



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Recombinant human arylsulfatase A for the treatment of metachromatic leukodystrophy

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in September 2011 on request of the sponsor.

On 9 July 2003, orphan designation (EU/3/03/158) was granted by the European Commission to HemeBiotech A/S, Denmark, for recombinant human arylsulfatase A for the treatment of metachromatic leukodystrophy.

The sponsorship was transferred to Shire Pharmaceuticals Ireland Limited, Ireland, in June 2008.

What is metachromatic leukodystrophy?

Metachromatic leukodystrophy is a chronic congenital disease. It is due to the lack of an enzyme, the arylsulfatase A. The result is the accumulation of certain substances called sulfatides, particularly in the cells of the nervous system. The nervous system is progressively damaged as a result. The damage causes walking difficulties, mental deterioration and other severe impairments of the nervous system. The disease is life-threatening especially in the early onset of the disease.

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

At the time of designation, metachromatic leukodystrophy affected approximately 0.01 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 400 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).

*Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 385,000,000 (Eurostat 2002) and may differ from the true number of patients affected by the condition.



What treatments are available?

No satisfactory methods exist that were authorised at the time of application. Only symptomatic treatments were available and in some cases, bone marrow transplant has been used.

How is this medicine expected to work?

Recombinant human arylsulfatase A is expected to replace the missing enzyme, avoiding the accumulation of sulfatides and this may be useful for the treatment of the disease.

What is the stage of development of this medicine?

The effects of recombinant human arylsulfatase A were evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients with metachromatic leukodystrophy were initiated.

At the time of submission, recombinant human arylsulfatase A was not marketed anywhere worldwide for metachromatic leukodystrophy 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 13 June 2003 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	Recombinant Human Arylsulfatase A	Treatment of metachromatic leukodystrophy
Bulgarian	Рекомбинантна човешка арилсулфатаза А	Лечение на метакроматична левкодистрофия
Czech	Rekombinantní lidská arylsulfatáza A	Léčba metachromatické leukodystrofie
Danish	Rekombinant human arylsulfatase A	Behandling af metakromatisk leukodytrofi
Dutch	Recombinant humaan arylsulphatase A	Behandeling van metachromatische leukodystrophie
Estonian	Rekombinantne inimese arülsulfataas A	Metakromaatilise leukotsütoosi ravi
Finnish	Rekombinantti humaani aryylisulfataasi A	Metakromaattisen leukodystrofian hoito
French	Arylsulphatase A recombinante humaine	Traitement de la leucodystrophie métachromatique
German	Recombinante menschliche arylsulfatase A	Behandlung der Metachromatischen Leukodystrophie
Greek	Ανασυνδρασμένη ανθρώπινη αρυλοσουλφατάση Α	Θεραπεία μεταχρωματικής λευκοδυστροφίας
Hungarian	Rekombinációs humán arylsulfatase A	Metachromasiás leukodystrophia kezelése
Italian	Arylsulfatasi A umana ricombinante	Trattamento della leucodistrofia metacromatica
Latvian	Rekombinētās cilvēka arilsulfatāze A	Metahromas leikodistrofijas ārstēšanai
Lithuanian	Rekombinantinis žmogaus arilsulfatazė A	Metachrominės leikodistrofijos gydymas
Maltese	<i>Arylsulfatase A</i> uman rikombinanti	Kura tal-leukodistrofija metakromatika
Polish	Ludzka rekombinowana arylosulfataza A	Leczenie zespołu leukodystrofii metachromatycznej
Portuguese	Arilsulfatase A Recombinante Humana	Tratamento da Leucodistrofia Metacromática
Romanian	Arilsulfatază A umană recombinantă	Tratamentul leucodistrofiei metacromatice
Slovak	Rekombinantná ľudská arylsulfatáza A	Liečba metachromatickej leukodystrofie
Slovenian	Rekombinantna humana arilsulfataza A	Zdravljenje metakromatske levkodistrofije
Spanish	Arilsulfatasa A recombinante humana	Tratamiento de la leucodistrofia metacromática
Swedish	Recombinant human arylsulfatase A	Behandling av metakromatisk leukodytrofi

¹ At the time of transfer of sponsorship