

29 January 2016
EMA/COMP/794963/2015
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Synthetic peptide L-cysteine, L-cysteinylglycyl-L-glutaminy-L-arginyl-L-.alpha.-glutamyl-L-threonyl-L-prolyl-L-.alpha.-glutamylglycyl-L-alanyl-L-.alpha.-glutamyl-L-alanyl-L-lysyl-L-prolyl-L-tryptophyl-L-tyrosyl-, cyclic (1.fwdarw.17)-disulfide for treatment of pseudohypoaldosteronism type 1B

On 14 December 2015, orphan designation (EU/3/15/1591) was granted by the European Commission to Apeptico Forschung und Entwicklung GmbH, Austria, for synthetic peptide L-cysteine, L-cysteinylglycyl-L-glutaminy-L-arginyl-L-.alpha.-glutamyl-L-threonyl-L-prolyl-L-.alpha.-glutamylglycyl-L-alanyl-L-.alpha.-glutamyl-L-alanyl-L-lysyl-L-prolyl-L-tryptophyl-L-tyrosyl-, cyclic (1.fwdarw.17)-disulfide (also known as solnatide) for treatment of pseudohypoaldosteronism type 1B.

What is pseudohypoaldosteronism 1B?

Pseudohypoaldosteronism 1B is an inherited disorder characterised by abnormal salt and water balance in the body. The disease is caused by a genetic mutation (change) leading to defects in certain proteins called sodium ion channels located in the cells of the gut, kidney and lungs. These ion channels regulate the transport of salt and water in the body. As a result of the mutation, patients have sodium loss from the body, dehydration and high blood levels of potassium and acid, which can lead to organ damage. In the lungs, fluid accumulation in the alveoli (tiny air sacs in the lungs where oxygen is absorbed) leads to cough and repeated infections.

Pseudohypoaldosteronism 1B is a long-lasting, debilitating disease and may be life threatening because of its damaging effects on the gut, lungs and kidneys.

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

At the time of designation, pseudohypoaldosteronism 1B affected approximately 0.02 in 10,000 people in the European Union (EU). This was equivalent to a total of around 1,000 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).

What treatments are available?

At the time of designation, no medicines were authorised in the EU for the treatment of pseudohypoaldosteronism 1B. Treatments were aimed at correcting potassium levels, fluid balance and other symptoms of the disease rather than correcting the underlying cause of the disease.

How is this medicine expected to work?

This medicine is intended to be given by inhalation. When inhaled, it is expected to activate sodium ion channels and facilitate the transport of sodium into the bloodstream. This is expected to help reduce the fluid in the alveoli and thereby reduce lung infections and improve cough, and also to improve the general electrolyte imbalance in patients with pseudohypoaldosteronism 1B.

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 pseudohypoaldosteronism 1B had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for pseudohypoaldosteronism 1 B 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 12 November 2015 recommending the granting of this designation.

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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Synthetic peptide L-cysteine, L-cysteinylglycyl-L-glutaminy-L-arginyl-L-.alpha.-glutamyl-L-threonyl-L-prolyl-L-.alpha.-glutamylglycyl-L-alanyl-L-.alpha.-glutamyl-L-alanyl-L-lysyl-L-prolyl-L-tryptophyl-L-tyrosyl-, cyclic (1.fwdarw.17)-disulfide	Treatment of pseudohypoaldosteronism type 1B
Bulgarian	Синтетичен пептид съдържащ L-цистеин, L-цистеинилглицил-L-глутамил-L-аргинил-L-алфа-глутамил-L-треонил-L-пролил-L-алфа-глутамилглицил-L-аланил-L-алфа-глутамил-L-аланил-L-лизил-L-пролил-L-триптофил-L-тирозил-, цикличен (1.fwdarw.17)-дисулфид	Лечение на псевдохипоалдостеронизъм тип 1B
Croatian	Sintetički peptid L-cistein, L-cisteinilglicil-L-glutaminil-L-arginil-L-.alfa.-glutamyl-L-treonil-L-prolil-L-.alfa.-glutamylglicil-L-alanil-L-.alfa.-glutamyl-L-alanil-L-lizil-L-prolil-L-triptofil-L-tirozil-, ciklični (1.fwdarw.17)-disulfid	Liječenje pseudohipoaldosteronizma tipa 1B
Czech	Syntetický peptid L-Cysteine, L-cysteinylglycyl-L-glutaminy-L-arginyl-L-.alpha.-glutamyl-L-threonyl-L-prolyl-L-.alpha.-glutamylglycyl-L-alanyl-L-.alpha.-glutamyl-L-alanyl-L-lysyl-L-prolyl-L-tryptophyl-L-tyrosyl-, cyclic (1.fwdarw.17)-disulfid	Léčba pseudohypoaldosteronismus typ 1B
Danish	Syntetisk peptid L-ystein, L-cysteinylglycyl-L-glutaminy-L-arginyl-L-.alpha.-glutamyl-L-threonyl-L-prolyl-L-.alpha.-glutamylglycyl-L-alanyl-L-.alpha.-glutamyl-L-alanyl-L-lysyl-L-prolyl-L-tryptophyl-L-tyrosyl-, cyclic (1.fwdarw.17)-disulfid	Behandling af pseudohypoaldosteronism type 1B
Dutch	Synthetisch peptide L-Cysteine, L-cysteinylglycyl-L-glutaminy-L-arginyl-L-.alpha.-glutamyl-L-threonyl-L-prolyl-L-.alpha.-glutamylglycyl-L-alanyl-L-.alpha.-glutamyl-L-alanyl-L-lysyl-L-prolyl-L-tryptophyl-L-tyrosyl-, cyclic (1.fwdarw.17)-disulfide	Behandeling van pseudohypoaldosteronisme type 1B
Estonian	Süntetiline peptiid L-tsüsteiin, L-tsüsteiinüülglüküül-L-glutaminüül-L-arginüül-L-.afa.-glutamüül-L-threonüül-L-prolül-L-.alfa.-glutamüülglicüül-L-alanüül-L-.alfa.-glutamüül-L-alanüül-L-lüsüül-L-prolül-L-trüptofüül-L-türosüül-, tsüklik (1.fwdarw.17)-disulfiid	1B tüüpi pseudohüpoaldosteronism ravi

