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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Ribavirin for the treatment of adenovirus infection in immunocompromised patients

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in September 2007 on request of the sponsor.

On 8 March 2001, orphan designation (EU/3/01/029) was granted by the European Commission to ICN Pharmaceuticals Limited, United Kingdom, for ribavirin for the treatment of adenovirus infection in immunocompromised patients.

The sponsor changed name to Valeant Pharmaceuticals Limited, United Kingdom, in June 2004.

What is adenovirus infection in immunocompromised patients?

Patients whose immune system has been impaired by disease or treatment are immunocompromised. Their immune system's ability to fight infections is compromised or entirely absent and these patients are easily infected by micro-organisms such as adenoviruses.

Adenovirus disease in immunocompromised patients has been attributed to both primary infection and reactivation of endogenous latent infection (virus is sleeping in the body after the first infection). In immunocompromised patients adenovirus infections can cause lesions (injuries) in the lung, the pancreas, the gastrointestinal tract, the liver, the kidney and the urinary system. Furthermore, it can cause throat infections and spread via the blood stream. Infection of adenovirus in immunocompromised patients can be chronically debilitating and life threatening.

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

At the time of designation, adenovirus infection in immunocompromised patients affected approximately 0.6 to 3 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of around 23,000 to 113,000 people, and is below the ceiling for orphan designation, which is 5 people in

[*]Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 377,000,000 (Eurostat 2001) and may differ from the true number of patients affected by the condition.

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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?

No medicinal products were authorised for the treatment of adenovirus infection in immunocompromised patients in the Community at the time of submission of the application for orphan drug designation.

How is this medicine expected to work?

Ribavirin is a purine nucleoside analogue. The purine nucleosides are part of the fundamental genetic material of human cells but also of viruses. Ribavirin stops the adenovirus from copying its genetic material (blocks adenovirus' replication) and thus prevents the virus from multiplying itself and spreading.

What is the stage of development of this medicine?

The effects of ribavirin were evaluated in experimental models. At the time of submission of the application for orphan designation, clinical trials in immunocompromised patients with adenovirus infection were completed.

Ribavirin was not marketed anywhere worldwide for treatment of adenovirus infection in immunocompromised patients 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 15 January 2001 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:

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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	Ribavirin	Treatment of adenovirus infection in immunocompromised patients
Danish	Ribavirin	Behandling af adenovirusinfektion hos patienter med nedsat immunforsvar
Dutch	Ribavirine	Behandeling van adenovirusinfectie bij immuungecompromiteerde patiënten
Finnish	Ribaviriini	Adenovirusinfektion hoito immuunipuutteisilla potilailla
French	Ribavirin	Traitement des infections à adénovirus, chez les patients immunodéprimés
German	Ribavirin	Behandlung von Infektionen mit Adenoviren bei immunkompromittierten Patienten
Greek	Ribavirin	Θεραπεία λοίμωξης από αδενοϊό σε ανοσοκατεσταλμένους ασθενείς
Italian	Ribavirina	Trattamento di infezioni da Adenovirus in pazienti immunocompromessi
Portuguese	Ribavarina	Tratamento da infecção por adenovírus em doentes imunocomprometidos
Spanish	Ribavirina	Tratamiento de la infección por adenovirus en pacientes inmunodeprimidos
Swedish	Ribavirin	Behandling av adenovirusinfektion hos patienter med försvagat immunsystem

[1] At the time of designation