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Public summary of opinion on orphan designation

N-(2-aminophenyl)-4-(1-[(1,3-dimethyl-1H-pyrazol-4-yl)methyl]piperidin)benzamide for the treatment of peripheral T-cell lymphoma

On 12 October 2017, orphan designation (EU/3/17/1942) was granted by the European Commission to Celleron Therapeutics Limited, United Kingdom, for N-(2-aminophenyl)-4-(1-[(1,3-dimethyl-1H-pyrazol-4-yl)methyl]piperidin)benzamide (also known as CXD101) for the treatment of peripheral T-cell lymphoma.

What is peripheral T-cell lymphoma?

Peripheral T-cell lymphoma is a cancer of the lymphatic system, a network of vessels that transport fluid from tissues through the lymph nodes and into the bloodstream. In peripheral T-cell lymphoma there is uncontrolled growth of T lymphocytes (T cells), a type of white blood cell found in the lymphatic system. Peripheral T-cell lymphomas include types that mainly occur in the lymph nodes (primary nodal) and types that occur mainly outside the lymph nodes (primary extranodal).

The symptoms of the disease vary according to the type of lymphoma, but the first sign may be a lump in the neck, under the arm or in the groin area, which is caused by an enlarged lymph node. The lymphoma may also affect other organs in the body such as the bone marrow, liver and the skin.

Peripheral T-cell lymphoma is a long-term debilitating and life-threatening condition because in most cases the disease does not respond well to therapy, usually comes back within one year and is associated with early death.

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

At the time of designation, peripheral T-cell lymphoma affected less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 52,000 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 515,700,000 (Eurostat 2017).

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, there were no specific treatments for peripheral T-cell lymphoma, but the disease was treated in the same way as the broader class of lymphomas known as non-Hodgkin's lymphomas, for which several medicines were authorised in the EU. The main treatment was chemotherapy (medicines to treat cancer), sometimes in combination with radiotherapy (treatment with radiation).

The sponsor has provided sufficient information to show that this medicine might be of benefit for patients with peripheral T-cell lymphoma because early results suggested that it might produce a response in patients whose disease had not responded to previous treatments or had come back after 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?

This medicine is an 'HDAC inhibitor'. This means that it blocks enzymes called histone deacetylases (HDACs), which are involved in turning genes 'on' and 'off' within cells. By blocking HDACs, the medicine is expected to 'switch on' the genes that suppress the division and growth of the tumour cells in peripheral T-cell lymphoma. This is expected to lead to a reduction in the growth and division of the cancer cells.

