



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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EMA/COMP/744439/2012  
Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

Cyclo(-gamma-aminobutyryl-L-phenylalanyl-L-tryptophanyl-D-tryptophanyl-L-lysyl-L-threonyl-L phenylalanyl-N-3-carboxypropyl)-glycine amide, acetate salt for the treatment of acromegaly

On 6 December 2012, orphan designation (EU/3/12/1075) was granted by the European Commission to Dr Ulrich Granzer, Germany, for cyclo(-gamma-aminobutyryl-L-phenylalanyl-L-tryptophanyl-D-tryptophanyl-L-lysyl-L-threonyl-L phenylalanyl-N-3-carboxypropyl)-glycine amide, acetate salt for the treatment of acromegaly.

### What is acromegaly?

Acromegaly is a disease in which the pituitary gland, a small gland located at the base of the brain, produces too much growth hormone. Acromegaly usually affects adults in middle age. In over 90% of patients, it is caused by a non-cancerous tumour of the pituitary gland called a pituitary adenoma. One of the most common symptoms of the disease is the abnormal growth of the hands and feet. The disease can result in serious complications, such as severe damage to the joints and problems affecting the cardiovascular (heart and blood vessels) and respiratory systems.

Acromegaly is a long-term debilitating disease because it can lead to respiratory problems and joint damage, and is life threatening because of the increased risk of cardiovascular disease.

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

At the time of designation, acromegaly affected less than 1.2 in 10,000 people in the European Union (EU)\*. This is equivalent to a total of less than 61,000 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).

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\*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 27), Norway, Iceland and Liechtenstein. This represents a population of 506,300,000 (Eurostat 2011).



## **What treatments are available?**

At the time of designation, several medicines were authorised in the EU to treat acromegaly, including bromocriptine and somatostatin analogues (medicines that block the release of growth hormone) such as octreotide and lanreotide, as well as pegvisomant (a medicine that blocks the effects of growth hormone). Other treatments include surgery and radiotherapy (treatment with radiation).

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with acromegaly based on early studies showing better responses in adenoma cells than octreotide and a potentially better safety profile. These assumptions 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 a somatostatin analogue, a copy of the natural hormone somatostatin. Somatostatin regulates growth hormone production in the body by blocking the release of growth hormone from the pituitary gland. The medicine is expected to attach to the receptors for somatostatin on the surface of cells, thus blocking the release of growth hormone and helping to reduce the symptoms and complications of acromegaly.

## **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 acromegaly had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for acromegaly 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 7 November 2012 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<sup>1</sup>, Norwegian and Icelandic

Language	Active substance	Indication
English	Cyclo(-gamma-aminobutyryl-L-phenylalanyl-L-tryptophanyl-D-tryptophanyl-L-lysyl-L-threonyl-L-phenylalanyl-N-3-carboxypropyl)-glycine amide, acetate salt	Treatment of acromegaly
Bulgarian	Цикло(-гама -аминобутирил-L-фенилаланил-L-триптофанил-D-триптофанил-L-лизил-L-треонил-L-фенилаланил-N-3-карбоксыпропил)-глицин амид, ацетатна сол	Лечение на акромегалия
Czech	Cyklo(-gamma -aminobutyryl-L-fenylalanyl-L-tryptofanyl-D-tryptofanyl-L-lysyl-L-threonyl-L-fenylalanyl-N-3-karboxypropyl)-glycinamid acetát	Léčba akromegalie
Danish	Cyclo(-gamma -aminobutyryl-L-phenylalanyl-L-tryptophanyl-D-tryptophanyl-L-lysyl-L-threonyl-L-phenylalanyl-N-3-carboxypropyl)-glycinamid, acetatsalt	Behandling af akromegali
Dutch	Cyclo(-gamma -aminobutyryl-L-fenylalanyl-L-tryptofanyl-D-tryptofanyl-L-lysyl-L-threonyl-L-fenylalanyl-N-3-carboxypropyl)-glycineamide acetaatzout	Behandeling van acromegalie
Estonian	Tsüklo(-gamma -aminobutüüül-L-fenüüalanüüül-L-trüptofanüüül-D-trüptofanüüül-L-lüsüül-L-treonüüül-L-fenüüalanüüül-N-3-karboksüpropüül)-glütsiinamiid, atsetaatsool	Akromegaalia ravi
Finnish	Syklo(- gamma -aminobutyryyli-L-fenyyliälanyyli-L-tryptofanyyli-D-tryptofanyyli-L-lysyli-L-treonyyli-L-fenyyliälanyyli-N-3-karboksiopropyli)-glysiiniamidi, asetaattisuola	Akromegalian hoito
French	Sel d'acétate du cyclo[(gamma -aminobutyryl-L-phénylalanil-L-tryptophanyl-D-tryptophanyl-L-lysyl-L-thréonyl-L-phénylalanil-N-3-carboxypropyl)-glycine]-amide	Traitement de l'acromégalie
German	Cyclo(-gamma -aminobutyryl-L-phenylalanyl-L-tryptophanyl-D-tryptophanyl-L-lysyl-L-threonyl-L-phenylalanyl-N-3-carboxypropyl)-glycinamid, Azetatsalz	Behandlung der Akromegalie
Greek	Κυκλο(-γάμμα -αμινοβουτυριλο-L-φαινυλαλανυλο-L-τριπτοφανυλο-D-τριπτοφανυλο-L-λυσυλο-L-θρεονυλο-L-φαινυλαλανυλο-N-3-καρβοξυπροπυλο)-γλυκινικό αμίδιο, οξικό άλας	Θεραπεία της μεγαλακρίας

