



European Medicines Agency
Pre-authorisation Evaluation of Medicines for Human Use

Document Date: London, 12 November 2009
Doc.Ref.: EMEA/COMP/152574/2004 Rev.1

Please note that this product was withdrawn from the Community Register of designated Orphan Medicinal Products in March 2009 on request of the Sponsor.

Committee for Orphan Medicinal Products

Public summary of positive opinion for orphan designation of Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptide, sequence 169-177, of proteinase 3) for the treatment of myelodysplastic syndromes

On 21 December 2004, orphan designation (EU/3/04/254) was granted by the European Commission to Accelsiors CRO & Consultancy Services GmbH, Germany, for Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptide, sequence 169-177, of proteinase 3) for the treatment of myelodysplastic syndromes.

What are myelodysplastic syndromes?

Myelodysplastic syndromes are a distinct group of disorders in which the production of blood cells by the bone marrow is abnormal. The bone marrow is the spongy tissue found in the large bones. It has the function of making red cells (which are the main carriers of oxygen to body tissues), white blood cells (which fight infection), and platelets (which make the blood clot). In myelodysplastic syndromes their production is affected because these cells do not grow and mature normally. Consequently several symptoms can develop: fatigue or weakness (due to anaemia, the red cells deficit), infections (due to decrease in white blood cells) or easy bruising or abnormal bleeding (platelets deficit). Myelodysplastic syndromes are life threatening because they can result in severe anaemia, infections or haemorrhages and can progress to acute leukaemia.

What is the estimated number of patients the condition?

At the time of designation, myelodysplastic syndromes affected between 1 and 3 in 10,000 people in the European Union (EU)*. This is equivalent to a total of between 46,000 and 138,000 people, and is below the threshold for orphan designation, which is 5 people in 10,000. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP).

What treatments are available?

At the time of submission of the application for the orphan drug designation there was no treatment authorised in the European Union. Available therapeutic options for myelodysplastic syndromes included supportive care methods (e.g. antibiotics to treat infections, blood or platelet transfusions for anaemia or bleeding respectively), the use of products such as erythropoietin (a substance that stimulates the bone marrow to produce red cells), chemotherapy (using drugs that can kill the abnormal cells), and bone-marrow transplantation.

*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 25), Norway, Iceland and Liechtenstein. This represents a population of 459,700,000 (Eurostat 2004).

How is this medicine expected to work?

Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptide, sequence 169-177, of proteinase 3) is a peptide, which is a part of a protein. It is similar to a part of a protein, present on the surface of certain cells, such as cancer cells. It is expected to work as a vaccine, thus upon administration of this peptide, the body's defense system (immune system) will recognize this peptide as a foreign body and react against this peptide. Since part of this foreign body is similar to parts of the protein on the cancer cells, it is expected that the immune system will not only recognize the vaccine as foreign, but also the cancer cells. The body's immune system could then kill these cancer cells.

What is the stage of development of this medicine?

The effects of Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptide, sequence 169-177, of proteinase 3) were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with myelodysplastic syndromes were ongoing.

Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptide, sequence 169-177, of proteinase 3) was not marketed anywhere worldwide for the treatment of myelodysplastic syndromes or designated as orphan medicinal product elsewhere for this condition, at the time of submission.

According to Regulation (EC) No 141/2000 of 16 December 1999, the Committee for Orphan Medicinal Products (COMP) adopted on 11 November 2004 a positive opinion recommending the grant of the above-mentioned 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 Community) 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:

Accelsiors CRO & Consultancy Services GmbH

Hammersbacher Fussweg 24

82467 Garmisch-Partenkirchen

Germany

Telephone: +49 88 21 94 30 271

Telefax: +49 88 21 94 30 272

E-mail: accelsiors@t-online.de

Patients' associations contact points:

Associazione Italiana contro le Leucemie-linfomi e mieloma ONLUS

Via Ravenna 34

00161 Roma

Italy

Telephone: +39 06 44 03 763

Telefax: +39 06 44 04 226

E-mail: ail@ail.it

Leukaemia Care

2 Shrubbery Avenue

Worcester

WR1 1QH

United Kingdom

0800 169 6680 (freephone for UK residents)

Telephone: +44 19 05 33 00 03 / + 44 84 57 67 32 03

Telefax: +44 19 05 33 00 90

E-mail: enquiries@leukaemiaCARE.org.uk

DLH : Deutsche Leukämie- und Lymphom-Hilfe e.V.

Thomas-Mann-Straße 40

53111 Bonn

Germany

Telephone: +49 22 83 90 440

Telefax: +49 22 83 90 44 22

E-mail: info@leukaemie-hilfe.de

**Translations of the active ingredient and indication in all EU languages
and Norwegian and Icelandic**

Language	Active Ingredient	Indication
English	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptide, sequence 169-177, of proteinase 3)	Treatment of myelodysplastic syndromes
Czech	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptid, sekvence 169-177, proteinázy 3)	Léčba myelodysplastického syndromu
Danish	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptid, sekvens 169-177, af proteinase 3) eukæmiassocieret PR1-antigen	Behandling af myelodysplastiske syndromer
Dutch	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptide, sequentie 169-177, van proteinase 3)	Behandeling van myelodysplastische syndromen
Estonian	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Proteinaas 3 PR1 nanopeptiid, järjestusega 169-177)	Müelodüsplastilise sündroomi ravi
Finnish	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (proteinaasi 3 Pr1 nanopeptidi, 169-177 sekvenssi)	Myelodysplastisten syndroomien hoito
French	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (nanopeptide Pr1, séquence 169-177 de la protéinase 3)	Traitement des syndromes myélodysplasiques
German	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 Nanopeptid, Sequenz 169-177, der Proteinase 3)	Behandlung der myelodysplastischen Syndrome
Greek	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 νοναπεπτίδιο, αλληλουχίας 169-177 της πρωτεΐνάσης 3)	Θεραπεία των μυελοδυσπλαστικών συνδρόμων
Hungarian	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptid, proteináz3 169-177 szekvenciája)	Myelodysplasiás szindrómák kezelése
Italian	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptide, sequenza 169-177, di proteinasi 3)	Trattamento delle sindromi mielodisplastiche
Latvian	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (proteināzes 3 Pr1 nanopeptīds, virkne 169-177)	Mielodisplastisko sindromu ārstēšana
Lithuanian	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptidas, proteinazės 3 seka 169- 177)	Mielodisplazijos sindromo gydymas
Polish	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (nanopeptyd w sekwencji 169-177 proteinazy 3)	Leczenie zespołów mielodysplastycznych
Portuguese	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptido, sequência 169-177, da proteinase 3)	Tratamento dos síndromes mielodisplásticos

Slovak	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptid, sekvencia 169-177, proteinázy 3)	Liečba myelodysplastických syndrómov
Slovenian	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptid, sekvencia 169-177 proteinaze 3)	Zdravljenje mielodisplastičnih sindromov
Spanish	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (nanopéptido Pr1, secuencia 169-177, de la proteínasa 3)	Tratamiento del síndrome mielodisplásico
Swedish	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptid, sekvens 169-177, av proteinasa 3)	Behandling av myelodysplastiska syndrom
Norwegian	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptid, sekvens 169-177 av proteinase 3)	Behandling av myelodysplastiske syndromer
Icelandic	Val-Leu-Gln-Glu-Leu-Asn-Val-Thr-Val (Pr1 nanopeptíð, röð 169-177, af próteinasa 3)	Meðferð við mergmisþroskaheilkenni