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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

5-(ethylsulfonyl)-2-(naphthalen-2-yl)benzo[d]oxazole for the treatment of Duchenne muscular dystrophy

First publication	22 June 2009
Rev.1: transfer of sponsorship	17 May 2011
Rev.2: sponsor's change of address	4 February 2014

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.

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On 4 December 2008, orphan designation (EU/3/08/591) was granted by the European Commission to Summit (Oxford) Limited, UK, for 5-(ethylsulfonyl)-2-(naphthalen-2-yl)benzo[d]oxazole for the treatment of Duchenne muscular dystrophy.

The sponsorship was transferred to BioMarin Europe Ltd., United Kingdom, in May 2009 and subsequently to Summit (Oxford) Limited, United Kingdom, in March 2011.

What is Duchenne muscular dystrophy?

Duchenne muscular dystrophy (DMD) is an inherited genetic disease characterised by progressive weakening of the muscles. It mainly affects boys, usually before the age of six years. The muscle weakness usually starts in the hips and legs, before reaching the chest, arms, and possibly the heart. Patients with DMD do not produce enough of a protein called dystrophin. As dystrophin is an important component of muscle fibres, the muscles of patients with DMD cannot grow, so they become weak and eventually stop working.

Duchenne muscular dystrophy causes long-term disability and is life-threatening because of its effects on the heart and on respiratory muscles (muscles that are used to breathe).

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

At the time of designation, DMD affected approximately 0.3 in 10,000 people in the European Union (EU). This was below the threshold for orphan designation, which is 5 in 10,000, and is equivalent to a total of around 15,000 people*. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of submission of the application for orphan drug designation, there were no treatments available in the EU that could cure DMD. The main treatments for the disease were corticosteroids to slow down the progression of the disease, and physiotherapy, which was used to relieve symptoms and improve the patient's general condition.

The sponsor has provided sufficient information to show that 5-(ethylsulfonyl)-2-(naphthalen-2-yl)benzo[d]oxazole might be of potential significant benefit for patients because of the way the medicine is expected to work. This could improve the overall outcome of patients with DMD. 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 medicine is thought to promote the production of utrophin A, a protein that is similar to dystrophin. This protein is expected have a similar effect to dystrophin on muscles and therefore relieve the symptoms of DMD.

What is the stage of development of this medicine?

The effects of 5-(ethylsulfonyl)-2-(naphthalen-2-yl)benzo[d]oxazole have been evaluated in experimental models.

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

At the time of submission, 5-(ethylsulfonyl)-2-(naphthalen-2-yl)benzo[d]oxazole was not authorised anywhere in the world for DMD or designated as 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 October 2008 recommending the granting of this designation.

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* 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 502,800,000 (Eurostat 2008).

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.

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Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active Ingredient	Indication
English	5-(ethylsulfonyl)-2-(naphthalen-2-yl)benzo[d]oxazole	Treatment of Duchenne muscular dystrophy
Bulgarian	5-(етилсулфонил)-2-(нафтален-2-ил)бензо[d]оксазол	Лечение на мускулна дистрофия на Duchenne
Czech	5-(etylsulfonyl)-2-(naftalen-2-yl)benzo[d]oxazol	Léčba pacientů s Duchennovou muskulární dystrofií
Danish	5-(ethylsulfonyl)-2-(naphthalen-2-yl)benzo[d]oxazol	Behandling af Duchenne muskeldystrofi
Dutch	5-(ethylsulfonyl)-2-(naftaleen-2-yl)benzo[d]oxazool	Behandeling van Duchenne spierdystrofie
Estonian	5-(etüülsulfonüül)-2-(naftaleen-2-üül)benso[d]oksasool	Duchenne'i lihasdüstroofia ravi
Finnish	5-(etyylisulfonyyli)-2-(naftaleeni-2-yyli)bentso[d]oksatsoli	Duchennen lihasdystrofian hoito
French	5-(éthyl sulfonyl)-2-(naphthalène-2-yl)benzo[d]oxazole	Traitement de la dystrophie musculaire de Duchenne
German	5-(Ethylsulfonyl)-2-(naphthalen-2-yl)benzo-[d]-oxazol	Behandlung der Duchenne-Muskeldystrophie
Greek	5-(αιθυλοσουλφονυλο)-2-(ναφθαλεν-2-υλ)βενζο[d]οξαζόλη	Θεραπεία της μυϊκής δυστροφίας Duchenne
Hungarian	5-(etilszulfonil)-2-(naftalin-2-il)benzo[d]oxazol	Duchenne dystrophia kezelése
Italian	5-(etilsulfonil)-2-(naftalen-2-il)benzo[d]ossazolo	Trattamento di distrofia muscolare di tipo Duchenne
Latvian	5-(etilsulfonil)-2-(naftalēn-2-il) benzo[d]oksazols	Dišēna muskuļu distrofijas ārstēšana
Lithuanian	5-(etilsulfonil)-2-(naftalen-2-il)benzo[d]oksazolas	Duchenne (Diušeno) raumenų distrofijos gydymas
Maltese	5-(ethylsulfonyl)-2-(naphthalen-2-yl)benzo[d]oxazole	Kura tad-distrofija muskolari tat-tip Duchenne
Polish	5-(etylosulfonylo)-2-(naftalen-2-ylo)benzo[d]oksazol	Leczenie zaniku mięśni typu Duchenne'a
Portuguese	5-(etilsulfonil)-2-(naftaleno-2-il)benzo[d]oxazol	Tratamento da distrofia muscular de Duchenne
Romanian	5-(etilsulfonil)-2-(naftalen-2-il) benzo[d]oxazol	Tratamentul distrofiei musculare Duchenne
Slovak	5-(etylsulfonyl)-2-(naftalén-2-yl)benzo[d]oxazol	Liečba Duchennovej muskulárnej dystrofie
Slovenian	5-(etilsulfonil)-2-(naftalen-2-il)benzo[d]oksazol	Zdravljenje Duchennove mišične distrofije
Spanish	5-(etilsulfonil)-2-(naftalen-2-il)benzo[d]oxazol	Tratamiento de la distrofia muscular de Duchenne

[1] At the time of designation

Language	Active Ingredient	Indication
Swedish	5-(etylsulfonyl)-2-(naftalen-2-yl)benzo[d]oxazol	Behandling av Duchennes muskeldystrofi
Norwegian	5-(etylsulfonyl)-2-(naftalen-2-yl)benzo[d]oksazol	Behandling av Duchennes muskeldystrofi
Icelandic	5-(etýlsúlfónýl)-2-(naftalen-2-ýl)benzó[d]oxazól	Meðferð á Duchenne vöðvarýrnun

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