Recommendation for maintenance of orphan designation at the time of marketing authorisation
Spinraza (nusinersen) for the treatment of 5q spinal muscular atrophy

On 25 April 2017, the Committee for Orphan Medicinal Products (COMP) concluded its review of the designation EU/3/12/976 for Spinraza (nusinersen, previously known as antisense oligonucleotide targeted to the SMN2 gene) as an orphan medicinal product for the treatment of 5q spinal muscular atrophy. 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.

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

The Committee for Medicinal Products for Human Use (CHMP) recommended the authorisation of Spinraza for: 'treatment of 5q spinal muscular atrophy (SMA)'.

This falls within the scope of the product’s designated orphan indication, which is: '5q spinal muscular atrophy'.

The COMP concluded that there had been no change in the seriousness of the condition since the orphan designation in 2012. 5q spinal muscular atrophy remains a condition that is long-term debilitating and life threatening because it causes muscle wasting, breathing problems and paralysis that worsen over time.

Prevalence of the condition

The sponsor provided an updated calculation of the prevalence of 5q spinal muscular atrophy based on available epidemiological studies.

On the basis of the information provided by the sponsor and the knowledge of the COMP, the COMP concluded that the prevalence of 5q spinal muscular atrophy 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 still estimated to be less than 0.4 people in 10,000. This is equivalent to a total of fewer than 21,000 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 treatments were authorised in the EU for patients affected by this condition.

Conclusions

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

More information on the COMP assessment can be found in the May 2017 COMP minutes.

Further information on Spinraza 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.