8 June 2016
EMA/COMP/260673/2016
Committee for Orphan Medicinal Products

# Recommendation for maintenance of orphan designation at the time of marketing authorisation

## Strimvelis (autologous CD34+ cells transfected with retroviral vector containing adenosine deaminase gene) for the treatment of severe combined immunodeficiency due to adenosine deaminase deficiency (ADA-SCID)

On 8 April 2016, the Committee for Orphan Medicinal Products (COMP) completed its review of the designation EU/3/05/313 for Strimvelis (autologous CD34+ cells transfected with retroviral vector containing adenosine deaminase gene) as an orphan medicinal product for the treatment of severe combined immunodeficiency due to adenosine deaminase deficiency (ADA-SCID). The COMP assessed whether, at the time of marketing authorisation, the medicinal product still met the criteria for orphan designation. The Committee looked at the seriousness and prevalence of the condition, and the existence of other methods of treatment. The COMP recommended that the orphan designation of the medicine be maintained[1].

### Life-threatening or long-term debilitating nature of the condition

The Committee for Medicinal Products for Human Use (CHMP) recommended the authorisation of Strimvelis for 'the treatment of patients with severe combined immunodeficiency due to adenosine deaminase deficiency (ADA-SCID), for whom no suitable human leukocyte antigen (HLA)-matched related stem cell donor is available'.

This falls within the scope of the product's designated orphan indication, which is treatment of severe combined immunodeficiency due to adenosine deaminase deficiency.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2005. ADA-SCID remains a condition that is long-term debilitating and life threatening, because it increases susceptibility to multiple infections and patients rarely survive without treatment beyond 1 or 2 years of age.

[1] The maintenance of the orphan designation at time of marketing authorisation would, except in specific situations, give an orphan medicinal product 10 years of market exclusivity in the EU. This means that in the 10 years after its authorisation similar products with the same therapeutic indication cannot be placed on the market.

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## Prevalence of the condition

The sponsor provided updated information on the prevalence of ADA-SCID based on data from the scientific literature.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of ADA-SCID remains below the ceiling for orphan designation, which is 5 people in 10,000. At the time of the review of the orphan designation, the prevalence was estimated to be 0.04 people in 10,000. This is equivalent to a total of around 2,000 people in the EU.

## Existence of other methods of treatment

The COMP noted that, at the time of the review of the orphan designation, no satisfactory treatments were authorised in the EU for patients with ADA-SCID.

## Conclusions

Based on the data submitted and the scientific discussion within the COMP, the COMP considered that Strimvelis still meets the criteria for designation as an orphan medicinal product and that it should remain in the Community Register of Orphan Medicinal Products.

Further information on the current regulatory status of Strimvelis can be found in the European public assessment report (EPAR) on the Agency's website ema.europa.eu/Find medicine/Human medicines/European Public Assessment Reports.