12 November 2013
EMA/COMP/553063/2013 Rev.1
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Naproxcinod for the treatment of Duchenne muscular dystrophy

First publication	29 October 2013
Rev.1: administrative update	12 November 2013
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 7 October 2013, orphan designation (EU/3/13/1194) was granted by the European Commission to Nicox, France, for naproxcinod for the treatment of Duchenne muscular dystrophy.

What is Duchenne muscular dystrophy?

Duchenne muscular dystrophy (DMD) is a genetic disease that gradually causes weakness and atrophy (wasting) of the muscles. It mainly affects boys, and usually starts before the age of six years. The muscle weakness usually starts in the hips and legs, before reaching the chest, arms, and in the late stage also the heart. Patients with DMD lack normal dystrophin, a protein found in muscles. Because this protein helps to strengthen and protect muscles from injury as muscles contract and relax, in patients with DMD the muscles become weak and eventually stop working.

DMD causes long-term disability and is life-threatening because of its effects on the heart and the respiratory muscles (muscles that are used to breathe). The disease usually leads to death in adolescence or early adulthood.

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

At the time of designation, DMD affected approximately 0.5 in 10,000 people in the European Union (EU). This was equivalent to a total of around 26,000 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).

*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 512,200,000 (Eurostat 2013).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7418 8416
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

What treatments are available?

At the time of designation, no satisfactory method had been authorised in the European Union to treat DMD. Treatment of patients with DMD primarily involved physiotherapy and other supportive treatments.

How is this medicine expected to work?

Naproxcinod is converted in the body into the medicine naproxen and a chemical that releases nitric oxide. Nitric oxide is a vasodilator, a substance that causes the widening of blood vessels and plays an important role at ensuring that sufficient oxygen reaches the muscle during exercise, thereby reducing or delaying muscle damage. Patients with DMD do not have enough nitric oxide which together with the absence of dystrophin leads to muscle damage and inflammation.

Naproxen is a non-steroidal anti-inflammatory drug (NSAID). It works by blocking an enzyme called cyclo-oxygenase, which produces prostaglandins, substances that are involved in the inflammation process. By reducing the production of prostaglandins, naproxen is expected to reduce the inflammation seen in DMD.

What is the stage of development of this medicine?

The effects of naproxcinod have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with naproxcinod in patients with DMD had been started.

At the time of submission, naproxcinod was not authorised anywhere in the EU for DMD 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 4 September 2013 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.

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:

Nicox S.A.
Drakkar 2- Bat D
2405 route des Dolines
CS 10313 Sophia Antipolis
06560 Valbonne
France
Telephone: +33 497 245 300
Telefax: +33 497 2453 09
E-mail: nicox@nicox.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 languages[1], Norwegian and Icelandic

Language	Active ingredient	Indication
English	Naproxcinod	Treatment of Duchenne muscular dystrophy
Bulgarian	Напроксцинод	Лечение на мускулна дистрофия на Duchenne
Czech	Naproxcinod	Léčba pacientů s Duchennovou muskulární dystrofií
Croatian	Naprokscinod	Liječenje Duchenneove mišićne distrofije
Danish	Naproxcinod	Behandling af Duchenne muskeldystrofi
Dutch	Naproxcinod	Behandeling van Duchenne spierdystrofie
Estonian	Naproxcinod	Duchenne'i lihasdüstroofia ravi
Finnish	Naprokssinodi	Duchennen lihasdystrofian hoito
French	Naproxcinod	Traitement de la dystrophie musculaire de Duchenne
German	Naproxcinod	Behandlung der Duchenne-Muskeldystrophie
Greek	Ναπροξινόδη	Θεραπεία της μυϊκής δυστροφίας Duchenne
Hungarian	Naproxcinod	Duchenne dystrophia kezelése
Italian	Naproxcinod	Trattamento della distrofia muscolare di tipo Duchenne
Latvian	Naprokscinods	Dišēna muskuļu distrofijas ārstēšana
Lithuanian	Naprokscinodas	Duchenne (Diušeno) raumenų distrofijos gydymas
Maltese	Naproxcinod	Kura tad-distrofija muskolari tat-tip Duchenne
Polish	Naprokscynod	Leczenie zaniku mięśni typu Duchenne'a
Portuguese	Naproxcinod	Tratamento da distrofia muscular de Duchenne
Romanian	Naproxcinod	Tratamentul distrofiei musculare Duchenne
Slovak	Naproxcinod	Liečba Duchennovej muskulárnej dystrofie
Slovenian	Naprokscinod	Zdravljenje Duchennove mišične distrofije
Spanish	Naproxcinod	Tratamiento de la distrofia muscular de Duchenne
Swedish	Naproxcinod	Behandling av Duchennes muskeldystrofi
Norwegian	Naprokscinod	Behandling av Duchennes muskeldystrofi
Icelandic	Naproxcínód	Meðferð á Duchenne vöðvarýrnun

[1] At the time of designation