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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Sodium acetate salt of the synthetic peptide H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH for the treatment of necrotising soft tissue infections

First publication	27 August 2014
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Disclaimer

Please note that revisions to the Public Summary of Opinion are purely administrative updates. Therefore, the scientific content of the document reflects the outcome of the Committee for Orphan Medicinal Products (COMP) at the time of designation and is not updated after first publication.

On 29 July 2014, orphan designation (EU/3/14/1294) was granted by the European Commission to Dr Ulrich Granzer, Germany, for sodium acetate salt of the synthetic peptide H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH for the treatment of necrotising soft tissue infections.

The sponsorship was transferred to FGK Representative Service GmbH, Germany, in March 2015.

What are necrotising soft tissue infections?

Necrotising soft tissue infections are severe and rapidly progressing bacterial infections that destroy skin, muscle, and other soft tissues. A necrotising infection causes areas of tissue to die and often leads to failure of several organs, which eventually may lead to death. The most dangerous type of these infections is commonly known as 'flesh-eating disease'.

Necrotising soft tissue infections are life threatening and long-term debilitating because of the extent of damage to soft tissues and high mortality rate.

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

At the time of designation, necrotising soft tissue infections affected less than 1 people in 10,000 in the European Union (EU). This was equivalent to fewer than 51,000 people*, and is below the ceiling for

*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.
At the time of designation, this represented a population of 512,900,000 (Eurostat 2014).

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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?

At the time of designation, antibiotics were authorised in the EU to treat necrotising soft tissue infections. Surgery (often repeated) and admission to critical care units were also used to try to control the infections.

The sponsor has provided sufficient information to show that this medicine might be of significant benefit for patients with necrotising soft tissue infections because it works in a different way to existing treatments and early studies show that it might improve the outcome of patients with the condition. 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?

Necrotising soft tissue infections can produce rapid organ failure due to a massive inflammatory response affecting the whole body. This inflammatory response is thought to be caused by an excess production of cytokines (substances produced by the body as a defence mechanism, for example against infections) and of toxins from bacteria.

This medicine is made of a small peptide that mimics part of a receptor called CD28, which is found on the surface of some white blood cells and is involved in their activation. Although its mechanism of action is not fully understood, the medicine is expected to block the excessive production of cytokines by white blood cells, thus improving the outcome of patients with necrotising soft tissue infections.

What is the stage of development of this medicine?

The effects of this medicine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, a clinical trial with the medicine in patients with necrotising soft tissue infections had been completed and further studies were planned.

At the time of submission, the medicine was not authorised anywhere in the EU for necrotising soft tissue infections. Orphan designation of the medicine had been granted in the United States for necrotising soft tissue infections.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 12 June 2014 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	Sodium acetate salt of the synthetic peptide H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH	Treatment for necrotising soft tissue infections
Bulgarian	Натриева ацетатна сол на синтетичния пептид H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH	Лечение на некротизиращи мекотъканни инфекции
Croatian	Natrijev acetat sintetičkog peptida H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tir-Asp-D-Ala-OH	Liječenje nekrotizirajućih infekcija mekih tkiva
Czech	Natrium acetátová sůl syntetického peptidu H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH	Léčba infekcí nekrotizující měkké tkáně
Danish	Natriumacetatsalt af det syntetiske peptid H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH	Behandling af nekrotiserende bløddelsinfektioner
Dutch	Natriumacetaatzout van het synthetische peptide H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH	Behandeling van necrotiserende weke delen infecties
Estonian	Sünteetilise peptiidi H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH naatriumatsetaatsool	Pehmete kudedee nekrotiseerivate infektsioonide ravi
Finnish	Synteettisen H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH-peptidin natriumasetaattisuola	Nekrotisoivien pehmytkudosinfektioiden hoito
French	Sel d'acétate de sodium du peptide synthétique H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH	Traitement des infections nécrosantes des tissus mous
German	Natriumacetatsalz des synthetischen Peptids H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH	Behandlung nekrotisierender Weichgewebeinfektionen
Greek	Άλας οξικού νατρίου του συνθετικού πεπτιδίου H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH	Θεραπεία των νεκρωτικών λοιμώξεων των μαλακών μορίων
Hungarian	A szintetikus, H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH peptid nátrium-acetát sója	Szövetelhalással járó lágyrészfertőzések kezelése
Italian	Sale acetato di sodio del peptide sintetico H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH	Trattamento delle infezioni necrotizzanti dei tessuti molli
Latvian	Sintētiskā peptīda H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH nātrija acetāta sāls	Nekrotizējošu mīksto audu infekciju ārstēšana
Lithuanian	Sintetinio peptido H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH natrio acetato druska	Minkštųjų audinių nekrotizuojančių infekcijų gydymas
Maltese	Melħ ta' sodium acetate tal-peptide sintetiku H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH	Kura għal infezzjonijiet tat-tessut artab bin-nekrosi
Polish	Sól sodowa octanu syntetycznego peptydu H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH	Leczenie martwiczych zakażeń tkanek miękkich

[1] At the time of designation

Language	Active ingredient	Indication
Portuguese	Sal de acetato de sódio do peptídeo sintético H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH	Tratamento de infecções necrozantes dos tecidos moles
Romanian	Sare acetat de sodiu a peptidei sintetice H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH	Tratamentul infecţiilor necrozante ale ţesuturilor moi
Slovak	Nátrium-acetátová soľ syntetického peptidu H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH	Liečba nekrotizujúcich infekcií mäkkého tkaniva
Slovenian	Sol natrijevega acetata sintetičnega peptida H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH	Zdravljenje nekrotizirajoči okužb mehkih tkiv
Spanish	Sal de acetato sódico del péptido sintético H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH	Tratamiento de infecciones necrotizantes de los tejidos conectivos celulares subcutáneos
Swedish	Natriumacetatsalt av den syntetiska peptiden H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH	Behandling av nekrotiserande mjukdelsinfektioner
Norwegian	Natriumacetatsalt av det syntetiske peptidet H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH	Behandling av nekrotiserende bløtdelsinfeksjoner
Icelandic	Natríumasetatsalt tilbúna peptíðsins H-D-Ala-Ser-Pro-Met-Leu-Val-Ala-Tyr-Asp-D-Ala-OH	Meðferð við sýkingum í mjúkvef með drepi