



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Acadesine for the treatment of multiple myeloma

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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 5 August 2011, orphan designation (EU/3/11/881) was granted by the European Commission to Advancell - Advanced In Vitro Cell Technologies S.A., Spain, for acadesine for the treatment of multiple myeloma.

What is multiple myeloma?

Multiple myeloma is a cancer of a type of white blood cell called plasma cells. Plasma cells are found in the bone marrow, the spongy tissue inside the large bones in the body. In multiple myeloma, the division of plasma cells becomes out of control, 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 that is associated with poor long-term survival.

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

At the time of designation, multiple myeloma affected approximately 1.6 in 10,000 people in the European Union (EU). This was equivalent to a total of around 81,000 people*, and is below the ceiling

*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 27), Norway, Iceland and Liechtenstein. At the time of designation, this represented a population of 507,700,000 (Eurostat 2011).



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 with steroids to reduce the activity of the immune system, the body's natural defences. Radiotherapy (treatment with radiation) was used to treat pain and weakened bones. Interferon alfa, a protein normally produced by the body during viral infections, was sometimes used in combination with chemotherapy. Stem cell transplantation (a complex procedure where the bone marrow of the patient is destroyed and replaced by stem cells that 're-colonise' the bone marrow) was used in some patients.

The sponsor has provided sufficient information to show that acadesine might be of significant benefit for patients with multiple myeloma because it works in a different way to existing treatments and early studies in experimental models show that it might improve the treatment of patients with this condition, when used in combination with existing treatments. 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?

Although the way that this medicine works in multiple myeloma is not fully understood, it is thought to interfere with the growth cycle of cancerous cells leading to 'apoptosis' (controlled cell death) of the cancerous cells

What is the stage of development of this medicine?

The effects of acadesine have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with acadesine in patients with multiple myeloma had been started.

At the time of submission, acadesine was not authorised anywhere in the EU for multiple myeloma or designated as an orphan medicinal product elsewhere for this condition. Orphan designation of acadesine had been granted in the EU for the treatment of B-cell chronic lymphocytic leukaemia.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 5 May 2011 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:

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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 languages¹, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Acadesine	Treatment of multiple myeloma
Bulgarian	Акадезин	Лечение на мултиплен миелом
Czech	Acadesine	Léčba mnohočetného myelomu
Danish	Acadesin	Behandling af multipelt myelom
Dutch	Acadesine	Behandeling van multipel myeloom
Estonian	Akadesiin	Multiibelse müeloomi ravi
Finnish	Akadesiini	Multippeli myelooman hoito
French	Acadésine	Traitement du myélome multiple
German	Acadesin	Behandlung des multiplen Myeloms
Greek	Ακαδεσίνη	Θεραπευτική αγωγή πολλαπλού μυελώματος
Hungarian	Acadesin	Myeloma multiplex kezelése
Italian	Acadesina	Trattamento del mieloma multiplo
Latvian	Akadezīns	Multiplās mielomas ārstēšana
Lithuanian	Akadezinas	Dauginės mielomos gydymas
Maltese	Acadesine	Kura tal-mjeloma multipla
Polish	Acadezyna	Leczenie szpiczaka mnogiego
Portuguese	Acadesina	Tratamento do mieloma múltiplo
Romanian	Acadesină	Tratamentul mielomului multiplu
Slovak	Acadesin	Liečba mnohopočetného myelómu
Slovenian	Akadezinc	Zdravljenje multiplega mieloma
Spanish	Acadesina	Tratamiento del mieloma múltiple
Swedish	Akadesin	Behandling av multipelt myelom
Norwegian	Akadesin	Behandling av myelomatose
Icelandic	Akadesín	Meðferð við mergfrumuæxli

¹ At the time of designation