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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Hydrocortisone (modified release tablet) for the treatment of adrenal insufficiency

First publication	30 November 2007
Rev.1: transfer of sponsorship	27 August 2009
Rev.2: sponsor's change of address	12 November 2009
Rev.3: sponsor's change of address	6 March 2015
Disclaimer Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.	

On 20 March 2007, orphan designation (EU/3/07/441) was granted by the European Commission to Phoqus Pharmaceuticals Ltd, United Kingdom, for hydrocortisone (modified release tablet) for the treatment of adrenal insufficiency.

The sponsorship was transferred to Diurnal Limited, United Kingdom, in February 2009.

What is adrenal insufficiency?

There are two adrenal glands in the abdomen, located above the kidneys. The adrenal glands secrete important hormones, called "steroid hormones"; these include cortisol (a glucocorticoid hormone), aldosterone (a mineralocorticoid hormone) and dehydroepiandrosterone (a weak androgen, or male hormone). Adrenal insufficiency occurs when adrenal glands do not produce enough of these hormones. Patients affected by this disease suffer of weight loss, muscle weakness, fatigue, low blood pressure, and sometimes darkening of the skin. Adrenal insufficiency can also cause irritability and depression. Some patients experience a reduced general health and impaired sexuality. Because the symptoms often worsen slowly, they are sometimes ignored until a stressful event such as an illness or an accident causes them to become clinically obvious and severe. This can develop into acute adrenal insufficiency ("Addisonian crisis"), which is a life-threatening condition. Some patients still have symptoms even if they are treated with currently available medications.

[1] Following a corrigendum procedure, the active ingredient was renamed from Hydrocortisone (modified release tablet) to Hydrocortisone in July 2020.

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

At the time of designation, adrenal insufficiency affected less than 4.5 in 10,000 people in the European Union (EU). This was equivalent to a total of 225,000 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?

Several products to treat adrenal insufficiency have been authorised, including hydrocortisone, as oral tablets administered in two or three daily doses. Various other steroid hormones can also be used to replace those that are insufficiently produced by the adrenal gland.

Hydrocortisone (modified release tablet) might be of potential significant benefit for the treatment of adrenal insufficiency because of its special way of releasing hydrocortisone into the body once it is administered. This assumption will have to be confirmed at the time of marketing authorisation, as this will be necessary to maintain the orphan status.

How is this medicine expected to work?

Hydrocortisone (also known as cortisol) is the main steroid hormone secreted by the adrenal gland. Hydrocortisone (modified release tablet) is expected to replace the natural cortisol that is missing in adrenal insufficiency, which helps to treat the symptoms of the disease. The product is designed to mimic more closely the level of cortisol in the body, which has a variable profile over the day. In particular, it may improve the early morning fatigues and the patient's compliance with the treatment, since the increase of plasma cortisol would start to occur before the patient awakens in the morning, as occurs in healthy individuals.

What is the stage of development of this medicine?

At the time of submission of the application for orphan designation, no clinical trials in patients with adrenal insufficiency were initiated.

This specific formulation of hydrocortisone (modified release tablet) was not authorised anywhere worldwide for adrenal insufficiency, or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 February 2007 recommending the granting of this designation.

*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 27), Norway, Iceland and Liechtenstein.
At the time of designation, this represented a population of 500,300,000 (Eurostat 2007).

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:

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http://www.diurnal.co.uk/contact

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[2], Norwegian and Icelandic

Language	Active Ingredient	Indication
English	Hydrocortisone (modified release tablet)	Treatment of adrenal insufficiency
Bulgarian	Хидрокортизон (таблетка с изменено освобождаване)	Лечение на надбъбречна недостатъчност
Czech	Hydrocortizon (upravené uvolňovací tablety)	Léčba nedostatečné funkce kůry nadledvin
Danish	Hydrocortison (tablet med modificeret udløsning)	Behandling af binyreinsufficiens
Dutch	Hydrocortisone (tablet met gereguleerde afgifte)	Behandeling van bijnierschorsinsufficiëntie
Estonian	Hüdrokortisoon (modifitseeritud vabanemisega tablett)	Neerupealiste puudulikkuse ravi
Finnish	Hydrokortisoni (depottabletti)	Lisämunuaisen vajaatoiminnan hoito
French	Hydrocortisone (comprimé à libération modifiée)	Traitement de l'insuffisance surrénalienne
German	Hydrocortison (Tabletter mit veraenderter Wirkstoffreisetzung)	Behandlung der Nebennierenrindeninsuffizienz
Greek	Υδροκορτιζόνη (δισκίο τροποποιημένης απελευθέρωσης)	Θεραπεία ανεπάρκειας επινεφριδίων
Hungarian	Hidrokortizon (módosított hatóanyagleadású tabletta)	Mellékveseelégtelenség kezelése
Italian	Idrocortisone (compressa a rilascio modificato)	Trattamento dell'insufficienza surrenalica
Latvian	Hidrokortizons (mainīgas darbības tabletes)	Virsnieru mazspējas ārstēšana
Lithuanian	Hidrokortizonas (modifikuota atpalaiduota tabletė)	Antinksčių nepakankamumo gydymas
Maltese	Hydrocortisone (pillola li terħi l-mediċina b'mod modifikat)	Kura ta' insuffiċjenza adrenali
Polish	Hydrokortyzon (tabletki o zmodyfikowanym uwalnianiu)	Leczenie niewydolności nadnerczy
Portuguese	Hydrocortisone (comprimido de libertação modificada)	Tratamento da insuficiência supra-renal
Romanian	Hidrocortizon(comprimate cu eliberare modificată)	Tratamentul insuficienţei suprarenaliene
Slovak	hydrokortizón (tableta s riadeným uvoľňovaním)	Liečba adrenokortikálnej nedostatočnosti
Slovenian	Hidrokortizon (tablete s prirejenim sproščanjem)	Zdravljenje nadledvične insufucuence

[2] At the time of transfer of sponsorship

Language	Active Ingredient	Indication
Spanish	Hidrocortisona (comprimido de liberación modificada)	Tratamiento de la insuficiencia suprarenal
Swedish	Hydrocortison (tablett med modifierad frisättning)	Behandling av binjureinsufficiens
Norwegian	Hydrokortison (tablett med modifisert frisetting)	Behandling av binyrebarksvikt
Icelandic	Hýdrókortisón (tafla með aðlagaðri losun)	Meðferð á vanstarfsemi nýrnahettna