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SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

## Public summary of opinion on orphan designation

### 6-thioguanine (oral liquid) for the treatment of acute lymphoblastic leukaemia

First publication	7 December 2009
Rev.1: sponsor's change of address	27 November 2013
<b>Disclaimer</b> 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 26 November 2009, orphan designation (EU/3/09/694) was granted by the European Commission to Only For Children Pharmaceuticals, France, for 6-thioguanine (oral liquid) for the treatment of acute lymphoblastic leukaemia.

#### What is acute lymphoblastic leukaemia?

Acute lymphoblastic leukaemia (ALL) is a cancer of the white blood cells called lymphocytes. In this disease, the lymphocytes multiply too quickly and live for too long, so there are too many of them circulating in the blood. These abnormal lymphocytes are not fully developed and do not work properly. Over a period of time, they replace the normal white cells and red blood cells and platelets in the bone marrow (the spongy tissue inside the large bones in the body).

ALL is the most common type of leukaemia in young children, but the disease also affects adults, especially those aged 65 years and older. Many people with ALL can be cured. However, despite the available treatments, ALL remains a serious and life-threatening disease in some patients.

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

At the time of designation, ALL affected less than 1 in 10,000 people in the European Union (EU). This was equivalent to a total of fewer than 50,000 people\*, and is below the ceiling for orphan designation,

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\*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 504,800,000 (Eurostat 2009).



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?**

Treatment for ALL is complex and depends on a number of factors including the extent of the disease, whether it has been treated before, and the patient's age, symptoms and general state of health. At the time of designation, the main treatment of ALL was chemotherapy (medicines to treat cancer) followed by or combined with radiotherapy (treatment with radiations). Bone marrow transplantation was also used. This is a complex procedure where the bone marrow of the patient is destroyed and replaced with healthy bone marrow from a matched donor.

The sponsor has provided sufficient information to show that 6-thioguanine (oral liquid) might be of significant benefit for patients with ALL because of the way the medicine is given. 6-Thioguanine is already available in the EU as tablets for the treatment of ALL. 6-Thioguanine (oral liquid) is a liquid form of 6-thioguanine, which could be given to children who have difficulty swallowing tablets. This may make it easier for children to take the medicine and allow the dose to be adjusted more precisely according to the patient's weight. 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?**

6-Thioguanine (oral liquid) is expected to work in the same way as 6-thioguanine tablets. 6-Thioguanine is an anticancer medicine that belongs to the group 'antimetabolites'. It works by interfering with the production of DNA within cells, preventing them from making more copies of their DNA. This means that cancer cells cannot divide and they eventually die.

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

At the time of submission of the application for orphan designation, the evaluation of the effects of 6-thioguanine (oral liquid) in experimental models was planned.

At the time of submission of the application for orphan designation, no clinical trials with the designated product in patients with ALL had been started.

At the time of submission, 6-thioguanine (oral liquid) was not authorised anywhere in the EU for ALL or designated as 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 7 October 2009 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:

Only For Children Pharmaceuticals  
Site Villemin, 10  
Avenue de Verdun  
75010 Paris  
France  
Tel. +33 1 57 27 86 86  
Fax +33 1 77 65 63 94  
E-mail: [contact@o4cp.com](mailto:contact@o4cp.com)

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	6-thioguanine (oral liquid)	Treatment of acute lymphoblastic leukaemia
Bulgarian	6-thioguanine (перорална течност)	Лечение на остра лимфобластна левкемия
Czech	6-thioguanine (perorální tekutina)	Léčba akutní lymfoblastické leukémie
Danish	6-thioguanin (oral væske)	Behandling af akut lymfoblastær leukæmi
Dutch	6-thioguanine (vloeistof voor oraal gebruik)	Behandeling van acute lymfoblastaire leukemie
Estonian	6-thioguanine (suukaudne vedelik)	Ägeda lümfoblastilise leukeemia ravi
Finnish	6-tioguanini (oraalineneste)	Akuutin lymfoblastileukemian hoito
French	6-thioguanine (liquide oral)	Traitement de la leucémie lymphoblastique aiguë
German	6-Thioguanin (Lösung zum Einnehmen)	Behandlung der akuten lymphatischen Leukämie
Greek	6-θειογουανίνη (στοματικό υγρό)	Θεραπεία της οξείας λεμφοβλαστικής λευχαιμίας
Hungarian	6-thioguanine (orális oldat)	Akut lymphoblastos leukaemia kezelése
Italian	6-tioguanina (liquido orale)	Trattamento della leucemia linfoblastica acuta
Latvian	6-tioguanīns (šķidrums iekšķīgai lietošanai)	Akūtas limfoblastiskas leikozes ārstēšana
Lithuanian	6-tioguaninas (geriamasis skystis)	Ūmios limfoblastinės leukemijos gydymas
Maltese	6-thioguanine (likwidu orali)	Kura tal-lewkimja limfoblastika akuta
Polish	6-tioguanina (płyn doustny)	Leczenie ostrej białaczki limfoblastycznej
Portuguese	6-thioguanine (líquido oral)	Tratamento da leucémia linfoblástica aguda
Romanian	6-tioguanină (formulare lichidă pentru administrare orală)	Tratamentul leucemiei limfoblastice acute
Slovak	6-tioguanín (perorálna kvapalina)	Liečba akútnej lymfoblastickej leukémie
Slovenian	6-thioguanine (peroralna tekočina)	Zdravljenje akutne limfoblastne levkemije
Spanish	6-tioguanina (líquido oral)	Tratamiento de la leucemia linfoblástica aguda
Swedish	6-thioguanine (oral vätska)	Behandling av akut lymfatisk leukemi
Norwegian	6-tioguanin (flytende, per os)	Behandling av akutt lymfoblastisk leukemi
Icelandic	6-thiógúanín (lausn til inntöku)	Meðferð við bráðu eitilfrumuhvítblæði

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