4 September 2020
EMADOC-628903358-2482

Public summary of opinion on orphan designation

(+)-Epicatechin for the treatment of Becker muscular dystrophy

On 26 June 2020, orphan designation EU/3/20/2293 was granted by the European Commission to MWB Consulting S.A.R.L., France, for (+)-epicatechin 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 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 working dystrophin, 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.

*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).

Official address Domenico Scarlattilaan 6 ● 1083 HS Amsterdam ● The Netherlands
Address for visits and deliveries Refer to www.ema.europa.eu/how-to-find-us
Send us a question Go to www.ema.europa.eu/contact **Telephone** +31 (0)88 781 6000 An agency of the European Union

How is this medicine expected to work?

(+)-Epicatechin is known to increase the production of follistatin, a substance that increases muscle growth, and reduce production of myostatin, which reduces muscle growth. This leads to improved muscle strength and function. The medicine is also expected to act on mitochondria (the bodies that generate energy inside cells) to improve the way in which muscles generate energy and their ability to prevent oxidative stress (damage caused by oxygen-containing molecules). These actions are expected to help improve the function of muscles in patients with Becker muscular dystrophy and so relieve some of the symptoms of the condition.

What is the stage of development of this medicine?

The effects of (+)-epicatechin have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with (+)-epicatechin in patients with Becker muscular dystrophy had been started.

At the time of submission, (+)-epicatechin was not authorised anywhere in the EU for the treatment of Becker 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 May 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

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	(+)-epicatechin	Treatment of Becker muscular dystrophy
Bulgarian	(+)-епикатехин	Лечение на мускулна дистрофия тип Бекер
Croatian	(+)-epikatehincatechin	Liječenje Beckerove mišićne distrofije
Czech	(+)-epicatechin	Léčba Beckerovy svalové dystrofie
Danish	(+)-epicatechin	Behandling af Beckers muskeldystrofi
Dutch	(+)-epicatechine	Behandeling van Becker spierdystrofie
Estonian	(+)-epikatehiin	Beckeri lihasdüstroofia ravi
Finnish	(+)-epikatekiini	Beckerin lihasdystrofian hoito
French	(+)-épicatéchine	Traitement de la dystrophie musculaire de Becker
German	(+)-epicatechin	Behandlung der Muskeldystrophie Typ Becker
Greek	(+)–επικατεχίνη	Θεραπεία της μυϊκής δυστροφίας του Becker
Hungarian	(+)-epikatekin	Becker-féle izomdisztrófia kezelése
Italian	(+)-epicatechina	Trattamento della distrofia muscolare di Becker
Latvian	(+)-epikatehīns	Bekera muskuļu distrofijas ārstēšanai
Lithuanian	(+)–epikatechinas	Bekerio raumenų distrofijos gydymas
Maltese	(+)-epikatekin	Kura tad-distrofija muskolari ta’ Becker
Polish	(+)-epikatechina	Leczenie dystrofii mięśniowej Beckera
Portuguese	(+)-epicatequina	Tratamento da distrofia muscular de Becker
Romanian	(+)-epicatechina	Tratamentul distrofiei musculare Becker
Slovak	(+)-epikatechín	Liečba Beckerovej svalovej dsystrofie
Slovenian	(+)-epikatekhin	Zdravljenje Beckerjeve mišične distrofije
Spanish	(+)-epicatequina	Tratamiento de la distrofia muscular de Becker
Swedish	(+)-epikatekin	Behandling av Beckers muskeldystrofi
Norwegian	(+)-epikatekin	Behandling av Beckers muskeldystrofi
Icelandic	(+)-epicatechin	Meðferð á Beckers vöðvakyrkingi

[1] At the time of designation