7 November 2016
EMA/620954/2016
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Ubiquinol for the treatment of primary coenzyme Q_{10} deficiency syndrome

On 14 October 2016, orphan designation (EU/3/16/1765) was granted by the European Commission to Centro de Investigación Biomédica en Red (CIBER), Spain, for ubiquinol for the treatment of primary coenzyme Q_{10} deficiency syndrome.

What is primary coenzyme Q_{10} deficiency syndrome?

Primary coenzyme Q_{10} deficiency syndrome is an inherited disease caused by defects in the genes for coenzyme Q_{10}, a vitamin-like substance vital for producing the energy that cells need to work properly. Deficiency of coenzyme Q_{10} causes damage to organs that are highly dependent on the availability of energy such as the brain, muscles, liver and the kidneys. This can lead to muscle weakness, kidney failure and encephalopathy. Encephalopathy, which includes effects such as fits and reduced mental capacity, is caused by the build-up in the brain of waste substances that the damaged liver is no longer able to remove.

The condition is long-term debilitating and life-threatening, mainly because it causes kidney failure and encephalopathy.

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

At the time of designation, primary coenzyme Q_{10} deficiency syndrome affected approximately 0.1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 5,000 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, ubiquinone was authorised in the EU to treat primary coenzyme Q_{10} deficiency 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 513,700,000 (Eurostat 2016).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

The sponsor has provided sufficient information to show that ubiquinol might be of significant benefit for patients with the condition because it is expected to be better absorbed than ubiquinone, allowing lower doses to be used, which may help patients to stick to their treatment. In addition, it may work in patients who do not improve on ubiquinone. 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?

Ubiquinol is a form of coenzyme Q_{10}. The medicine is expected to replace the natural coenzyme Q_{10}, thereby helping the cells to produce energy and improving the symptoms of the disease.

What is the stage of development of this medicine?

The effects of ubiquinol have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with ubiquinol in patients with primary coenzyme Q_{10} deficiency syndrome were ongoing.

At the time of submission, ubiquinol was not authorised anywhere in the EU for primary coenzyme Q_{10} deficiency syndrome 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 8 September 2016 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	Ubiquinol	Treatment of primary coenzyme Q_{10} deficiency syndrome
Bulgarian	Убикинол	Лечение на синдрома на първичен дефицит на коензим Q_{10}
Croatian	Ubikvinol	Liječenje sindroma primarnog nedostatka koenzima Q_{10}
Czech	Ubichinol	Léčba primárního syndromu deficience koenzymu Q_{10}
Danish	Ubiquinol	Behandling af primære syndrom med mangel på coenzym Q_{10}
Dutch	Ubiquinol	Behandeling van het primaire co-enzym Q_{10}-deficiëntie syndroom
Estonian	Ubikinool	Primaarse koensüümi Q_{10} puudulikkuse sündroomi ravi
Finnish	Ubikinoli	Primaarisen koentsyymi Q_{10}:n puutosoireyhtymän hoito
French	Ubiquinol	Syndrome du déficit primaire en coenzyme Q_{10}
German	Ubichinol	Behandlung des primären Koenzymmangelsyndroms Q_{10}
Greek	Ουμπικινόλη	Θεραπεία του πρωτογενούς συνδρόμου ανεπάρκειας του συνενζύμου Q_{10}
Hungarian	Ubiquinol	Primér Q_{10}-koenzim-hiányos tünetegyüttes kezelése
Italian	Ubiquinol	Trattamento della sindrome da deficit primario del coenzima Q10
Latvian	Ubikvinols	Koenzīma Q_{10} primāra deficīta sindroma ārstēšana
Lithuanian	Ubichinolis	Pirminio kofermento Q_{10} stokos sindromo gydymas
Maltese	Ubiquinol	Kura tas-sindrome primarju ta' nuqqas ta' koenżima Q_{10}
Polish	Ubichinol	Leczenie pierwotnego syndromu niedoboru koenzymu Q_{10}
Portuguese	Ubiquinol	Tratamento da síndrome do déficit primário da coenzima Q_{10}
Romanian	Ubiquinol	Tratamentul sindromului deficienţei primare de coenzimă Q_{10}
Slovak	Ubichinol	Liečba syndrómu primárnej deficiencie koenzýmu Q_{10}
Slovenian	Ubikinol	Zdravljenje sindroma primarnega pomanjkanja koencima Q_{10}
Spanish	Ubiquinol	Tratamiento de síndrome primaria de deficiencia de coenzima Q_{10}
Swedish	Ubiquinol	Behandling av primär koenzym Q_{10}-brist
Norwegian	Ubikinol	Behandling av primær koenzym Q_{10} mangel
Icelandic	Úbiquínól	Meðferð við fromkomnu kóensím Q_{10} skorts heilkenni

[1] At the time of designation