4 August 2010
EMA/COMP/327903/2010
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Molgramostim for the treatment of cystic fibrosis

On 5 November 2009, the Committee for Orphan Medicinal Products (COMP) adopted a negative opinion on the orphan designation application for molgramostim for the treatment of cystic fibrosis. A negative decision was issued by the European Commission on 16 July 2010.

The sponsor applied for orphan designation on the basis of the seriousness and the rarity of the condition, as well as an assumption of potential benefit over currently available methods of treatment.

The negative opinion was based on the following reason:

The sponsor had not provided sufficient evidence to justify the claim that molgramostim might be of significant benefit for patients with cystic fibrosis. The sponsor provided preclinical data from an animal model that was not relevant to the condition and anecdotal data from only one cystic fibrosis patient. These data were not considered sufficient to show that the product for which designation was applied might have a clinically relevant advantage over existing treatments or that the product might represent a major contribution to patient care. This justification was necessary because other satisfactory methods of treatment for cystic fibrosis had already been authorised in the European Union.

Requests for designation as an orphan medicinal product are made for investigational products. Absence of orphan designation does not preclude the development of this product, including its use in clinical trials. A marketing authorisation can still be obtained if quality, safety and efficacy are demonstrated.

7 Westferry Circus ● Canary Wharf ● London E14 4HB ● United Kingdom
Telephone +44 (0)20 7418 8400 **Facsimile** +44 (0)20 7523 7040
E-mail info@ema.europa.eu **Website** www.ema.europa.eu
An agency of the European Union

For more information:

Sponsor's contact details:

Drugrecure Aps
c/o Serendex ApS
Cobis Building
Ole Maaløes vej 3
2200 Copenhagen
Denmark
Telephone: +45 43 58 37 59
Telefax: +45 43 58 37 69
E-mail: fiala@serendex.com

Patient associations' contact points:

CF : Cystic Fibrosis Association of Ireland
24 Lower Rathmines Road
Dublin 6
Dublin
Ireland
Telephone: +353 1 49 62 433
Telefax: +353 1 49 62 201
E-mail: info@cfireland.ie

Cystic Fibrosis Europe
c/o Mukoviszidose eV
In den Dauen 6
Germany
Telephone: +49 228 98780 31
Telefax: +49 228 98780 77
E-mail: info@cf-europe.info

Vaincre la Mucoviscidose
181, rue de Tolbiac
75013 Paris
France
Telephone : +33 1 40 78 91 91
Telefax : +33 1 45 80 86 44
E-mail : info@vaincrelamuco.org