



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

15 June 2010
EMA/COMP/1537/2003 Rev.3
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

vasoactive intestinal peptide for the treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension

On 22 December 2003, orphan designation (EU/3/03/173) was granted by the European Commission to Mondobiotec Laboratories Anstalt, Liechtenstein, for vasoactive intestinal peptide for the treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension.

The sponsorship was transferred to Biogen Idec Limited, United Kingdom, in December 2006 and subsequently to Mondobiotec Laboratories AG, Fürstentum Liechtenstein, in June 2010.

What is pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension?

Pulmonary arterial hypertension is a rare blood vessel disorder of the lung in which the pressure in the pulmonary artery (the blood vessel that leads from the heart to the lungs) rises above normal levels. An increase of smooth muscle cells in the lung microcirculation (so called cell proliferation) are remodelling the vessels and may lead to obstructions in the microcirculation, which will then lead to an increase in the pressure.

Chronic thromboembolic pulmonary hypertension is a complication representing less than 1% of all cases of acute pulmonary embolism (the sudden blocking of an artery by a clot or foreign material which has been brought to its site by the blood current), which directly leads to pulmonary hypertension. Pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension are chronically debilitating and life-threatening.

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

At the time of designation, pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension affected not more than 2 in 10,000 people in the European Union (EU)*. This is equivalent to a total of not more than 78,000 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).

*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 385,000,000 (Eurostat 2002) and may differ from the true number of patients affected by the condition.



What treatments are available?

Several medicinal products were authorised for the treatment of pulmonary arterial hypertension in the Community at the time of submission of the application for orphan drug designation.

Vasoactive intestinal peptide might be of potential significant benefit for the treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension in particular with regards to the new mechanism of action. The preliminary preclinical and clinical results indicate that this product may also offer a major contribution to patient care because of the new route of administration and a different safety profile. This benefit will have to be confirmed at the time of marketing authorisation and will be necessary to maintain the orphan status.

How is this medicine expected to work?

It has vasodilating (enlargement of the vessel) properties, including an effect on pulmonary arteries. Furthermore it inhibits the proliferation of muscle cells in the vessels and in the bronchi. Vasoactive intestinal peptide has also anti-platelet properties, which may play a beneficial role in preventing embolism (platelets are important for blood coagulation).

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, clinical trials in patients with pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension were ongoing.

Vasoactive intestinal peptide was not marketed anywhere worldwide for pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension 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 10 October 2003 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 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:

Mondobiotech Laboratories AG
Herrengasse 21
9490 Vaduz
Fürstentum Liechtenstein
Telephone: +41 840 200 030
Telefax: +41 840 200 010
E-mail: info@mondobiotech.com

Patient associations' contact points

The Pulmonary Hypertension Association

PO Box 2760
Lewes
Sussex BN8 4WA
United Kingdom
Telephone: +44 870 321 37 51
Helpline (UK only): 0800 389 81 56
Telefax: +44 701 071 57 23

Association Française d'aide aux patients souffrant d'Hypertension Artérielle Pulmonaire

31 Rue Jacques Cellier
F-51100 Reims
France
Telephone: (33-3) 26 36 93 32
E-mail: contact@htapfrance.com

Pulmonale Hypertonie e.V.

Wormser Str. 20
D-76287 Rheinstetten
Germany
Telephone: (49-7242) 72 94
Telefax: (49-7242) 95 26 67
E-mail : pphev@aol.com

Translations of the active ingredient and indication in all official EU languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Vasoactive Intestinal Peptide	Treatment of pulmonary arterial hypertension and chronic thromboembolic pulmonary hypertension
Bulgarian	Вазоактивен интестинален пептид	Лечение на белодробна артериална хипертензия и хронична тромбемболична белодробна хипертензия
Czech	Vasoaktivní intestinální peptid	Léčba plicní arteriální hypertenze a chronické tromboembolické plicní hypertenze
Danish	Vasoaktiv Intestinal Peptid	Behandling af pulmonal arteriel hypertension samt kronisk tromboembolisk pulmonal hypertension
Dutch	Vasoactief Intestinaal Peptide	Behandeling van pulmonale arteriële hypertensie en chronische trombo-embolische pulmonale hypertensie
Estonian	Vasoaktiivne intestinaalne peptiid.	Pulmonaalhüpertensiooni ja kroonilise tromboemboolse pulmonaalhüpertensiooni ravi
Finnish	Vasoaktiivinen Intestinaalinen Peptidi	Kohonneen keuhkovaltimopaineen ja kroonisen tromboembolisen pulmonaarisen hypertension hoito
French	Peptide Intestinal Vasoactif	Traitement de l'hypertension artérielle pulmonaire et de l'hypertension pulmonaire chronique thrombo-embolique
German	Vasoaktives Intestinales Peptid	Behandlung der pulmonalen arteriellen Hypertonie und der chronischen thromboembolischen pulmonalen Hypertonie
Greek	Αγγειοενεργό εντερικό πεπτιδιο	Θεραπεία της αρτηριακής πνευμονικής υπέρτασης και της χρόνιας θρομβοεμβολικής πνευμονικής υπέρτασης
Hungarian	Vasoactiv Intestinalis Peptid	Pulmonális arteriális hipertónia és krónikus tromboembólia okozta pulmonális hipertónia kezelése
Italian	Peptide intestinale vasoattivo	Trattamento dell'ipertensione arteriosa polmonare e l'ipertensione polmonare cronica tromboembolica
Latvian	Vazoaktīvais intestinālais peptīds	Plaušu arteriālās hipertensijas un hroniskās tromboemboliskās plaušu hipertensijas ārstēšanai
Lithuanian	Vazoaktyvus žarnų peptidas	Plaučių arterinės hipertenzijos ir lėtinės tromboembolinės plaučių hipertenzijos gydymas
Maltese	Peptidu intestinali vasoattiv	Kura ta' pressjoni arterjali pulmonari għolja u ta' pressjoni pulmonari trombo-embolika għolja kronika

¹ At the time of transfer of sponsorship

Language	Active ingredient	Indication
Polish	Naczyniowoaktywny peptyd jelitowy	Leczenie tętniczego nadciśnienia płucnego oraz przewlekłego zakrzepowo-zatorowego nadciśnienia płucnego
Portuguese	Peptídeo Intestinal Vasoactivo	Tratamento da hipertensão arterial pulmonar e Hipertensão pulmonar tromboembólica crónica
Romanian	Peptidul vasointestinal activ (VIP)	Tratamentul hipertensiunii arteriale pulmonare și al hipertensiunii pulmonare tromboembolice cronice
Slovak	Vazoaktívny intestinálny peptid	Liečba pľúcnej arteriálnej hypertenzie a chronickej tromboembolickej pľúcnej hypertenzie
Slovenian	Vazoaktivni intestinalni peptid	Zdravljenje pljučne arterijske hipertenzije in kronične tromboembolične pljučne hipertenzije
Spanish	Péptido Intestinal Vasoactivo	Tratamiento de la hipertensión arterial pulmonar e hipertensión pulmonar tromboembólica crónica
Swedish	Vasoaktiv intestinal peptid	Behandling av förhöjt pulmonärartärtryck och kronisk tromboembolisk pulmonell hypertoni