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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Repertaxin L-lysinate salt for the prevention of delayed graft function in organ transplant

First publication	9 February 2009
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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 19 September 2001, orphan designation (EU/3/01/058) was granted by the European Commission to Dompé s.p.a., Italy, for repertaxin L-lysinate salt for the prevention of delayed graft function in organ transplant.

The sponsorship was transferred to Dompé pha.r.ma s.p.a, Italy, in April 2007 and back to Dompé s.p.a., Italy, in February 2010. Finally, the sponsorship was transferred to Dompé farmaceutici s.p.a., Italy, in March 2015.

What is delayed graft function in organ transplant?

Delayed graft function (DGF) is a complication that occurs in the first few days after the transplant of a solid organ, such as a kidney, when the transplanted organ does not start to work properly. DGF may be caused by events occurring after the restoration of blood flow to the transplanted organ. The damage to the organ caused by the interruption and restoration of blood flow is called ischaemia/reperfusion injury and is associated with an inflammatory reaction, characterised by the invasion of neutrophils (a type of white blood cell) into the transplanted organ. DFG is a life-threatening condition because of the risk of losing the transplanted organ.

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What is the estimated number of patients at risk of developing the condition?

At the time of designation, the number of patients at risk of delayed graft function in organ transplant was estimated to be approximately 1 people in 10,000 in the European Union (EU). This was equivalent to a total of around 38,000 people[*], which 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 methods of prevention are available?

At the time of submission of application for orphan drug designation there were no satisfactory methods for the prevention of delayed graft function in organ transplant. The preventive measures to reduce delayed graft function in organ transplant consisted of donor management and organ preservation.

How is this medicine expected to work?

Repertaxin L-lysinate salt blocks the activity of of interleukin-8, a type of protein called chemokine that is responsible for the reaction of the immune system (body's natural defences) to unknown factors. IL-8 is a mediator of an inflammatory response that can occur after restoring blood flow in a transplanted organ and cause DGF. Repertaxin L-lysinate salt is suggested to inhibit the inflammatory reaction of tissue associated with restored blood flow and thus prevent the delayed graft function in organ transplant.

What is the stage of development of this medicine?

The effects of repertaxin L-lysinate salt were evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials in patients with delayed graft function in organ transplant were initiated.

Repertaxin L-lysinate salt was not marketed anywhere worldwide for the prevention of delayed graft function in organ transplant or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 18 July 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]

Language	Active Ingredient	Indication
English	Repertaxin L-lysine salt	Prevention of delayed graft function in organ transplant
Bulgarian	Репертаксин L-лизинова сол	Превенция на забавена функция на присадката при органа трансплантация
Croatian	Repertaksin L-lizinat	Prevencija odgođene funkcije presatka kod transplantacije organa
Czech	Repertaxin L- lysinová sůl	Léčba protrahované funkce transplantovaných oogánů.
Danish	Repertaxin L-lysin, salt	Forebyggelse af forsinket transplanteringsfunktion i forbindelse med organtransplantation
Dutch	Repertaxin L-lysine zout	Preventie van vertraagde orgaan transplantaatfunctie
Estonian	Repertaksiin-L-lüsiini sool	Hilise siirdistutuse funktsiooni preventsioon organ transplantatsioonis.
Finnish	Repertaksiini L-lysiini suola	Siirrännäisen toimintaviiveen ehkäisy elinsiirrossa
French	Répertaxine L-lysine (sel)	Prévention des retards fonctionnels du greffon dans la greffe d'organe.
German	Repertaxin –L-Lyzin Salz	Vorbeugung der verzögerten Transplantatfunktion bei der Organtransplantation
Greek	Repertaxin, άλας L-λυσίνης	Πρόληψη της αργοπορημένης λειτουργίας του μοσχεύματος κατά τη μεταμόσχευση οργάνων
Hungarian	Repertaxin L-lizin só	Késve induló transzplantátum működés megelőzése
Italian	Repertaxin sale di L-lisina	Prevenzione del ritardo nella funzionalita' nell'organo trapiantato
Latvian	Repertaksīna L-lizīna sāls	Aizkavētas transplantāta funkcijas profilakse pēc orgānu transplantācijas
Lithuanian	Repertaksino L – lizino druska	Vėlyvos transplantuoto organo funkcionavimo pradžios profilaktikai
Polish	Repertaksyna z L-lizyną	Zapobieganie opóźnieniu podjęcia czynności przez przeszczepiony narząd
Portuguese	Repertaxina, sal de L-lisina	Prevenção do atraso funcional do órgão transplantado
Romanian	Repertaxin L-lizină sare	Prevenirea funcţionării întârziate a grefei în transplantul de organ
Slovak	Soľ repertaxínu s L-lyzínom	Prevencia oneskorenej funkcie štepu po transplantácii orgánu
Slovenian	Repertaksin (L-lizin)	Preprečevanje zakasnjenega delovanja presadka
Spanish	Repertaxina, sal de L-lisina	Prevención del retraso de la funcionalidad del órgano transplantado
Swedish	Repertaxin L-lysinsalt	Förebyggande av fördröjd transplantatfunktion i samband med organtransplantation

[1] At the time of transfer of sponsorship