25 February 2019
EMA/806419/2018

Public summary of opinion on orphan designation

Lonafarnib for the treatment of Hutchinson-Gilford progeria syndrome

On 14 December 2018, orphan designation (EU/3/18/2118) was granted by the European Commission to Eiger Biopharmaceuticals Europe Limited, United Kingdom, for lonafarnib for the treatment of Hutchinson-Gilford progeria syndrome.

What is Hutchinson-Gilford progeria syndrome?

Hutchinson-Gilford progeria syndrome is a genetic condition in which features resembling aging appear in childhood.

Children born with Hutchinson-Gilford progeria syndrome 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 syndrome 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 syndrome affected less than 0.01 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 500 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?

At the time of designation, no satisfactory methods were authorised in the EU for the treatment of Hutchinson-Gilford progeria syndrome. 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 28), Norway, Iceland and Liechtenstein. This represents a population of 517,400,000 (Eurostat 2018).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

How is this medicine expected to work?

Hutchinson-Gilford progeria syndrome is caused by mutations (changes) in the *LMNA* gene, which produces lamin A, a protein that helps to keep cells of the body strong and stable. People with Hutchinson-Gilford progeria syndrome have abnormal lamin A, which causes damage to cells and leads to symptoms of aging early in life.

It is thought that lonafarnib may help prevent formation of abnormal lamin A thereby improving symptoms of the disease.

What is the stage of development of this medicine?

The effects of lonafarnib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with the medicine in patients with Hutchinson-Gilford progeria syndrome were ongoing.

At the time of submission, lonafarnib was not authorised anywhere in the EU for Hutchinson-Gilford progeria syndrome. Orphan designation of the medicine had been granted in the United States for Hutchinson-Gilford progeria syndrome.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 8 November 2018 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:

Contact details of the current sponsor for this orphan designation can be found on the EMA website.

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	Lonafarnib	Treatment of Hutchinson-Gilford progeria syndrome
Bulgarian	Лонафарниб	Лечение на синдром на Hutchison-Gilford Progeria (HGPS) и прогероидни ламинопатии
Croatian	Lonafarnib	Liječenje Hutchison-Gilfordovog sindroma progerije
Czech	Lonafarnib	Léčba syndromu Hutchison-Gilford Progerie (HGPS)
Danish	Lonafarnib	Behandling af Hutchison-Gilford Progeria Syndrome (HGPS) og Progeroid Laminopathies
Dutch	Lonafarnib	Behandeling van Hutchison-Gilford Progeria Syndroom
Estonian	Lonafarniib	Hutchison-Gilford progeeria sündroomi ravi
Finnish	Lonafarnibi	Hutchison-Gilfordin progeria-oireyhtymän (HGPS) hoito
French	Lonafarnib	Traitement du syndrome de progeria de Hutchinson-Gilford (HGPS)
German	Lonafarnib	Behandlung von Hutchison-Gilford Progeria Syndrom
Greek	Λοναφαρνίμπη	Θεραπεία του συνδρόμου προγηρείας Hutchison-Gilford (HGPS
Hungarian	Lonafarnib	Hutchison-Gilford Progeria-szindróma (HGPS) kezelése
Italian	Lonafarnib	Trattamento della sindrome della progeria di Hutchison-Gilford (HGPS) e delle laminopatie progeroidi
Latvian	Lonafarnibs	Hatčinsona-Gilforda progērijas sindroma (HGPS) ārstēšana
Lithuanian	Lonafarnibas	*Hutchinson-Gilford* progerijos sindromo gydymas
Maltese	Lonafarnib	Trattament tas-Sindromu Progeria ta' Hutchison-Gilford (HGPS)
Polish	Lonafarnib	Leczenie zespołu progerii Hutchisona-Gilforda (HGPS) i laminopatii progeroidowych
Portuguese	Lonafarnib	Tratamento da Síndrome de Progéria de Hutchison-Gilford (HGPS) e Laminopatias Progeróides
Romanian	Lonafarnib	Tratamentul sindromului de progerie Hutchinson-Gilford Progeria
Slovak	Lonafarnib	Liečba syndrómu Hutchison-Gilford Progeria (HGPS) a progeroidných laminopatií
Slovenian	Lonafarnib	Zdravljenje sindroma Hutchison-Gilford Progeria (HGPS) in progeroidnih laminopatij
Spanish	Lonafarnib	Tratamiento del síndrome de progeria de Hutchison-Gilford (HGPS) y de laminopatías progeroides
Swedish	Lonafarnib	Behandling av Hutchison-Gilford progeria sndrom
Norwegian	Lonafarnib	Behandling av Hutchinson-Gilford progeria syndrom
Icelandic	Lónafarníb	Meðferð á Hutchison-Gilford Progeria heilkenni (HGPS) og Progeroid Laminopathies

[1] At the time of designation