10 June 2010
EMA/COMP/165397/2010
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Pravastatin / zoledronic acid for the treatment of Hutchinson-Gilford progeria

On 9 June 2010, orphan designation (EU/3/10/748) was granted by the European Commission to Prenyl BIO SAS, France, for pravastatin / zoledronic acid for the treatment of Hutchinson-Gilford progeria.

What is Hutchinson-Gilford progeria?

Hutchinson-Gilford progeria is a severe, genetic condition in which features resembling aging appear in childhood. Children born with Hutchinson-Gilford progeria live for around 13 years. They appear healthy at birth, but in the first few years of life they develop symptoms such as limited growth, a distinctive appearance with a small face and a pinched nose, loss of hair and body fat, prominent scalp veins, crowded teeth, small and fragile bones, and stiffness of joints. Later, the condition causes wrinkled skin and problems with the heart.

Hutchinson-Gilford progeria is a severe and life-threatening condition particularly because of the problems with the heart, which lead to premature death.

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

At the time of designation, Hutchinson-Gilford progeria affected less than 0.05 in 10,000 people in the European Union (EU)[*]. This is equivalent to a total of fewer than 3,000 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).

What treatments are available?

At the time of designation, no satisfactory methods were authorised in the EU for the treatment of Hutchinson-Gilford progeria. Patients received supportive treatment to help them and their families to cope with the symptoms of the condition. This included psychological support, painkillers, nutritional supplements, physical aids, sealing of the teeth and use of wigs.

[*]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 506,500,000 (Eurostat 2010).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

How is this medicine expected to work?

Hutchinson-Gilford progeria is caused by abnormalities in the *LMNA* gene, which is needed to produce lamina A, a protein in the nucleus of cells that helps in producing and repairing genetic material. Children with Hutchinson-Gilford progeria have an abnormal form of this protein, which disrupts the normal functioning of the nucleus, leading to symptoms of aging.

The two active substances in this medicine, pravastatin and zoledronic acid, are already authorised in the EU on their own for the treatment of high blood cholesterol and for the prevention of bone fractures. In Hutchinson-Gilford progeria, they are expected to work together by reducing the production of the abnormal protein. This is expected to restore the normal functioning of the nucleus, relieving the symptoms of Hutchinson-Gilford progeria.

What is the stage of development of this medicine?

The effects of pravastatin / zoledronic acid have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with pravastatin / zoledronic acid in patients with Hutchinson-Gilford progeria were ongoing.

At the time of submission, pravastatin / zoledronic acid was not authorised anywhere in the EU for Hutchinson-Gilford progeria 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 3 March 2010 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:

Prenyl BIO SAS
avenue Vacher CD6
Immeuble CCE
ZI de Rousset Peynier
13106 Rousset
France
Telephone: +33 491 25 40 46
Telefax: +33 486 95 81 00
E-mail: adm@prenylbio.com

Patient associations' contact points

Progeria Family Circle - Stiftung für Kinder mit Progeria
Delbrückstrasse 36
12051 Berlin
Germany
Telephone: +49 30 69568236
Telefax: +49 30 65498913
E-mail: info@progeriafamilycircle.de

Translations of the active ingredient and indication in all official EU languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Pravastatin / zoledronic acid	Treatment of Hutchinson-Gilford progeria
Bulgarian	Правастатин / золедронова киселина	Лечение на прогерия на Hutchinson-Gilford
Czech	Pravastatin / acidum zoledronicum	Léčba Hutchinson-Gilford progerie
Danish	Pravastatin / zoledronsyre	Behandling af Hutchinson-Gilfords syndrom
Dutch	Pravastatine / zoledroninezuur	Behandeling van Hutchinson-Gilford progeria
Estonian	Pravastatiin / veevaba zoledroonhapet	Hutchinson-Gilfordi progeeria ravi
Finnish	Pravastatiini / vedetön tsoledronihappo	Hutchinson-Gilfordin oireyhtymän hoito
French	Pravastatine / acide zolédronique	Traitement de la progeria de Hutchinson-Gilford
German	Pravastatin / Zoledronsäure	Behandlung von Hutchinson-Gilford-Progerie
Greek	Πραβαστατίνη / ζολεδρονικό οξύ	Θεραπεία της προγηρίας Hutchinson-Gilford
Hungarian	Pravasztatin / zoledronsav	Hutchinson-Gilford progeria kezelése
Italian	Pravastatina / acido zoledronico	Trattamento della progeria di Hutchinson-Gilford
Latvian	Pravastatīna / zoledronskābe	Hutchinsona- Gilforda progērijas ārstēšana
Lithuanian	Pravastatinas /zoledrono rūgštis	*Hutchinson-Gilford* progerijos gydymas
Maltese	Pravastatin / zoledronic acid	Kura ta' proġerja ta’ Hutchinson-Gilford
Polish	Prawastatyna / kwas zoledronowy	Leczenia progerii Hutchinsona-Gilforda
Portuguese	Pravastatina / ácido zoledrónico	Tratamento da progeria de Hutchinson-Gilford
Romanian	Pravastatin/acid zoledronic	Tratamentul progeriei Hutchinson-Gilford
Slovak	Pravastatín / kyselina zoledrónová	Liečba Hutchinsonovej - Gilfordovej progerie
Slovenian	Pravastatín / zoledronska kislina	Zdravljenje progerije Hutchinson-Gilford
Spanish	Pravastatina / ácido zoledrónico	Tratamiento de la progeria de Hutchinson-Gilford
Swedish	Pravastatin / zoledronsyra	Behandling av progeria Hutchinson-Gilford
Norwegian	Pravastatin / zoledronsyre	Behandling av Hutchinson-Gilfords progeria syndrom
Icelandic	Pravastatin / zóledrónic sýra	Meðferð á Hutchinson-Gilford progeria

[1] At the time of designation