Spinraza

(nusinersen)

New Product
Slideshow

MPR

Introduction

- **Brand name:** Spinraza
- **Generic name:** Nusinersen
- **Pharmacological class:** Antisense oligonucleotide
- **Strength and Formulation:** 12mg/5mL; solution for intrathecal injection; preservative-free
- **Manufacturer:** Biogen
- **How supplied:** Single-dose vial—1
- **Legal Classification:** Rx

SPINRAZA


Spinraza
(nusinersen)
Injection
12 mg /5 mL
(2.4 mg/mL)
NDC 64406-058-01
Spinraza
(nusinersen)
Injection
12 mg /5 mL
(2.4 mg/mL)
Sterile solution for
Intrathecal Use Only
Rx Only
1 Single Dose Vial
Discard Unused Portion

Indications

- Spinal muscular atrophy

Dosage & Administration

- Consider sedation and ultrasound or other imaging techniques as guidance
- Remove 5mL of cerebrospinal fluid prior to inj
- Give as intrathecal bolus inj over 1–3 mins
- 12mg (5mL) per dose
- **Initially:** give 3 loading doses at 14-day intervals then give 4th loading dose 30 days after
- **Maintenance:** give dose once every 4 months thereafter

Considerations for Special Populations

- **Pregnancy:** No human data to inform drug-associated risks
- **Nursing mothers:** Consider benefits and adverse effects
- **Pediatric:** Newborn to 17yrs: established

Warnings/Precautions

- **Do not administer** in areas of infected or inflamed skin
- Increased risk of **bleeding complications**
- **Perform** platelet count, prothrombin time, aPTT, quantitative spot urine protein testing at baseline, prior to each dose, and as clinically needed; consider repeat testing if urine protein >0.2g/L

Adverse Reactions

- Lower/upper respiratory infection
- Constipation
- Teething
- Thrombocytopenia
- Coagulation abnormalities
- Renal toxicity

Mechanism of Action

- Spinraza is a survival motor neuron-2 (SMN2)-directed antisense oligonucleotide
- It binds to a specific sequence in the intron downstream of exon 7 of the *SMN2* transcript
- Spinraza was shown to increase exon 7 inclusion in *SMN2* mRNA transcripts and production of full-length SMN protein

Clinical Trials

- Spinraza was evaluated in a multicenter, double-blind, sham-procedure controlled study (n=121) in infants ≤7 months of age diagnosed with SMA
- Patients were randomized to receive either Spinraza or sham injection
- An interim efficacy analysis was conducted based on patients who died, withdrew or completed ≥183 days of treatment

Clinical Trials

- The **primary endpoint** assessed at the time of interim analysis was the proportion of responders defined as patients with an improvement in motor milestones according to Section 2 of the Hammersmith Infant Neurologic Exam (HINE)

Clinical Trials

- A **statistically significant greater percentage** of patients achieved a motor milestone response in the Spinraza group (40%) vs. the sham-control group (0%)

- For more clinical trial data, see full labeling

New Product Monograph

- For more information view the product monograph available at:

  http://www.empr.com/spinraza/drug/34633/