



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

22 March 2011
EMA/COMP/473573/2007 Rev.3
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

17-(allylamino)-17-demethoxygeldanamycin hydroquinone hydrochloride for the treatment of malignant gastrointestinal stromal tumours

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in January 2011 on request of the Sponsor.

On 29 November 2007, orphan designation (EU/3/07/512) was granted by the European Commission to MedImmune Oncology, Inc., Netherlands, for 17-(allylamino)-17-demethoxygeldanamycin hydroquinone hydrochloride for the treatment of malignant gastrointestinal stromal tumours.

The sponsorship was transferred to AstraZeneca AB, Sweden, in June 2008 and subsequently to Voising Consulting S.A.R.L., France, in April 2009.

What are malignant gastrointestinal stromal tumours?

Malignant gastrointestinal stromal tumours (GIST) belong to a special group of gastro-intestinal tumours, called sarcomas. Sarcomas may occur also in other soft tissues of the body; they are cancers of the tissues that support, surround and protect the organs. The cause of GIST is largely unknown. GIST are most common in the stomach (60-70%), followed by the small intestine (20-30%), and then the colon and rectum (5%). GIST occur predominantly in middle-aged and older persons, and are considered as a life-threatening condition.

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

At the time of designation, GIST affected approximately 1.3 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 65,000 people, and is below the threshold 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).

*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. This represents a population of 498,000,000 (Eurostat 2006).



What treatments are available?

The current methods of treatment of GIST are surgical removal of the tumour, and treatment with two (authorised) medicinal products, which belong to a group of substances called tyrosine kinase inhibitors. These products are aimed at slowing down and potentially stopping tumour growth.

17-(allylamino)-17-demethoxygeldanamycin hydroquinone hydrochloride could be of potential significant benefit for the treatment of malignant GIST, mainly because it could act in a different way to currently authorised products, and thus might improve the long-term outcome of the patients. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

17-(allylamino)-17-demethoxygeldanamycin hydroquinone hydrochloride acts by blocking the function of a protein (a chemical substance within the cells) called HSP90. This protein is involved in the growth and division processes of the cells; by blocking its action, the medicinal product is expected to slow down or stop the growth of GIST.

What is the stage of development of this medicine?

The effects of 17-(allylamino)-17-demethoxygeldanamycin hydroquinone hydrochloride were evaluated in experimental models.

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

The medicinal product was not authorised anywhere in the world for the treatment of GIST, or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 10 October 2007 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	17-(allylamino)-17-demethoxygeldanamycin hydroquinone, hydrochloride	Treatment of malignant gastro intestinal stromal tumours
Bulgarian	17-(алиламино)-17-диметоксигелданамицин хидроквинон, хидрохлорид	Лечение на злокачествени гастроинтестинални стромални тумори
Czech	17-allylamino-17-demethoxygeldanamycinhydrochloride	Léčba gastrointestinálních stromálních tumorů
Danish	17-(allylamino)-17-demethoxygeldanamycin hydroquinon, hydrochlorid	Behandling af malign gastrointestinal stromal tumor
Dutch	17-(allylamino)-17-demethoxygeldanamycin hydroquinone hydrochloride	Behandeling van kwaadaardige gastro-intestinale stromale tumoren
Estonian	17-(allüülamiin)-17-demetoksügeldanamütsiin hüdrokinoon, hüdrokloriid	Pahaloomuliste seedetrakti stroomaalsete kasvajate ravi.
Finnish	17-(allyyliamino)-17-demetoksyyligeldanamysiini hydrokinoni, hydrokloridi	Ruuansulatuskanavan pahanlaatuisten stroomatuumorien hoito
French	Chlorhydrate de 17-(allylamino)-17-deméthoxygeldanamycine hydroquinone	Traitement des tumeurs malignes stromales gastrointestinales
German	17-(allylamino)-17-demethoxygeldanamycin-hydroquinon-hydrochlorid	Behandlung von bösartigen gastrointestinalen Stromatumoren
Greek	17-(αλλυλαμινo)-17-δεμεθοξυγκελνταναμικιν υδροκινόνη, υδροχλωριούχος	Θεραπευτική αγωγή των κακοήθων γαστρεντερικών στρωματικών όγκων
Hungarian	17-(allilamino)-17-demetoxigeldanamicin-hidrokinon, hidroklorid	Rosszindulatú gasztrointesztinális stromalis tumorok kezelése
Italian	17-(allilamino)-17-demetossigeldanamicina idrochinone cloridrato	Trattamento dei tumori stromali gastrointestinali maligni
Latvian	17-(alilamino)-17-demetoksigeldanamicīna hidrohinons, hidrohlorīds	Ļaundabīga kuņģa-zarnu trakta stromas audzēju terapija

¹ At the time of designation

Language	Active Ingredient	Indication
Lithuanian	17-(alilamino)-17-demetoksigeldanamicino hidrochinonas, hidrochloridas	Skrandžio ir žarnyno stromos piktybių auglių gydymas
Maltese	17-(allylamino)-17-demethoxygeldanamycin hydroquinone, hydrochloride	Kura ta' tumuri malinni gastrointestinali li jiżviluppaw fit-tessuti konnettivi
Polish	Hydrochinon 17-alliloamino-17-demetoksygeldanamycyny, chlorowodorek	Leczenie złośliwych nowotworów podścieliska przewodu pokarmowego
Portuguese	17-alilamino-17-demetoxigeldanamicina-hidroquinona, cloridrato	Tratamento de tumores estromais gastrintestinais malignos
Romanian	clorhidrat de 17-(alilamină)-17-demetoxigeldanamicină hidrochinonă	Tratamentul tumorilor stromale gastrointestinale maligne
Slovak	Chlorid 17-alylamino-17-demetoxygeldanamycinhydrochinónu	Liečba malígnych gastrointestinálnych stromatóznych nádorov
Slovenian	17-(alilamino)-17-demetoksigeldanamicin hidrokinon, hidroklorid	Zdravljenje malignih gastrointestinalnih stromalnih tumorjev
Spanish	17-(alilamino)-17-demetoxigeldanamicina-hidroquinona, clorhidrato	Tratamiento de los tumores malignos del estroma gastrointestinal
Swedish	17-(allylamino)-17-demetoxygeldanamycin hydroquinon, hydroklorid	Behandling av maligna gastrointestinala stromala tumörer
Norwegian	17-(allylamino)-17-demetoksygeldanamycinhydrokinon, hydroklorid	Behandling av maligne gastrointestinale stromale tumorer
Icelandic	17-(allýlamínó)-17-demethoxýgeldanamýcín hýdrókínón, hýdróklóríð	Til meðferðar við grunnfrumuæxlum í meltingarfærum