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EMA/COMP/1257/03

COMMITTEE FOR ORPHAN MEDICINAL PRODUCTS

PUBLIC SUMMARY OF POSITIVE OPINION FOR ORPHAN DESIGNATION OF prasterone for the treatment of adrenal insufficiency

On 28 July 2003, orphan designation (EU/3/03/156) was granted by the European Commission to Medicom Healthcare BV, the Netherlands, for prasterone (Fidelin) for the treatment of adrenal insufficiency.

What is adrenal insufficiency?

The adrenal gland consists of two small glands that are located above the kidneys, in the abdomen. The outer wall (cortex) of the adrenal gland secretes important steroid hormones. These include cortisol, aldosterone and dehydroepiandrosterone. Adrenal insufficiency is a rare disease that occurs when the 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 worsen slowly, they are usually ignored until a stressful event like an illness or an accident causes them to become worse. This can develop into acute adrenal insufficiency (“addisonian crisis”), which is life-threatening.

What are the methods of treatment available?

Several products to treat adrenal insufficiency have been authorised. In particular, various steroid hormones can be used to replace those which are insufficiently produced by the adrenal gland. Prasterone, as adjunct therapy, might be of potential significant benefit for the treatment of adrenal insufficiency, particular in terms of improving the quality of life. This assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

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

According to the information provided by the sponsor, adrenal insufficiency was considered to affect about 113,000 persons in the European Union.

How is this medicinal product expected to act?

Prasterone contains the hormone dehydroepiandrosterone. Dehydroepiandrosterone is a precursor of sexual hormones (so called androgens and estrogens). It acts on peripheral target tissues of sex steroids, but also as a neurosteroid in the brain. Prasterone is expected to replace the dehydroepiandrosterone that is missing due to the adrenal insufficiency, and this may help to treat the symptoms of the disease.

What is the stage of development of this medicinal product?

The effects of prasterone were evaluated in experimental models.

At the time of submission of the application for orphan designation, preliminary clinical trials in patients with adrenal insufficiency were completed.

Prasterone was not marketed anywhere worldwide for adrenal insufficiency, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 13 June 2003 a positive opinion recommending the grant of the above-mentioned designation.

Opinions on orphan medicinal products designations are based on the following cumulative criteria: (i) the seriousness of the condition, (ii) the existence or not of alternative methods of diagnosis, prevention or treatment and (iii) either the rarity of the condition (considered to affect not more than five in ten thousand persons in the Community) or the insufficient return of development investments.

Designated orphan medicinal products are still investigational products, which were considered for designation on the basis of potential activity. An orphan designation is not a marketing authorisation. As a consequence, demonstration of the quality, safety and efficacy will be necessary before this product can be granted a marketing authorisation.

For more information:

Sponsor's contact details:

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*Disclaimer: The number of patients affected by the condition is estimated and assessed for the purpose of the designation, for a European Community population of 377,000,000 (Eurostat 2001) and may differ from the true number of patients affected by the condition. This estimate is based on available information and calculations presented by the sponsor at the time of the application.

Patients associations' contact points:

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Translations of the active ingredient and indication in all EU languages

Language	Active Ingredient	Indication
English	Prasterone	Treatment of Adrenal Insufficiency
Danish	Prasteron	Behandling af binyreinsufficiens
Dutch	Prasterone	Behandeling van bijnierschorsinsufficiëntie
Finnish	Prasteroni	Lisämunuaisen vajaatoiminnan hoito
French	Prastérone	Traitement de l'insuffisance surrénalienne
German	Prasteron	Behandlung der Nebennierenrindeninsuffizienz
Greek	Πραστερόνη	Θεραπεία ανεπάρκειας επινεφριδίων
Italian	Prasterone	Trattamento dell'insufficienza surrenalica
Portuguese	Prasterona	Tratamento da insuficiência supra-renal
Spanish	Prasterona	Tratamiento de la insuficiencia suprarrenal
Swedish	Prasteron	Behandling av binjureinsufficiens