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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Alpha-1 proteinase inhibitor (for inhalation use) for the treatment of congenital alpha-1 antitrypsin deficiency

First publication	18 August 2008
Rev.1: sponsor's name change	19 April 2012
Rev.2: administrative update	30 April 2012
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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 3 June 2008, orphan designation (EU/3/08/546) was granted by the European Commission to Talecris Biotherapeutics GmbH, Germany, for alpha-1 proteinase inhibitor (for inhalation use) for the treatment of congenital alpha-1 antitrypsin deficiency.

In February 2012, Talecris Biotherapeutics GmbH changed name to Grifols Deutschland GmbH.

What is congenital alpha-1 antitrypsin deficiency?

Congenital alpha-1 antitrypsin deficiency is an inherited disease that is characterised by a lack (deficiency) of a protein in the blood called 'alpha-1 proteinase inhibitor' or 'alpha-1 antitrypsin' (AAT). AAT is produced in the liver and its main function is to control another protein called elastase. Elastase is an enzyme that breaks down a cell constituent, elastin, which is present in the lungs. Because AAT is missing in patients with congenital alpha-1 antitrypsin deficiency, elastase can accumulate in the lungs, and the patients can develop a lung disease called emphysema, resulting in shortness of breath, coughing and wheezing.

Congenital AAT deficiency is a debilitating disease that is long lasting and can be life-threatening.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

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

At the time of designation, congenital AAT deficiency affected approximately 2.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 126,000 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 orphan drug designation, treatment for lung disease due to AAT deficiency included medicines that help patients to breathe, oxygen, and medicines containing human AAT given as an injection into a vein. In the most severe cases of lung disease, a lung transplant might be considered.

The sponsor has provided sufficient information to show that the assumption that alpha-1 proteinase inhibitor (for inhalation use) might be of significant benefit for patients with congenital AAT deficiency because it can be administered by breathing.

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?

Alpha-1 proteinase inhibitor (for inhalation use) contains human AAT in a form that can be breathed directly into the lungs. In the lungs, it works in the same way as AAT to replace the missing enzyme and stopping the accumulation of elastase. This helps to reduce the symptoms of lung disease due to AAT deficiency.

What is the stage of development of this medicine?

The effects of alpha-1 proteinase inhibitor (for inhalation use) have been evaluated in experimental models. At the time of submission of the application for orphan designation, no clinical trials in patients with congenital AAT deficiency were ongoing.

At the time of submission, alpha-1 proteinase inhibitor (for inhalation use) was not authorised anywhere in the world for congenital AAT deficiency 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 April 2008 recommending the granting of this designation.

*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:

Grifols Deutschland GmbH
Lyoner Str. 15
D-60528 Frankfurt am Main
Germany
Tel.: +49 69 66 05 93 401
Fax: + 49 69 66 05 93 110
E-mail: wolfgang.schulten@grifols.com

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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Alpha-1 proteinase inhibitor (for inhalation use)	Treatment of congenital alpha-1 antitrypsin deficiency
Bulgarian	Инхибитор на алфа-1 протеиназа (за инхалационна употреба)	Лечение на вроден алфа-1 антитрипсинов дефицит
Czech	Inhibitor alfa-1 proteinázy (určeno k inhalaci)	Léčba vrozeném deficitu alfa-1 antitrypsinu
Danish	Alfa1-proteinasehæmmer (til inhalation)	Behandling af medfødt alfa-1 antitrypsinmangel
Dutch	Alfa-1 proteinaseremmer (voor inhalatiegebruik)	Behandeling van aangeboren alfa-1 antitrypsine deficiëntie
Estonian	Alfa-1 proteinaasi inhibiitor (inhalatsiooniks)	Kaasasündinud alfa-1 antitrüpsiini puudulikkuse ravi
Finnish	Alfa-1-proteaasinestäjä (inhalaatiokäyttöön)	Synnynnäisen alfa-1 antitrypsiinin puutteen hoito
French	Inhibiteur de la protéinase alpha-1 (voie inhalée)	Traitement du déficit congénital en alpha-1 antitrypsine
German	Alpha-1-Proteinase-Inhibitor (zur Inhalation)	Behandlung von erblichem Alpha-1 Antitrypsinmangel
Greek	Αναστολέας της α-1 πρωτεϊνάσης (για χορήγηση μέσω της αναπνευστικής οδού)	Θεραπεία της συγγενούς ανεπάρκειας άλφα-1 αντιθρυψίνης
Hungarian	Alfa-1 proteáz inhibitor (inhalációs készítmény)	Kongenitális alfa-1 antitripszin hiány kezelése
Italian	Inibitore dell'alfa-1 proteinasi (per uso inalatorio)	Trattamento del deficit congenito di alfa-1 antitripsina
Latvian	Alfa-1 proteināzes inhibitors (inhalāciju veikšanai)	Iedzimta alfa -1 antitripsīna deficīta ārstēšana
Lithuanian	Alfa-1 proteinazės inhibitorius (inhaliuoti)	Įgimtas alfa -1 antitripsino deficito gydymas
Maltese	Inibitur ta' l-alpha-1 proteinase (għal biex jingibed man-nifs)	Kura tan-nuqqas konġenitu ta' l-alpha-1 antitrypsin
Polish	Inhibitor proteinazy alfa 1 (do inhalacji	Leczenie wrodzonego niedoboru alfa-1antytrypsyny
Portuguese	Inibidor de Alfa-1 Proteinase (para uso de inhalational)	Tratamento da deficiência congénita em antitripsina alfa-1
Romanian	Inhibitor al alfa-1 proteinazei (administrare inhalatorie)	Tratamentul deficitului congenital de alfa-1 antitripsină
Slovak	Inhibítor alfa-1 proteinázy (na inhalačné použitie)	Liečba vrodenom deficitu alfa-1 antitrypsínu
Slovenian	Inhibitor al alfa-1 proteinazei (pentru inhalational folos)	Zdravljenje kongenitalnega pomanjkanja alfa-1 antitripsina

[1] At the time of designation

Language	Active ingredient	Indication
Spanish	Inhibidor de la alfa 1 proteinasa (vía inhalatoria)	Tratamiento del déficit congénito de alfa-1antitripsina
Swedish	Alfa-1-proteinashämmare (användning för inhalation)	Behandling av kongenital alpha-1 antitrypsin brist
Norwegian	alfa-1-proteinasehemmer (bruk til inhalasjon)	Behandling av alfa-1 antitrypsinmangel
Icelandic	Alfa-1 próteinasahemill (til innöndunar)	Meðferð á meðfæddum alfa-1 andtrýpsínskorti