Interview with Megan Fookes, Director, Rare Voices

08.01.2013

Tags: Rare Voices

Rare Voices Australia is a new organisation that has been established after the national rare diseases symposium in 2011. Can you please begin with giving our readers an introduction to Rare Voices and explain to our readers why the organization was needed?

It is estimated that there are disease community in Australia makes up approximately 8 percent of the total population.

The fact that we do not know the exact percentage is a clear sign that Rare Voices is needed. A lot has been going on around the world in the rare disease area. Some Australian states have had effective rare diseases and genetic peak groups, but it was unanimously decided at the first Australian National Rare Disease symposium that there was a need for a National Rare Disease Organisation. Australia needed one organization to represent the needs of all Australians living with rare diseases, regardless of age, background or where they live.

RVA is there for the bigger voices, the middle voices and the smaller voices from the loudest to the softest. Some rare diseases have better organized support groups, while other rare diseases only affect one or a very few Australians, these people live all over the nation, in the cities and in some cases in very isolated areas. Rare Voices Australia is a National organization and aims to educate the country on the impact of rare diseases and to communicate with the federal government. The wish for one voice also came from the federal health department; as they basically said, ‘We want to help, but in order to help as effectively as possible we need you to come up to us as one representative body for the whole sector rather than in various small groups. .’

What would you say are the most important issues that people with rare diseases are dealing with in Australia?

Getting a diagnosis is one major issue for many diseases They are all very different in their clinical presentation and in how they are managed. The wait for diagnosis can be anywhere from a period of months to 40+ years in some cases.

Coordination is another issue. The diseases are multi-systemic, so conditions affect more than one organ or system of the body. That in itself is complicated enough, and identifying a way of coordinating the clinical care and management and providing improved service in that area is long overdue.

For researchers who are trying to assist and do more work for the rare disease sector there is no framework to identify the rare diseases. As rare diseases are not listed as a key health priority, it is very hard for researchers to accurately direct their funding applications. This makes applying for the funding in order to research a specific rare disease extremely difficult. One thing that we are really trying to do at the moment is push for a national rare disease plan. Australia is one of the last developed nations to establish such a plan and to enact supporting policies.

How much of a step was the symposium towards establishing a national rare diseases policy?

Everybody was absolutely flabbergasted by the response. We had attendees from patients, patient support groups, and GPs, specialists, industry, policy makers; all stakeholders were present and all could see the gap that needed to be closed.

We had a lot of support from international equivalents – EURORDIS from Europe and the National Organization for Rare Disorders and Genetic Alliance from the US. They advised us on how to make sure that we get our voice heard and that we have our organization up and running. RVA has met and spoken with all of them and they are very supportive and engaging strongly with RVA to ensure the Australian rare diseases community is working with these key global communities and organisations An international agreement has been set up between all these companies on the ten top recommendations of what are the needs for the rare disease sector and how we can internationally link together and work as one.

We are already on committees with NHMRC and having conversations about genetic testing and the associated ethical issues In less than six months from founding Rare Voices Australia we have been able to make great steps in getting rare diseases on the agenda.

The Symposium registration session included the launch of ‘Rare Friends’, a non-partisan network of State politicians, formed to raise awareness. Could you tell us more about Rare Friends?

Rare Friends was initiated at the time of the Rare Disease Symposium in 2011 and was set up to identify politicians that were very passionate and personally touched by people with a rare disease. Somebody who has a personal connection generally has a deeper understanding of the issue and problems, of what it is like on a day to day basis to live with a rare disease. Rare Friends brought them together and is going forward to identify federal MPs that would fit into that category.

What is on top of your agenda today?

At the moment we are talking about building up our campaign in order for us to start advocating for a National Rare Diseases Plan what is on the draft of the national rare diseases plan that we have had input into the draft of this plan we hope to present to AHMAC early in 2013.

International Rare Disease Day is coming at the end of February. This day first took place five years ago, and in 2013 we will be holding a launch of Rare Voices Australia in at Canberra. MP’s will be invited and we look forward to introducing Rare Diseases to the Federal Parliament and raising the issues surrounding living with a rare disease.

