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EMA/695502/2017

# Public summary of opinion on orphan designation

## Tiratricol for the treatment of Allan-Herndon-Dudley syndrome

On 12 October 2017, orphan designation (EU/3/17/1945) was granted by the European Commission to Medical Need Europe AB, Sweden, for tiratricol 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 gene is found only on the X chromosomes and boys have only one X chromosome. 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 less than 0.01 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 500 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?

No satisfactory methods of treatments for Allan-Herndon-Dudley syndrome were authorised in the EU at the time of orphan designation. Patients were given supportive care including feeding via a tube where necessary.

[*]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 515,700,000 (Eurostat 2017).

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## How is this medicine expected to work?

This medicine has a similar structure to and works in the same way as the thyroid hormone T3. The difference is that, unlike T3, it can enter developing nerve cells without the MCT8 transporter protein. This is expected to allow the medicine to enter nerve cells in patients with Allan-Herndon-Dudley syndrome, replacing the hormone that they cannot transport, and thereby allowing the nerves to develop properly and relieving symptoms of the disease.

## What is the stage of development of this medicine?

The effects of tiratricol have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with tiratricol in patients with Allan-Herndon-Dudley syndrome were ongoing.

At the time of submission, tiratricol was approved in France for a condition known as thyroid hormone resistance syndrome. It was not authorised anywhere in the EU for Allan-Herndon-Dudley syndrome 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 5 October 2017 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:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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 | Tiratricol | Treatment of Allan-Herndon-Dudley syndrome |
| Bulgarian | Тиратрикол | Лечение на синдрома на Алън-Херндън-Дъдли |
| Croatian | Tiratricol | Liječenje Allan-Herndon-Dudleyevog sindroma |
| Czech | Tiratricol | Léčba Allan-Herndon-Dudleyho syndrome |
| Danish | Tiratricol | Behandling af Allan-Herndon-Dudley-syndrom |
| Dutch | Tiratricol | Behandeling van het Allan-Herndon-Dudley syndroom |
| Estonian | Tiratrikool | Allan-Herndon-Dudley sündroomi ravi |
| Finnish | Tiratrikoli | Allan-Herndon-Dudleyn oireyhtymän hoito |
| French | Tiratricol | Traitement du syndrome d'Allan-Herndon-Dudley |
| German | Tiratricol | Behandlung des Allan-Herndon-Dudley Syndroms |
| Greek | Τιρατρικόλη | Θεραπεία του συνδρόμου Allan-Herndon-Dudley |
| Hungarian | Tiratricol | Allan-Herndon-Dudley szindróma kezelése |
| Italian | Tiratricol | Trattamento della sindrome di Allan-Herndon-Dudley |
| Latvian | Tiratrikols | Allana-Herndona-Dadlija sindroma ārstēšanai |
| Lithuanian | Tiratrikolis | *Allan-Herndon-Dudley* sindromo gydymas |
| Maltese | Tiratrikol | Kura tas-sindrome t'Allan-Herndon-Dudley |
| Polish | Tyratrykol | Leczenie zespołu Allana-Herndona-Dudleya |
| Portuguese | Tiratricol | Tratamento da síndrome de Allan-Herndon-Dudley |
| Romanian | Tiratricol | Tratamentul sindromului Allan-Herndon-Dudley |
| Slovak | Tiratrikol | Liečba Allanovho-Herndonovho-Dudleyovej syndrómu |
| Slovenian | Tiratrikol | Zdravljenje Allan-Herndon-Dudleyjevega sindroma |
| Spanish | Tiratricol | Tratamiento del síndrome de Allan-Herndon-Dudley |
| Swedish | Tiratricol | Behandling av Allan-Herndon-Dudley syndrom |
| Norwegian | Tiratrikol | Behandling av Allan-Herndon-Dudleys syndrom |
| Icelandic | Tíratrícól | Meðferð við Allan-Herndon-Dudleys heilkenni |

[1] At the time of designation