¹ At the time of designation

Language	Active ingredient	Indication
Finnish	Synteettinen L-kysteiniini, L-kysteinylglysyyl-L-glutaminyyli-L-arginyyli-L-.alpha.-glutamyyli-L-treonyyli-L-prolyyli-L-.alpha.-glutamyyliglysyyl-L-alanyyli-L-.alpha.-glutamyyli-L-alanyyli-L-lysyli-L-prolyyli-L-tryptofyyli-L-tyrosyyli-, syklinen (1.fwdarw.17)-disulfiidi	Tyypin 1B pseudohypoaldosteronismien hoito
French	Peptide synthétique L-Cysteine, L-cysteinylglycyl-L-glutaminyli-L-arginyl-L-.alpha.-glutamyl-L-threonyl-L-prolyl-L-.alpha.-glutamylglycyl-L-alanyl-L-.alpha.-glutamyl-L-alanyl-L-lysyl-L-prolyl-L-tryptophyl-L-tyrosyl-, cyclic (1.fwdarw.17)-disulfide	Traitement du pseudohypoaldostéronisme de type 1B
German	Synthetisches Peptid L-Cysteine, L-cysteinylglycyl-L-glutaminyli-L-arginyl-L-.alpha.-glutamyl-L-threonyl-L-prolyl-L-.alpha.-glutamylglycyl-L-alanyl-L-.alpha.-glutamyl-L-alanyl-L-lysyl-L-prolyl-L-tryptophyl-L-tyrosyl-, cyclic (1.fwdarw.17)-disulfid	Behandlung des Pseudohypoaldosteronismus Typ 1B
Greek	Συνθετικό πεπτιδιο L-κυστεϊνη, L-κυστεϊνυλγλυκυλ-L-γλουταμινυλ-L-αργινυλ-L-α-γλουταμυλ-L-θρεονυλ-L-προλυλ-L-α γλουταμυλγλυκυλ-L-αλανυλ-L-α-γλουταμυλ-L-αλανυλ-L-λυσυλ-L-προλυλ-L-τρυπτοφυλ-L-τυροσυλ-, κυκλικό (1.fwdarw.17)-δισουλφίδιο	Θεραπεία του ψευδοϋποαλδοστερονισμού τύπου 1B
Hungarian	Szintetikus L-cisztein, _ciszteinilglicil-L-glutaminil-L-arginil-L-.alfa.-glutamil-L-treonil-L-propil-L-.alf.-glutamilglicil-L-alanil-L-.alfa.-glutamil-L-alanil-L-lizil-L-propil-L-tripofil-L-tirozil-, ciklikus (1.fwdarw.17) diszulfid	B1-típusú pseudohypoaldosteronismus kezelése
Italian	Peptide sintetico L-Cysteine, L-cysteinylglycyl-L-glutaminyli-L-arginyl-L-.alpha.-glutamyl-L-threonyl-L-prolyl-L-.alpha.-glutamylglycyl-L-alanyl-L-.alpha.-glutamyl-L-alanyl-L-lysyl-L-prolyl-L-tryptophyl-L-tyrosyl-, cyclic (1.fwdarw.17)-disulfide	Trattamento dello pseudoipoaldosteronismo tipo 1B
Latvian	Sintētisks L-cisteīna, L-cisteīnīlglicil-L-glutaminil-L-arginil-L-.alfa.-glutamil-L-treonil-L-prolil-L-.alfa.-glutamilglicil-L-alanil-L-.alfa.-glutamil-L-alanil-L-lizil-L-prolil-L-triptofil-L-tirozil-, ciklisks (1.fwdarw.17)-disulfida peptīds	1B tipa pseidohipoaldosteronisma ārstēšana
Lithuanian	Sintetinis L-cisteino, L-cisteinilglycyl-L-glutaminil-L-arginil-L-.alfa.-glutamil-L-treonil-L-prolil-L-.alfa.-glutamilglycyl-L-alanil-L-.alfa.-glutamil-L-alanil-L-lysyl-L-prolil-L-triptofil-L-tirozil-, ciklic (1.fwdarw.17)-disulfido peptidas	Pseudohipoaldosteronismo 1B tipo gydymas
Maltese	Peptide L-cysteine, L-cysteinylglycyl-L-glutaminyli-L-arginyl-L-.alpha.-glutamyl-L-threonyl-L-prolyl-L-.alpha.-glutamylglycyl-L-alanyl-L-.alpha.-glutamyl-L-alanyl-L-lysyl-L-prolyl-L-tryptophyl-L-tyrosyl-, cyclic (1.fwdarw.17)-disulfide sintetiku	Kura ta' psewdoipoaldosteroniżmu tat-tip 1B

