

## **FSMA NZ meeting transcript**

Kate Edwards started by opening the meeting. All attendees introduced themselves. In attendance:

Rakesh Patel, Consultant Paediatric Neurologist, Starship  
Tracey, Consultant Paediatric Neurologist, Starship  
Miriam Rodrigues, MDA liaison officer  
Kate Edwards – Present of FSMANZ and mother of Natalie (SMA II)  
Steve Reeves – Kate’s partner  
Arama Gummer – Trustee of FSMANZ and mother of angel Zane  
Narelle Greig – mother of angel Tristan  
Susan – Narelle’s friend  
Tina Bell – FSMA NZ Website and Fundraising and mother of angel Tylah  
Hayden and Emily Beswick with Stella (SMA II) and Harry  
Jessie Russell with Maija (SMA II) and George  
Graham Edwards with Natalie (SMA II)  
Arran Kupu – father of Lisa and Melino  
Waimon Thant-Cyn (SMA III) – Trustee of FSMANZ  
Waimon’s father  
Kerry Hills (SMA III)  
Judy Lawrence – Support worker for 0800 FSMANZ and grandmother of Natalie

Rakesh: Before we start I wondered if we could talk about why form the Group and what your aims are, and what you hope to achieve. I think it is important because it helps us to help you.

Kate: OK, from my point of view, my main driver was that there didn’t seem to be anything in NZ that was aimed specifically at SMA. The MDA is great but there is no specific SMA assistance, and whilst the MDA had a SMA support group listing it didn’t seem to be very active. I never had a single phone call from anyone on the list. I just thought it would be good if we could get people more proactive in talking to other people to provide support. I think the aim from my point of view is also to fund research for a cure – one of the reasons I wanted to try Natalie on Valproic Acid was because someone’s get to try it basically and we were willing to take the risk. Also people like Tina will be very keen on getting funding for equipment.

Tina: When Tylah was diagnosed with SMA type 1, we were told that we couldn’t get any funding because she wouldn’t live for a year and what child shouldn’t be given the opportunity for equipment to make their life easier just because they haven’t got a long life span.

Arama: When Zane was diagnosed, there wasn’t a lot of information at Starship. Doctors weren’t able to provide much information and it takes a lot of time and resources to go and search the internet. With the type 1s you don’t have a lot of time to do that research. What I want is to be able to provide all that information to the parents so that the time they’ve got with their children has more quality. We spent a lot of time and have a lot of regrets because

there was so much time wasted. Also, from Tina's point of view down in Christchurch they didn't have the resources. She wasn't given a speech therapist, or any bottles, or any equipment, so we want to be able to raise funds so that we can get all that equipment on hand, so that we can provide it to families so they do not have to go through the same dramas that we had to. That's where we're coming from, that's what's driving us.

Narelle: For myself, on the other hand, we only had 16 hours to deal with SMA, because my son was diagnosed in the morning and died that night. I want to know why I didn't have choices to find out that I was even a carrier. They are so quick to test your baby for Downes Syndrome, Spina Bifida, but I had to deal with all of that in one day so I didn't get any options. I'm sure if did I would probably have ended up in the same boat as these people, as my baby died of SMA I too. I want to help them with their funding provision but at the same time I want families to have choices of being able to get tested not just now that they are aware that it is in your family. It should be an option available to all parents regardless, just like everything else.

Emily: I had Stella's diagnosis when I was pregnant with Harry, so he was tested the day he was born, through the cord.

Kate: Just following on from testing, Tina was saying that she has been denied funding for even getting her grandparents tested for SMA, so that she can find out which side of the family it sits, to know which of her cousins is at risk. But they have been denied funding which to me just seems bizarre.

Tina: I had to fight with them constantly making excuse after excuse after excuse. I was thinking "I don't care about that, lets just do it". And in the end they said that they didn't have the funding to cover this.

Arama: As well there was another lady down in Christchurch who wasn't even told that she was entitled to a disability allowance immediately. These are things that Starship provided for me because I am in Auckland but there are other towns and cities in this country that do not have that information and you shouldn't have to be fighting for this stuff, its so basic.

Tina: That was the good thing for me to come up from Christchurch to speak to you, especially to find out what we are entitled to. For two months I fought for a bottle.

Rakesh: Yes, that is not acceptable.

Narelle: And, I have a 2½ year old daughter and I am just waiting and waiting for somebody to tell me when I can get her tested, because I mean as far as I am concerned we are not out of the woods yet. But no one has bothered to come to us about it. My GP has written away and I can't ask much more than that.

Tina: The attitude of the genetic counsellor in Christchurch said that until the age of [ ] they can't be tested because they [couldn't catch this]

Rakesh: Ok, that is different

Tina: It seems that Starship gives the best to offer in NZ

Miriam: I didn't think that was the case for carrier testing

Narelle: I haven't heard from anybody. I got told when Tristan was diagnosed what I could then do. I had to go back home, because we are Australians, and let my family say their goodbyes, and I came back and I've seen my doctor since and she has written away to the genetics team and I haven't heard a word in four weeks.

Rakesh: OK, well I don't know about your child. I did hear about it.

Narelle: I only got a 16 hours period.

Rakesh: And your paediatrician is Ross?

Narelle: Yes. [Something about prenatal testing]

Rakesh: Yes, There is some testing now available.

Narelle: But not for carrier testing?

Graham Edwards: Why is there a difference? I think they suggest that it is quite complex. What is the difference in cost?

Rakesh: I couldn't tell you what the cost difference is. There was a study in 2003/04, which looked at ante-natal testing, and they say that it looks cost effective but there has been no promotion of that. There has been no advocacy of ante-natal testing and I don't know why. It needs to be looked into and we need to lobby and advocate.

Graham: With the other things that they pour money into, the frequency of deaths can be a lot less with other disabilities

Rakesh: Exactly. That's because children don't have a say. And NZ has just recently got a child commissioner. Most countries do have a minister for child health or children and we don't.

Graham Edwards: Although this is not medicine related, if I want to bet a mobility vehicle for Natalie, I can't get funding from the Government because she can't drive it. But they say in their mission statement that disabled children and adults have the same rights. But they don't. And unfortunately sad as it may seem you have to fight to get what you want and its not right but you've got no choice.

Rakesh: No you don't. And these are things that you can't plan for – you don't start off with health insurance here. In some countries parents' health insurance policies do cover disabilities, and some countries don't. We rely on our government and we pay our taxes and you expect a certain standard of care and provision.

