EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

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Public summary of opinion on orphan designation

Autologous skeletal myoblasts expanded ex vivo for the treatment of oculopharyngeal muscular dystrophy

On 7 December 2017, the Committee for Orphan Medicinal Products (COMP) adopted a negative opinion on the orphan designation application for autologous skeletal myoblasts expanded ex vivo for the treatment of oculopharyngeal muscular dystrophy. A negative decision was issued by the European Commission on 13 April 2018.

The sponsor applied for orphan designation on the basis of the seriousness and the rarity of the condition. The COMP confirmed that oculopharyngeal muscular dystrophy is a long-term debilitating condition because of its symptoms such as drooping eyelids and difficulty swallowing, and because it can spread to other parts of the body affecting mobility. The condition was estimated to affect approximately 0.1 in 10,000 people in the European Union (EU).

The negative opinion was based on the following reason:

- The COMP considered that the preliminary data in patients provided by the sponsor did not demonstrate that the medicine improves swallowing when used together with surgery (‘cricopharyngeal myotomy’) compared with surgery alone.

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.

For more information:

Sponsor's contact details:

Contact details of the current sponsor for this orphan designation can be found on EMA website, on the medicine's rare disease designations page.

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.