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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Tafamidis for treatment of senile systemic amyloidosis

On 8 November 2012, orphan designation (EU/3/12/1066) was granted by the European Commission to Pfizer Limited, United Kingdom, for tafamidis for the treatment of senile systemic amyloidosis.

What is senile systemic amyloidosis?

Senile systemic amyloidosis is an age-related type of amyloid disease, in which deposits of proteins (called amyloids) accumulate in the heart and, more rarely, in other organs.

The disease mainly affects the elderly, particularly elderly men, and is linked to ageing. As people age, a protein called transthyretin, which is involved in the transport of substances in the blood, starts to break up and lose its function. The broken up parts of the protein then form deposits that accumulate as amyloids in the heart, where they can cause damage to the heart muscle.

Senile systemic amyloidosis is a life-threatening disease because of the severe damage to the heart that leads to heart failure.

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

At the time of designation, senile systemic amyloidosis affected approximately 3 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 152,000 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 treating senile systemic amyloidosis. Treatments focused at improving the functioning of the heart, and included diuretics and pacemakers.

[*]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 506,300,000 (Eurostat 2011).

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
An agency of the European Union

How is this medicine expected to work?

Tafamidis is expected to attach to and stabilise the transthyretin protein. The normal form of the protein is as a homotetramer, which means that it is formed of four identical protein chains linked to each other. By attaching to transthyretin, the medicine is expected to keep the protein in its homotetramer form, preventing it from breaking up and thereby decreasing the amount of harmful amyloid deposits in the heart.

What is the stage of development of this medicine?

The effects of tafamidis have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with tafamidis in patients with senile systemic amyloidosis were ongoing.

At the time of submission, tafamidis was authorised in the EU for the treatment of transthyretin amyloidosis.

At the time of submission, tafamidis was not authorised anywhere in the EU for senile systemic amyloidosis. Orphan designation of tafamidis had been granted in the United States of America for symptomatic transthyretin amyloid cardiomyopathy.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 October 2012 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:

Pfizer Limited
Ramsgate Road
Sandwich
Kent CT13 9NJ
United Kingdom
Telephone: +44 1304 616161
Telefax: +44 1304 656 221
E-mail: ORPHAN_Enquiries@pfizer.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 substance	Indication
English	Tafamidis	Treatment of senile systemic amyloidosis
Bulgarian	Тафамидис	Лечение на сенилна системна амилоидоза
Czech	Tafamidis	Léčba senilní systemové amyloidózy
Danish	Tafamidis	Behandling af senil systemisk amyloidose
Dutch	Tafamidis	Behandeling van seniele systemische amyloïdosis
Estonian	Tafamidis	Süsteemse seniilse amüloidoosi ravi
Finnish	Tafamidis	Ikääntymiseen liittyvän systeemisen amyloidoosin hoito
French	Tafamidis	Traitement de l'amylose systémique sénile
German	Tafamidis	Behandlung der senilen systemischen Amyloidose
Greek	Tafamidis	Θεραπεία της γεροντικής συστηματικής αμυλοείδωσης
Hungarian	Tafamidisz	Időskori szisztémás amyloidosis kezelése
Italian	Tafamidis	Trattamento dell' amiloidosi sistemica senile
Latvian	Tafamidis	Senilas sistēmiskas amiloidozes ārstēšana
Lithuanian	Tafamidis	Senatvinės sisteminės amiloidozės gydymas
Maltese	Tafamidis	Kura tal-amilojdosi sistemika senili
Polish	Tafamidis	Leczenie starczej amyloidozy układowej
Portuguese	Tafamidis	Tratamento da amiloidose sistémica senil
Romanian	Tafamidis	Tratamentul amiloidozei sistemice senile
Slovak	Tafamidis	Liečba senilnej systémovej amyloidózy
Slovenian	Tafamidis	Zdravljenje starostne sistemske amiloidoze
Spanish	Tafamidis	Tratamiento de la amiloidosis sistémica senil
Swedish	Tafamidis	Behandling av senil systemamyloidos (SSA)
Norwegian	Tafamidis	Behandling av senil systemisk amyloidose
Icelandic	Tafamidis	Meðferð við aldurstengdu altæku mýlildi

[1] At the time of designation