Graham: You're allowed to smoke and get funding

Rakesh: Yes – that is self inflicted and you often wonder. So it does come down to....

Graham Edwards: What about these guys in Cambridge? What sort of support do they have – Hayden?

Rakesh: Well, they are a bit more fortunate because they hopefully have both a good GP and a good paediatrician who advocates, because he has rung us several times and emailed us. And we go down to Hamilton to do clinics and that's because Hamilton pays ADHB to provide paediatric neurology services. We only go down to the upper part of the North Island, I don't know what happens in Wellington or Palmerston North, or anywhere in the South Island. I know that there is a paediatric neurology service in Christchurch

Graham Edward: I don't think things are that good in Wellington because my friend who lives in Wellington has a child with Downes Syndrome, and they are better off in Auckland.

Rakesh: Exactly.

Kate: It is an interesting fact that I have a database of the SMA people in NZ and there is not a single SMA person in Wellington, which has always struck me as quite strange, because there are quite a few in Christchurch, and quite a few in Auckland and upper North Island. I was wondering whether that is because it has never been diagnosed. It strikes me as a bit bizarre that there can be no SMA people in Wellington

Rakesh: Good point, and they have a fair population. There are issues of inequality of health provision in NZ, and that goes against the mission statement at the end of the day. At the moment, we are trying to advocate a national service where we can provide. And the Government has been looking at using us as a pilot team to see what happens. But the momentum has been lost at the moment and I don't know why. But certainly if we were a national service then I think we would be able to provide more equal care. There are pros and cons.

Graham Edwards: I mean to get this wheelchair was a bit of a fight at the age of 2.5. But to have the wheelchair early must help her life expectancy, and other things like that. And even getting the high low function was based on carer safety, nothing to do with her needs.

Rakesh: The problem is that we don't get asked by Mobility Solutions, because I think they are afraid that we will advocate for the child, but I think it is important that we get asked by them.

Jessie: Maija's had a trial in a wheelchair, and is meant to be getting a trial chair in January, when she will be 2. I've been pushing for 6 months.

Rakesh: You guys have probably broken ground and made it easier for others, especially in the Auckland region but I don't know what it will be like south of Auckland because every area seems to have a different provider.

Judy: Have you done anything about getting a wheelchair for Stella?

Emily: Yes, she is supposed to be getting a trial one soon.

Rakesh: I think the Waikato region is a bit better than Auckland. It also depends on your therapist a lot – it depends on your OT and your physio. If they are proactive, that's good. If they are not, then you are in a bit of trouble. We are trying to get round that by doing these neuromuscular clinics, by having our OTs and physios there providing input for your own OT and physio.

Graham: There's the Wilson Home

Rakesh: Well, that's where we are doing our neuromuscular clinics. It's a minefield and I think as awareness increases, we are going to be breaking new ground.

Kate: Waimon, what about the adult perspective? I don't know what the funding is like for adults, whether it is any different.

Waimon: I've been trying to get a wheelchair with a tilt and everything and I've been on a waiting list for about 6 years, and I've been contacted by Mobility Solutions for a trial chair.

Rakesh: That's too long

Jessie: I was really surprised we waited for ages for our trial and we had 6 specialists involved and got there and it was so wrong, I just turned round and walked out. I mean Maija can't hold her head up when she's sitting up straight and she was in an upright position without a tilt and no head support and stuff like that. There are 5 or 6 professionals here all to do with CT and chairs and physiotherapists and occupational therapists and to get it so wrong!

Rakesh: Maybe it comes down to us writing a statement saying this is what is appropriate for children with SMA.

Judy: That would be really useful for me to have on the end of the phone line to suggest to people.

Rakesh: and something that we can post out or keep on the website and appropriateness of provisions.

Kate: Well this is it, you can even say type I and type II, but within each type there is such a wide variety that it is very hard for any professionals to work out exactly how much support they need, in the wheelchair for example.

Tina: Because I was prepared to pay for a special chair for Tylah, we had the company who made it, the physio and the OT all come to our house and actually go through this and they were really good once I said that I'd would pay for it myself.

Jessie: The thing is that they have the OT there, the physio there, the seating solution person, the person from the wheelchair base place and one other person I can't remember, and they

still can't get it right.

Rakesh: It depends on the experience. It does.

Graham: I remember that when we only had the OT and the wheelchair guy, we didn't have that many people.

Rakesh: It depends on who it is, I mean if you have the OT from GSE they are absolutely useless (that's Group Special Education). Their aim is to get a child in front of a desk at school, that's it, and as long as they are there, they have fulfilled their job, and children over the age of 5 usually only get OTs and physios from GSE. So it's really not very good. I don't know what Christchurch is like.

Tina: [something about Medifab]

Kate: Natalie's stander is Medifab.

Tina: Well, that's a private company, isn't it? Just a little chair that Tylah sat in, I managed to get them down to cost at \$696 and that's just a little chair. But this would be suitable for type I, type II and type III. I've made enquiries of a company in Christchurch whether they can actually copy it because it comes from Germany. Otherwise they were going to charge us \$1200. At least it's down to cost, but some families can't afford it.

Rakesh: Well they shouldn't be in a position where they should have to buy it.

Tina: Well what parent isn't going to think that I'll just cover that cost right now because it's my child.

Rakesh: Exactly.

Judy: But not everyone can afford to.

Tina: No exactly, and that's why we want to have a full set of equipment so that the minute a child is diagnosed we can loan it to them until something else comes up

Rakesh: The other person is the Social Worker, who knows what you are entitled to.

Graham: You should get the social worker the same day your child is diagnosed.

Arama: Was that at Starship? I got given a name but never got to meet her. I've still got it somewhere.

Graham: It's not just about getting equipment, it's also bureaucracy.

Rakesh: Exactly, they're rushing through the minefield of all the paperwork.

Graham: I wanted a high-low chair that was on wheels, but they said I couldn't have it initially because she was going to get the high-low function of the wheelchair because the

chair was mobility but I got it eventually.

Tina: For type I especially, they very much say well this is your diagnosis so go home and care for them, and that's it.

Kate: We had that with our previous neurologist – Rakesh is ours now. The previous guy was quite old-school and when we said “well what can we do to help her quality of life, for example take her to the osteopath to prevent scoliosis”, he said we had to work out what we were going to do when she gets really sick, whether we want her to survive and take life measures to save her life or whether we just want to let her go, and I thought, my child has just been diagnosed with type II and that's not necessary. Let's be a bit proactive here.