<sup>1</sup> At the time of designation

Language	Active substance	Indication
Hungarian	Ciklo(gamma -aminovajsav-L-fenilalanin-L-triptofán-D-triptofán-L-lizin-L-threonin-L-fenilalanin-N-3-karboxipropil)glicin-amid, acetát só	Acromegália kezelésére
Italian	Ciclo(-gamma -aminobutiril-L-fenilalanil-L-triptofanil-D-triptofanil-L-lisil-L-treonil-L fenilalanil-N-3-carbossipropil)-glicinamide, sale acetato	Trattamento dell'acromegalia
Latvian	Ciklo(-gamma -aminobutiril-L-fenilalanil-L-triptofanil-D-triptofanil-L-lizil-L-treonil-L fenilalanil-N-3-karboksipropil)-glicīnamīda, acetāta sāls	Akromegālijas ārstēšana
Lithuanian	Ciklo(-gama -aminobutiril-L-fenilalanil-L-triptofanil-D-triptofanil-L-lisil-L-treonil-L fenilalanil-N-3-karboksipropil)-glicino amidas, acetato druska	Akromegalijos gydymas
Maltese	Cyclo(-gamma -aminobutyryl-L-phenylalanyl-L-tryptophanyl-D-tryptophanyl-L-lysyl-L-threonyl-L phenylalanyl-N-3-carboxypropyl)-glycine amide, acetate salt	Kura ta' l-akromegalija
Polish	Cyklo(-gamma -aminobutyrylo-L-fenylolanylo-L-tryptofanylo-D-tryptofanylo-L-lizylo-L-treonylo-L fenyolanylo-N-3-karboksypropylo)-glicynamidu octan	Leczenie akromegalii
Portuguese	Ciclo(-gama -aminobutiril-L-fenilalanila-L-triptofanil-D-triptofanil-L-lisil-L-treonil-L fenilalanila-N-3-carboxipropil)-amido-glicina, sal de acetato	Tratamento da acromegalia
Romanian	Ciclo(-gamma -aminobutiril-L-fenilalanil-L-triptofanil-D-triptofanil-L-lisil-L-treonil-L fenilalanil-N-3-carboxiproxil)-glicin amidă, sare acetat	Tratamentul acromegaliei
Slovak	Cyklo(-gama -aminobutyryl-L-fenylalanyl-L-tryptofyl-D-tryptofyl-L-lyzyl-L-treonyl-L-fenylalanyl-N-3-karboxypropyl)-glycīnamid acetát	Liečba akromegálie
Slovenian	Ciklo(-gama -aminobutiril-L-fenilalanil-L-triptofanil-D-triptofanil-L-lizil-L-treonil-L fenilalanil-N-3-karboksipropil)-glicin amid, acetatna sol	Zdravljenje akromegalije
Spanish	Ciclo(-gamma-aminobutiril-L-fenilalanil-L-triptofanil-D-triptofanil-L-lisil-L-threonil-L fenilalanil-N-3-carboxipropil) -glicina amida, sal de acetato	Tratamiento de la acromegalia
Swedish	Cyklo(-gamma -aminobutyryl-L-fenylalanyl-L-tryptofanyl-D-tryptofanyl-L-lysyl-L-treonyl-L-fenylalanyl-N-3-karboxypropyl)-glycinamid, acetatsalt	Behandling av akromegali
Norwegian	Syklo(-gamma -aminobutyryl-L-fenylalanyl-L-tryptofanyl-D-tryptofanyl-L-lysyl-L-treonyl-L-fenylalanyl-N-3-karboksypropyl)-glysinamid, acetatsalt	Behandling af akromegali

Language	Active substance	Indication
Icelandic	Cýkló(-gamma -amínóbútýrýl-L-fenýlalanýl-L-tryptófanýl-D-tryptófanýl-L-lysýl-L-treónýl-L-fenýlalanýl-N-3-karboxýprópýl)-glýsínamíð, asetatsalt	Meðhöndlun æsavvaxtar