



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

EMA/COMP/158609/2011 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)-3,6,11',12b'-tetramethyl-2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'-icosahydro-1'H,3H-spiro[furo[3,2-b]pyridine-2,9'-naphtho[2,1-a]azulene]-3'-yl)methanesulfonamide hydrochloride] for the treatment of chondrosarcoma

First publication	19 May 2011
Rev.1: withdrawal from the Community Register	23 July 2013
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.	

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in July 2013 on request of the Sponsor.

On 13 May 2011, orphan designation (EU/3/11/859) was granted by the European Commission to Voisin Consulting S.A.R.L., France, for [N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)-3,6,11',12b'-tetramethyl-2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'-icosahydro-1'H,3H-spiro[furo[3,2-b]pyridine-2,9'-naphtho[2,1-a]azulene]-3'-yl)methanesulfonamide hydrochloride] for the treatment of chondrosarcoma.

What is chondrosarcoma?

Chondrosarcoma is cancer of the cartilage (the tissue that covers the ends of bones in joints). Chondrosarcoma predominantly affects middle-aged and older adults, and most commonly occurs in the long bones of the arms or legs. Patients with chondrosarcoma do not usually have symptoms in the early stages of the disease. Symptoms appear when the tumour grows large enough to cause swelling and pain. The bone may also ultimately break.

Chondrosarcoma is a long-term debilitating disease because it requires surgery including amputations, and is life threatening particularly when the cancer has spread to other parts of the body.



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

At the time of designation, chondrosarcoma affected less than 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 25,000 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, no satisfactory methods were authorised in the EU for the treatment of chondrosarcoma. Some patients were treated with surgery. Radiotherapy (treatment with radiation) and chemotherapy (medicines to treat cancer) were sometimes used if surgery was not possible or the disease had spread to other parts of the body.

How is this medicine expected to work?

This medicine is expected to work by blocking 'the Hedgehog signalling pathway'. This is a mechanism through which embryos control the development of their cells, but it also plays a role in the development of some cells, including cartilage cells in children and adults. When this pathway is disrupted and becomes abnormally active, it can lead to the development of cancers, including chondrosarcoma. The medicine is expected to attach to a protein called 'SMO' which is involved in the Hedgehog signalling pathway. By attaching to SMO, the medicine is expected to block this pathway, thereby slowing down the growth of the cancer cells.

What is the stage of development of this medicine?

The effects of this medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, one clinical trial with this medicine in patients with chondrosarcoma was ongoing.

At the time of submission, this medicine was not authorised anywhere in the EU for the treatment of chondrosarcoma 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 9 February 2011 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 507,700,000 (Eurostat 2011).

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	[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)-3,6,11',12b'-tetramethyl-2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'-icosahydro-1'H,3H-spiro[furo[3,2-b]pyridine-2,9'-naphtho[2,1-a]azulene]-3'-yl)methanesulfonamide hydrochloride]	Treatment of chondrosarcoma
Bulgarian	[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)-3,6,11',12b'-тетраметил-2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'-икозахидро-1'H,3H-спиро[фуро[3,2-b]пиридин-2,9'-нафто[2,1-a]азулен]-3'-yl)метансулфонамид хидрохлорид]	Лечение на хондросаркома
Czech	[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)-3,6,11',12b'-tetramethyl-2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'-icosahydro-1'H,3H-spiro[furo[3,2-b]pyridine-2,9'-naphtho[2,1-a]azulene]-3'-yl)methanesulfonamide hydrochloride]	Léčba chondrosarkomu
Danish	[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)-3,6,11',12b'-tetramethyl-2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'-icosahydro-1'H,3H-spiro[furo[3,2-b]pyridin-2,9'-naphtho[2,1-a]azulen]-3'-yl)methansulfonamid hydrochlorid]	Behandling af chondrosarkom
Dutch	[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)-3,6,11',12b'-tetramethyl-2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'-icosahydro-1'H,3H-spiro[furo[3,2-b]pyridine-2,9'-naphtho[2,1-a]azulene]-3'-yl)methanesulfonamide hydrochloride]	Behandeling van chondrosarcoma
Estonian	[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)-3,6,11',12b'-tetrametüül-2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'-ikosahüdro-1'H,3H-spiro[furo[3,2-b]püridiin-2,9'-naftho[2,1-a]azulene]-3'-yl)metaansulfoonamiid hüdrokloriid]	Kondrosarkoomi ravi
Finnish	[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)-3,6,11',12b'-tetrametyyli-2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'-ikosahydro-1'H,3H-spiro[furo[3,2-b]pyridiini-2,9'-nafto[2,1-a]atsulene]-3'-yl)metaanisulfonamidi hydrokloridi]	Kondrosarkooman hoito
French	Cloridrate de [N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)-3,6,11',12b'-tetramethyl-2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'-icosahydro-1'H,3H-spiro[furo[3,2-b]pyridine-2,9'-naphtho[2,1-a]azulene]-3'-yl)methanesulfonamide]	Traitement du chondrosarcome

