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Public summary of opinion on orphan designation

Losmapimod for the treatment of facioscapulohumeral muscular dystrophy

On 24 March 2020, orphan designation EU/3/20/2263 was granted by the European Commission to Pharma Gateway AB, Sweden, for losmapimod for the treatment of facioscapulohumeral muscular dystrophy.

What is facioscapulohumeral muscular dystrophy?

Facioscapulohumeral muscular dystrophy is an inherited condition that causes weakness and wasting of the muscles, usually starting with the muscles of the face, shoulders and arms and gradually extending to the muscles of the torso and lower limbs. Symptoms usually start in early adulthood but there is also a more severe form of the disease that starts in childhood (infantile onset).

The condition is long-term debilitating due to the progressive muscle weakness and resulting difficulty moving around. There may also be problems with vision and hearing, and the infantile onset form is considered life threatening.

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

At the time of designation, facioscapulohumeral muscular dystrophy affected less than 1.5 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 78,000 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?

At the time of designation, no satisfactory methods were authorised in the EU to treat facioscapulohumeral muscular dystrophy. Patients mainly received supportive treatment including physical therapies such as physiotherapy and medicines to manage muscle pain and inflammation.

*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).

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How is this medicine expected to work?

Muscle weakness in patients with facioscapulohumeral muscular dystrophy is caused by abnormal activity of a protein called DUX4 which damages and destroys muscles cells. Losmapimod is expected to work by blocking of DUX4, thereby preventing muscle cell damage.

What is the stage of development of this medicine?

The effects of losmapimod have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with losmapimod in patients with facioscapulohumeral muscular dystrophy were ongoing.

At the time of submission, losmapimod was not authorised anywhere in the EU for the treatment of facioscapulohumeral muscular dystrophy or designated as an orphan medicinal product elsewhere for this condition.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 20 February 2020, 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 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	Losmapimod	Treatment of facioscapulohumeral muscular dystrophy
Bulgarian	Лосмапимод	Лечение на фацио-скапуло-хумерална мускулна дистрофия
Croatian	Losmapimod	Liječenje fascioskapulohumeralne mišićne distrofije
Czech	Losmapimod	Léčba facioskapulohumerální svalové dystrofie
Danish	Losmapimod	Behandling af facioscapulohumeral muskeldystrofi
Dutch	Losmapimod	Behandeling van facioscapulohumerale spierdystrofie
Estonian	Losmapimood	Fatsioskapulohumeraalse lihasdüstroofia ravi
Finnish	Losmapimodi	Fasioskapulohumeraalisen lihasdystrofian hoito
French	Losmapimod	Traitement de la dystrophie musculaire facio-scapulo-humérale
German	Losmapimod	Behandlung der fazioskapulohumeralen Muskeldystrophie
Greek	Λοσμαπιμόδη	Θεραπεία της προσωποωμοβραχιόνιας μυϊκής δυστροφίας
Hungarian	Losmapimod	Fascioscapulohumeralis izomdisztrófia kezelése
Italian	Losmapimod	Trattamento della distrofia muscolare facio-scapolo-omerale
Latvian	Losmapimods	Facioskapulohumerālās muskuļu distrofijas ārstēšana
Lithuanian	Losmapimodas	Veido-mentės-žasto raumenų distrofijos gydymas
Maltese	Losmapimod	Kura ta' distrofija muskolari faċjo-skapulo-umerali
Polish	Losmapimod	Leczenie dystrofii mięśniowej twarzowo-łopatkowo-ramieniowej
Portuguese	Losmapimod	Tratamento da distrofia muscular facioescapuloumeral
Romanian	Losmapimod	Tratamentul distrofiei musculare facio-scapulo-humerale
Slovak	Losmapimod	Liečba facioskapulohumerálnej svalovej dystrofie
Slovenian	Losmapimod	Zdravljenje facioskapulohumeralne mišične distrofije
Spanish	Losmapimod	Tratamiento de la distrofia muscular facioescapulohumeral
Swedish	Losmapimod	Behandling av facioskapulohumeral muskeldystrofi
Norwegian	Losmapimod	Behandling av facioscapulohumeral muskeldystrofi
Icelandic	Losmapímód	Meðferð á facioscapulohumeral-vöðvarýrnun

[1] At the time of designation