

(PAS) is utilised or where the list/contract price is equivalent or lower than the PAS price.

- **MEDICINAL FORMS** There can be variation in the licensing of different medicines containing the same drug.

Granules

- ▶ **Translarna** (PTC Therapeutics Ltd) ▼

Ataluren 125 mg Translarna 125mg granules for oral suspension sachets | 30 sachet [PoM] £2,532.00

Ataluren 250 mg Translarna 250mg granules for oral suspension sachets | 30 sachet [PoM] £5,064.00

Ataluren 1 gram Translarna 1,000mg granules for oral suspension sachets | 30 sachet [PoM] £20,256.00

Nusinersen

31-Aug-2018

- **DRUG ACTION** Nusinersen is an antisense oligonucleotide that increases the production of survival motor neurone (SMN) protein, thereby helping to compensate for the defect in the SMN1 gene found in 5q spinal muscular atrophy.

● INDICATIONS AND DOSE

5q spinal muscular atrophy (initiated by a specialist)

- ▶ BY INTRATHECAL INJECTION

▶ **Neonate:** Initially 12 mg for 4 doses on days 0, 14, 28 and 63, then 12 mg every 4 months, for advice on missed doses—consult product literature.

▶ **Child:** Initially 12 mg for 4 doses on days 0, 14, 28 and 63, then 12 mg every 4 months, for advice on missed doses—consult product literature

- **UNLICENSED USE** Licensed for use in children (age range not specified by manufacturer).

IMPORTANT SAFETY INFORMATION

MHRA/CHM ADVICE: NUSINERSEN (*SPINRAZA*®): REPORTS OF COMMUNICATING HYDROCEPHALUS NOT RELATED TO MENINGITIS OR BLEEDING (JULY 2018)

Communicating hydrocephalus not related to meningitis or bleeding has been reported in patients treated with *Spinraza*®. Patients and caregivers should be informed about the signs and symptoms of hydrocephalus before *Spinraza*® is started and should be instructed to seek medical attention in case of: persistent vomiting or headache, unexplained decrease in consciousness, and in children increase in head circumference. Patients with signs and symptoms suggestive of hydrocephalus should be further investigated by a physician with expertise in its management.

- **CAUTIONS** Risk factors for renal toxicity—monitor urine protein (preferably using a first morning urine specimen) · risk factors for thrombocytopenia and coagulation disorders—monitor platelet and coagulation profile before treatment
- **PREGNANCY** Manufacturer advises avoid—no information available.
- **BREAST FEEDING** Manufacturer advises avoid—no information available.
- **RENAL IMPAIRMENT** Manufacturer advises close monitoring—safety and efficacy not established.
- **HANDLING AND STORAGE** Manufacturer advises store in a refrigerator (2–8 °C); may be stored (in the original carton, protected from light) at or below 30 °C, for up to 14 days.
- **NATIONAL FUNDING/ACCESS DECISIONS**
- **NICE decisions**
- ▶ **Nusinersen** for treating spinal muscular atrophy (July 2019) NICE TA588
Nusinersen (*Spinraza*®) is recommended as an option for treating 5q spinal muscular atrophy (SMA) only if:

- patients have pre-symptomatic SMA, or SMA types 1, 2 or 3, **and**
- the conditions in the managed access agreement are followed.

www.nice.org.uk/guidance/ta588

Scottish Medicines Consortium (SMC) decisions

SMC No. 1318/18

The *Scottish Medicines Consortium* has advised (May 2018) that nusinersen (*Spinraza*®) is accepted for restricted use within NHS Scotland for the treatment of 5q spinal muscular atrophy (SMA) in patients with symptomatic type 1 SMA (infantile onset). This advice is contingent upon the continuing availability of the patient access scheme in NHS Scotland or a list price that is equivalent or lower.

- **MEDICINAL FORMS** There can be variation in the licensing of different medicines containing the same drug.

Solution for injection

- ▶ **Spinraza** (Biogen Idec Ltd) ▼

Nusinersen (as Nusinersen sodium) 2.4 mg per 1 ml Spinraza 12mg/5ml solution for injection vials | 1 vial [PoM] £75,000.00

2.2 Myasthenia gravis and Lambert-Eaton myasthenic syndrome

ANTICHOLINESTERASES

Anticholinesterases



- **DRUG ACTION** They prolong the action of acetylcholine by inhibiting the action of the enzyme acetylcholinesterase.
- **CONTRA-INDICATIONS** Intestinal obstruction · urinary obstruction
- **CAUTIONS** Arrhythmias · asthma (extreme caution) · atropine or other antidote to muscarinic effects may be necessary (particularly when neostigmine is given by injection) but not given routinely because it may mask signs of overdosage · bradycardia · epilepsy · hyperthyroidism · hypotension · parkinsonism · peptic ulceration · recent myocardial infarction · vagotonia
- **SIDE-EFFECTS** Abdominal cramps · diarrhoea · excessive tearing · hypersalivation · nausea · vomiting
- **Overdose** Signs of overdose include bronchoconstriction, increased bronchial secretions, lacrimation, excessive sweating, involuntary defaecation, involuntary micturition, miosis, nystagmus, bradycardia, heart block, arrhythmias, hypotension, agitation, excessive dreaming, and weakness eventually leading to fasciculation and paralysis.
- **PREGNANCY** Manufacturer advises use only if potential benefit outweighs risk.
- **BREAST FEEDING** Amount probably too small to be harmful.

▶ above

Neostigmine

(Neostigmine methylsulfate)

● INDICATIONS AND DOSE

Treatment of myasthenia gravis

- ▶ BY MOUTH

▶ **Neonate:** Initially 1–2 mg, then 1–5 mg every 4 hours, given 30 minutes before feeds.