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The Bulletin from the Clinical Pharmacist

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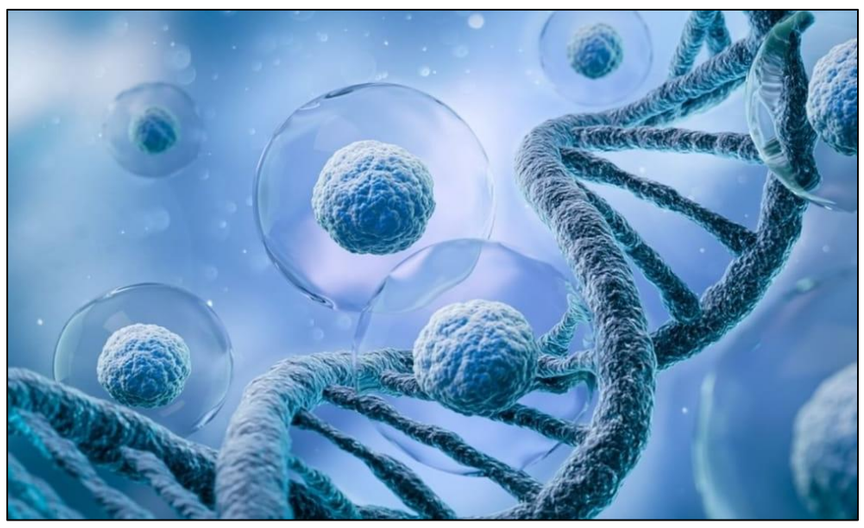

"One Dose, One Gene, One Cure? Itvisma as the World's most expensive drug"

Brand name: Itvisma

Generic name: Onasemnogene abeparvovec-*brve*

Category: Gene replacement therapy

Indication: Spinal muscular atrophy (SMA) in children aged 2 years and above

SMA is an autosomal-recessive neurodegenerative disorder caused by mutations in the *SMN*1 gene, characterized by irreversible and progressive motor neuron loss, leading to progressive muscle atrophy and weakness, and subsequent paralysis and death in the most severe cases.

Advantages:

- It is administered as a single dose intrathecally.
- The fixed dose does not require adjustment based on age or body weight.
- It has a rapid onset of action and directly targets the genetic root cause of SMA.
- By replacing the *SMN1* gene, Itvisma can improve motor function, and reduce the need for chronic ongoing treatment.

Adverse effects: Upper respiratory tract infection, gastrointestinal symptoms, pyrexia and headache.

Warnings & Precautions: Hepatotoxicity, thrombocytopenia, ganglionopathy, peripheral sensory neuropathy, thrombotic microangiopathy, elevated cardiac troponin I and tumourogenicity.

Available strength: Single dose vial containing $1.2x10^{14}$ vg / 3ml suspension.

Cost: $2.59 million

Note: Prophylactic immune suppression with prednisone, tacrolimus, sirolimus or rapamycin is imperative to sustain gene expression without immune reactions.

References:

1. https://www.rheumatologyadvisor.com/news/one-time-gene-therapy-itvisma-approved-for-spinal-muscular-atrophy/
2. https://checkrare.com/fda-approves-intrathecal-gene-therapy-for-patients-with-spinal-muscular-atrophy/
3. Kagiava A and Kleopa KA. Facing the challenge of effective dosing, safety, and timing of intrathecal gene therapy for neurological disorders. eBioMedicine 2026;123: 106045.