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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Antisense NF- κ Bp65 oligonucleotide for the treatment of active ulcerative colitis

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Rev.1: administrative update	23 January 2003
Rev.2: sponsor's change of address	16 December 2011
Rev.3: sponsor's change of address	14 March 2013
Rev.4: sponsor's change of address	10 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 30 July 2002, orphan designation (EU/3/02/106) was granted by the European Commission to InDex Pharmaceuticals AB, Sweden, for antisense NF- κ Bp65 oligonucleotide (Kappaproct) for the treatment of active ulcerative colitis.

What is active ulcerative colitis?

Ulcerative colitis is a disorder of the digestive tract. The lower-bowel becomes inflamed, slow-healing sores appear in the lining of the bowel. Patients experience progressive loosening of the stools and increased bowel movement frequency. The stools contain yellowish liquid produced by infection, mixed with slimy substances that normally protect the lining of the bowel, and blood. Often, there are cramps and pressing need to expel faeces. The disease can give trouble on and off throughout life. Most of the time, the disease is inactive (in remission). When the disease is in remission, most patients feel well and have no symptoms. The disease flares up from time to time and becomes active. That is when inflammation is at its maximum. The disease mostly occurs in young adults.

The condition is chronically debilitating due to severe symptoms, such as general feeling of illness, weight loss, and this has a large impact on the quality of life of patients.



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

At the time of designation, active ulcerative colitis affected approximately 4.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 171,000 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 medicines had been authorised for the treatment of ulcerative colitis in the Community at the time of submission of the application for orphan drug designation.

The oligonucleotide may act on the disease using a different mechanism. This might be of potential significant benefit for the treatment of the active phase of the disease.

How is this medicine expected to work?

Oligonucleotides are short sequences of acid substances called nucleic acids. They are similar to those that carry the genetic information in the cells. The genetic information is used to produce proteins. Oligonucleotides can be used to block a cell from producing a protein. In the case of ulcerative colitis, the oligonucleotide aims to block the production of a protein called transcription factor NF-κB. This is a critical element in the inflammatory process.

What is the stage of development of this medicine?

The effects of Kappaproct have been evaluated in experimental models. At the time of submission of the application for orphan designation, one pilot study in patients with active ulcerative colitis had been initiated.

This product had not been marketed as a medicine anywhere worldwide for treatment of active ulcerative colitis 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 27 June 2002 recommending the granting of this 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;
- either the rarity of the condition (affecting not more than 5 in 10,000 people in the EU) or insufficient returns on investment.

*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. At the time of designation, this represented a population of 380,600,000 (Eurostat 2002).

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:

InDex Pharmaceuticals AB
Retzius väg 8
171 77 Stockholm
Sweden
Tel. +46 8 50 88 47 34
Fax +46 8 50 88 47 39
E-mail: info@indexpharmab.com

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Antisense NF-ΚΒ p65 Oligonucleotide	Treatment of active ulcerative colitis
Danish	Antisense NF-ΚΒ p65 Oligonucleotid	Behandling af ulcerøs colitis
Dutch	Antisense NF-ΚΒ p65 Oligonucleotide	Behandeling van actieve ulceratieve colitis
Finnish	Antisense NF-ΚΒ p65 Oligonukleotidi	Ulserratiivisen koliitin hoito
French	Oligonucléotide antisens NF-ΚΒ p65	Traitement de la colite ulcéreuse active
German	Antisense NF-ΚΒ p65 Oligonucleotid	Behandlung der activen ulcerativen Colitis
Greek	αντιπληροφοριακό ολιγονουκλεοτίδιο NF-ΚΒ p65	Θεραπεία της ενεργούς ελκώδης κολίτιδας
Italian	Oligonucleotide Antisense NF-ΚΒ p65	Trattamento delle colite ulcerativa attiva
Portuguese	Oligonucleótido Antisense NF-ΚΒ p65	Tratamento da colite ulcerosa activa
Spanish	Oligonucleótido NF-ΚΒ p65 Antisense	Tratamiento para colitis ulcerosa activa
Swedish	Antisens NF-ΚΒ p65 oligonukletid	Behandling av aktiv ulcerös kolit

¹ At the time of designation