29 October 2013
EMA/COMP/562044/2013
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

3,5-diiodothyropropionic acid for the treatment of Allan-Herndon-Dudley syndrome

On 7 October 2013, orphan designation (EU/3/13/1193) was granted by the European Commission to CATS Consultants GmbH, Germany, for 3,5-diiodothyropropionic acid for the treatment of Allan-Herndon-Dudley syndrome.

What is Allan-Herndon-Dudley syndrome?

Allan-Herndon-Dudley syndrome is a brain disorder marked by impaired brain development and intellectual disability. Other symptoms include weak muscle tone, impaired muscle development, poor head control and faulty or involuntary movements. The symptoms start in early childhood.

The condition, which is seen only in boys, is caused by a defective gene for a protein called MCT8 which transports the thyroid hormone T3 into nerve cells, where this hormone is needed for normal nerve development. Only boys are affected because the faulty genes are found only on the X chromosomes and as boys have only one X chromosome, a single copy of the gene is enough to cause the condition. In girls, who have two X chromosomes, a second undamaged copy of the gene can compensate for the faulty one.

Allan-Herndon-Dudley syndrome is a long-term debilitating and life-threatening condition because of its effects on the nervous system and is associated with poor survival.

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

At the time of designation, Allan-Herndon-Dudley syndrome affected approximately 0.2 in 10,000 people in the European Union (EU). This was equivalent to a total of around 10,000 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).

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What treatments are available?

No satisfactory methods of treatments for Allan-Herndon-Dudley syndrome were authorised in the EU at the time of orphan designation.

How is this medicine expected to work?

This medicine has a similar structure and works in the same way as the thyroid hormone T3. The difference is that, unlike T3, it is believed to be able to enter developing nerve cells without the MCT8 transporter protein. As the MCT8 protein is faulty in patients with Allan-Herndon-Dudley syndrome, the medicine is expected to help overcome the body's inability to transport the hormone into the nerves, thereby allowing the nerves to develop properly and relieving symptoms of the disease.

What is the stage of development of this medicine?

The sponsor has provided clinical and non-clinical data from the published literature to support its application for orphan designation.

At the time of submission of the application for orphan designation, no clinical trials with 3,5-diiodothyropropionic acid in patients with Allan-Herndon-Dudley syndrome had started.

At the time of submission, 3,5-diiodothyropropionic acid was not authorised anywhere in the EU for Allan-Herndon-Dudley syndrome. Orphan designation of 3,5-diiodothyropropionic had been granted in the United States for Allan-Herndon-Dudley syndrome.

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.

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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	3,5-diiodothyropropionic acid	Treatment of Allan-Herndon-Dudley syndrome
Bulgarian	3,5-Дийодотиропропионова киселина	Лечение на синдрома на Алън-Херндън-Дъдли
Czech	Kyselina 3,5-dijodothyropropionová	Léčba Allan-Herndon-Dudleyho syndromu
Croatian	3,5-dijodotiropropionatna kiselina	Liječenje Allan-Herndon-Dudleyevog sindroma
Danish	3,5-diiodothyropropionsyre dijodthyropropionsyre	Behandling af Allan-Herndon-Dudley-syndrom
Dutch	3,5-Dijoodthyropropionzuur	Behandeling van het Allan-Herndon-Dudley Syndroom
Estonian	3,5-dijodotüropropioonhape	Allan-Herndon-Dudley sündroomi ravi
Finnish	3,5-dijodi-tyro-propionihappo	Allan-Herndon-Dudleyn oireyhtymän hoito
French	Acide 3,5-diiodothyropropionique	Traitement du Syndrome d'Allan-Herndon-Dudley
German	3,5-Dijodthyropropionsäure	Behandlung des Allan-Herndon-Dudley Syndroms
Greek	3,5-διϊωδοθυροπροπιονικό οξύ	Θεραπεία του συνδρόμου Allan-Herndon-Dudley
Hungarian	3,5-dijódtiropropionsav	Allan-Herndon-Dudley szindróma kezelése
Italian	Acido 3,5-diiodotiropropionico	Trattamento della sindrome di Allan-Herndon-Dudley
Latvian	3,5-dijodtiropropionskābe	Allana-Herndona-Dadlija sindroma ārstēšanai
Lithuanian	3,5-dijodotiropropioninė rūgštis	*Allan-Herndon-Dudley* sindromo gydymas
Maltese	3,5-diiodothyropropionic acid	Kura tas-sindrome t'Allan-Herndon-Dudley
Polish	Kwas 3,5-dijodotyropropionowy	Leczenie zespołu Allana-Herndona-Dudleya
Portuguese	Ácido 3,5-diiodotiropropiónico	Tratamento da síndrome de Allan-Herndon-Dudley
Romanian	Acidul 3,5- diiodotiropropionic	Tratamentul sindromului Allan-Herndon-Dudley
Slovak	Kyselina 3,5-dijódtyropropiónová	Liečba Allanovho-Herndonovho-Dudleyovej syndrómu
Slovenian	3,5-dijodotiropropionska kislina	Zdravljenje Allan-Herndon-Dudleyjevega sindroma
Spanish	Ácido 3,5-Diiodotiropropiónico	Tratamiento del síndrome de Allan-Herndon-Dudley
Swedish	3,5-dijodtyropropionsyra	Behandling av Allan-Herndon-Dudley syndrom
Norwegian	3,5-dijodtyropropionsyre	Behandling av Allan-Herndon-Dudleys syndrom
Icelandic	3,5-díjódótýróprópíónsýra	Meðferð við Allan-Herndon-Dudleys heilkenni

[1] At the time of designation