



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Sildenafil citrate for the treatment of postcardiotomy right ventricular failure

On 26 November 2010, orphan designation (EU/3/10/815) was granted by the European Commission to Pfizer Limited, United Kingdom, for sildenafil citrate for the treatment of postcardiotomy right ventricular failure.

What is postcardiotomy right ventricular failure?

Right ventricular failure occurs when the right part of the heart stops working properly. The right part of the heart pumps blood that has come from all over the body towards the lungs. Patients with right ventricular failure may have hypoxaemia (poor blood oxygenation), low blood pressure, poor blood circulation and accumulation of fluid in parts of the body such as legs and feet which may become swollen. Postcardiotomy right ventricular failure is the term to describe this condition when it occurs in patients who have recently had heart surgery.

Postcardiotomy right ventricular failure is a life-threatening condition with a high mortality rate.

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

At the time of designation, postcardiotomy right ventricular failure affected less than 1 person in 10,000 people in the European Union (EU)*. This is equivalent to a total of fewer than 51,000 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 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 for the treatment of postcardiotomy right ventricular failure. Patients were treated with standard methods to improve blood circulation.

*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. This represents a population of 506,500,000 (Eurostat 2010).



How is this medicine expected to work?

Sildenafil is a medicine that has been used for a number of years to improve blood flow in patients with erectile dysfunction (impotence) and with pulmonary arterial hypertension (high blood pressure in the artery that leads from the heart to the lungs). Sildenafil works by blocking an enzyme called phosphodiesterase type 5. When this enzyme is blocked, a substance called 'cyclic guanine monophosphate' (cGMP) cannot be broken down, so that it remains in the vessels where it causes the relaxation and widening of the blood vessels. In postcardiotomy right ventricular failure, sildenafil is expected to work by widening the blood vessels of the lungs thus making the pumping action of the right side of the heart easier.

What is the stage of development of this medicine?

The effects of sildenafil citrate have been evaluated in experimental models.

At the time of submission of the application for orphan designation, no clinical trials with sildenafil in patients with postcardiotomy right ventricular failure had been started.

At the time of submission, sildenafil was not authorised anywhere in the EU for postcardiotomy right ventricular failure 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 9 September 2010 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 European Union) 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:

Pfizer Limited
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United Kingdom
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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	Sildenafil citrate	Treatment of postcardiotomy right ventricular failure
Bulgarian	Силденафил цитрат	Лечение на посткардиотомна деснокамерна недостатъчност
Czech	Sildenafil citras	Léčba pravostranného srdečního selhání po kardiometrii
Danish	Sildenafil citrat	Behandling af højresidigt hjertesvigt efter kardiometri
Dutch	Sildenafil citraat	Behandeling van post-cardiometrie rechterventrikelfalen
Estonian	Sildenafiltsitraat	Kardiotoomiajärgse parema vatsakese puudulikkuse ravi
Finnish	Sildenafilisitraatti	Kardiometriän jälkeisen oikean kammion vajaatoiminnan hoito
French	Citrate de sildénafil	Traitement de l'insuffisance cardiaque droite post-cardiometrie
German	Sildenafil citrat	Behandlung eines Rechtsherzversagens nach Kardiometrie
Greek	Κιτρική σιλντεναφίλη	Θεραπεία δεξιάς κοιλιακής ανεπάρκειας μετά από καρδιοτομή
Hungarian	Sildenafil-citrát	Kardiometriát követő jobbkamra-elégtelenség kezelése
Italian	Sildenafil citrato	Trattamento dell'insufficienza ventricolare destra post-cardiometria
Latvian	Sildenafil citrāts	Labā kambara mazspējas ārstēšanai pēc kardiometrijas
Lithuanian	Sildenafilio citratas	Dešiniojo skilvelio nepakankamumo po kardiometrijos gydymas
Maltese	Sildenafil citrate	Kura ta' insuffiċjenza tal-ventrikl u lemini tal-qalb wara kardjotomija
Polish	Cytrynian sildenafilu	Leczenie niewydolności prawokomorowej serca po kardiometrii
Portuguese	Citrato de sildenafil	Tratamento da insuficiência ventricular direita pós cardiometria
Romanian	Citrat de sildenafil	Tratamentul insuficienței ventriculare drepte postcardiometrie
Slovak	Sildenafilcitrát	Liečba zlyhania pravej komory srdca po kardiochirurgickom výkone
Slovenian	Sildenafilijev citrate	Zdravljenje popuščanja desnega srca po kardiometriji
Spanish	Citrato de sildenafilo	Tratamiento del fallo ventricular derecho postcardiometría
Swedish	Sildenafilcitrat	Behandling av högersidig hjärtsvikt efter kardiometri
Norwegian	Sildenafilsitrat	Behandling av høyre ventrikkelsvikt etter kardiometri
Icelandic	Síldenafl sítrat	Meðferð hægri hjartabilunar eftir opna hjartaaðgerð

¹ At the time of designation