Language	Active ingredient	Indication
Polish	Syntetyczny peptyd L-Cysteine, L-cysteinylglycyl-L-glutaminył-L-arginyl-L-.alpha.-glutamyl-L-threonył-L-prolył-L-.alpha.-glutamylglycyl-L-alanyl-L-.alpha.-glutamyl-L-alanyl-L-lysyl-L-prolył-L-tryptophyl-L-tyrosyl-, cyclic (1.fwdarw.17)-disulfide	Leczenie pseudohypoaldosteronizmu typu 1B
Portuguese	Peptídeo sintético L-cisteína, L-cisteinilglicil-L-glutaminil-L-arginil-L-.alfa.-glutamil-L-treonil-L-prolil-L-.alfa.-glutamilglicil-L-alanil-L-.alfa.-glutamil-L-alanil-L-lisil-L-prolil-L-triptofil-L-tirosil-, ciclico (1.fwdarw.17)-dissulfeto	Tratamento de pseudohipoaldosteronismo tipo 1B
Romanian	Peptid sintetic L-Cistein, L-cisteinilglicil-L-glutaminil-L-arginil-L-.alfa.-glutamil-L-treonil-L-prolil-L-.alfa.-glutamilglicil-L-alanil-L-.alfa.-glutamil-L-alanil-L-lizil-L-prolil-L-triptofil-L-tirozil-, ciclic (1.fwdarw.17)-disulfid	Tratamentul pseudohipoaldosteronismului de tip 1B
Slovak	Syntetický L-Cysteine, L-cysteinylglycyl-L-glutaminył-L-arginyl-L-.alpha.-glutamyl-L-threonył-L-prolył-L-.alpha.-glutamylglycyl-L-alanyl-L-.alpha.-glutamyl-L-alanyl-L-lysyl-L-prolył-L-tryptophyl-L-tyrosyl-, cyclic (1.fwdarw.17)-disulfide peptid	Liečba pseudohypoaldosteronizmu typ 1B
Slovenian	Sintetični peptid L-cistein, L-cisteinilglicil-L-glutaminil-L-arginil-L-.alfa.-glutamil-L-treonil-L-prolil-L-.alfa.-glutamilglicil-L-alanil-L-.alfa.-glutamil-L-alanil-L-lisil-L-prolil-L-triptofil-L-tirosil-, ciklični (1.fwdarw.17)-disulfid	Zdravljenje pseudohypoaldosteronism tipa 1B
Spanish	Peptido sintético L-cisteína, L-cisteinilglicil-L-glutaminil-L-arginil-L-.alfa.-glutamil-L-treonil-L-prolil-L-.alfa.-glutamilglicil-L-alanil-L-.alfa.-glutamil-L-alanil-L-lisil-L-prolil-L-triptofil-L-tirosil-, ciclico (1.fwdarw.17)-disulfuro	Tratamiento del pseudohipoaldosteronismo tipo 1B
Swedish	Synthetisk peptid L-Cysteine, L-cysteinylglycyl-L-glutaminył-L-arginyl-L-.alpha.-glutamyl-L-threonył-L-prolył-L-.alpha.-glutamylglycyl-L-alanyl-L-.alpha.-glutamyl-L-alanyl-L-lysyl-L-prolył-L-tryptophyl-L-tyrosyl-, cyclic (1.fwdarw.17)-disulfide	Behandling av pseudohypoaldosteronism typ 1B
Norwegian	Syntetisk peptid L-Cysteine, L-cysteinylglycyl-L-glutaminył-L-arginyl-L-.alpha.-glutamyl-L-threonył-L-prolył-L-.alpha.-glutamylglycyl-L-alanyl-L-.alpha.-glutamyl-L-alanyl-L-lysyl-L-prolył-L-tryptophyl-L-tyrosyl-, cyclic (1.fwdarw.17)-disulfide	Behandling av pseudohypoaldosteronism type 1B
Icelandic	Samtengt peptíð L-Cýstein, L-cýsteinýlglýcýl-L-glútamínýl-L-arginýl-L-.alfa.-glútamýl-L-threónýl-L-prólýl-L-.alfa.-glútamýlglýcýl-L-alanýl-L-.alfa.-glútamýl-L-alanýl-L-lýsýl-L-prólýl-L-trýptóphýl-L-týrósfýl-, cýclic (1.fwdarw.17)-dísúlfíð	Meðferð við pseudóhýpóaldósterónisma gerð 1B