

The table below provides an overview of the three medicines available in Australia for people with SMA that can help slow or stop the progression of SMA. While there is no cure for SMA, treatment focuses on managing symptoms and improving quality of life

**SMA Australia does not recommend any specific treatment for people living with SMA.** Your healthcare team will provide detailed information about available treatments and develop a personalised management plan tailored to you or your child's needs.

TREATMENT	Evrysdi® (risdiplam)	Spinraza® (nusinersen)	Zolgensma® (onasemnogene abeparvovec)
<b>Type of treatment</b>	mRNA splicing modifier	Antisense oligonucleotide	Single stranded <i>SMN1</i> DNA packaged in a viral vector
<b>How it works</b>	Targets the body's working <i>SMN2</i> gene, causing it to produce more SMN protein	Targets the body's working <i>SMN2</i> gene, causing it to produce more SMN protein	Replaces the function of the body's <i>SMN1</i> gene, which is missing or faulty
<b>Method of treatment delivery</b>	Available as a liquid or a tablet, taken by mouth	Injection into spinal fluid (lumbar puncture)	Injection into vein (intravenous infusion)
<b>Location of treatment</b>	At home	Hospital setting, managed by a doctor experienced in giving lumbar punctures	Hospital setting, managed by a doctor or nurse experienced in treating SMA
<b>Frequency of treatment</b>	Daily, after a meal, at the same time each day. Can be taken with or without food.	4 doses in the first 2 months, then 1 dose every 4 months thereafter	One time only
<b>Treatment storage</b>	Store the liquid in the fridge. The tablets should be stored below 30°C, in the original packaging.	N/A	N/A
<b>TGA approval date</b>	Liquid: June 2021 Tablet: October 2025	November 2017	February 2021
<b>Initial PBS listing date</b>	Liquid: Children – 1 August 2021; Adults – 1 October 2023 Tablet: 1 April 2026	Children – 1 June 2018 Adults – 1 August 2022	Children – 1 May 2022

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<b>Available on PBS?</b>	Yes, for people with SMA symptoms and a diagnosis of SMA types 1–3, or presymptomatic people with 1, 2 or 3 copies of the <i>SMN2</i> gene. The tablet is available for patients aged 2 years or older weighing 20 kg or more, who have been prescribed the 5mg dose.	Yes, for people with SMA symptoms and a diagnosis of SMA types 1–3, or pre-symptomatic people with 1, 2 or 3 copies of the <i>SMN2</i> gene	Yes, for children under 9 months of age with SMA symptoms and a diagnosis of SMA Type 1, or pre-symptomatic children with 1, 2 or 3 copies of the <i>SMN2</i> gene