¹ At the time of designation

Language	Active ingredient	Indication
German	[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)-3,6,11',12b'-tetramethyl-2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'-icosahydro-1'H,3H-spiro[furo[3,2-b]pyridine-2,9'-naphtho[2,1-a]azulene]-3'-yl)methanesulfonamide hydrochloride]	Behandlung des Chondrosarkoms
Greek	[υδροχλωρική N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)-3,6,11',12b'-τετραμεθυλ-2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'-εικοσαυδρο-1'H,3H-σπυρο[φουρο3,2-b]πυριδινό-2,9'-ναφθο[2,1-a]αζυλενο]-3'-yl)μεθανοσουλφοναμίδη]	Θεραπεία του χονδροσάρκωματος
Hungarian	[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)-3,6,11',12b'-tetrametil-2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'-icosahydro-1'H,3H-spiro[furo[3,2-b]pyridine-2,9'-naphtho[2,1-a]azulene]-3'-yl)metánszulfonamid hidroklorid]	Chondrosarcoma kezelése
Italian	[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)-3,6,11',12b'-tetramethyl-2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'-icosahydro-1'H,3H-spiro[furo[3,2-b]pyridine-2,9'-naphtho[2,1-a]azulene]-3'-yl)metansulfonamide idrocloruro]	Trattamento del condrosarcoma
Latvian	[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)-3,6,11',12b'-tetrametil-2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'-ikosahidro-1'H,3H-spiro[furo[3,2-b]pyridine-2,9'-nafto[2,1-a]azulena]-3'-yl)metāna sulfonamīda hidrohlorīds	Hondrosarkomas ārstēšana
Lithuanian	[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)-3,6,11',12b'-tetrametil-2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'-ikozahidro-1'H,3H-spiro[furo[3,2-b]piridino-2,9'-nafto[2,1-a]azuleno]-3'-il)metansulfonamido hidrochloridas]	Chondrosarkomos gydymas
Maltese	[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)-3,6,11',12b'-tetramethyl-2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'-icosahydro-1'H,3H-spiro[furo[3,2-b]pyridine-2,9'-naphtho[2,1-a]azulene]-3'-yl)methanesulfonamide hydrochloride]	Kura tal-kondrosarkoma
Polish	[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)-3,6,11',12b'-tetrametylo-2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'-ikozahydro-1'H,3H-spiro[furo[3,2-b]pirydyno-2,9'-nafto[2,1-a]azulen]-3'-yl)metanosulfonamidu chlorowodorek]	Leczenie chrzęstniaka mięsakowego
Portuguese	[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)-3,6,11',12b'-tetrametil-2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'-icosahidro-1'H,3H-spiro[furo[3,2-b] piridina-2,9'-nafto[2,1-a]azuleno]-3'-il) cloridrato de metanosulfonamida]	Tratamento do condrossarcoma

Language	Active ingredient	Indication
Romanian	[Clorhidrat de N- ((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)- 3,6,11',12b'-tetrametil- 2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'- icosahidro-1'H,3H-spiro[furo[3,2-b]piridin-2,9'-nafto[2,1- a]azulen]-3'-yl)metansulfonamidă]	Tratamentul condrosarcomului
Slovak	[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)- 3,6,11',12b'-tetrametyl- 2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'- eikozahydro-1'H,3H-spiro[furo[3,2-b]pyridín-2,9'-nafto[2,1- a]azulén]-3'-yl)metánsulfonamid hydrochlorid]	Liečba chondrosarkómu
Slovenian	[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)- 3,6,11',12b'-tetrametil- 2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'- ikosahidro-1'H,3H-spiro[furo[3,2-b]piridin-2,9'-nafto[2,1- a]azulen]-3'-il)metansulfonamid hidroklorid]	Zdravljenje hondrosarkoma
Spanish	Clorhidrato de [N- ((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)- 3,6,11',12b'-tetrametil- 2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'- icosahidro-1'H,3H-spiro[furo[3,2-b]piridina-2,9'-naphtho[2,1- a]azuleno]-3'-yl)methanesulfonamida]	Tratamiento del condrosarcoma
Swedish	[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)- 3,6,11',12b'-tetrametyl- 2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'- ikosahydro-1'H,3H-spiro[furo[3,2-b]pyridin-2,9'-nafto[2,1- a]azulen]-3'-yl)metansulfonamid hydroklorid]	Behandling av kondrosarkom
Norwegian	[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)- 3,6,11',12b'-tetrametyl- 2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'- ikosahydro-1'H,3H-spiro[furo[3,2-b]pyridin-2,9'-nafto[2,1- a]azulen]-3'-yl)metanesulfonamid hydroklorid]	Behandling av kondrosarkom
Icelandic	[N-((2S,3R,3aS,3'R,4a'R,6S,6a'R,6b'S,7aR,12a'S,12b'S,Z)- 3,6,11',12b'-tetrametyl- 2',3a,3',4,4',4a',5,5',6,6',6a',6b',7,7a,7',8',10',12',12a',12b'- ícósahýdró-1'H,3H-spiró[fúró[3,2-b]pýridín-2,9'-naphthó[2,1- a]azúlen]-3'-ýl)methanesúlfónamíð hýdróklóríð]	Meðferð við chondrósarkmeini