



Muscular Dystrophy
New Zealand

SMA Community Newsletter

Update on SMA Treatments

Roche Community Statement on RG7916 and Olesoxime Programmes

Roche Pharmaceuticals shared an update on their SMA programmes; investigational molecules in development for people with SMA, olesoxime and RG7916. While the molecules work in different ways both drugs are administered orally.

RG7916 is an SMN2 splicing modifier and is being studied in three ongoing clinical studies, FIREFISH, SUNFISH and JEWELFISH.

FIREFISH is evaluating RG7916 in babies aged 1-7 months with Type 1 SMA. It is an open-label study, all babies receive RG7916, and there is no placebo.

The first part of the study confirmed that RG7916 was safe and well tolerated, as well as finding the optimum

dosage. Currently, the efficacy is being studied in 40 babies over the course of two years, after which all babies enrolled will continue to receive RG7916 until it is available in their country.

SUNFISH is a similar clinical study but for people with Type 2 and 3 SMA who are non-ambulant, aged 2-25 years.

JEWELFISH is an exploratory study assessing safety and tolerability of RG7916 in people with Type 2 and 3 SMA who have previously taken part in a study with another SMN2-targeting molecule.

Olesoxime supports the function of mitochondria, the powerhouses of cells, therefore supporting the continued role of cells.

The OLEOS study is an ongoing

open-label extension study with non-ambulant Type 2 and 3 SMA patients who took part in earlier olesoxime studies. Patients will be followed over several years. Roche is working on plans for a phase 3 study with olesoxime.

SMA FAMILY DAY

RSVP Now!

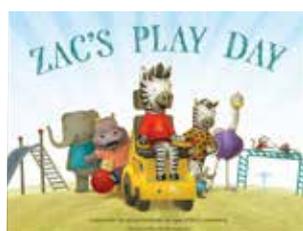
On Saturday 14th April 2018, 11am-4.00pm at the Link Centre, Corner River Road and Te Aroha Street, Hamilton.

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.

RSVP info@mda.org.nz or call 0800 800 337



Zac's Play Day Available Now!

This is a book aimed at young children to provide them with an understanding of SMA. Pick up a copy at Family Day or email us.

Together in SMA Healthcare Professionals Forum

The inaugural Australasian 'Together in SMA' Forum held on the 16th -18th March in Sydney was an independently coordinated medical, educational event targeted at paediatric neuromuscular specialists along with paediatric multidisciplinary team members (such as nurses, physiotherapists, pharmacists, respiratory consultants and other related healthcare personnel). The objective of this event was to present a high quality, interactive educational program that will ultimately ensure the best possible standards of care and outcomes for patients with SMA. The meeting brought together approximately 70 members of the multidisciplinary

neuromuscular teams involved in the care and treatment of patients with SMA in Australia and New Zealand. This meeting focused on the changing landscape of care through scientific exchange and practical workshops. There were two internationally renowned presenters from the USA: Dr Thomas Crawford, Professor of Neurology and Professor of Pediatrics at Johns Hopkins University and Dr Nancy Kuntz, Professor of Pediatrics and Neurology from the Ann & Robert H. Lurie Children's Hospital of Chicago. Four healthcare professionals from New Zealand were in attendance including Miriam Rodrigues who will provide us with an update from the forum at the SMA Family Day.

MDANZ's SMA Member Reference Group

The Reference Group met again on the 6th March 2018. There was plenty to discuss with the SMA Family Day on the 14th April.

Two members of the group, Fiona and Anna will be attending the CureSMA conference in Dallas, Texas in June.

The reference group members are looking forward to meeting you all in Hamilton at the SMA family day.

Reference group members are pictured below; Tania Woodmass, Brent Walker, Emily Beswick, Fiona Tolich, Jo Truscott, Belinda Old and Anna Sutherland.



Miriam., Meghann, Tracey and Rakesh at the Together in SMA inaugural Healthcare Professionals Forum.



The SMA Reference Group meeting at the MDA Office in Auckland on the 6th March 2018.