12 August 2015
EMA/COMP/432098/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Lanreotide acetate for the treatment of autosomal dominant polycystic kidney disease

On 10 August 2015, orphan designation (EU/3/15/1514) was granted by the European Commission to Prof. Dr R.T.Gansevoort, the Netherlands, for lanreotide acetate for the treatment of autosomal dominant polycystic kidney disease.

What is autosomal dominant polycystic kidney disease?

Polycystic kidney disease is an inherited condition marked by the growth of numerous fluid-filled cysts mainly in the kidneys. The growth of cysts eventually affects kidney function and can cause the kidneys to fail. Symptoms include abdominal pain, problems with urinating, high blood pressure and infection.

In most cases polycystic kidney disease is 'autosomal dominant', which means that it is caused by gene mutations (defects) that are 'dominant' because a person can have the disease even if they have inherited a defective gene from only one parent. Autosomal dominant polycystic kidney disease is caused by a mutation of either of two genes, PKD1 and PKD2.

Autosomal dominant polycystic kidney disease is debilitating in the long term and life threatening because patients can develop kidney failure and also problems in other organs such as the heart and the gut.

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

At the time of designation, autosomal dominant polycystic kidney disease affected between 4.2 and 4.7 in 10,000 people in the European Union (EU). This was equivalent to a total of between 215,000 and 241,000 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 512,900,000 (Eurostat 2015).

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What treatments are available?

At the time of designation, Jinarc (tolvaptan) was authorised in the EU for the treatment of autosomal dominant polycystic kidney disease.

The sponsor has provided sufficient information to show that lanreotide acetate might be of significant benefit for patients with autosomal dominant polycystic kidney disease because it works in a different way to the currently authorised treatment, and published data indicate that it may have beneficial effects not only on the kidneys but also on other organs such as the liver. 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?

Lanreotide acetate is expected to work in autosomal dominant polycystic kidney disease by blocking the production inside cells of a substance called cAMP. An excess of cAMP in cells is thought to be involved in the processes that lead to cyst formation. By blocking the production of cAMP, lanreotide acetate is expected to reduce the formation of cysts in the kidney and also in the liver. This is expected to improve the symptoms of autosomal dominant polycystic kidney disease.

What is the stage of development of this medicine?

The effects of lanreotide acetate have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with lanreotide in patients with autosomal dominant polycystic kidney disease were ongoing.

At the time of submission, lanreotide was authorised in several countries worldwide for acromegaly and neuroendocrine tumours.

At the time of submission, lanreotide acetate was not authorised anywhere in the EU for autosomal dominant polycystic kidney disease 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 18 June 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	Lanreotide acetate	Treatment of autosomal dominant polycystic kidney disease
Bulgarian	Ланреотид ацетат	Лечение на автозомно-доминантна поликостозна бъбречна болест
Croatian	Lanerotidacetat	Liječenje autosomno dominantne policistične bolesti bubrega
Czech	Lanreotid acetát	Léčba autozomálně dominantní polycystózy ledvin
Danish	Lanreotid acetat	Behandling af autosomal dominant polycystisk nyresygdom
Dutch	Lanreotideacetaat	Behandeling van autosomale dominante polycystische nierziekte
Estonian	Lanreotiid atsetaat	Autosoom-dominantse polütsüstilise neeruhaiguse ravi
Finnish	Lanreotidiasetaatti	Autosomaalisen dominantin polykystisen munuaistaudin hoito
French	Lanréotide acétate	Traitement de la polykystose rénale autosomique dominante
German	Lanreotid Acetat	Behandlung der autosomal-dominanten polyzystischen Nierenerkrankung
Greek	Λανρεοτίδη οξεική	Θεραπεία της αυτοσωματικής κυρίαρχης πολυκυστικής νόσου των νεφρών
Hungarian	Lanreotid acetát	Autoszomális domináns policisztás vesebetegség kezelése
Italian	Lanreotide acetato	Trattamento della malattia renale policistica autosomica dominante
Latvian	Lanreotīda acetāts	Autosomāli dominantas nieru policistozes ārstēšana
Lithuanian	Lanreotido acetatas	Autosominės dominantinės policistinės inkstų ligos gydymas
Maltese	Lanreotide acetate	Kura tal-marda poliċistika tal-kliewi awtosomali dominanti
Polish	Octan lanreotydu	Leczenie autosomalnie dominującej wielotorbielowatości nerek
Portuguese	Acetato de lanreotida	Tratamento da doença renal poliquística autossómica dominante
Romanian	Acetat de lanreotidă	Tratamentul bolii polichistice renale cu transmitere autozomal dominantă
Slovak	Lanreotid acetát	Liečba autozomálneho dominantného polycystického ochorenia obličiek
Slovenian	lanreotid acetat	Zdravljenje avtosomne dominantne policistične bolezni ledvic
Spanish	Acetato de lanreótida	Tratamiento de la poliquistosis renal autosómica dominante
Swedish	Lanreotid acetat	Behandling av autosomalt dominant polycystisk njursjukdom
Norwegian	Lanreotid acetat	Behandling av autosomal dominant polycystisk nyresykdom
Icelandic	Lanreótíð asetat	Meðferð við nýrnafjölblöðrusjúkdómi með ríkjandi erfðamáta

[1] At the time of designation