



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Cardiotrophin-1 for the treatment of acute liver failure

On 30 August 2011, orphan designation (EU/3/11/893) was granted by the European Commission to Digna Biotech S.L., Spain, for cardiotrophin-1 for the treatment of acute liver failure.

What is acute liver failure?

Acute liver failure is the sudden loss of the normal liver functions in a patient with a previously normal liver and without evidence of chronic liver disease. The most common first sign of liver failure is yellowing of the skin (jaundice). Acute liver failure has serious complications such as bruising and bleeding due to impaired blood clotting as well as cerebral oedema (swelling around the brain), convulsions (fits) and coma. The most common causes of acute liver failure in the EU are toxic damage (e.g. due to consumption of large amounts of alcohol or overdose of drugs such as paracetamol) or viral hepatitis (an infectious disease that affects the liver).

Acute liver failure is a long-term debilitating and life-threatening disease because of its damaging effects on the brain and other organs.

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

At the time of designation, acute liver failure affected less than 1 in 10,000 people in the European Union (EU)*. This is equivalent to a total of fewer than 50,000 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, the main treatment option for acute liver failure was transplantation. Patients with acute liver failure caused by paracetamol overdose can be treated with N-acetylcysteine. The sponsor has provided sufficient information to show that cardiotrophin-1 might be of significant benefit for patients with acute liver failure because it works in a different way to existing treatments

*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).



and might reduce or delay the need for liver transplantation. These assumptions 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?

Cardiotrophin-1 is a naturally occurring chemical messenger in the blood that has antioxidant (a molecule that prevents the oxidation of other molecules) and anti-inflammatory properties. Liver cells actively produce cardiotrophin-1 as part of the defence mechanism against injury.

The medicine is expected to work the same way as naturally occurring cardiotrophin-1 by acting as a messenger to the damaged liver cells, helping them to recover and restoring their function.

What is the stage of development of this medicine?

The effects of cardiotrophin-1 have been evaluated in experimental models was ongoing.

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with acute liver failure had started.

At the time of submission, the medicine was not authorised anywhere in the EU for acute liver failure or designated as an 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 10 June 2011 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:

Digna Biotech S.L.
Avenida Pio XII, 22
Oficina 2
31008 Pamplona
Navarra
Spain
Telephone: + 34 91 18 52 510
Telefax: + 34 91 18 52 519
E-mail: info@dignabiotech.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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Cardiotrophin-1	Treatment of acute liver failure
Bulgarian	Кардиотрофин - 1	Лечение на остра чернодробна недостатъчност
Czech	Kardiotropin-1	Léčba Akutní jaterní insuficience
Danish	Kardiotrophin-1	Behandling af akut leversvigt
Dutch	Cardiotrofine-1	Behandeling van acuut leverfalen
Estonian	Kardiotrofiin-1	Akuutse maksapuudulikkuse ravi
Finnish	Kardiotropiini-1	Akuutin maksan vajaatoiminnan hoito
French	Cardiotrophine-1	Traitement de l'insuffisance hépatique aiguë
German	Cardiotrofine-1	Behandlung des akuten Leberversagens
Greek	καρδιотροφίνη-1	Θεραπεία της οξείας ηπατικής ανεπάρκειας
Hungarian	Cardiotrophin-1	Akut májelégtelenség kezelése
Italian	Cardiotrofina-1	Trattamento della insufficienza epatica acuta
Latvian	Kardiotrofīns-1	Akūtās aknu mazspējas ārstēšana
Lithuanian	Kardiotrofinas-1	Ūmaus kepenų nepakankamumo gydymas
Maltese	Cardiotrophin-1	Kura ta' insuffiċjenza akuta tal-fwied
Polish	Kardiotropina-1	Leczenie ostrej niewydolności wątroby
Portuguese	Cardiotrofina-1	Tratamento da insuficiência hepática aguda
Romanian	Cardiotrofina-1	Tratamentul insuficienței hepatice acute
Slovak	Kardiotrofín-1	Liečba akútneho zlyhania pečene
Slovenian	Kardiotrofin-1	Zdravljenje akutne jetrne odpovedi
Spanish	Cardiotrofina-1	Tratamiento de la insuficiencia hepática aguda
Swedish	Cardiotrophin-1	Behandling av akut leversvikt
Norwegian	Kardiotrofin-1	Behandling av akutt leversvikt
Icelandic	Kardíótrófín-1	Meðferð bráðrar lifrabilunar

¹ At the time of designation