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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Apomorphine hydrochloride for the treatment of moderate and severe traumatic brain injury

On 13 May 2011, orphan designation (EU/3/11/862) was granted by the European Commission to Dr Elkan Raphael Gamzu, United Kingdom, for apomorphine hydrochloride for the treatment of moderate and severe traumatic brain injury.

What is moderate and severe traumatic brain injury?

Traumatic brain injury is brain damage caused by a head injury (such as a blow to the head in a traffic accident or a fall). The initial injury to the head and brain usually goes on to cause 'secondary' problems, most frequently due to inflammation and swelling of the brain tissue increasing the pressure within the skull. Traumatic brain injury is classified as mild, moderate or severe according to the patient's level of consciousness: patients with moderate injury are lethargic (lacking in energy) or stuporous (unaware of their surroundings), and those with severe injury are comatose (unconscious). People with moderate or severe traumatic brain injury need to be admitted to hospital for observation and examination, in case the condition gets worse.

Moderate and severe traumatic brain injuries are long-term debilitating and life threatening because they may lead to permanent disability and death.

What is the estimated number of patients affected by moderate and severe traumatic brain injury?

At the time of designation, moderate and severe traumatic brain injury affected approximately 4 in 10,000 people in the European Union (EU)*. This is equivalent to a total of around 203,000 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 27), Norway, Iceland and Liechtenstein. This represents a population of 506,300,000 (Eurostat 2011).

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu

What treatments are available?

At the time of designation, various methods were used to reduce the pressure within the skull in patients with moderate and severe traumatic brain injury, including medicines such as mannitol and surgery.

The sponsor has provided sufficient information to show that apomorphine hydrochloride might be of significant benefit for patients with moderate and severe traumatic brain injury because this medicine works in a different way to existing treatments and early studies show that it might improve the treatment of patients with this condition. This assumption will need to be confirmed at the time of marketing authorisation, in order to maintain the orphan status.

How is this medicine expected to work?

Apomorphine is a well known dopamine agonist; this means that it acts like a substance in the brain called dopamine. Dopamine is involved in higher brain functions, such as consciousness. This medicine is expected to be given by slow injection under the skin to help patients to regain consciousness and improve the functioning of the brain.

What is the stage of development of this medicine?

The sponsor of this application has not conducted any studies in experimental models with apomorphine hydrochloride. However, it has provided the results of studies from the published literature to support its application for orphan designation.

At the time of submission of the application for orphan designation, clinical trials with apomorphine hydrochloride in patients with moderate and severe traumatic brain injury were ongoing.

At the time of submission, apomorphine hydrochloride was not authorised anywhere in the EU for moderate and severe traumatic brain injury. Orphan designation of apomorphine hydrochloride had been granted in the United States of America for treatment of patients in a vegetative state or minimally conscious state for up to twelve months following brain injury (traumatic or spontaneous).

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 9 February 2011 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:

Dr Elkan Raphael Gamzu
Little Barn, Lodge Farm, Elvetham
Fleet Road
Hartley Wintney
Hants RG27 8AS
Tel: +44 1252 842 255

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	Apomorphine hydrochloride	Treatment of moderate and severe traumatic brain injury
Bulgarian	Апоморфин хидрохлорид	Лечение на средно тежка и тежка, травматична мозъчна увреда
Czech	Apomorfin hydrochlorid	Léčba středně závažného až závažného traumatického poranění mozku
Danish	Apomorphinhydrochlorid	Behandling af moderat til svær traumatisk hjerneskade
Dutch	Apomorfinehydrochloride	Behandeling van matig tot ernstig traumatisch hersenletsel
Estonian	Apomorfiinvesinikkloriid	Mõõduka ja raske traumaatilise ajukahjustuse ravi
Finnish	Apomorfiinihydrokloridi	Kohtalaisen ja vaikean traumaattisen aivovaurion hoito
French	Chlorhydrate d'apomorphine	Traitement de blessures traumatiques modérées et sévères du cerveau
German	Apomorphinhydrochlorid	Behandlung mittelschwerer und schwerer traumatischer Hirnverletzungen
Greek	Υδροχλωρική απομορφίνη	Θεραπεία μέτριας και σοβαρής τραυματικής κάκωσης εγκεφάλου
Hungarian	Apomorfin hidroklorid	Középsúlyos vagy súlyos traumás agysérülések kezelése
Italian	Apomorfina cloridrato	Trattamento delle lesioni cerebrali traumatiche gravi e moderate
Latvian	Apomorfīna hidrohlorīds	Smadzeņu vidēji smagu un smagu traumatisku bojājumu ārstēšana
Lithuanian	Apomorfino hidrochloridas	Vidutinio sunkumo ir sunkaus trauminio galvos smegenų pažeidimo gydymas
Maltese	Apomorphine hydrochloride	Kura ta' feriti trawmatiċi tal-moħħ moderati u severi
Polish	Chlorowodorek apomorfiny	Leczenie umiarkowanych i ciężkich urazów mózgu
Portuguese	Cloridrato de Apomorfina	Tratamento de danos cerebrais traumáticos moderados a graves
Romanian	Clorhidrat de apomorfină	Tratamentul leziunilor cerebrale traumatice moderate şi severe
Slovak	Apomorfínchlorid	Liečba stredne závažného až závažného traumatického poškodenia mozgu
Slovenian	Apomorfinijev hidroklorid	Zdravljenje zmernih in težkih travmatskih možganskih poškodb
Spanish	Clorhidrato de apomorfina	Tratamiento de lesiones traumáticas cerebrales moderadas y graves
Swedish	Apomorfinhydroklorid	Behandling av måttlig och svår traumatisk hjärnskada
Norwegian	Apomorfinhydroklorid	Behandling av moderat og alvorlig traumatisk hjerneskade
Icelandic	Apómorfín-hýdróklóríð	Meðferð miðlungs- og alvarlegs heilaáverka á heila

[1] At the time of designation