Rakesh: But the thought has changed recently, it's becoming more proactive. At the end of the day, it's not just quantity, it is also quality of life. You've got to provide as much as you can.

Jessie: I was also wondering about the cough assist?

Rakesh: The cough assist device.

Jessie: Because we've been to hospital 3 times this year and she really struggles.

Rakesh: Who does she come in under?

Jessie: She comes under Liz Edwards.

Rakesh: Well, I talked to Liz yesterday about cough assist.

[?]: What is cough assist?

Rakesh: It's a device that augments your cough, OK, so it's not positive pressure, it generates a negative pressure which helps you cough your secretions up. It augments your own cough, which is useful.

Jessie: We said we'd buy one, then Liz said “well, hold off because the Muscular Dystrophy Association are buying one and then I kept talking to the suppliers and they said that if I could get two orders, I could get two to the price of one.

Rakesh: Yes, two for one

Jessie: And then I told Liz and she said “well I wouldn't spend 5 grand on it if you're not sure it's going to work” and I talked to a couple of respiratory physiotherapists who've used it in Canada and in England and they said it's not always successful.

Kate: It's not. I've heard a lot of children get problems with it, stomach problems. There is a fair bit of information on the US FSMA website.

Miriam: Did Liz think it would be useful for Maija or not?

Jessie: She hasn't got any experience with it, so she doesn't know, but she said it would be good to have one in the hospital and one to be able to lend out to the community, and it's just that she'll cough for 2 hours. You just sort of get to the stage when you feel that you want to do something else for her; it's just not working and you need to help her to cough.

Kate: It's amazing that a respiratory specialist has no experience of the cough assist

Rakesh: No I don't think she has, but our two respiratory physios have, which is good, that's Rachel and I can't remember the other lady's name, and I think what we will end up doing is trialling them on individual children and seeing if they are useful or not.

Kate: Because I think this is New Zealand's problem. It's that we have such a small population, that a lot of the specialists just can't get the experience.

Jessie: It should be useful for other conditions as well.

Arama: Did the MDA actually buy one?

Miriam: We've bought 5.

Rakesh: You bought 5 – not 4 or 6 (2 for 1)?

Miriam: We've bought 6 and on-sold one to these guys (*pointed to Beswicks from Cambridge*). So that's why 5 sounded funny because it is an odd number, it's because we've actually bought 6.

Rakesh: I think we had better sit down and then go through when to use it and who to use it on.

Miriam: I have asked Jonathan Pinder, who is a respiratory physician, who I've seen a couple of times in other places, he's a big provider of cough assist machines, and I've asked his advice on uses.

Rakesh: Well, we've got a new respiratory physician at Starship from the UK, who has some experience.

Miriam: I mean, I know that Liz Edwards is supportive of the idea of the cough assist and what I would like to do is to be able to gather some data from using the ones that we have that would be useful in lobbying the DHBs to buy them themselves.

Rakesh: Bi-pap and v-pap aren't an issue – they are funded quite readily – so that's not an issue, so if she hypo-ventilates, that's not an issue. Cough assist is different. I think it still has to prove itself, prove its worth.

Tina: It's useful to know about the cough assist because it's only the stuff that Tylah directly needed like IHD that I really know about. For everyone else, obviously they need other things. I'm going to ANZ for a huge grant, but whether we get it is another thing. I mean as

long as I've got quotes for equipment, I've got something to base it on.

Rakesh: Yes, exactly. The other people to try are Vodafone. We know they've got a few funds to donate.

Jessie: My uncle works quite high up in Vodafone. He's heard of SMA, so that's a start. His name is Mike Davies.

Graham: It takes a family member

Rakesh: Yes, it takes a family member sometimes

Kate: We are now tax registered, so we have tax-exempt status, so companies can get their tax back.

Rakesh: Yes, exactly

Graham: What can the MDA do to support the SMA charity? What do they do for funding?

Rakesh: MDA used to be helped out by Ferrari because NZ Ferrari's son, Dino, had Muscular Dystrophy. It sometimes takes things like that. I don't know if there are any big benefactors at the moment.

Miriam: The person to talk to – and I'll give you her details – is Rayma Ingles, who is our Grants Co-ordinator. She's really happy to give advice and she is very knowledgeable about a whole variety of organisations to go to and ask for funds, basically. So she would be able to give you the details and give you advice on how to write the grants as well if that would be useful.

Rakesh: I know the person from Cure Kids and I know how they do that. They are very very proactive

Kate: Yes, I have spoken to the Chairlady, Kaye

Rakesh: Yes, I know Kaye and certainly they would fund any research we wanted to do as they're keen on funding research. If there's anything we can come up with, we can always approach them. In effect, she told us that – that they would be willing to fund.

Arama: Who's going to do what, we need some sort of action plan and who is going to co-ordinate this?

Rakesh: Somebody needs to allocate things to do

Arama: Well, maybe when we get the minutes we can work out what needs to be done and sit down with the MDA to allocate tasks.

Rakesh: The other person to invite from Christchurch is a new paediatric neurologist who's just started, Cameron. Do you know Cameron?

Tina: Yes, I don't like him.

Rakesh: OK, we won't invite him then.

Tina: He's very good but he basically told me that my daughter wouldn't get a good quality of life as a type II, and there are certain ways to say things

Rakesh: Yes there are.

Tina: and [name] let us do what was best for us, whereas Cameron tried to pull me down into reality. But I wasn't giving up on her and she was going to beat this.

Rakesh: And you need that hope don't you.

Graham: I guess the thing with SMA is that 60% of kids with SMA are type I, aren't they, so I guess it's about proportion

Judy: And are all of them diagnosed?

Narelle: Well, it's funny you say that because I spoke to my GP about that, because a lot goes through your head and the question I have is – children who pass away from SIDS, who's to say it isn't SMA because I was told that SMA is a clinical diagnosis, and of course they need to do the blood testing, but it is all the signs first to say that's the path you are on, and obviously they can start deteriorating while they are in your womb, so when they come out, if the parent is a first time parent, or even a second time parent – I mean I was a second time parent and I still didn't know anything was that abnormal until he got a little bit older, but it hasn't been ruled out that you don't even know that SIDS isn't in fact SMA, because when they pass away they can't clinically diagnose it and they don't get a blood test on it and it's just put down to “well we think that's what they just passed away from”.

Rakesh: I think it all depends on where you are and who you see. I hope I can say this as a generalisation – if you see a paediatric neurologist, he will make a diagnosis.

Narelle: Yes, I realise that but what I am saying is that if your child is only 3 or 4 weeks old

Rakesh: Yes, then it's difficult.

