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Public summary of opinion on orphan designation

Miransertib for the treatment of Proteus syndrome

On 21 March 2018, orphan designation (EU/3/18/1997) was granted by the European Commission to QRC Consultants Ltd, United Kingdom, for miransertib for the treatment of Proteus syndrome.

What is Proteus syndrome?

Proteus syndrome is a genetic condition where patients' organs and tissues grow out of proportion to the rest of the body. The disease usually starts in infancy and can involve any tissue or organ. The overgrowth is usually asymmetric, which means that it affects the right and left sides of the body differently.

Proteus syndrome is a long-term debilitating and life-threatening condition because of bone deformities and problems with lung, heart, kidney and gut function.

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

At the time of designation, Proteus 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 to treat Proteus syndrome. Treatment of patients consisted mainly of surgery to control the overgrowth.

How is this medicine expected to work?

Miransertib is expected to work by blocking an enzyme known as AKT1. This enzyme is involved in stimulating cells to grow excessively in patients with Proteus syndrome. By blocking AKT1, the medicine is expected to help control the growth of cells and slow down the overgrowth of tissues in patients with Proteus syndrome.

*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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What is the stage of development of this medicine?

The effects of miransertib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with miransertib in patients with Proteus syndrome were ongoing.

At the time of submission, miransertib was not authorised anywhere in the EU for Proteus syndrome. Orphan designation of miransertib had been granted in the United States for Proteus syndrome.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 15 February 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	Miransertib	Treatment of Proteus syndrome
Bulgarian	Мирансертиб	Лечение на синдрома на Протеус
Croatian	Miransertib	Liječenje Proteusovog sindroma
Czech	Miransertib	Léčba Proteus syndromu
Danish	Miransertib	Behandling af Proteus syndrom
Dutch	Miransertib	Behandeling van Proteus syndroom
Estonian	Miransertiib	Proteuse sündroomi ravi
Finnish	Miransertibi	Proteus-oireyhtymän hoito
French	Miransertib	Traitement du syndrome de Protée
German	Miransertib	Behandlung des Proteus-Syndroms
Greek	Μιρανσερτίμπη	Θερπεία του συνδρόμου του Πρωτέα
Hungarian	Miransertib	Proteus-szindróma kezelése
Italian	Miransertib	Trattamento della syndrome di Proteus
Latvian	Miransertibs	Proteja sindroma ārstēšana
Lithuanian	Miransertibas	*Proteus* sindromo gydymas
Maltese	Miransertib	Kura tas-sindromu ta' Proteus
Polish	Miransertib	Leczenie zespołu Proteusza
Portuguese	Miransertib	Tratamento da síndrome de Proteus
Romanian	Miransertib	Tratatmentul sindromului Proteus
Slovak	Miransertib	Liečba Proteus syndrómu
Slovenian	Miransertib	Zdravljenje proteusnega sindroma
Spanish	Miransertib	Tratamiento del Síndrome de Proteus
Swedish	Miransertib	Behandling av Proteus syndrom
Norwegian	Miransertib	Behandling av Proteus syndrom
Icelandic	Míransertíb	Meðferð á Proteus heilkenni

[1] At the time of designation