

Summary of Patient Organisations supported by Medicines Australia Member Companies

Company: *PTC Therapeutics Australia Pty Ltd*
For the period: *1 Jan 2025 - 31 Dec 2025*
Total support: *\$68,000*

Name of Patient Organisation	Description of and/or purpose of support	Nature of support (monetary value or equivalent) or description of non-financial support
Rare Voices Australia (RVA)	RVA 2025 Rare Disease Day Parliamentary Event 11 February 2025 12pm-1.30pm. Parliamentary Events provide key stakeholders with the opportunity to meet and speak with parliamentarians to further highlight rare diseases. Enabling parliamentarians to gain a better understanding and awareness about rare diseases is even more important as 2025 is a Federal Election year.	\$10,000
Rare Voices Australia (RVA)	RVA 2025 Round Table of Companies Membership. RTC membership is an opportunity for companies to support RVA’s work as the national peak body for Australians living with a rare disease. This work includes rare disease policy advocacy, including HTA policy; leadership of the collaborative implementation of the Australian Government’s National Strategic Action Plan for Rare Diseases; building key relationships with other peak bodies and participation in advisory panels, including research steering committees; white paper development; and consumer co-design activities. This work enables RVA to build the profile and recognition of the needs of Australians living with a rare disease.	\$20,000

Name of Patient Organisation	Description of and/or purpose of support	Nature of support (monetary value or equivalent) or description of non-financial support
Metabolic Dietary Disorders Association (MDDA)	MDDA 2025 Industry Partnership. MDDA industry partners work in collaboration with MDDA with the following opportunities to improve the lives of Australian inborn errors of protein metabolism patients: (1) advocate for the work MDDA provides to the patient community with all relevant health bodies, government representatives and health professionals (2) promote MDDA partnership and events via any patient centric websites and social media channels (3) provide the opportunity to include MDDA fliers in occasional product deliveries (4) provide invitations any cooking workshops (or ability for MDDA to provide flier to distribute) (5) share summary of results of any relevant & ethically approved local and international research paper/studies with MDDA	\$3,500

Name of Patient Organisation	Description of and/or purpose of support	Nature of support (monetary value or equivalent) or description of non-financial support
Metabolic Dietary Disorders Association (MDDA)	MDDA IEpM National Forum 19-21 November 2025 Fairmont Resort Blue Mountains. The MDDA IEpM National Forum is a dedicated gathering to connect leaders, healthcare professionals, and industry representatives focused on advancing care, resources, and advocacy for those with Inborn Errors of Metabolism (IEpM). This inaugural event precedes MDDA's annual family retreat, creating a unique opportunity to foster stronger engagement between healthcare professionals (HCPs) and the IEpM community across Australia and New Zealand. As a sponsor, PTC benefited from enhanced visibility within a specialised audience, showcasing the company's dedication to IEpM treatment and supporting the patient community. This forum offers PTC a role in critical discussions with national representatives from the Australasian Society of Inborn Errors of Metabolism (ASiEM), alongside MDDA's executive team and our "to be" reformed Scientific Advisory Council, other patient representatives and industry stakeholders.	\$21,000

Name of Patient Organisation	Description of and/or purpose of support	Nature of support (monetary value or equivalent) or description of non-financial support
Metabolic Dietary Disorders Association (MDDA)	MDDA 2025 National Family Retreat 21-23 November 2025, Fairmont Resort Blue Mountains NSW. The MDDA's National Conference and Family Retreat is the hallmark event that occurs just once a year in a prime interstate location around Australia. It attracts all types of family groups and members from the IEPM Australian community including; newly diagnosed infants, kids and teens through to well experienced adults, those returning to diet, supportive family members, spouses, parents, grandparents, siblings and carers representing all protein IEPMs. It also welcomes special guests and speakers from IEPM clinics, research and other health institutions from around the country. For patients and their families, the retreat offers scientific and educational sessions, developmental sessions, transitional sessions & program workshops, IEPM rare streams, product showcase, supplement tasting session and social functions & meals.	\$13,500