Narelle: Because obviously there is no age as to when they pass away and Tristan was only 10 weeks. I mean everyone's child has been a different age. I'm saying that if they are only 2 or 3 weeks, of course you don't expect them to keep their heads up or kick their legs around.

Graham: It was the same with Natalie. Dr Dominick just thought she was a slow developer. I don't know if there is anything we could have done if we had known earlier, but possibly not.

Narelle: No, I'm not complaining about the service of my doctors, because they were

brilliant, and in the amount of time I had to deal with everything, I really couldn't fault or knock anybody, after everything they did do for us, but what I am saying is that the percentage doesn't really mean anything because there is still a big gap as to the fact that SIDS children aren't tested. We really don't know what the numbers are

Rakesh: Yes, we don't really know the true incidence, and then you don't know what might happen pre-birth either.

Miriam: And that's something that Monique Ryan is trying to ascertain.

Rakesh: What we are trying to put into place is a National reporting system. We already have one for infectious diseases – it's in the New Zealand paediatric surveillance unit. What the Australian and the New Zealand one are trying to do is where every weak child is reported to a central location and every weak child is hopefully seen by a paediatric neurologist and certain tests will be performed. Again that may not pick up all the ones that we talked about – the early ones.

Kate: But it's still amazing that there is no central register of people with SMA. There must be people falling through the gaps.

Rakesh: There are people falling through the gaps. It's not just must be – I'm sure there are.

Narelle: Tristan was only 5 or 6 weeks old when we first ended up in hospital and they just put it down to him having tummy issues, and that's why he wasn't feeding properly, because they didn't know what signs to look for, so obviously there is no – I mean I know that everyone specialises in different things, but I was under a paediatrician and when I went and saw him and said that he hadn't gained any weight, I hear that he's got to turn a big corner because he hasn't been eating properly but he hasn't been eating properly because he couldn't swallow. And it was 3 weeks later that we ended up in hospital again due to the major weight loss and I was there for a week before I got told what Tristan had. Now I think they knew beforehand, but we had problems with the blood testing and I can understand that they didn't want to scare me or upset me or anything, so I did cop it all in one day, but they did come and tell me what was wrong even before blood tests had come back.

Graham: It depends – I mean good old Rakesh here, he told us.

Narelle: He was actually in Napier on the day, he was actually meant to be there. We were waiting on you to come back from your holidays.

Rakesh: I think I was doing a clinics around the upper North Island and was in Palmerston North that day

Graham: He actually told us what he thought it was even before he did the blood tests, rather than waiting, which was the right thing to do.

Narelle: Well, I think he was in a situation – I don't think he was really allowed to tell me – I mean you can understand they've got ethics too and I can appreciate that. He was waiting on a neurologist to come and see me but he did tell me because of the situation. This is what I

think it is; I do have to have neurologist confirm it. And I thank him for that I mean he doesn't need his butt sued off for telling me wrong information either. I do understand that there is protocol.

Rakesh: Yes, yes

Tina: We were in the same situation when we took Tylah to a paediatrician. He obviously thought that's what it was, because he told Paul (our GP?), but he never told us – he told us our baby was going to go. For five days I held her not knowing whether she was going to go that night. He should have told us there and then. I think it's this, but it's got to be confirmed.

Narelle: I think that's what they didn't want to do to me.

Tina: But instead I was terrified, I was holding her, would sleep with her 24/7, because I didn't know anything. Paul was actually really angry when we told him that for five days we'd sat there not knowing.

Rakesh: Yes, he should have told you.

Tina: He was very angry, because he said he actually told me this is what it was but he could have told us straight away, but he didn't say it's got to be SMA.

Narelle: How long did it take you guys to get a confirmed diagnosis?

*A couple of people responded with a week and a few said between 7 and 8 weeks.*

Rakesh: It's changed a little, it's a little bit faster

Judy: Is it still going to Australia?

Rakesh: No, we are doing it here. There is a new assay that's starting.

Tina: Isn't it coming to Christchurch?

Rakesh: There's a new one that's about to start this very ?

Narelle: Because they told us that the first lot they took of Tristan clotted.

Rakesh: Yes, I heard about that. Quick diagnosis I know they pushed hard.

Narelle: They did, they did do everything

Rakesh: That's why the new assay has just been developed by Liz Harris, so hopefully that will here in the future.

Miriam: And that's going to be in Christchurch.

Rakesh: It's at the cusp of technology at the moment. Things have really changed.

Jessie: Something I was not clear about and she says she has asked the geneticist how many copies of the SMN2 gene she has.

Rakesh: We can try to estimate that by doing the SMN2 protein. I think that's something you can assay.

Kate: Yes, I'd be interested to know that as well.

Miriam: They will do it at NCRI .

Rakesh: It would be good if we could get pre-treatment and then treatment .... I think it's going to be important that you see a geneticist.

Narelle: Well we're waiting. I mean a lot of people have said to me 'do you really want to find out about your daughter' but yes, I do. I mean to know quality of life. Today yes she's fine, but obviously I thought Tristan was fine too but had a 'formula' issue, because we ended up on Peptic Junior, because they thought "what's wrong with him, why wasn't he eating?"

Tina: It seems everyone has different stories, though, there are still families in Christchurch, us being one of them, one was told not to get any more injections, you know 5 month jabs and all that, we were told nothing and the other were told to definitely get them, all by the same neurologist.

Rakesh: It's probably the questions that the parents ask.

Tina: Well, he told them to get them and the other people asked and he said 'no, I wouldn't' and I checked the tape last night because Paul was also told that with one family that if you have a type I child then you will always have type I children, whereas Kate said that's not always the case.

Rakesh: There are intermediate forms. It depends on your SMN2 level and the tests we do don't determine that.

Kate: I was told generally that if you have a type II and you have more children they will probably be type II but its not certain and then there's the family from Rotorua where they had a type II and then their little 3 year old who was type II has just died recently, and the older child is quite well, so there is definitely no guarantees.

Rakesh: It depends on what people understand. It's up to us to update the paediatricians now.

Tracey: It very much depends on how much experience they have had and also their training.

Rakesh: There are priorities. Having a national group I think will help. It will certainly get you in touch with the right people, but somebody has got to make that diagnosis and tell the parents this is what might be done, and somebody's got to tell the parent 'yes, there is an

issue’.

Kate: Yes, I don’t know whether most paediatric neurologists know about us.

Tina: When Narelle contacted us and that was one of the first things I asked her – how did she find out about us?

