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EMA/COMP/209764/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Sodium 2-hydroxylinoleate for the treatment of neuroblastoma

On 24 April 2015, orphan designation (EU/3/15/1485) was granted by the European Commission to Ability Pharmaceuticals SL, Spain, for sodium 2-hydroxylinoleate for the treatment of neuroblastoma.

What is neuroblastoma?

Neuroblastoma is a cancer of certain nerve cells which is usually seen as a lump in the abdomen or around the spine. Symptoms may include weakness, bone pain, loss of appetite and fever.

Neuroblastoma is the most common solid tumour outside the brain in children. In many cases it is present at birth but is diagnosed later when the cancer has spread to other parts of the body and the child begins to show symptoms of the disease.

Neuroblastoma is a long-term debilitating and life-threatening disease that is associated with poor long-term survival.

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

At the time of designation, neuroblastoma affected approximately 1.1 in 10,000 people in the European Union (EU). This was equivalent to a total of around 56,000 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?

At the time of designation, several medicines were authorised in the EU for the treatment of neuroblastoma. Treatments for neuroblastoma included surgery, chemotherapy (medicines to treat cancer) and radiotherapy (treatment with radiation).

The sponsor has provided sufficient information to show that sodium 2-hydroxylinoleate might be of significant benefit for patients with neuroblastoma because it works in a different way to currently authorised treatments and may increase their effectiveness when given together. It may also have an

*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 512,900,000 (Eurostat 2015).

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action against tumours that are resistant to cisplatin, one of the authorised medicines usually given for the 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?

This medicine is expected to work by reducing the activity of several proteins in the 'Akt/mTOR signalling pathway'. This is a mechanism within cells which is important in regulating their growth and survival. In many cancers, including in neuroblastoma, this pathway is overactive, allowing the cancer cells to grow and survive uncontrollably. By reducing the activity of this pathway, the medicine is expected to slow down the progression of the cancer.

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, no clinical trials with the medicine in patients with neuroblastoma had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for neuroblastoma 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 25 March 2015 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:

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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	Sodium 2-hydroxylinoleate	Treatment of neuroblastoma
Bulgarian	Натрий 2-хидроксилинолеат	Лечение на невробластом
Croatian	Natrijev 2-hidroksilinoleat	Liječenje neuroblastoma
Czech	2-hydroxilinoleát sodný	Léčba neuroblastomu
Danish	Natrium 2-hydroxylinolat	Behandling af neuroblastom
Dutch	2-hydroxilinoleaat natrium	Behandeling van neuroblastoom
Estonian	Naatrium 2-hüdroksülinolaat	Neuroblastoomi ravi
Finnish	Natrium 2-hydroksilinolaatti	Neuroblastooman hoito
French	2-hydroxylinoléate de sodium	Traitement du neuroblastome
German	Natrium 2-hydroxilinolat	Behandlung des Neuroblastoms
Greek	2-υδροξυλινελαϊκο νάτριο	Θεραπεία του νευροβλαστώματος
Hungarian	Nátrium 2-hidroxilinolát	Neuroblastoma kezelése
Italian	2-idrossilinoleato di sodio	Trattamento del neuroblastoma
Latvian	Nātrija 2-hidroksilinoleāts	Neiroblastomas ārstēšana
Lithuanian	Natrio 2-hidroksilinoleatas	Neuroblastomos gydymas
Maltese	Sodium 2-hydroxylinoleate	Kura tan-newroblastoma
Polish	2-hydroksylinolean sodu	Leczenie nerwiaka płodowego
Portuguese	2-hidroxilinoleato de sódio	Tratamento do neuroblastoma
Romanian	2-hidroxilinoleat de sodiu	Tratamentul neuroblastomului
Slovak	2-hydroxilinoleát sodný	Liečba neuroblastómu
Slovenian	Natrijev 2-hidroksilinolat	Zdravljenje nevroblastoma
Spanish	2-hidroxilonoleato sódico	Tratamiento del neuroblastoma
Swedish	Natrium 2-hydroxilinolat	Behandling av neuroblastom
Norwegian	Natrium-2-hydroksylinoelat	Behandling av nevroblastom
Icelandic	Natríum 2-hýdroxýlínólati	Meðferð við taugakímfrumuæxli

[1] At the time of designation