

## **SMA pathology: current treatments**

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Several companies have developed therapeutic approaches against spinal muscular atrophy. The following compounds are currently applied or are considered as potential treatments:

- A. SPINRAZA (Nusinersen): antisense oligonucleotide
- B. Zolgensma: gene therapy (scAAV9-cba-SMN)
- C. Risdiplam (RO7034067): small molecule

Based on the mechanism of action of each treatment, address the following questions:

Which treatment do you think is more appropriate for SMA type I ? (by order of priority)

- A. Spinraza (ASO)
- B. Risdiplam (small molecule)
- C. Zolgensma (gene therapy)

**Answer**

- 1 Zolgensma (gene therapy)
- 2 Risdiplam (small molecule)
- 2 Spinraza (ASO)

Which treatment do you think is more appropriate for SMA type II and III ? (by order of priority)

- A. Spinraza (ASO)
- B. Risdiplam (small molecule)
- C. Zolgensma (gene therapy)

**Answer**

- 1 Risdiplam (small molecule)
- 1 Spinraza (ASO)
- 2 Zolgensma (gene therapy)

For which treatment is the risk of side effects the most important ? (by order of priority)

- A. Spinraza (ASO)
- B. Risdiplam (small molecule)
- C. Zolgensma (gene therapy)

**Answer**

- 1 Zolgensma (gene therapy)
- 1 Risdiplam (small molecule)
- 2 Spinraza (ASO)

Which treatment is the most effective in SMA type I ?

- A. Spinraza (ASO)
- B. Risdiplam (small molecule)
- C. Zolgensma (gene therapy)

**Answer**

Zolgensma (gene therapy)

Which treatment is the most accessible to patients ?

- A. Spinraza (ASO)
- B. Risdiplam (small molecule)
- C. Zolgensma (gene therapy)

**Answer**

Risdiplam (small molecule)