Rakesh: Was that through the paediatric society?

Tina: I was hoping that she was going to say that they gave her our number.

Rakesh: It should have been but we could send out details of the SMA group again.

Judy: Sorry, how did you find out, Narelle?

Susan: I got onto the internet as soon as I found out Tristan had SMA, and then I found the New Zealand site. I had found the American site. I emailed somebody explaining our situation the night Tristan died but I never got an email back. I was just asking ‘can we get some support for Narelle’ but nothing came back and that was through the SMA site, but it was someone in Auckland and I emailed ... but anyway that was just what happened but I just think that because it’s new.

Kate: Narelle emailed me and I definitely got that one. I don’t know what happened to the first.

Susan: I just assumed that it was because I wasn’t the parent.

Kate: No, I never got anything.

Tina: So I have linked that FSMA national email straight to my inbox, Narelle, so there are now two of us receiving emails.

Rakesh: We can increase knowledge of the group. I can send another flyer out to the paediatric society – that’s not a problem. There’s a paediatric update that happens every year, so if you provide me with some flyers and a stand or whatever, it’s easy enough to do.

Narelle: [Name of Doctor] offered to come out to see me of his own accord. The personal touch I think was brilliant. If Susan hadn’t told me about the website, I wouldn’t have known about it. Then it took me a couple of weeks before I looked at it because I wasn’t sure I was ready to read other people’s stories.

Rakesh: I just hope that, certainly in the Auckland Region, that should quickly be done. The problem is that you’ve got awareness within the group, but you need awareness in the doctors and paediatricians, nursing staff and whoever the support people are. You’ve got to disseminate the information.

Narelle: I might be way out of line here, but I remember you get a brochure for anything, influenza, a cold, meningococcal B; why is there no information for SMA – the support group,

the website? I mean at the time of diagnosis, parents don't want everything in your face, but why couldn't there be something basic, very bland – something shoved in your face when you go, because I would have read it when I went home.

Miriam: Well, there can be.

Rakesh: There's no reason why we can't.

Narelle: What does it take to get it, because I know people that could create one, but I don't want to without all the correct information.

Rakesh: We can always plagiarise.

Tracey: We could always get the UK one and have a look at it.

Kate: We are a branch of the US FSMA and fully entitled to use all of their literature and Tina is in the process of changing it to a NZ version.

Judy: The Jennifer Trust for SMA have said that any information they have they are happy to send us. They are a different organisation from FSMA, who are very focussed on support of the family.

*Talk about profile in the media and public awareness.*

Rakesh: We need something. See, at the moment the media is not interested in just saving lives. They want something they can grasp.

Tina: Isn't it something they can grasp that all these children with type I especially, and I'm not knocking type II at all, because you have your struggles with the equipment that you need as well, but all those children with type I are going without any funding? Surely that's a story that should be told to the New Zealand public, because it's not just tragic, it's absolutely outrageous that a human life is regarded as being so unworthy for assistance and that should be a big hook

Rakesh: Yes, that should be the case with the media.

Narelle: I can get out to the public as being a Mum who lost her child, but at the end of the day the media need to badger somebody. If we go to John Campbell, he is going to want to badger someone.

Susan: But they are going to badger the Minister of Health, they are not going to badger the parents or the doctors.

Narelle: But that's what I'm saying. Unfortunately, I can put my proposal together but they are going to want to get some sort of professional advice.

Graham: I'm sure that if 70% of SMA kids were type II or III, there would be a lot more awareness.

Rakesh: What we need to do is to sit down and write what the rights of the parent and the rights of the child should be. This is a kind of gold standard statement that we could work on, so where your funds aren't provided or your chairs aren't provided or appropriate feedings aren't provided, you can say "look there are children that should be getting a quality of life – sure you may not be able to provide a quantity".

Tina: If we got a TV slot and you are prepared to say that you will go on it, then we will fight to get on TV.

Rakesh: I am sure me and Tracey could. There is no reason why we shouldn't. But you want somebody vibrant and good looking, so Tracey can do it! I'll sit at the back and write something out for you!

Narelle: And the ambition is to get John Campbell to badger the Health Minister and say "where is our funding?" Because as soon as people get put in a corner they say "oh we'll help" but you need to attack them like that. I won't give up until ..

Kate: I think the other thing we need to do is to raise the awareness. Everybody has heard of cystic fibrosis, this is almost as common.

Susan: I was just saying to Narelle "when are you going to do a week for SMA, like the SMA week?"

Narelle: It could be like the daffodil week or ..

Kate: worldwide August is SMA awareness week, but there just haven't been enough people who have been wanting to actually do anything.

Narelle: I mean creating the awareness is great, don't get me wrong, but at the moment they are not prepared to let us be tested.

Kate: Well that's another thing that the actual awareness is going to create, though, because if people have never heard of the disease, they are not going to request it.

Narelle: But they are not going to be allowed to anyway. This is what Tina was trying to say before. We've got to get over that funding hurdle first because then even when we have heard of it, you're not allowed to get it done anyway.

Kate: True, yes.

Arama: So raising the awareness will eventually show them the figures and statistics and how many people are out there that need it and put pressure on the Ministry to provide it.

Tina: So we'll just do it alongside each other – raising the awareness and we'll have a TV slot hammering them.

Kate: Yes, well that's what we're going to do

Waimon: Is it OK to ask a question?

Rakesh: Yes, say what you like.

Waimon: Kerry and I want to know – are there any drug trials going on at the moment that we can try?

Rakesh: There are some trials going on and I'll talk about that. The big one at the moment is Sodium Valproate, that's the anti-epileptic drug, Epilim. There have been trials using about 5ml per kilo twice a day and what they are trying to achieve is increase your expression of SMN2 gene. And SMN2, some of that gets converted into SMN1 protein, which will hopefully be protective, so that shows some promise. OK.

Waimon: Is that available in New Zealand?

Rakesh: Epilim - it's cheaply available in New Zealand.

Miriam: So can you take it and not be in a clinical drug trial.

Rakesh: We use it off-label.

Rakesh: There are some side effects that you need to be aware of – it can cause liver problems, it can cause blood problems and it can make you increase weight, which may be a big problem for individuals with SMA.

Kate: I would just like to say that Natalie has been on it for about 2 months. I don't know how long Stella has been on it.

Rakesh: Probably about the same – less maybe – 6 weeks?

Kate: Initially, she had a bowel upset but other than that I'd say she's not had any negative effects from it at all. In my opinion, it's definitely given her more energy and had a positive impact on her health.