Last year the theme was solidarity, working together as one. Although we have different diseases and problems, we also share a lot of challenges such as access to treatment and services. Rare diseases are complicated and often chronic diseases and 80 percent of them are genetic based. A good percentage of them are diagnosed in childhood but often diagnosis is delayed.

The pharmaceutical industry is trying to regain trust of the public, and many of our interviewees indicate that cooperation with patient and disease organizations is getting more important. How do you see cooperation between big pharma and patients group?

There is a perceived mistrust against the pharmaceutical industry. What is unique about rare diseases and the pharmaceutical industry which is that they have had to work hard to establish and maintain relationships between the support groups and the companies. This has been necessary in order to advocate for new therapies and get those treatments over the line.

Innovator association Medicines Australia has actually put together some fantastic documents that highlight the need for executing such cooperation under an agreement identifying commonalities. Industry obviously has to make a profit while patient organizations are not for profit, but it is possible to make an agreement to bridge these differences and allow them to work together.

An example is how the Fabry Support Group Australia (www.fabry.com.au) has been working with the pharmaceutical industry for the past 10 plus years.

Could you give examples of cooperation between Rare Voices and the pharmaceutical industry?

Many Pharmaceutical Companies have been extremely supportive of Rare Voices including; Genzyme, Shire Australasia and Novartis. Many other industry companies have been approached including; Pfizer, Bayer, Roche, UCB – over 25 companies in total.

Rare Diseases UK deals with the various companies as a reference group in a system that deals with them as one through rotating meetings in which everyone puts in support towards projects, so no one can have more say than another. We are setting up a similar system in Australia and look forward to working collaboratively in a similar fashion.

Do you feel that the level of commitment of industry is at a high enough level?

Industry involvement differentiates depending on their portfolio. Some companies have very targeted therapies and have been involved in the rare disease space for some time. They have established relationships and understand the need for collaborative relationships with rare disease community

A challenge many companies face with some of the treatments that have been funded under the Lifesaving Drug Program is that they have to provide proof that a rare diseases drug is lifesaving. For this proof the government asks for large cohorts of patients with data that cannot be produced in the rare disease sector. We therefore need some kind of agreement to protect so-called orphan drugs.

At the moment the orphan drugs listed for reimbursement is pretty minimal. The worry is that lots of companies come to Australia and feel that it is too hard to receive funding here and therefore decide to stay away altogether.

To which extent is Rare Voices already recognized by the industry as the player to look at?

RVA is already recognised by the pharmaceutical industry as the player to look at and collaborate with. RVA has been working hard to establish itself as a formal company and build its website and employ staff and this all takes a lot of hard work time and effort. 2013 is promising to be a fantastic year for RVA as many international Rare Disease Organisations are reaching out to RVA and as the ‘word gets out industry are fast approaching RVA with interest in learning more and offering their combined support.

What is your personal mission behind your involvement in Rare Voices?

Personally I would like to see that, when a person gets diagnosed with a rare disease, they can easily and quickly find a place to go for support, information and the health services they need Speed is crucial with any disease, but especially with rare diseases there is a lot to be won in this regard. Sometimes it is even a matter of hours.

My father was diagnosed at the late age of 48 – it took him 48 years to get diagnosis of his rare disease, and there was absolutely nothing written about the disease. He actually invited an American researcher to come to Australia to do a presentation, and together they raised a lot of money and funded a research student to do a paper on the particular disease.

Patients can be empowered to do a great deal of things, and it is amazing what can be done if we unite. Giving back to the patient community, the empowerment and the feeling of being in control are all crucial factors.

Most GPs would never have heard of the different diseases, and the patients often are the experts. Indeed we encourage people with rare diseases to be as well informed as possible about their condition. Going forward we can utilize modern technology and capitalise on the technological advances of our day and age? Innovation is a top value and priority to RVA.

How will you measure success four years from now?

Australia is the only developed country that does not have a national policy for rare diseases, and RVA is looking to having this implemented as soon as possible.

Rare Voices would like to have state branches as well as its national office. The aim is for someone who is diagnosed with a rare disease to know straight away where to go. Life will be a lot easier for patients and carers. It will not be such a maze to find out where to turn for support. There are models in existence in other parts of the world and it is just a matter of picking the most suitable one and adapting it to the Australian reality!

See more interviews