των μυελοδυσπλαστικών συνδρόμων
Hungarian	(E)-2,4,6-Trimethoxystyryl-3-karboximetilamino-4-methoxybenzylsulfone, nátrium sója,	Myelodysplasias syndroma kezelése
Italian	(E)-2,4,6-Trimethoxystyryl-3-carbossimetilammino-4-methoxybenzylsulfone, sale di sodio	Trattamento delle sindromi mielodisplastiche
Latvian	(E)-2,4,6,-trimetoksistiril-3-karboksimetilamino-4-metoksibenzil-sulfona nātrija sāls	Mielodisplastisko sindromu ārstēšana

¹ At the time of transfer of sponsorship

Language	Active ingredient	Indication
Lithuanian	(E)-2,4,6-trimetoksistiril-3-karboksimetilamino-4-metoksibenzil-sulfono natrio druska	Mielodisplastinių sindromų gydymas
Maltese	Melħ tas-sodju ta' (E)-2,4,6-trimethoxystyryl-3-carboxymethylamino-4-methoxybenzyl-sulfone	Kura tas-sindromi mjelodisplastici
Polish	Sól sodowa (E)-2,4,6-trimetoksystyrenylo-3-karboksymetyloamino-4-metoksybenzylo-sulfonu	Leczenie zespołów mielodysplastycznych
Portuguese	Sal sódico de (E)-2,4,6-trimetoxistiril-3-carboximetilamino-4-metoxibenzil-sulfona	Tratamento dos síndromes mielodisplásicos
Romanian	Sare sodică a (E)-2,4,6-trimetoxistiril-3-carboximetilamino-4-metoxibenzil-sulfonei	Tratamentul sindromului mielodisplazic
Slovak	Sodná soľ (E)-2,4,6-trimetoxystryryl-3-karboxymetylaminó-4-metoxybenzylsulfónu	Liečba myelodysplastického syndrómu
Slovenian	(E)-2,4,6-trimetoksistiril-3-karboksimetilamino-4-metoksibenzil-sulfone natrij	Zdravljenje mielodisplastičnega sindroma
Spanish	Sal sódica de (E)-2,4,6-Trimethoxistiril-3-carboximetilamino-4-methoxibenzilsulfona	Tratamiento de los síndromes mielodisplásicos
Swedish	(E)-2,4,6-trimetoxystryryl-3-karboximetylaminó-4-metoxybensylsulfon, natriumsalt	Behandling av myelodysplastiska syndrom
Norwegian	(E)-2,4,6-trimetoksystryryl-3-karboksymetylaminó-4-metoksybenzylsulfon natriumsalt	Behandling av myelodysplastisk syndrom
Icelandic	(E)-2,4,6-trímethóxýstýrýl-3-carboxýmethýlamínó-4-methoxýbenzýl-súlfón natríum salt	Til meðferðar við mergmisproskaheilkenni