INAHTA Brief

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Title SPINRAZA – A Health Technology Assessment

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Reference link to full report in French
https://www.has-sante.fr/jcms/pprd_2983397/fr/spinraza

Aim

Assessment of SPINRAZA (nusinersen) with a view to funding by the French national health insurance system and of its clinical contribution compared to other strategies in the indication treatment of 5q spinal muscular atrophy in infants and children in the pre-symptomatic stage.

Conclusions of Transparency Committee

Clinical Benefit

- 5Q spinal muscular atrophy is a serious, life-threatening disease in types I and II. Most patients with 2 to 3 copies of the SMN2 gene progress to type I or II.
- This is a curative treatment.
- The efficacy/adverse effects ratio of SPINRAZA (nusinersen) is high.
- There is only one alternative medicinal product available via a CUP: ZOLGENSMA (onasemnogene abeparvovec) reserved for patients with biallelic mutation of the SMN1 gene and up to three copies of the SMN2 gene. Nevertheless, the Transparency Committee emphasises that it has not yet assessed it. For the other patients with 5q spinal muscular atrophy in the pre-symptomatic stage and not eligible for the ZOLGENSMA CUP, there is no alternative.
- SPINRAZA is a first-line treatment in pre-symptomatic infants and children with genetically-confirmed spinal muscular atrophy with 2 to 3 copies of the SMN2 gene.
- SPINRAZA (nusinersen) is likely to have an additional impact on public health.

Considering all of the above, the Committee considers that the clinical benefit of SPINRAZA (nusinersen) is substantial in pre-symptomatic infants and children with genetically-confirmed 5q spinal muscular atrophy with 2 or 3 copies of the SMN2 gene.

Clinical Added Value
Considering:
-data suggesting a change in the natural course of the disease from a phase 2, non-comparative study in pre-symptomatic children with 2 to 3 copies of the SMN2 gene, especially in terms:

- ventilation-free survival (primary endpoint) for 21 of the 25 patients,
- survival (for all 25 patients at a median age of 26 months),
- acquisition of motor functions such as the ability to sit without support for all of these children;

-hindsight at a median age of 34.8 months showing a more positive outcome in these children than without treatment, but without curing the disease,
-the unmet or partially met medical need (via the ZOLGENSMA (onasemnogene abeparvovec) cohort CUP for patients with biallelic mutation of the SMN1 gene and up to three copies of the SMN22 gene) in the treatment of 5q spinal muscular atrophy in pre-symptomatic infants and children,
and despite:
-the lack of robust analysis with pairing in terms of children from the same siblings, which cannot be used to accurately estimate the effect size,
the Committee considers that SPINRAZA (nusinersen) provides moderate Clinical Added Value (CAV III) in pre-symptomatic infants and children with genetically-confirmed 5q spinal muscular atrophy and with 2 to 3 copies of the SMN2 gene.

Recommendations

The Transparency Committee issued its approval for the funding of SPINRAZA (nusinersen) by the French national health insurance system (hospital only) in the indication treatment of 5q spinal muscular atrophy in pre-symptomatic infants and children.

Methods

The assessment of SPINRAZA (nusinersen) was founded on evidence-based medicine with a critical analysis of the clinical data.

Written by

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