*{NOTE: If anyone wants additional details regarding my opinion on the impact of valproic acid on Natalie they are welcome to contact Kate directly at [stevenandkate@clear.net.nz](mailto:stevenandkate@clear.net.nz)}*

Rakesh: What we are trying to do is stabilise strength. I don't know if we can say we would like to increase the strength.

Kate: I think the purpose of Valproic Acid is basically to try to hold them at the level they are already at.

Tina: So how long has this been available?

Rakesh: It's not been available per se, it's still under trial. We are using it off label. The medication has been around for about 20 years but not for SMA.

Tina: But how long in New Zealand?

Kate: Rakesh is the only one prescribing it.

Rakesh: We're the only guys prescribing it and that's only in the past year. The evidence has just come up in the past year or two.

Kate: These are the only 2 in New Zealand on it – Natalie and Stella.

Tina: But you said the past year.

Rakesh: Yes, for the past year we have been saying we were willing to give it a go.

Tina: You see, we asked if there was something that Tylah could take and Cameron said no.

Kate: It is only available for children over 2 because it has been shown to be dangerous for children under 2 (liver toxicity is more likely) and therefore it is only government approved for over 2s.

Rakesh: It's off label as well, so I'm asking the parents to take a risk, by using this medication off label.

Arama: So you're the only one really who has stepped outside the square.

Kate: Yes, he is

Arama: That's what we need – someone like yourselves to step outside that square and give us something that ..

Tina: Because Cameron told me that he doesn't agree with intervening, because there is no cure and it's not giving them quality of life, so depending on what doctor you are under depends on whether your child gets a better quality of life or the option.

Rakesh: Yes, I know what you're saying

Tina: He told me she would have no quality of life as a type II child, but look at Natalie.

Kate: Well that's why I asked Rakesh to come and talk to us, because there are different individuals who will take their own decisions within what they are legally allowed to do.

Tina: But it means that the type II's in Christchurch will have to fly to Auckland to see Rakesh for access to the drug.

Rakesh: Well, the Government does fund Epilim.

Tina: If your neurologist doesn't offer the information

Tracey: Then you don't know what's out there .

Tina: We didn't know what we were offered until I got talking to people. I've got a cousin whose mother said to me "hey you should be getting a disability allowance". I got my first payment the day my baby passed away.

Rakesh: That should have been advocated

Tina: We didn't have any advice. Tylah was 6.5 months. But I never saw a social worker.

Arama: Who is responsible for providing that information to the parents?

Rakesh: Paediatricians should take the time to do that. I'm afraid to say that would be my colleagues.

Tina: That is why I spoke to Lis Robertson from MDA Christchurch about having our own fieldworker, because we had great support from the team but it was the same as Kate said before, there is nothing SMA specific. Even Lis said "you as a parent are the best person to go to for information".

Rakesh: In the past we have relied on family to family or parent to parent.

Jessie: I feel if someone rang me I am still not in a place where I could be constructive.

Judy: That's why I think I am far enough removed that I can handle that.

Tina: Well, I am just not prepared to let another family go through the shock that we went through. I do find it hard and I do break down.

Rakesh: [*to Judy*] You are based in Auckland, right, so maybe in the next few months, we could get together with a social worker, maybe somebody from WINZ, ourselves, maybe somebody like Rosie who knows the ins and outs of obtaining things for kids – Rosie Marks is a developmental paediatrician who is quite an advocate for children with disability – maybe the guys at Wilson Centre who are providing the neuromuscular clinics, and physios, and maybe the palliative care people; and seeing what should be provided.

Arama: Once you do all that, how will you let us know what the outcome is?

Rakesh: We would have to put it on paper and from there we would start formulating guidelines and a checklist, and maybe we could have some of those checklists and guidelines available for their paediatric update meeting.

Judy: When is that?

Rakesh: February/March, usually the first week in March. Certainly that would target most of the paediatricians.

Judy: Do any of the neurologists here go to the FSMA conference?

Rakesh: Not yet. I mean we're relatively new. We've only been consultants for a couple of years, 2 or 3 years.

Kate: Because I think actually another priority for NZ FSMA is to send a neurologist to one of the conferences to get that specialist knowledge.

Rakesh: It might be worth sending somebody more independent; I think that's more important. A parent advocate is just as important.

Tina: Because they've got toy libraries in England for SMA children and that's what I would like to see for these type I children and find out what they have available.

Judy: I think that the SMA conference in America is more important for a neurologist to go to, because they are the ones who do the research and provide all the information.

Kate: Yes, they do all the research

Tina: I don't mind going to that, but I would like to go just to see

Judy: You would absolutely love the JTSMA conference. It is a really supportive family conference.

Rakesh: What's the JTSMA?

Judy: It's the Jennifer Trust. It's the UK one - very family orientated and lots of SMA 1 parents there.

Kate: They are actually quite different. The US is very research driven, the UK is equipment/support driven. This is in some ways why we want the neurologist to go to the US one but maybe somebody else who wants to look at the equipment, maybe an OT or physio, to go to the UK one, so that we can look at the different equipment and the toys and everything. Yes, that's true - Steve just said maybe an engineer who can try to design some equipment for us.

Judy: Yes, because there's a little girl in England with SMA II and her physio and her OT have built her a sort of standing frame so that she can go dancing with her sister. It's supportive back and front and a frame round her and her legs hang free so that she can dance.

Kate: And they designed that, and apparently there's a group in the UK that have a retired engineers association or something and they basically will give their time to designing equipment, and I'm sure we could do something similar in New Zealand.

Rakesh: Well, you can - you've got that New Zealand IPENZ, a professional engineers group who are equivalent to the colleges. We could approach a professional society like them.

Miriam: So there are quite a number of different things that as a group you want to be working on. Do you think you've got the people to be looking at all the different aspects, or

maybe you might have to narrow down a few of them or get some more people involved.

Rakesh: You can only do so much.

Judy: Kate and Waimon and Arama put a lot of work into setting it up and Tina has done an amazing job on the website. Really there's been no one else that has been actually doing, doing, doing.

Jessie: I'd like to help but then again I still don't feel like I'm all that strong dealing with other people. But I do work in homecare and know about all the funding and that sort of thing, so any help I'm really happy to give.

Narelle: You don't need a massive packet of information when you've just found out a diagnosis, just something giving us the pointers of what we are entitled to, so that when you get to "OK all right I'm going to ring these people because they can organise the homecare I need it right now".

Tina: It doesn't happen like that ...

