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Public summary of opinion on orphan designation

Asp-Arg-Val-Tyr-Ile-His-Pro for the treatment of epidermolysis bullosa

On 20 June 2017, orphan designation (EU/3/17/1879) was granted by the European Commission to Envigo Pharma Consulting Limited, United Kingdom, for Asp-Arg-Val-Tyr-Ile-His-Pro (also known as TXA127) for the treatment of epidermolysis bullosa.

What is epidermolysis bullosa?

Epidermolysis bullosa is a group of inherited diseases of the skin, in which the skin is very fragile and forms severe blisters after even minor friction (rubbing) or injury. In most cases, symptoms of epidermolysis bullosa appear from birth, but for some forms, symptoms may not occur until adulthood. The diseases are caused by mutations (changes) in the genes responsible for the production of certain proteins that make the skin strong and elastic, such as collagen or keratins.

Epidermolysis bullosa is a long-term debilitating and life-threatening condition because the severe blistering and associated scarring and deformities result in poor quality of life and may also reduce life expectancy.

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

At the time of designation, epidermolysis bullosa affected approximately 0.7 in 10,000 people in the European Union (EU). This was equivalent to a total of around 36,000 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).

What treatments are available?

At the time of designation, no satisfactory methods were authorised in the EU to treat epidermolysis bullosa. Good personal hygiene and skincare were recommended to help blisters heal, to avoid infections and to protect the skin from damage. Painkillers were also used. Surgery was sometimes necessary for complications such as deformed hands or the development of skin cancer.

*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).

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How is this medicine expected to work?

The medicine is a synthetic form of angiotensin-(1-7), which is a substance naturally found in the body that reduces the development of scar tissue (fibrosis) and inflammation. How it works in the treatment of epidermolysis bullosa is not completely understood but is thought to involve reducing fibrosis in the skin by stimulating a receptor (target) on cells called the Mas receptor. The medicine also reduces inflammation. It is expected that these effects will help to reduce the symptoms of epidermolysis bullosa.

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 epidermolysis bullosa had been started.

At the time of submission, the medicine was not authorised anywhere in the EU for epidermolysis bullosa 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 May 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	Asp-Arg-Val-Tyr-Ile-His-Pro	Treatment of epidermolysis bullosa
Bulgarian	Asp-Arg-Val-Tyr-Ile-His-Pro	Лечение на булозна епидермолиза
Croatian	Asp-Arg-Val-Tyr-Ile-His-Pro	Liječenje bulozne epidermolize
Czech	Asp-Arg-Val-Tyr-Ile-His-Pro	Léčba bulózní epidermolýzy
Danish	Asp-Arg-Val-Tyr-Ile-His-Pro	Behandling af epidermolysis bullosa
Dutch	Asp-Arg-Val-Tyr-Ile-His-Pro	Behandeling van epidermolysis bullosa
Estonian	Asp-Arg-Val-Tyr-Ile-His-Pro	Bulloosse epidermolüüsi ravi
Finnish	Asp-Arg-Val-Tyr-Ile-His-Pro	Epidermolysis bullosan hoito
French	Asp-Arg-Val-Tyr-Ile-His-Pro	Traitement de l'épidermolyse bulleuse
German	Asp-Arg-Val-Tyr-Ile-His-Pro	Behandlung der Epidermolysis bullosa
Greek	Asp-Arg-Val-Tyr-Ile-His-Pro	Θεραπεία της πομφολυγώδους επιδερμόλυσης
Hungarian	Asp-Arg-Val-Tyr-Ile-His-Pro	Epidermolysis bullosa kezelése
Italian	Asp-Arg-Val-Tyr-Ile-His-Pro	Trattamento della epidermolisi bollosa
Latvian	Asp-Arg-Val-Tyr-Ile-His-Pro	Bulozās epidermolīzes ārstēšanai
Lithuanian	Asp-Arg-Val-Tyr-Ile-His-Pro	Pūslinės epidermolizės gydymas
Maltese	Asp-Arg-Val-Tyr-Ile-His-Pro	Kura tal-epidermolisi bullosa
Polish	Asp-Arg-Val-Tyr-Ile-His-Pro	Pęcherzowe oddzielanie się naskórka
Portuguese	Asp-Arg-Val-Tyr-Ile-His-Pro	Tratamento da epidermólise bulhosa
Romanian	Asp-Arg-Val-Tyr-Ile-His-Pro	Tratamentul epidermolizei buloase
Slovak	Asp-Arg-Val-Tyr-Ile-His-Pro	Liečba epidermolysis bullosa
Slovenian	Asp-Arg-Val-Tyr-Ile-His-Pro	Zdravljenje bulozne epidrmolize
Spanish	Asp-Arg-Val-Tir-Ile-His-Pro	Tratamiento de la epidermolisis bullosa
Swedish	Asp-Arg-Val-Tyr-Ile-His-Pro	Behandling av epidermolysis bullosa
Norwegian	Asp-Arg-Val-Tyr-Ile-His-Pro	Behandling av epidermolysis bullosa
Icelandic	Asp-Arg-Val-Tyr-Ile-His-Pro	Meðferð á epidermolysis bullosa

[1] At the time of designation