



EUROPEAN MEDICINES AGENCY  
SCIENCE MEDICINES HEALTH

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## Public summary of opinion on orphan designation

### Asciminib for the treatment of chronic myeloid leukaemia

On 24 March 2020, orphan designation EU/3/20/2261 was granted by the European Commission to Novartis Europharm Limited, Ireland, for asciminib for the treatment of chronic myeloid leukaemia.

#### What is chronic myeloid leukaemia?

Chronic myeloid leukaemia (CML) is a cancer of the white blood cells (cells that fight against infections). In patients with CML, the bone marrow (the spongy tissue inside the large bones) produces large numbers of abnormal white blood cells, called blasts, that are not fully developed and do not work properly. Gradually these blast cells replace the normal blood cells in the bone marrow. The disease usually develops very slowly, which is why it is called 'chronic'. However, when it progresses, CML is a severe and life-threatening disease that is associated with poor overall survival. CML is most common in adults and older people, but children may also be affected.

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

At the time of designation, CML affected approximately 1.2 in 10,000 people in the European Union (EU). This is equivalent to a total of around 62,000 people\*, and is below the ceiling for orphan designation, which is 5 in 10,000 people. 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, authorised treatments included various types of cancer medicines, including tyrosine kinase inhibitors (medicines that block enzymes called tyrosine kinases) and immunotherapy (medicines that stimulate the body's immune system to target cancer cells). Patients might also receive stem cell transplantation (a complex procedure where the patient receives stem cells from a matched donor to restore the bone marrow and produce healthy blood cells).

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\*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, Iceland, Liechtenstein, Norway and the United Kingdom. This represents a population of 519,200,000 (Eurostat 2020).



The sponsor has provided sufficient information to show that the medicine might be of significant benefit for patients with CML because early results showed that asciminib had an effect on the disease in patients who did not respond to other available therapies.

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?**

Asciminib is a tyrosine kinase inhibitor. It blocks the action of BCR-ABL, a tyrosine kinase produced by CML cells that helps them to grow and reproduce uncontrollably. By blocking BCR-ABL in this way, the medicine is expected to control the growth of the leukaemia cells.

## **What is the stage of development of this medicine?**

The effects of asciminib have been evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials with asciminib in patients with chronic myeloid leukaemia were ongoing.

At the time of submission, asciminib was not authorised anywhere in the EU for the treatment of chronic myeloid leukaemia. Orphan designation of asciminib had been granted in the United States for chronic myelogenous leukaemia.

In accordance with Regulation (EC) No 141/2000, the COMP adopted a positive opinion on 20 February 2020, 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](#).

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<sup>1</sup>, Norwegian and Icelandic

Language	Active ingredient	Indication
English	Asciminib	Treatment of chronic myeloid leukaemia
Bulgarian	Асциминиб	Лечение на хронична миелоидна левкемия
Croatian	Asciminib	Liječenje bolesnika s kroničnom mijeloičnom leukemijom
Czech	Asciminib	Léčba chronické myeloidní leukémie
Danish	Asciminib	Behandling af kronisk myeloid leukæmi
Dutch	Asciminib	Behandeling van chronische myeloïde leukemie
Estonian	Astsiminiib	Kroonilise müeloidse leukeemia ravi
Finnish	Askiminibi	Kroonisen myeloosien leukemian hoito
French	Asciminib	Traitement de la leucémie myéloïde chronique
German	Asciminib	Behandlung der chronischen myeloischen Leukämie
Greek	Ασμινιβήνη	Θεραπεία της χρόνιας μυελοειδούς λευχαιμίας
Hungarian	Asciminib	Krónikus myeloid leukémia kezelése
Italian	Asciminib	Trattamento della leucemia mieloide cronica
Latvian	Asciminibs	Hroniskas mieloleikozes ārstēšana
Lithuanian	Asciminibas	Lėtinės mielocitinės leukemijos gydymas
Maltese	Askiminib	Kura tal-lewkimja mjelojda kronika
Polish	Ascyminib	Leczenie przewlekłej białaczki szpikowej
Portuguese	Asciminib	Tratamento da leucemia mielóide crónica
Romanian	Asciminib	Tratamentul leucemiei mieloide cronice
Slovak	Asciminib	Liečba chronickej myeloidnej leukémie
Slovenian	Askiminib	Zdravljenje kronične mieloične levkemije
Spanish	Asciminib	Tratamiento de la leucemia mieloide crónica
Swedish	Askiminib	Behandling av kronisk myeloid leukemi
Norwegian	Asciminib	Behandling av kronisk myelogen leukemi
Icelandic	Asciminib	Meðferð við langvinnu kyrningahvítblæði

<sup>1</sup> At the time of designation