11 July 2017
EMA/325369/2017

# Public summary of opinion on orphan designation

## Ibutamoren mesilate for the treatment of growth hormone deficiency

On 20 June 2017, orphan designation (EU/3/17/1882) was granted by the European Commission to Richardson Associates Regulatory Affairs Ltd., United Kingdom, for ibutamoren mesilate (also known as MK-0677) for the treatment of growth hormone deficiency.

### What is growth hormone deficiency?

Growth hormone deficiency is a condition where the patient lacks a sufficient amount of growth hormone, which is normally secreted by the pituitary gland (at the base of the brain). Growth hormone promotes growth during childhood and adolescence, and also acts on the way the body handles proteins, fat and carbohydrates (sugars). The condition can be caused by a genetic mutation (change) or other factors such as trauma and inflammation, or it may have no known cause. It can affect people of any age. In childhood, the main signs include failure to grow normally and impaired development of bones and skeletal muscle. In adulthood, the condition can affect the heart, muscles and bones, alter metabolism and cause psychological symptoms such as anxiety and depression.

Growth-hormone deficiency is a long-term debilitating condition that includes decreased bone mass, bone fractures and psychological symptoms. The disease can be life-threatening due to the risk of problems with the heart and blood circulation.

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

At the time of designation, growth hormone deficiency affected approximately 4.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 237,000 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, medicines containing recombinant human growth hormone were authorised in the EU to treat growth hormone deficiency. These were given to patients by daily injection.

*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 515,700,000 (Eurostat 2017).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
**Telephone** +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
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The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with growth hormone deficiency. Early studies show that the medicine, which can be given by mouth and works in a different way to authorised medicines, can improve growth rate in children with the condition. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

## How is this medicine expected to work?

The release of growth hormone in the body is regulated by a complex system of hormones. Ibutamoren mesilate acts similarly to GHRP6, a regulating hormone which stimulates the body to produce more growth hormone. Taking the medicine is therefore expected to increase the body's natural production of growth hormone, and so help overcome the effects of this condition.

## What is the stage of development of this medicine?

The effects of ibutamoren mesilate 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 growth hormone deficiency were ongoing.

At the time of submission, ibutamoren mesilate was not authorised anywhere in the EU for growth hormone deficiency 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 12 May 2017 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 | Ibutamoren mesilate | Treatment of growth hormone deficiency |
| Bulgarian | Ибутаморен мезилат | Лечение на дефицит на растежния хормон |
| Croatian | Ibutamoren mesilat | Liječenje manjka hormona rasta |
| Czech | Ibutamoren mesylát | Léčba deficitu růstového hormonu |
| Danish | Ibutamorenmesilat | Behandling af væksthormonmangel |
| Dutch | Ibutamoren mesilaat | Behandeling van groeihormoondeficiëntie |
| Estonian | Ibutamoreenmesülaat | Kasvuhormooni puudulikkuse ravi |
| Finnish | Ibutamoreeni mesilaatti | Kasvuhormonin puutoksen hoito |
| French | Ibutamoren mésylate | Le traitement de la déficience en hormone de croissance |
| German | Ibutamorenmesilat | Behandlung eines Wachstumshormonmangels |
| Greek | Ιβουταμορένη μεσυλική | Θεραπεία της ανεπάρκειας της αυξητικής ορμόνης |
| Hungarian | Ibutamoren-mezilát | Növekedési hormon hiány kezelése |
| Italian | Ibutamoren mesilato | Trattamento del deficit di ormone della crescita |
| Latvian | Ibutamorēna mesilāts | Augšanas hormona deficīta ārstēšana |
| Lithuanian | Ibutamoreno mesilatas | Augimo hormono stokos gydymas |
| Maltese | Ibutamoren mesilate | Kura ta' nuqqas tal-ormon tat-tkabbir |
| Polish | Mesylan ibutamorenu | Leczenie niedoboru hormonu wzrostu |
| Portuguese | Mesilato de ibutamoreno | Tratamento do défice de hormona de crescimento |
| Romanian | Mesilat de ibutamoren | Tratamentul deficienţei de hormon de creştere |
| Slovak | Ibutamoren-mezylát | Liečba nedostatku rastového hormónu |
| Slovenian | Ibutamorenov mesilat | Zdravljenje pomanjkanja rastnega hormona |
| Spanish | Mesilato de ibutamoren | Tratamiento de la deficiencia de la hormona del crecimiento |
| Swedish | Ibutamorenmesylat | Behandling av tillväxthormonbrist |
| Norwegian | Ibutamorenmesilat | Behandling av veksthormonmangel |
| Icelandic | Íbútamórenmesýlat | Meðferð við vaxtarhormónskorti |

[1] At the time of designation