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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Adenovirus serotype 5 containing partial E1A deletion and an integrin-binding domain for the treatment of glioma

On 16 December 2014, orphan designation (EU/3/14/1396) was granted by the European Commission to Alan Boyd Consultants Ltd, United Kingdom, for adenovirus serotype 5 containing partial E1A deletion and an integrin-binding domain for the treatment of glioma.

What is glioma?

Glioma is a type of brain tumour that affects the 'glial' cells (the cells that surround and support the nerve cells). Patients with glioma can have severe symptoms, but the types of symptoms experienced depend on where the tumour develops in the brain.

Symptoms can include headaches, nausea (feeling sick), loss of appetite, vomiting, and changes in personality, mood, mental capacity and concentration. About one fifth of patients with glioma have seizures (fits) for months or years before the disease is diagnosed.

Glioma is a long-term debilitating and life-threatening disease because of the severe damage to the brain, and is associated with poor long-term survival.

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

At the time of designation, glioma affected approximately 2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 102,000 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 511,100,000 (Eurostat 2014).

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

What treatments are available?

At the time of designation, several medicines were authorised for the treatment of glioma in the EU. Treatments included surgery, radiotherapy (treatment with radiation), and chemotherapy (medicines to treat cancer) to improve survival. Patients also received treatments for the symptoms of glioma, including corticosteroids to reduce pressure within the skull and medicines to prevent seizures.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with glioma because early studies showed a good response in patients whose disease came back after standard treatment. 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?

The medicine is made up of an 'oncolytic' virus, a virus that has been genetically modified so that it is able to specifically target, replicate itself within and destroy glioma cells but not normal cells. When inside a tumour cell, the virus is expected to take over the cell's replication apparatus and use it to make more copies of itself. This action of the virus is expected to kill the cell, leaving the virus to spread to neighbouring tumour cells.

The medicine is expected to be delivered directly into the brain.

What is the stage of development of this medicine?

The effects of the medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with this medicine in patients with glioma were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for glioma or 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 13 November 2014 recommending the granting of this designation.

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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:

Alan Boyd Consultants Ltd
Electra House
Crewe Business Park
Crewe
Cheshire CW1 6GL
United Kingdom
Tel. +44 (0)1270 270 010
Fax +44 (0)127 0253 832
E-mail: info@boydconsultants.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	Adenovirus serotype 5 containing partial E1A deletion and an integrin-binding domain	Treatment of glioma
Bulgarian	Аденовирус серотип 5, с частично премахнат E1A и интегрин-свързваща част	Лечение на глиома
Croatian	Adenovirus serotipa 5 koji ima parcijalnu deleciju E1A i domenu koja veže integrin	Liječenje glioma
Czech	5. sérotyp adenovirózy, který částečně ničí proteiny E1A a přitahuje molekuly integrinů	Léčba gliomů
Danish	Adenovirus serotype 5, der indeholder delvis E1A sletning og et integrinbindende område	Behandling af gliom
Dutch	Adenovirus van het serotype 5 met daarin een partiële E1A deletie en toevoeging van een integrine-bindend domein	Behandeling van glioma
Estonian	Adenoviirus serotüüp 5, mis sisaldab osalist E1A deletsiooni ja integriini siduvat domeeni	Glioomi ravi
Finnish	Adenoviruksen serotyyppi 5 sisältää osittaisen E1A-poiston sekä integriiniä sitovan domeenin	Glioman hoito
French	Adénovirus serotype 5 comprenant suppression partielle de E1A et un domaine avec integrin.	Traitement des gliomes
German	Adenovirus vom Serotyp 5 mit einer teilweisen Deletion von E1A und einer Integrin-bindenden Domäne	Behandlung von Gliomen
Greek	Αδενοϊός οροτύπου 5 που περιέχειμερική διαγραφή της περιοχής E1A καθως και μία περιοχή δέσμευσης της ιντεγκρίνης	Θεραπεία του γλοιώματος
Hungarian	Részleges E1A deléciót és egy integrin-kötő domént tartalmazó 5-ös szerotípusú adenovirus	Glioma kezelése
Italian	Un adenovirus di sierotipo 5 che incorpora una delezione parziale di E1A e un dominio legante integrina	Trattamento del glioma
Latvian	Adenovīrusa 5. serotips, kas satur daļēji dzēstu E1A un ar integrīnu saistošu domēnu	Gliomas ārstēšana
Lithuanian	Adenoviruso 5 serotipas su daline E1A iškrita ir integriną surišančiu domenu	Gliomos gydymas
Maltese	Adenovirus tas-serotip 5 li fih tħassir parzjali tal-E1A u qasam li jorbot l-integrin	Kura tal-glioma
Polish	Adenowirus serotypu 5 zawierający częściową delecje E1A i domeny wiążącej integrynę	Leczenie glejaka
Portuguese	Adenovirus serotipo 5 contendo uma deleção parcial de E1A e um domínio de ligação à integrina	Tratamento do glioma

[1] At the time of designation

Language	Active ingredient	Indication
Romanian	Adenovirus de serotip 5 ce conține deleţia parțială a E1A și un domeniu de legare a integrinei	Tratamentul gliomului
Slovak	Adenovírus sérotypu 5 s čiastočne odstráneným E1A a s integrín viažucou doménou	Liečba gliómu
Slovenian	Adenovirus serotipa 5, ki vsebuje delni izbris E1A in domeno vezano z integrinom	Zdravljenje glioma
Spanish	Un adenovirus serotipo 5 que contiene una supresión parcial de E1A y un dominio de unión de integrina	Tratamiento del glioma
Swedish	Adenovirus serotyp 5 som innefattar en partiell deletion av E1A samt en integrinbindande domän	Behandling av gliom
Norwegian	Adenovirus serotype 5 som inneholder partiell E1A-delesjon og et integrinbindende område	Behandling av gliom
Icelandic	Adenóveira af sermisgerð sem 5 inniheldur hluta af E1A úrfellingu og integrín-bindihneppi	Meðferð á glíóma