



Muscular Dystrophy  
New Zealand

## SMA Community Newsletter

# Update on SMA Treatments

Keeping you informed of new developments

### Gene therapy

Avexis is moving ahead with its programme for AVXS-101, which to date appears to be safe and efficacious in SMA type 1. AVXS-101 is a genetic therapy delivered via intrathecal injection (into the spinal cord).

In December the U.S. Food and Drug Administration (FDA) approved initiation of Avexis' planned Phase 1 clinical trial of AVXS-101 in patients with SMA Type 2 via intrathecal injection.

This will allow the company to evaluate safety, optimal dosing and proof-of-concept for SMA type 2.

### Spinraza

Disappointingly on the 15th December Australia's Pharmaceutical Benefits Advisory Committee (PBAC) decided against recommending Spinraza for funding. Biogen, the company marketing Spinraza, will continue to work with the PBAC with an aim to list Spinraza for use in SMA Type 1 by mid to late 2018.

What does this mean for New Zealand?

While the declined funding recommendation in Australia may impact Pharmac's decision-making there are a number of steps that need to take place prior to Pharmac even receiving an application

for funding. These include registration of the new drug with MedSafe. This application, supported by MDANZ, has now been submitted by Biogen.

### SAVE THE DATE

For an SMA Family Day!

On Saturday 17th  
March 2018, 12pm-  
5pm at the Hamilton  
Gardens.

Join us for lunch and  
hear from an exciting  
range of speakers.

Meet new families and  
ask questions from our  
panel of experts.

Free childcare and  
travel assistance is  
available – contact  
info@mda.org.nz to  
find out more.

International Congress on Spinal Muscular Atrophy SMA Europe are holding a conference 25-27 January 2018 in Poland. Visit the conference website for more information and links to presentations: <https://krakow2018.sma-europe.eu/urope>

## Our Priorities

Thank you to the MDANZ members who contributed to the SMA survey asking about what is important to you.

Your responses helped us to identify priorities for the reference group. These are;

1. Achieving funded access to Spinraza for New Zealanders with SMA
2. Developing resources and working with stakeholders to ensure better and more timely supports for newly diagnosed families
3. Creating informal opportunities for peer to peer networking in the New Zealand SMA community

4. Support other SMA related initiatives being undertaken by health or research professionals.

The group plans to meet again in February and is already working on two exciting initiatives, our SMA Family Day (Advertised on page one) and our New stories booklet.

### SMA Stories Booklet

We are seeking expressions of interest from families who wish to share their stories in a booklet that will be used to educate others about SMA. The booklet will be used to inform a wide range of audiences including other families who are new to the SMA community, government departments and Pharmac.

## MDANZ Member SMA Reference Group

The newly formed Member Reference Group for SMA met for the first time at our National Office in Auckland on Tuesday 12th December.

Reference group members are Tania Woodmass, Brent Walker, Emily Beswick, Fiona Tolich, Jo Truscott, Belinda Old and Anna Sutherland. The group will help MDANZ identify priorities specific to families with SMA and for the benefit of the SMA community. Meetings will be facilitated by Miriam Rodrigues supported by the team at National Office.

## Books for kids

We are looking for SMA friendly resources that can help parents to have conversations with their children about handling different situations in their childhood lives. Please get in touch with us at [info@mda.org.nz](mailto:info@mda.org.nz) if you know of any books we could share with other families.

