



EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

21 April 2017
EMA/181654/2017
Committee for Medicinal Products for Human Use (CHMP)

Summary of opinion¹ (initial authorisation)

Spinraza

nusinersen

On 21 April 2017, the Committee for Medicinal Products for Human Use (CHMP) adopted a positive opinion, recommending the granting of a marketing authorisation for the medicinal product Spinraza, intended for the treatment of 5q spinal muscular atrophy (SMA). Spinraza, which was designated as an orphan medicinal product on 2 April 2012, was reviewed under EMA's accelerated assessment programme. The applicant for this medicinal product is Biogen Idec Ltd.

Spinraza will be available as a 2.4 mg/ml solution for injection. The active substance of Spinraza is nusinersen, an antisense oligonucleotide (ATC code: N07) that can make the *SMN2* gene produce adequate levels of full-length SMN protein, thus improving neuronal survival.

The benefits with Spinraza are its ability to allow achievement of motor milestones and improvement in muscle function which is not observed during the natural course of the disease. The most common side effects are related to its administration via lumbar puncture.

The full indication is: "Spinraza is indicated for the treatment of 5q spinal muscular atrophy." It is proposed that Spinraza should only be started by a physician with experience in the management of spinal muscular atrophy.

Detailed recommendations for the use of this product will be described in the summary of product characteristics (SmPC), which will be published in the European public assessment report (EPAR) and made available in all official European Union languages after the marketing authorisation has been granted by the European Commission.

¹ Summaries of positive opinion are published without prejudice to the Commission decision, which will normally be issued 67 days from adoption of the opinion