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 the medicine in patients with peripheral T-cell lymphoma were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for peripheral T-cell lymphoma 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	N-(2-aminophenyl)-4-(1-[(1,3-dimethyl-1H-pyrazol-4-yl)methyl]piperidin)benzamide	Treatment of peripheral T-cell lymphoma
Bulgarian	N-(2-аминофенил)-4-(1-[(1,3-диметил-1H-пиразол-4-ил)метил]пиперидин)бензамид	Лечение на периферен Т-клетъчен лимфом
Croatian	N-(2-aminofenil)-4-(1-[(1,3-dimetil-1H-pirazol-4-il)metil]piperidin)benzamid	Liječenje perifernog limfoma T-stanica
Czech	N-(2-aminophenyl)-4-(1-[(1,3-dimethyl-1H-pyrazol-4-yl)methyl]piperidin)benzamide	Léčba periferních T-lymfomů
Danish	N-(2-aminophenyl)-4-(1-[(1,3-dimethyl-1H-pyrazol-4-yl)methyl]piperidin)benzamid	Behandling af perifer T-celle lymfom
Dutch	N-(2-aminophenyl)-4-(1-[(1,3-dimethyl-1H-pyrazol-4-yl)methyl]piperidin)benzamide	Behandeling van perifere T-cel lymfomen
Estonian	N-(2-aminofenüül)-4-(1-[(1,3-dimetüül-1H-pürazool-4-üül)metüül]piperidiin)benzamiid	Perifeerse T-rakulise lümfoomi ravi
Finnish	N-(2-aminofenyyli)-4-(1-[(1,3-dimetyyli-1H-pyratsol-4-yyli)metyyli]piperidiini)bentsamidi	Perifeerisen T-solulymfooman hoito
French	N-(2-aminophenyl)-4-(1-[(1,3-dimethyl-1H-pyrazol-4-yl)methyl]piperidine)benzamide	Traitement du lymphome périphérique à cellules T
German	N-(2-aminophenyl)-4-(1-[(1,3-dimethyl-1H-pyrazol-4-yl)methyl]piperidin)benzamide	Behandlung des peripheren T-Zell-Lymphoms
Greek	N-(2-αμινοφαινυλ)-4-(1-[(1,3-διμεθυλ-1H-πυραζολ-4-yl)μεθυλ]πιπεριδινο)βενζαμίδη	Θεραπεία του λεμφώματος περιφερικών κυττάρων Τ
Hungarian	N-(2-aminofenil)-4-(1-[(1,3-dimetil-1H-pirazol-4-il)metil]piperidin)benzamid	Perifériás T-sejtes lymphoma kezelése
Italian	N-(2-aminophenyl)-4-(1-[(1,3-dimethyl-1H-pyrazol-4-yl)methyl]piperidin)benzamide	Trattamento del linfoma periferico a cellule T
Latvian	N-(2-aminofenil)-4-(1-[(1,3-dimetil-1H-pirazol-4-il)metil]piperidīn)benzamīds	Perifēriskās T-šūnu limfomas ārstēšana
Lithuanian	N-(2-aminofenil)-4-(1-[(1,3-dimetil-1H-pirazol-4-il)metil]piperidin)benzamidas	Periferinės T-ląstelių limfomos gydymas
Maltese	N-(2-amminofenil)-4-(1-[(1,3-dimetil-1H-pirażol-4-il)metil]piperidin)benżammid	Kura tal-limfoma taċ-ċelloli T periferali
Polish	N-(2-aminofenylo)-4-(1-[(1,3-dimetylo-1H-pyrazolo-4-yl)metylo]piperydyno)benzamid	Leczenie obwodowego chłoniaka T-komórkowego
Portuguese	N-(2-aminofenil)-4-(1-[(1,3-dimetil-1*H*-pirazol-4-il)metil]piperidina)benzamida	Tratamento do linfoma periférico das células T
Romanian	N-(2-aminofenil)-4-(1-[(1,3-dimetil-1H-pirazol-4-il)metil]piperidin)benzamidă	Tratamentul limfomului periferic cu celule T
Slovak	N-(2-aminofenyl)-4-(1-[(1,3-dimetyl-1H-pyrazol-4-yl)metyl]piperidín)benzamid	Liečba periférneho T-bunkového lymfómu

[1] At the time of designation

Language	Active ingredient	Indication
Slovenian	N-(2-aminophenil)-4-(1-[(1,3-dimetil-1H-pirazol-4-il)metil]piperidin)benzamid	Zdravljenje perifernega limfoma celic T
Spanish	N-(2-aminofenil)-4-(1-[(1,3-dimetil-1H-pirazol-4-il)met5l]piperidin)benzamida	Tratamiento del linfoma periférico de células T
Swedish	N-(2-aminophenyl)-4-(1-[(1,3-dimethyl-1H-pyrazol-4-yl)methyl]piperidin)benzamide	Behandling av perifert T-cellslymfom
Norwegian	N-(2-aminofenyl)-4-(1-[(1,3-dimetyl-1H-pyrazol-4-yl)metyl]piperidin)benzamid	Behandling av perifert T-celle-lymfom
Icelandic	N-(2-amínóphenýl)-4-(1-[(1,3-dímethýl-1H-pýrazoól-4-ýl)metýl]píperidín)benzamíð	Meðferð við útlægu T-eitilfrumukrabbameini