



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

3 May 2011  
EMA/COMP/125/2004 Rev.1  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### Ribavirin for the treatment of haemorrhagic fever with renal syndrome

***Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in February 2009 on request of the sponsor.***

On 14 February 2001, orphan designation (EU/3/01/024) was granted by the European Commission to ICN Pharmaceuticals Limited, United Kingdom, for ribavirin for the treatment of haemorrhagic fever with renal syndrome. ICN Pharmaceuticals Limited subsequently changed name to Valeant Pharmaceuticals Ltd.

#### **What is haemorrhagic fever with renal syndrome?**

Haemorrhagic fever with renal syndrome is a disease characterised by fever, kidney failure and bleeding. In severe cases patients may suffer from shock. Humans can contract the disease by inhalation of particles from secretions (urine, droppings and saliva) of rodents (such as mice and rats) infected with viruses, called Hantavirus. The disease is not known to transmit from one person to another. The disease is potentially life threatening and chronically debilitating condition.

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

At the time of designation, haemorrhagic fever with renal syndrome affected approximately 3.7 in 10,000 people in the European Union (EU)\*. This is equivalent to a total of around 139,000 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?**

No medicinal products were authorised for the treatment of haemorrhagic fever with renal syndrome in the Community at the time of submission of the application for orphan drug designation.

---

\*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.



## **How is this medicine expected to work?**

Ribavirin is structurally similar to purine nucleosides. The purine nucleosides are part of the fundamental genetic material of human cells but also of viruses. Ribavirin inhibits the replication (the copying of the genetic material) of viruses causing haemorrhagic fever with renal syndrome. Therefore it is thought to prevent the viruses from multiplying and causing harm, ultimately limit the disease.

## **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, no clinical trials in patients with haemorrhagic fever with renal syndrome were initiated.

Ribavirin received orphan drug designation in the United States in 1991 for the treatment of haemorrhagic fever with renal syndrome.

Ribavirin was not marketed or designated as orphan medicinal product in the EU for the treatment of haemorrhagic fever and renal syndrome, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 19 December 2000 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:

Valeant Pharmaceuticals Ltd  
Cedarwood, Chineham Business Park  
Crockford Lane  
Basingstoke RG24 8WD  
United Kingdom  
Telephone: +44 1256 70 77 44  
Telefax: +44 1256 70 73 34

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<sup>1</sup>, Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Ribavirin	Treatment of Haemorrhagic Fever with Renal Syndrome
Danish	Ribavirin	Behandling af hæmoragisk feber med nyresyndrom.
Dutch	Ribavirine	Behandeling van hemorragische koorts met renaal syndroom
Finnish	Ribaviriini	Verenvuotokuumeen, johon liittyy munuaissyndrooma, hoito
French	Ribavirin	Traitement de la fièvre hémorragique avec syndrome rénal
German	Ribavirin	Behandlung des hämorrhagischen Fiebers mit renalem Syndrom
Greek	Ribavirin	Θεραπεία Αιμορραγικού Πυρετού με Νεφρική Προσβολή.
Italian	Ribavirina	Trattamento di febbre emorragica con sindrome renale
Portuguese	Ribavarina	Tratamento da febre hemorrágica com Síndrome Renal
Spanish	Ribavirina	Tratamiento de fiebre hemorrágica con síndrome renal
Swedish	Ribavirin	Behandling av hemorragisk feber med njursyndrom.

<sup>1</sup> At the time of designation