Narelle: But I haven't been there, I didn't get that option OK, so I'm just trying to help by saying "well this is available". I've just sat here and listened to so many people saying "I didn't know this was available I didn't know that was available", so at least the brochure at least tells you it's available. Then you know where to start your fight. I know that it doesn't get handed to you and I know you have all had to work for it – I don't take that away from you – but if you don't even know where your fight begins...

Rakesh: Exactly

Jessie: I would have thought that being in a homecare type job for 6 years before having Maija that that would have helped me, as far as knowing where to go, needs assessments, all that sort of stuff, and it didn't at all and I am so concerned about people that don't know the system, because it is such a struggle.

Tina: That's why you need Social Workers

Miriam: One aspect of this is that it's not specific to people or children with SMA at all.

Graham: It's disability in general isn't it?

Arama: Miriam, have you guys got anything at MDA that provides information like that to families?

Miriam: No, I don't think we do.

Rakesh: Kids in the past have been neglected

Miriam: But I think it would be a good thing to develop that sort of resource, so what I was saying is that, because this isn't something specific to Families of SMA, perhaps it doesn't

need to be a priority of SMA – you could pass it on to MDA and you guys could work on something more specific.

Kate: I have to admit that when we first had Natalie diagnosed, when we spoke to our Social Worker, she was saying “well, this is what I think you are entitled to” and I said “you don’t actually sound certain” and she said “actually we don’t understand it”.

Miriam: And it changes and it’s different depending on where in the country you are.

Rakesh: The problem is that the rules change and it’s different from area to area – that’s the big problem.

Kate: Which just shows that it needs to become a national thing.

Narelle: You should be able to ring that 0800 number and say “OK, I live in Christchurch and I need some home help – where can I start”?

Judy: That’s what I want to be able to do.

Miriam: You have to know to ask for home help, first of all

Rakesh: You’ve got to educate the primary health providers, the GPs, paediatricians, Plunket nurses. Actually, Plunket nurses ..

Miriam: I think that’s a really good idea though, we do need to ..

Rakesh: Educate the Plunket Nurses? You see, we’ve got a different system compared to Australia here. Plunket nurses who work throughout the country.

#### *Question about midwives*

Rakesh: Midwives, maybe not. They are too busy trying to deal with the babies

Narelle: Yes, but they’re the people that come and see you for the first 4 weeks.

Rakesh: Doesn’t that depend? Is it always midwives who come to see you?

Narelle: The midwives hand the paperwork over to Plunket and then Plunket step in when your child is 6 weeks.

Rakesh: I’d like to target the Plunket nurses. I think that would be a lot easier.

Judy: Because actually it was a Plunket nurse who first listened to Kate when we thought there was something wrong with Natalie.

Rakesh: I know somebody who is a Plunket nurse and she would be a good point of contact in the first instance.

Kate: Having taken Natalie to the GP to say “there is something wrong with my child” and the GP had said “I think you are worried about nothing”, and it was only when we came to the 12 month Plunket check that I’m like ‘there’s something wrong with her’ and they’re like “yes, actually I think you are right”, so they are really quite a lot more in touch, I think, with how a child should be doing at any particular age.

Judy: I think Wai and Kerry had some more questions.

Rakesh: Yes, I hadn’t finished their answer yet either! There’s another drug as well using salbutamol, which is Ventolin or Volmax, and that’s shown to possibly increase muscle strength. There is one trial from 2004, which has shown some benefit in a limited number of individuals with SMA. It needs to be formally trialled. The biggest side effect from that is tremor. So there are two simple medications here that are available here that could be used.

Kate: This is a summary of drug trials. I don’t know whether you’ve got this anywhere in your files. It’s something that I got off the internet and basically this talks about every single drug that’s in trial as at March 2006 and what stage they are at.

Miriam: And if you are thrown that clinical trials page and you can update that like every month

Kate: Yes that’s true - some of them are written up and some of them are not but I just thought you might like the file.

Rakesh: You don’t have salbutamol. Did you miss that paper? I should have it printed out in full. It’s only 2 years ago it was published

Graham: They want to know how to get it by the way.

Rakesh: Who’s your neurologist?

Wai: We don’t have one.

Rakesh: OK, Where do you guys live?

Wai: I live on the Shore and Kerry lives in South Auckland.

Rakesh: OK, same people will be involved. You need to see Richard Roxburgh. Get your GP to refer you to Richard Roxburgh or David Hutchinson. I’ll give you their names. They are both neurologists who do neuromuscular work. Richard is more neurogenetic and David is more neuromuscular, but yes, they should be watching you on a yearly or two-yearly basis.

Judy: So they do the same job as you but for adults?

Rakesh: Yes, they need to see an adult neurologist.

Wai: Are they from Auckland Hospital?

Rakesh: They are from Auckland Hospital. They work at North Shore as well as South Auckland, at Middlemore. You can ask to go through them, or a physician rather than your family doctor, but if you guys are keen and you don't get to see them, then you get your family doctor to ring one of us. The problem is that you fall outside of the paediatric age range. – Just!

Arran: Adrian is our children's neurologist. How long have you guys been trialling this?

Rakesh: We've only been saying we'll give it a go, if the parents agree, over the past year, not very long at all. I'll talk to Adrian anyway.

Arran: If I hadn't come to this meeting, I never would have known.

Rakesh: Exactly, that's why we are having the meeting – Advocacy. You guys would have to agree on trying the medicine, OK, because it will be off-label. The medicine salbutamol's first use is for asthma and Epilim is used for epilepsy, so you will be off-label.

Arama: It stimulates the SMN2 gene.

Rakesh: Salbutamol is thought to increase muscle bulk – it's a beta2 adrenergic receptor agonist and valproic acid is thought to stimulate SMN2 gene expression – a bit like Hydroxyurea and Phenylbutyrate.

Miriam: What about Carnitine?

Rakesh: That's an American thing. Monique is probably advocating that because she trained in America. Up to you guys.

Judy: But what's that, sorry?

Rakesh: With valproic acid, the Americans think that it depletes Carnitine, whilst the Australians, New Zealanders and English/Europeans don't believe that, so it depends where you were trained in what you recommend. We certainly don't supplement with Carnitine in epilepsy, where we are using valproic acid in much higher doses.

Miriam: We were just saying as well that Monique thought it helped with the shuttling and she seemed to think that kids with SMA were probably deficient in Carnitine naturally.

Rakesh: We can always do a measurement – that's an easy assay.

Miriam: We did some measurements and it was within the normal range. But valproic can deplete the levels of Carnitine very quickly.

