

NEW ZEALAND

## Pharmac refuses funding for life-saving drug Spinraza

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Gore High School pupil Ryan O'Rourke is hoping Pharmac will approve funding for the drug Spinraza which will help stop the wasting of his muscles due to the motor neuron disease Spinal Muscular Atrophy type three.

Otago Daily Times

Knowing his body is slowly deteriorating is "the scariest feeling ever".

However, what is worse for Ryan O'Rourke is that a drug that could help him is not funded by Pharmac.

Pharmac is a New Zealand Crown entity that decides which medicines and pharmaceutical products are subsidised for use in the community and public hospitals.

Ryan said he was about 5 when he was diagnosed with spinal muscular atrophy type 3.

"Mum noticed I was starting to trip and fall a lot more."

Over time, the motor neuron disease interfered with his ability to use his arms and legs.

Ryan is now 16 and the condition has meant he has to use a wheelchair.

"In the past two years I have lost my ability to walk.

"Who knows what will happen in the next two years."

It was a "terrifying" position to be in.

"You can feel yourself getting weaker and weaker . . .

"It's the scariest feeling ever."

Taking a drug called Spinraza would stop the atrophy.

"That would be worst-case scenario."

If his body reacted well to it, he could rebuild some of the muscle use he had lost and "have that ability to do more for myself".

Since he had been using a wheelchair, one thing he missed was being able to "look people eye to eye".

"It's one thing that I strongly believe is important, face-to face connections."

He believed it was not right Pharmac did not fund the drug.

"They're more worried about money than people's quality of life, it feels like."

For Ryan, quality of life was the issue. "If I take Spinraza I have much better quality of life.

"I want to have a job. I want to be able to get out. I want to make my own money.

"I want to be an accountant."

However, time was running out for him because if the drug was approved, it would be available only for those 18 and under, he said.

It was especially hard to understand why the Government did not provide the funding to Pharmac when the racing industry had recently been given a \$72.5 million emergency support package, he said.

"I think it is important the Government considers more funding for Pharmac."

Patient Voice Aotearoa spokeswoman Fiona Tolich said about 35 children aged 18 and under and about 75 others had the disease in New Zealand.

People with the condition have healthy muscles but because they lack the gene that creates the protein necessary for sending the signals to those muscles, the muscles waste.

"When the nerves stop sending the messages, the muscle cannot get the signal to do what it needs to do."

A funding application to provide Spinraza for those 18 and under was made in 2018.

"The evidence and data is there highlighting its effectiveness and it is funded in over 50 countries."

The group estimated it would cost about \$6 million a year to fund the 35 children who needed treatment.

Unfortunately, the calculation Pharmac used to work out which drugs to fund was based on the most cost-effective medicine for the greatest number of New Zealanders, Tolich said.

As SMA is a rare disease, it does not fit this calculation model.

"I think the thing with this story is that Ryan might have had his ability to walk saved had they funded this treatment two years ago when the application went in for funding."

Pharmac director of operations Lisa Williams said the organisation worked to get the best health outcomes for New Zealanders.

"Our funding model is the only one of its kind globally, combining both decision making about which medicines are funded and managing a fixed budget for spending on medicines.

"While we recognise the challenges faced by patients and their whānau, and their understandable desire to try new treatments, our job is to look at all the evidence and make a decision that is in the interests of all New Zealanders."

Possible opportunities for investment in new medicines would always exceed the budget Pharmac had available, she said.

Therefore, comparative ranking was an intrinsic part of the organisation's work.

"Every medicine that is recommended for funding by our advisers is compared and ranked against all other medicine funding options, and no one group of medicines is given priority over the others.

"We assess all funding applications against our factors for consideration to ensure that every application is treated fairly.

"At this time, Pharmac will focus on progressing medicine funding options that are ranked higher than Spinraza."

A Minister of Health spokesman said decisions on which medicines were publicly funded were made by Pharmac "at arm's length from the politicians".

"It is not really appropriate for the Minister of Health or the Prime Minister to comment."

The Government had consistently increased Pharmac's budget for medicines and medical devices which included \$60 million in September last year, over two years, and \$160 million, over four years, in this year's Budget, he said.

Since Budget 2019, 13 new medicines have been funded, including six new cancer treatments.

- Sandy Eggleston