EMA/COMP/249/2004 Rev.3
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

1,3-propanedisulfonic acid, disodium salt for the treatment of systemic secondary amyloidosis

First publication	13 May 2009
Rev.1: sponsor's name change	11 June 2010
Rev.2: transfer of sponsorship	7 March 2011
Rev.3: transfer of sponsorship	13 September 2013

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 31 July 2001, orphan designation (EU/3/01/051) was granted by the European Commission to Quintiles Limited, United Kingdom, for 1,3-propanedisulfonic acid, disodium salt for the treatment of systemic secondary amyloidosis.

The sponsorship was transferred to Neurochem Luxco II S.A.R.L, Luxembourg on September 2005.

The sponsor changed its name to BELLUS Health Luxo II S.à r.l. in February 2010.

The sponsorship was transferred to Kiacta Europe Ltd, United Kingdom, in December 2010 and subsequently to Phinco S.à r.l. in July 2013.

What is systemic secondary amyloidosis?

Amyloid is the name of a group of proteins that can be found in the body. These proteins are attached to sugar units, and are similar to starch (a protein found in plants). Normally, these proteins easily melt in water and liquids of the body, like in the blood. In some cases, however, the amyloid proteins lose their shape, become solid and deposit in certain parts of the body, which is unable to degrade them. These deposits can be harmful to organs in the body and this condition is called amyloidosis. To make the diagnosis a biopsy is required (a small piece of tissue is taken from the body and studied with a microscope).

The symptoms of amyloidosis depend on the organs affected and on the extent of the deposit. In systemic amyloidosis deposits are found throughout the body. Systemic amyloidosis can be classified into three major types: primary systemic amyloidosis, secondary systemic amyloidosis and hereditary

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systemic amyloidosis. Secondary systemic amyloidosis occurs in patients who have a chronic infection or inflammatory disease such as tuberculosis, a genetic disease called familial Mediterranean fever, bone infections (osteomyelitis), rheumatoid arthritis, inflammation of the small intestine (granulomatous ileitis), Hodgkin's disease or leprosy. The amyloid tissue deposits in secondary amyloidosis consist of AA amyloid proteins. Systemic secondary amyloidosis is a life-threatening condition.

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

At the time of designation, systemic secondary amyloidosis affected approximately 1.7 in 10,000 people in the European Union (EU). This was equivalent to a total of around 64,000 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).

What treatments are available?

At the time of submission of application for orphan drug designation there was no treatment of systemic secondary amyloidosis in the European Union except colchicine, which was authorised for treatment of familial Mediterranean fever.

Satisfactory argumentation has been submitted by the sponsor to justify the assumption that 1,3-propanedisulfonic acid, disodium salt might be of potential significant benefit for the treatment of systemic secondary amyloidosis. The 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?

The AA amyloid proteins that cause systemic secondary amyloidosis are protected from degradation because they are hidden within hard structures called fibrillar aggregate deposits. These contain different substances such as sulfated glycosaminoglycans. 1,3-propanedisulfonic acid is expected to compete with sulfated glycosaminoglycans, and thus prevent formation of aggregates improving the symptoms of the disease.

What is the stage of development of this medicine?

The effects of 1,3-propanedisulfonic acid, disodium salt were evaluated in experimental models.

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

1,3-propanedisulfonic acid, disodium salt was not marketed anywhere worldwide for treatment of systemic secondary amyloidosis, at the time of submission. Orphan designation of 1,3-propanedisulfonic acid, disodium salt was granted in April 1999 in the United States for the treatment of secondary (AA) amyloidosis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 June 2001 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.
At the time of designation, this represented a population of 378,800,000 (Eurostat 2001).

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 European Union) 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 languages[1], Norwegian and Icelandic

Language	Active Ingredient	Indication
English	1,3-Propanedisulfonic acid, disodium salt	Treatment of systemic secondary Amyloidosis
Bulgarian	Двунатриева сол на 1,3-пропандисулфоновата киселина	Лечение на системна вторична амилоидоза
Croatian	Dinatrijev 1,3-propandisulfonat	Liječenje sistemne sekundarne amiloidoze
Czech	Dvojsodná sůl kyseliny 1,3-propandisulfonové	Léčba systémové sekundární amyloidosy
Danish	1,3-Propandisulfonylsyre, dinatrium salt	Behandling af systemisk sekundær amyloidose.
Dutch	Dinatriumzout van 1,3-propaandisulfonzuur	Behandeling van systemische secundaire amyloïdose
Estonian	1,3-propaandisulfoonhappe dinaatiumsool	Süsteemse sekundaarse amüloidoosi ravi
Finnish	1,3-propaanidisulfonihappo, dinatriumsuola	Sekundaarisen systeemisen amyloidoosin hoito
French	Acide 1,3-propanedisulfonique, sel disodique	Traitement de l'amyloïdose systémique secondaire
German	Di-Natrium-1,3 Propandisulfonat	Behandlung systemischer sekundärer Amyloidose
Greek	1,3-Προπανδισουλφονικό οξύ, δινατριούχο άλας	Θεραπεία δευτερογενούς συστηματικής αμυλοείδωσης
Hungarian	Dinátrium 1,3-propándiszulfonsav	Szisztémás secunder amyloidosis
Italian	Acido 1,3-Propanodisulfonico, sale disodico	Trattamento dell'amiloidosi sistemica secondaria
Latvian	1,3-propāndisulfonskābe, dinātrija sāls	Sekundāras sistēmiskas amiloidozes ārstēšana
Lithuanian	1,3-propano disulfonrūgštis, dinatrio druska	Sisteminės antrinės amiloidozės gydymas
Maltese	1,3-Propanedisulfonic acid, disodium salt	Kura ta' l-amilojdosi sistemika sekondarja
Polish	Sól disodowa kwasu 1,3-propandisulfonowego	Leczenie wtórnej układowej amyloidozy
Portuguese	Ácido 1,3 propanodisulfónico, sal disódico	Tratamento da amiloidose sistémica secundária
Romanian	Sare disodică a acidului 1,3-propandisulfonic	Tratamentul amiloidozei secundare sistemice
Slovak	Dvojsodná soľ 1,3-propándisulfónovej kyseliny	Liečba systémovej sekundárnej amyloidózy
Slovenian	Dinatrijev 1,3-propandisulfonat	Zdravljenje sekundarne sistemske amiloidoze
Spanish	Ácido 1,3 propanodisulfónico, sal disódica	Tratamiento de la amiloidosis secundaria sistémica

[1] At the time of transfer of sponsorship

Language	Active Ingredient	Indication
Swedish	1,3-Propandisulfonsyra, dinatriumsalt	Behandling av systemisk sekundär amyloiddegeneration