Rakesh: Well, we'll do a repeat level whilst on valproic and see what it does. You can try supplementation with Carnitine. Carnitine as a medical product is expensive, but you can go to a health food shop or a body building shop and L-carnitine is quite cheap. It comes in big tins but is probably not as good.

Miriam: Yes, she thought that the quality was not very good – it's unregulated even.

Rakesh: It's unregulated, yes. I don't know if we would be able to get it through special authority. Carnitine, I think you have to get it specially approved. I'd have to check with Callum – Callum is our metabolic physician.

Judy: I think Wai and Kerry had some more questions.

Kerry: What about nutrition? Is there a recommended diet?

Rakesh: OK, there's a balance you have to keep against strength and weight gain, OK? So, have you guys seen a dietician first?

Kerry: Years ago

Kate: To be honest, when Natalie saw a dietician, it wasn't much use.

Rakesh: Yes, it's a balance between gaining strength and putting on fat and too much mass. I'd have to look into that as to what to recommend. I wouldn't say to go and eat Macdonalds, put it that way. As long as you have a balanced healthy diet at the moment, that's important.

Judy: The dietician thought Natalie should be on some extra protein, but the drink they would have prescribed for her had Casein in it and I gather that most SMA children have a problem with Casein.

Graham: They've got intolerance to it.

Rakesh: Yes, I think there's some, not all.

Judy: And to get a drink, an equivalent drink, which is Casein free, you can't get it funded, so you have to buy it yourselves. But would you say that protein levels need to be supplemented

Rakesh: I think a good healthy diet is important.

Tracey: We always used to say try to keep your protein intake up and your carbohydrates and fats at a healthy level, so you fill up on chicken and that sort of thing, rather than filling up on lost calories, in bread and carbohydrates.

Wai: Exercise. I am trying Pilates, which seems to help a little bit with breathing, but it's very expensive. We were just wondering if there is funding around this.

Rakesh: Uum, I'm just thinking. I'm sure there would be, there was somebody who got it done. I need to check on that

Tracey: In the UK we used to be able to get exercise on prescription, but I don't know about here.

Rakesh: Should be able to.

Miriam: I think when kids are at school they can get it through some sort of funding, but not when you get to adults.

Rakesh: For these guys there must be something.

Wai: With nutrition, we don't really know where to go. With a lot of dieticians, they don't understand our condition, so they may not give accurate advice.

Rakesh: Let me talk to Richard and David and we should start doing something for you guys as well. Surely there are many more adults with SMA than just you two.

Wai: Yes, we've got a few friends with SMA who want to know how we maintain our health.

Rakesh: Yes, exactly, and somebody who coordinates your care with your orthopaedic surgeons and neurologists.

Wai: We don't have a neurologist. Everything from Starship was taken away from us, so we're just sort of in limbo.

Rakesh: Yes, in limbo. OK, so I know Richard would gladly see you guys, that's not a problem. We just need to get your GPs to refer you guys or if you guys drop us an email, I could easily shoot off a letter to Richard. OK? That's not a problem. So even if you just drop me your names and dates of birth, I'll send a referral to Richard.

Wai: Do you work with Cas Burns?

Rakesh: Yes, Cas is the respiratory person. Does she still look after you?

Wai: She used to work very closely with me at Starship, but not anymore. I use the bi-pap during the night and when I was at Starship they used to check up on my breathing and things every year sort of thing, but after Starship we rarely get tested or looked after.

Rakesh: So you are looked after by the adult respiratory team?

Wai: Yes, but we don't get regular check-ups anymore.

Rakesh: So you can't be too sure if your ventilation is correct or not. It should be done on an annual basis at least. See, that's why we need to provide some sort of protocol – standards.

Tracey: They almost need to have transition clinics when they get to adolescence to make sure

Rakesh: A joint clinic would be good, wouldn't it. There's no transition team at the moment. We were in Melbourne just last week. They've just employed a transition nurse there for one year, just to see how things go. It's a big complaint.

Tracey: It's a big problem in the UK. We have just started doing it for neuromuscular patients and neurometabolic patients, where, when they got to a certain age, we discussed with them whether they thought they were ready to go to the adults and then we would have about two clinics where we would see them jointly. The adult consultants used to come over to the children's hospital to do the first clinic and the second clinic we would go over to the adults hospital, so that they would have two clinics where they had familiar faces before they then went on their own fully to the adult system. But it had only just started.

Miriam: Did it work well?

Tracey: It did work quite well, but we had only just started doing it. It carried on but we had only just started doing it and it had been a big problem before then that people were getting lost.

Rakesh: Certainly the adult neurologists here expressed an interest that we carry on with some of the other neuromuscular disorders into adulthood. Maybe we could expand that because it's a big complaint, isn't it. We in paediatrics do things quite differently from the adult physicians.

Wai: When we were Starship, when I got pneumonia and if I go into Emergency I have to show them a letter and they take me straight to a ward.

Rakesh: Exactly – open letter status.

Wai: I had an emergency a month ago and I had to wait in the emergency department for over 12 hours before I got transferred to the ward and they wouldn't start any medication and I had to tell them to start IV straight away, because they wouldn't do anything.

Wai's father: That is exactly right, because every time I take my daughter to Starship Emergency Department, still we have to wait there a couple of hours. Last time we were admitted at 11 o'clock at night and then until 4 o'clock in the morning until we were taken upstairs.

Arama: My paediatrician gave me a letter which stipulated what the problem was with Zane and how serious it was, so that if I had to go to ED we raced through that process, so I wouldn't have to wait.

Rakesh: Yes, open letter status. You've all got the open letter. Do you guys have any more questions – how many older individuals with SMA are there that you guys know of?

Wai: We have a group of friends in Auckland.

Kate: There's a real group of you around, about the same age as well, whereas then there doesn't seem to be as much of an active group outside that age group. I think because you are a similar age, you are more pro-active as well.

Rakesh: You can almost harness their energy can't you.

Graham: There's a girl of 6 with SMA III in Warkworth who has just got a wheelchair.

Rakesh: Yes I've seen that. She's under a general paediatrician. We don't have them all and there are some paediatricians who don't want to hand them to a paediatric neurologist.

Jessie: We have to go. Thank you. Lovely to meet you all and I'm sure we will be in touch by email. And the website's amazing, so well done.

Judy: Isn't it just

Kate: Well done, Tina.

End of meeting.

*We would like to thank Rakesh, Tracey and Miriam for the kind donation of their time in attending this meeting.*