5 March 2015
EMA/COMP/736555/2014
Committee for Orphan Medicinal Products

Public summary of opinion on orphan designation

Sodium ascorbate and menadione sodium bisulfite for the treatment of autosomal dominant polycystic kidney disease

On 21 July 2014, the Committee for Orphan Medicinal Products (COMP) adopted a negative opinion on the orphan designation application for sodium ascorbate and menadione sodium bisulfite for the treatment of autosomal dominant polycystic kidney disease. A negative decision was issued by the European Commission on 11 December 2014.

The sponsor applied for orphan designation on the basis of the seriousness and the rarity of the condition.

The negative opinion was based on the following reasons:

- The sponsor calculated the prevalence of autosomal dominant polycystic kidney disease on the basis of a limited number of publications, and the methodology used to estimate the prevalence was not adequately justified.
- The COMP noted that the prevalence of the genotype for the disease is higher than 5 in 10,000 people in the European Union and that the sponsor could not prove that the disease itself does not have a prevalence higher than this figure. 5 in 10,000 people is the maximum allowable prevalence for orphan designation.

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.

30 Churchill Place ● Canary Wharf ● London E14 5EU ● United Kingdom
Telephone +44 (0)20 3660 6000 **Facsimile** +44 (0)20 3660 5555
Send a question via our website www.ema.europa.eu/contact

For more information:

Sponsor's contact details:

JJGConsultancy Ltd
Sherston House
High Street
Evercreech
Somerset BA4 6HZ
United Kingdom
Tel. +44 (0)174 9838 886
Fax +44 (0)174 9838 887
E-mail: jjgconsultancy@btconnect.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.