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Public summary of opinion on orphan designation

Peptides YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL and PGCNKRYFKLSHLQMHSRKHTG for treatment of multiple myeloma

On 26 October 2018, orphan designation (EU/3/18/2078) was granted by the European Commission to Sellas Life Sciences Limited, Ireland, for peptides YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL and PGCNKRYFKLSHLQMHSRKHTG (also known as SLS-001) for treatment of multiple myeloma.

What is multiple myeloma?

Multiple myeloma (also called plasma cell myeloma) is a cancer of a type of white blood cell called plasma cells. Plasma cells are produced in the bone marrow, the spongy tissue inside the large bones in the body. In multiple myeloma, the division of plasma cells becomes uncontrolled, resulting in abnormal, immature plasma cells multiplying and filling up the bone marrow. This interferes with production of normal white blood cells, red blood cells and platelets (components that help the blood to clot), leading to complications such as anaemia (low red blood cell counts), bone pain and fractures, raised blood calcium levels and kidney disease.

Multiple myeloma is a debilitating and life-threatening disease particularly because it disrupts the normal functioning of the bone marrow, damages the bones and causes kidney failure.

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

At the time of designation, multiple myeloma affected 4.7 in 10,000 people in the European Union (EU). This was equivalent to a total of around 243,000 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, several medicines were authorised for multiple myeloma in the EU. The main treatment for multiple myeloma was chemotherapy (medicines to treat cancer) usually combined

*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 517,400,000 (Eurostat 2018).

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with corticosteroids to reduce the activity of the immune system, the body's natural defences. After chemotherapy patients received a stem-cell transplant if they were considered suitable for it. Stem-cell transplantation is a procedure where the patient's bone marrow is replaced with stem cells to form new bone marrow that produces healthy blood cells.

The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with multiple myeloma. Early studies in patients after a stem-cell transplant showed that the medicine in combination with existing treatments can help prolong the length of time patients lived without their disease getting worse. 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?

This medicine is expected to work by making the immune system (the body's natural defences) attack WT1, a protein that is present in high levels in multiple myeloma cells and helps them to grow.

The medicine contains 4 amino acid chains which are similar to parts of WT1. When injected into the patient, the immune system recognises them as 'foreign' and attack them, and because they are so similar to WT1 the immune system will also target WT1 in the cancer cells.

The medicine also contains a substance known as an adjuvant which helps to stimulate a strong immune response.

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, clinical trials with the medicine in patients with multiple myeloma were ongoing.

At the time of submission, the medicine was not authorised anywhere in the EU for multiple myeloma. Orphan designation of the medicine had been granted in the United States for this condition.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 13 September 2018 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	Peptides YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL and PGCNKRYFKLSHLQMHSRKHTG	Treatment of multiple myeloma
Bulgarian	Пептиди YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL, PGCNKRYFKLSHLQMHSRKHTG	Лечение на мултиплен миелом
Croatian	Peptidi YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL i PGCNKRYFKLSHLQMHSRKHTG	Liječenje multiplog mijeloma
Czech	Peptidy YMFPNAPYL, SGQAYMFPNAPYLPSCLES, PGCNKRYFKLSHLQMHSRKHTG	Léčba mnohočetného myelomu
Danish	Peptider YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL og PGCNKRYFKLSHLQMHSRKHTG	Behandling af multipelt myelom
Dutch	Peptiden YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL, PGCNKRYFKLSHLQMHSRKHTG	Behandeling van multipel myeloom
Estonian	YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL, PGCNKRYFKLSHLQMHSRKHTG peptiidid	Multiibelse müeloomi ravi
Finnish	Peptidit YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL, PGCNKRYFKLSHLQMHSRKHTG	Multippeli myelooman hoito
French	Peptides YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL, PGCNKRYFKLSHLQMHSRKHTG	Traitement du myélome multiple
German	Peptide YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL, PGCNKRYFKLSHLQMHSRKHTG	Behandlung des multiplen Myeloms
Greek	Πεπτίδιαν YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL, και PGCNKRYFKLSHLQMHSRKHTG	Θεραπεία πολλαπλού μυελώματος
Hungarian	YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL és PGCNKRYFKLSHLQMHSRKHTG peptidek	Myeloma multiplex kezelése
Italian	Peptidi YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL, PGCNKRYFKLSHLQMHSRKHTG	Trattamento del mieloma multiplo
Latvian	Peptīdi YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL, PGCNKRYFKLSHLQMHSRKHTG	Multiplās mielomas ārstēšana

[1] At the time of designation

Language	Active ingredient	Indication
Lithuanian	Peptidai YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL, PGCNKRYFKLSHLQMHSRKHTG	Dauginės mielomos gydymas
Maltese	Peptides YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL u PGCNKRYFKLSHLQMHSRKHTG	Kura tal-mjeloma multipla
Polish	Peptydy YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL, PGCNKRYFKLSHLQMHSRKHTG	Leczenie szpiczaka mnogiego
Portuguese	Peptidos YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL e PGCNKRYFKLSHLQMHSRKHTG	Tratamento do mieloma múltiplo
Romanian	Peptide YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL, PGCNKRYFKLSHLQMHSRKHTG	Tratamentul mielomului multiplu
Slovak	Peptidy YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL, PGCNKRYFKLSHLQMHSRKHTG	Liečba mnohopočetného myelómu
Slovenian	Peptidi YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL, PGCNKRYFKLSHLQMHSRKHTG	Zdravljenje multiplega mieloma
Spanish	Péptidos YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL, PGCNKRYFKLSHLQMHSRKHTG	Tratamiento del mieloma múltiple
Swedish	Peptider YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL, PGCNKRYFKLSHLQMHSRKHTG	Behandling av multipelt myelom
Norwegian	Peptider YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL, PGCNKRYFKLSHLQMHSRKHTG	Behandling av myelomatose
Icelandic	Peptíð YMFPNAPYL, SGQAYMFPNAPYLPSCLES, RSDELVRHHNMHQRNMTKL, PGCNKRYFKLSHLQMHSRKHTG	Meðferð við mergfrumuæxli