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Public summary of opinion on orphan designation

Givinostat for the treatment of Becker muscular dystrophy

On 31 July 2018, orphan designation (EU/3/18/2046) was granted by the European Commission to Italfarmaco S.p.A., Italy, for givinostat for the treatment of Becker muscular dystrophy.

What is Becker muscular dystrophy?

Becker muscular dystrophy is an inherited disease that gradually causes the muscles to become weaker. The muscle weakness usually starts in the hips and legs and may later on involve the chest and the heart. The disease mainly affects boys, and usually starts between the ages of 10 and 15 years.

Becker muscular dystrophy is caused by abnormalities in the gene responsible for the production of dystrophin, a protein that forms an important component of muscle fibres. As the patients do not have enough of the functional protein, muscle fibres gradually break down leading to muscle weakness.

Becker muscular dystrophy causes long-term disability and is life-threatening because of its effects on the heart and the muscles that are used to breathe.

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

At the time of designation, Becker muscular dystrophy affected approximately 0.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 10,000 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, there were no treatments authorised in the EU for Becker muscular dystrophy. Patients received supportive treatment to relieve symptoms and improve the patient's general condition. In addition, corticosteroids were used in an attempt to improve symptoms, although they were not authorised for use in this disease.

*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).

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How is this medicine expected to work?

Givinostat is an 'HDAC inhibitor' medicine. This means that it blocks enzymes called histone deacetylases (HDACs), which are involved in turning genes 'on' and 'off' within cells. The activity of HDACs is increased in patients with Becker muscular dystrophy. By blocking HDAC enzymes, givinostat 'switches on' a gene that increases the amount of a protein called follistatin in muscle cells. Follistatin is expected to increase muscle mass and prevent muscle degeneration and thereby improve the symptoms of Becker muscular dystrophy.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, a clinical trial with the medicine in patients with Becker muscular dystrophy was ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for Becker muscular dystrophy. Orphan designation of the medicine had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 21 June 2018 recommending the granting of this designation.

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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, on the medicine's rare disease designations page.

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	Givinostat	Treatment of Becker muscular dystrophy
Bulgarian	Гивиностат	Лечение на мускулна дистрофия тип Бекер
Croatian	Givinostat	Liječenje Beckerove mišićne distrofije
Czech	Givinostat	Léčba Beckerovy svalové dystrofie
Danish	Givinostat	Behandling af Beckers muskeldystrofi
Dutch	Givinostat	Behandeling van Becker spierdystrofie
Estonian	Givinostat	Beckeri lihasdüstroofia ravi
Finnish	Givinostaatti	Beckerin lihasdystrofian hoito
French	Givinostat	Traitement de la dystrophie musculaire de Becker
German	Givinostat	Behandlung der Muskeldystrophie Typ Becker
Greek	Τζιβινοστάτη	Θεραπεία της μυϊκής δυστροφίας του Becker
Hungarian	Givinostat	Becker-féle izomdisztrófia kezelése
Italian	Givinostat	Trattamento della distrofia muscolare di Becker
Latvian	Givinostats	Bekera muskuļu distrofijas ārstēšanai
Lithuanian	Givinostatas	Bekerio raumenų distrofijos gydymas
Maltese	Givinostat	Kura tad-distrofija muskolari ta' Becker
Polish	Giwinostat	Leczenie dystrofii mięśniowej Beckera
Portuguese	Givinostate	Tratamento da distrofia muscular de Becker
Romanian	Givinostat	Tratamentul distrofiei musculare Becker
Slovak	Givinostat	Liečba Beckerovej svalovej dsystrofie
Slovenian	Givinostat	Zdravljenje Beckerjeve mišične distrofije
Spanish	Givinostat	Tratamiento de la distrofia muscular de Becker
Swedish	Givinostat	Behandling av Beckers muskeldystrofi
Norwegian	Givinostat	Behandling av Beckers muskeldystrofi
Icelandic	Gívónóstat	Meðferð á Beckers vöðvakyrkingi

[1] At the time of designation