



Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of phenylephrine hydrochloride for the treatment of ileal pouch anal anastomosis related faecal incontinence

On, 20 November 2001 orphan designation (EU/3/01/069) was granted by the European Commission to S.L.A. Pharma (UK) Limited, United Kingdom, for phenylephrine hydrochloride for the treatment of ileal pouch anal anastomosis related faecal incontinence.

What is ileal pouch anal anastomosis related faecal incontinence?

Ileal pouch anal anastomosis is an operation to treat ulcerative colitis (a disease characterised by ulcers/open sores in the large intestine/colon) and familial adenomatous polyposis (inherited condition in which numerous polyps form in the large intestine). The operation consists of the removal of the diseased large intestine and creating a pouch as 'new rectum', which is the last portion of the large intestine. Patients having undergone such an operation may have faecal incontinence (inability to control excrement discharged from the intestines) due to the structure of the pouch. Ileal pouch anal anastomosis related faecal incontinence is a chronically debilitating condition.

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

At the time of designation ileal pouch anal anastomosis related faecal incontinence affected approximately 0.3 in 10,000 people in the European Union (EU)*. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP). This is below the threshold for orphan designation which is 5 in 10,000. This is equivalent to a total of around 11,000 people.

What treatments are available?

At the time of submission of the application for the orphan drug designation there were anti-diarrhoeal medicines authorised for the condition in the Community. Treatments have also consisted of dietary adjustments.

Satisfactory argumentation has been submitted by the sponsor to justify the assumption that phenylephrine hydrochloride might be of potential significant benefit for the treatment of ileal pouch anal anastomosis related faecal incontinence. The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Phenylephrine hydrochloride is a selective alpha-1 adrenergic receptor agonist (a drug that binds the so called alpha-1 adrenergic receptor and activates it). Through this mechanism, the drug is expected to make internal anal sphincter to contract (tighten) and thus prevent incontinence.

* 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.

What is the stage of development of this medicine?

The effects of phenylephrine hydrochloride were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with ileal pouch anal anastomosis related faecal incontinence were ongoing.

Phenylephrine hydrochloride was not marketed anywhere worldwide for the treatment of ileal pouch anal anastomosis related faecal incontinence or designated as orphan medicinal product elsewhere for this condition, 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 7 September 2001 a positive opinion recommending the grant of the above-mentioned designation.

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;
- and either the rarity of the condition (affecting not more than five in 10,000 people in the Community) or the 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 the 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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Patients associations contact points: Not available

Translations of the active ingredient and indication in all EU languages

| Language | Active Ingredient | Indication |
|------------|-----------------------------------|--|
| English | Phenylephrine Hydrochloride | Treatment of ileal pouch anal anastomosis related faecal incontinence |
| Danish | Fenylphrin hydroklorid | Behandling af ileum–anal anastomose med reservoir relateret til fæces inkontinens |
| Dutch | fenylefrine waterstofchloride | Behandeling van faecale incontinentie die verband houdt met anostomose met ileumzak |
| Finnish | Fenylefriinihydrokloridi | Ileoanaalisen pussin anastomoosiin liittyvän ulosteenpidätyskyvyttömyyden hoito |
| French | Hydrochlorure de phényléphrine | Traitement de l'incontinence fécale liée à une anastomose iléo-anale avec poche |
| German | Phenylephrin-Hydrochlorid | Behandlung der fäkalen Inkontinenz im Zusammenhang mit Ileumblasen-Analanastomose |
| Greek | Φαινυληφρίνη υδροχλωριδίου | Αγωγή ακράτειας κοπράνων που έχει προκληθεί από την πρωκτική αναστόμωση που έχει προκληθεί από τον θύλακα ειλεού |
| Italian | Fenilefedrina idroclorato | Trattamento dell'incontinenza fecale associata ad anastomosi ileo-anale con 'pouch' |
| Portuguese | Cloridrato de fenilefrina | Tratamento da incontinência fecal relacionada com a anastomose anal e bolsa-íleo |
| Spanish | Clorhidrato de fenilefrina | Tratamiento de la anastomosis ileoanal con incontinencia fecal |
| Swedish | Fenylefrin hydroklorid | Behandling av fekal inkontinens relaterad till bäckenreservoar och ileoanal anastomos |