Australia's first gene therapy for babies born with spinal muscular atrophy (SMA), Zolgensma®, becomes available on the PBS

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- Zolgensma® becomes the first gene therapy in Australia for children under the age of 9 months with spinal muscular atrophy (SMA) to be listed on the Pharmaceutical Benefits Scheme (PBS)[1]
- 1 in 10,000 babies are born in Australia with SMA – the leading cause of death for babies born with genetic conditions[2]
- Zolgensma is a potentially lifesaving therapy, delivered in one single dose, with the potential to transform the lives of young babies[1]

Sydney – May 1, 2022 – The Honourable Greg Hunt, Minister for Health, has today made a landmark announcement in Australia, with Zolgensma (onasemnogene abeparvovec) becoming the first gene therapy to be listed on the Pharmaceutical Benefits Scheme (PBS), for children under the age of 9 months born with pre-symptomatic and Type 1 spinal muscular atrophy (SMA).[1]

Gene therapies like Zolgensma represent a breakthrough in medicine, with the aim of addressing the root cause of some genetic conditions with one treatment.[1] By doing so, they may stop a disease in its tracks. This contrasts with more conventional medicines that may need to be taken continually for life.

SMA is the leading cause of death for babies born with genetic conditions. Babies with SMA are born with a missing or faulty SMN1 gene, resulting in muscle weakness and progressive loss of movement.[2] SMA prevents babies reaching important developmental milestones such as sitting and walking.[2] 1 in 10,000 babies born in Australia are diagnosed with SMA.[2]

"The announcement today is a monumental step forward for the SMA community – particularly for babies and their families – who will now be able to access a revolutionary therapy for the cost of just one script," said Julie Cini, CEO of SMA Australia. *"We know that the earlier we can detect and treat SMA – the better outcomes are for families, so parents across Australia will be extremely pleased to have another option available for young babies born with this very challenging disease."*[3]

Zolgensma is delivered in one single infusion, which addresses the root cause of the disease by replacing the missing or faulty gene.[1] Gene therapies like Zolgensma seek to reduce the burden of treatment for patients and their families by replacing recurring, lifetime therapies with one single treatment.

Dr Ian Woodcock: *"Ultimately these babies are missing a single gene, which is causing their disease. With Zolgensma, we have the ability to treat the root cause of the disease and give that gene back to them. And not only are we giving them back a gene, but we're giving them the chance to go to school, learn and live out their full potential. For clinicians like myself, there is no greater motivation or pleasure than to be able to potentially give a child its life back."*

Without treatment, children with SMA, their families and carers face a difficult battle for survival; at constant

risk of respiratory infection and pneumonia alongside issues with feeding, constipation, airway maintenance and ensuring healthy bodily function, which presents real emotional, physical and financial challenges for parents.[3]

"We sincerely thank Minister Hunt, the Pharmaceutical Benefits Advisory Committee and the Australian Government for listing this important therapy. Novartis is a global leader in the field of gene therapy, having made a considerable investment in gene therapy research and development. With Zolgensma now available in Australia, we can already see gene therapy transforming the lives of patients and families," said Richard Tew, General Manager and Country President, Novartis Australia and New Zealand.

"Our leadership in innovative medicines is demonstrated by today's announcement, but the hard work continues for Novartis – we are committed to advocating for the SMA community and support their calls for nationwide equitable access to newborn screening for SMA – to ensure that no baby is left behind."

Approved Zolgensma Consumer Medical Information

See approved Consumer Medical Information. Approved Consumer Information available on request. For the most up to date Consumer Information go to link: Consumer Information | Novartis Australia

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Issued on behalf of Novartis Pharmaceuticals Australia Pty Limited by Cube

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References

1. Zolgensma Product Information. Available from: https://www.ebs.tga.gov.au/ebs/picmi/picmirepository.nsf/pdf?OpenAgent&id=CP-2021-PI-01276-1&d=202105041016933. Accessed May 2021.
2. Spinal Muscular Atrophy Australia. SMA Information Guide. Available online: https://smaaustralia.org.au/wp-content/uploads/SMA_2020_A4_Information_Guide-compressed.pdf. Accessed Feb 2022.
3. Farrar MA, Carey KA, Paguinto S, et al. Financial, opportunity and psychosocial costs of spinal muscular atrophy: an exploratory qualitative analysis of Australian carer perspectives. BMJ Open 2018;8:e020907.
4. Mendell et al 2021, Five-Year Extension Results of the Phase I START Trial of Onasemnogene Abeparvovec in Spinal Muscular Atrophy

About Zolgensma®

ZOLGENSMA (onasemnogene abeparvovec) is indicated for the treatment of paediatric patients less than 9 months of age with symptomatic or pre-symptomatic spinal muscular atrophy with bi-allelic mutations in the survival motor neuron 1 (SMN1) gene and 1 to 3 copies of the SMN2 gene.

About Novartis

Novartis is reimagining medicine to improve and extend people's lives. As a leading global medicines company, we use innovative science and digital technologies to create transformative treatments in areas of great medical need. In our quest to find new medicines, we consistently rank among the world's top companies investing in research and development. We believe continued R&D is essential to innovation and in Australia last year we invested 9.4M in clinical trials.

Novartis products reach nearly 1 billion people globally and we are finding innovative ways to expand access to our latest treatments. About 125,000 people of more than 140 nationalities work at Novartis around the world. Novartis has more than 60 years' history in Australia and employ around 600 people across its two divisions. For more information, please visit https://www.novartis.com/au-en/.

PBS Information: Section 100 Public and Private Hospital Authority Required for the presymptomatic treatment of SMA patients with 1 to 2 copies of the SMN2 gene and for treatment of patients with Type 1 SMA under 9 months of age. Refer to PBS Schedule for full authority information. This product is not listed for the presymptomatic treatment of SMA patients with 3 copies of the SMN2 gene.

WARNING: HEPATOTOXICITY

- Acute serious liver injury, acute liver failure, and elevated aminotransferases can occur with ZOLGENSMA.
- Patients with pre-existing hepatic impairment may be at higher risk.
- Prior to infusion, assess liver function of all patients by clinical examination and laboratory testing (e.g., hepatic aminotransferases [aspartate aminotransferase (AST) and alanine aminotransferase (ALT)], total bilirubin, and prothrombin time). Administer systemic corticosteroid to all patients before and after ZOLGENSMA infusion. Continue to monitor liver function for at least 3 months after infusion.

For healthcare professionals only.

Please review full Product Information before prescribing, Scan QR code for full Zolgensma product information.

Alternatively, please contact med info at 1 800 671 203 or visit https://www.novartis.com/au-en/about/product-list to access the full product information.

For medical enquiries please contact 1800 671 203 (phone) or medinfo.phauno@novartis.com (email) Novartis Pharmaceuticals Pty Limited. 54 Waterloo Road, Macquarie Park NSW 2113. Ph (02) 9805 3555. ® Registered Trademark.

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List of links present in page

- https://www.novartis.com/au-en/au-en/news/media-releases/australias-first-gene-therapy-babies-born-spinal-muscular-atrophy-sma-zolgensma-becomes-available-pbs
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