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Muscular Dystrophy Association of NZ Inc.

Unfair criticism of community group challenged by CEO

Muscular Dystrophy Association of NZ Inc. (MDANZ) Chief Executive Ronelle Baker has hit back at criticism that the support organisation is using media tactics to pressure Pharmac to fund the first established treatment for Spinal Muscular Atrophy (SMA), and by default, helping to grow the profit margin of pharmaceutical company Biogen.

"It is Pharmac's job to negotiate pricing for pharmaceuticals," Baker says, "and it is our job to empower our community and support them to access treatment. Families have willingly shared their stories and this community deserves our support to gain access to Spinraza which is the only established treatment for SMA. There is no alternative for them and this is a breakthrough that we have waited decades for."

MDANZ is a non-profit organisation that provides information, support and advocacy for New Zealanders with a range of rare genetic neuromuscular conditions. The organisation fundraises 92% of what it needs to run every year.

"Suggesting that MDANZ has received financial support from Biogen to lobby Pharmac to fund their expensive new treatment for SMA is both unfair and inaccurate," claims Baker. "We have been fortunate to receive a patient education grant from Biogen this year, and the grant application was only partially funded to just under $7,800."

Given the group's audited financial accounts show an over $2 million annual picture for 2017, this amount is a drop in the bucket for the organisation, which Baker claims has been misrepresented, saying, "The Biogen grant money has been used to fund the development of resources that will help newly diagnosed people and families, and the hosting of two North Island based SMA family days in 2018 that included education about nutrition, resilience and coping, adapted sports and yoga. The organisation has received grants from other pharmaceutical companies for a range of educational purposes."

In carrying out its advocacy role, MDANZ has also written to Pharmac about funding for deflazacort as a corticosteroid alternative for Duchenne muscular dystrophy, Rituximab for Myasthenia Gravis, and have joined with other

groups to lobby for funded access to Myozyme for Pompe's disease. Myozyme was also being considered by Pharmac's Rare disorders subcommittee on 6th November.

Baker agrees that the subcommittee and Pharmac has some difficult decisions to make around funding recommendations and priorities. However she also points out that the stories that have been shared in media about children with SMA are real, and that Pharmac are currently considering Biogen's application for funding Spinraza for those under 18years. This means around 32 young New Zealanders may benefit if funding is approved.

"We are not sitting around waiting for a miracle, we are asking for access to a scientifically proven treatment for a small and important group of New Zealanders. These young people are vulnerable. They have a life limiting and disabling progressive condition that should be prioritised," says Baker. "There was a competitor on Australian Ninja Warrior last night, who lives with cystic fibrosis", she says. "It is clear that we are not comparing apples with apples in this space."