



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Abetimus sodium for the treatment of lupus nephritis

Please note that this product was withdrawn from the Community Register of designated orphan medicinal products in November 2009 on request of the sponsor.

On, 20 November 2001 orphan designation (EU/3/01/064) was granted by the European Commission to Icon Clinical Research UK Ltd., United Kingdom, for abetimus sodium for the treatment of lupus nephritis.

The sponsorship was transferred to La Jolla Limited, United Kingdom in September 2005.

What is lupus nephritis?

Lupus nephritis is a disease characterised by inflammation of the kidney caused by systemic lupus erythematosus (SLE), a disease of the immune system. SLE affects the skin, joints, kidneys and brain. The causes of SLE are unknown. Patients with SLE produce autoantibodies. Antibodies are proteins of the immune system that normally target bacteria and viruses. Autoantibodies mistakenly attack tissues. Lupus nephritis may cause weight gain, high blood pressure, dark urine, or swelling around the eyes, legs, ankles or fingers. This inflammation can damage the internal organs. Lupus nephritis is a chronically debilitating and life-threatening condition because of complications occurring during the disease process such as chronic renal failure and infections.

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

At the time of designation lupus nephritis affected approximately 0.6 to 1.8 in 10,000 people in the European Union (EU)*. This is based on the information provided by the sponsor and knowledge of the Committee for Orphan Medicinal Products (COMP). This is below the threshold for orphan designation which is 5 in 10,000. This is equivalent to a total of around 23,000 to 69,000 people.

*Disclaimer: For the purpose of the designation, the number of patients affected by the condition is estimated and assessed based on data from the European Union. This represents a population of 377,000,000 (Eurostat 2001).



What treatments are available?

At the time of submission of the application for the orphan drug designation there were glucocorticoids and other immunosuppressive agents (compounds that reduce the activity of the immune system) authorised for the condition in the Community.

Satisfactory argumentation has been submitted by the sponsor to justify the assumption that abetimus sodium might be of potential significant benefit for the treatment of lupus nephritis.

The assumption will have to be confirmed at the time of marketing authorisation. This will be necessary to maintain the orphan status.

How is this medicine expected to work?

Abetimus sodium is a chemically synthesised double-stranded oligodeoxyribonucleotide (oligonucleotides are the building blocks of the genetic material, DNA), which is targeted by the autoantibodies produced in patients with lupus nephritis. According to the sponsor, the medicinal product interferes with the function of B lymphocyte cells (type of immune cells) and it is expected to prevent the immune system from attacking and damaging patients' own tissues.

What is the stage of development of this medicine?

The effects of abetimus sodium were evaluated in experimental models.

At the time of submission of the application for orphan designation, clinical trials in patients with lupus nephritis were ongoing.

Abetimus sodium was not marketed anywhere worldwide for the treatment of lupus nephritis. Orphan designation of abetimus sodium was granted in the United States in 2000 for the treatment of lupus nephritis.

In accordance with Regulation (EC) No 141/2000 of 16 December 1999, the COMP adopted a positive opinion on 7 September 2001 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

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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	Abetimus sodium	Treatment of lupus nephritis
Czech	Abetimus sodný	Léčba nefritidy vzniklé na podkladě lupus erythematosus
Danish	Abetimusnatrium	Behandling af lupus nephritis
Dutch	Abetimusnatrium	Behandeling van lupusnephritis
Estonian	Abetimus-naatrium	Luupusnefriidi ravi
Finnish	Abetimuusinatrium	Lupusnefriitin hoito
French	Abetimus sodium	Traitement de la néphropathie lupique
German	Abetimus Natrium	Behandlung des Lupus nephritis
Greek	Abetimus sodium	Θεραπεία νεφριτίδας εξ ερυθματώδους λύκου
Hungarian	Abetimus nátrium	Lupus nephritis kezelése
Italian	Sodio abetimus	Trattamento della nefrite lupoide
Latvian	Abetimus nātrija sāls	Sistēmiskas sarkanās vilkēdes izraisīta nefrīta ārstēšana
Lithuanian	Natrio abetimas	Vilkligės sąlygoto nefrito gydymas
Polish	Abetymus sodowy	Leczenie toczniowego zapalenia nerek
Portuguese	Abetimus de sódio	Tratamento do nefrite por lúpus
Slovak	Abetimus sodný	Liečba lupusovej nefritídy
Slovenian	Abetimus natrij	Zdravljenje lupusnega nefritisa
Spanish	Sodio de Abetimus	Tratamiento de nefritis del lupus
Swedish	Abetimusnatrium	Behandling av lupusnephrit

¹ At the time of transfer of sponsorship