

### **2014 Projects, Events, Engagements**

The following projects, events have been executed and reported;

1. Rare Disease Day 28 Feb
2. Parliamentary Event 6 March
3. National Roadshow
  - QLD - Wesley Hospital Brisbane 27-28 Feb
  - VIC - Children's Murdoch Institute Melbourne, Vic 30 April
  - SA / NT – Muscular Dystrophy Office, SA 20 August
  - NSW – TBC, 29 October
4. RVA Website – Upgrade (Stage 2)
5. The Australian Experience of Living with a Rare Disease publication

### **ONGOING PROJECTS**

#### **International Partnerships**

##### **APARD (Asia Pacific Alliance Rare Disease)**

Singapore meeting held in 2012 (attended by L Murphy of RVA), 2014 – no invitation received. Sponsor – Novartis. Richard of Rare Cancers Australia and RVA gave a presentation from both organisations.

A paper has been drafted highlighting the formation of the group. Based in Singapore headed up by someone with little experience in Rare Disease.

Megan would like to attend next meeting at IRDiRC in China – Nov (pending funding). Have approached Alexion for funding to attend. Awaiting response.

Countries included in APARD – Australia, New Zealand, Taiwan, Thailand, Indonesia, Hong Kong, Philippines, Malaysia

##### **Indian Rare Disease Patient Organisation**

Approached RVA via EURORDIS contact. Met via Skype. Are interested in RVA presenting at their conference in Sep 2014 in India. Awaiting detail.

##### **EURORDIS**

1. ECRD Conference- Berlin, 8-10 May (**withdrawn**)
2. EURORDIS Summer School Barcelona (**withdrawn**)

Membership – RVA is not formally recognized at this point as the Australian national rare disease alliance. Need to clarify this further.

##### **RDUK**

Parliamentary Event / meeting / international mentor  
Scotland announced its Implementation Plan for Rare Disease  
Wales – Implementation strategy  
Ireland – National Plan

##### **NZORD**

Pharmac / PBAC / LSDP  
App / Registry / Orphan

### **Australian Partnerships / cross-border partnerships**

Rare Disease Survey – adults

- GaRDN, GSNV, AGSA, RVA, OPHG – partnership
- Draft complete
- 2 meetings
- Ethics
- Survey will be disseminated from

### **The Gay'wu (Dillybag) Women's Program**

- Carers Australia put forward M Fookes to attend The Gay'wu (Dillybag) Women's Program
- 8 women leaders from Telstra Australia, Anglicare, ACCAN, ACOSS, Financial Counselling Australia and RVA attended. Was funded by Telstra.

### **Launch of 'The Australian Experience of Living with a Rare Disease' booklet**

- NSW, Patron event – venue/ date TBC
- Female Patron – Dame Quentin Bryce – having trouble locating her contact details since moved. Need some support here.

### **MEETINGS:**

#### **RVA Pharmaceutical Industry Round Table Meetings**

- Shire 22 May – Economic study / LSDP review
- Genzyme 11 Sep
- 13 Nov – venue TBA
- New member – CSL (Melb)

#### **Federal Committees**

LSDP (Life Saving Drugs Program) Review

- 18 months
- Lesley Murphy – RVA rep

#### **Federal Dept of Health**

RFQ (Request for Quote) details coming to prepare additional piece of work to inform the Review – formal report from the consumer perspective relating to two terms of reference

- a) Establish a framework for data collection on rare diseases in Australia and assess how this could function internationally
- b) Compare the subsidization and equity principles of the Pharmaceutical Benefits Scheme (PBS) and the LSDP (Life Saving Drugs Program)

Item a) will contribute to work being undertaken by the external contractor.

Item b) RVA's report will be provided to the Reference Group for their consideration, particularly in relation to equity principles.

The Dept will fund RVA to undertake the work – to ensure perspectives of a broad range of people with a rare diseases are included in the report.

### **Scientific Medical Advisory Committee (SMAC)**

The National Rare Disease Co-ordinating Committee (NRDCC) Chair; Prof Hugh Dawkins and Vice Chair; Prof Elizabeth Elliott and RVA Ex Director; Megan Fookes – met by TC with Facilitator Lisa Adams. Originally this was proposed and invited to be a face to face meeting – but due to inability to attend, was adjoined to a small steering group to draft a proposal for the NRDCC to consider. Proposal from APSU – to reformat the NRDCC and call SMAC of RVA. Terms of Reference being considered 18<sup>th</sup> July. Megan to update at strategy day.

### **Other Meetings**

- Ogilvy
- APSU
- AWCH
- Missing School
- GSNV
- AGSA/RDUK
- PWC
- Rare Cancers Australia – 2014 partnership - cancelled
- Variety NSW / Genzyme – Rare Disease Day 2015 and beyond
- Dept of Health – Fed / LSDP Review briefing
- Minister of Health Qld
- Federal Treasurer – Joe Hockey
- Medicines Australia – Code of Conduct Review
- OPHG – Projects Evaluation / reports
- Dept of Health – Office for Health & Medical Research – Dr Antonio (Tony) Penna / OPHG meeting – talking to collaborations for rare disease work
- Jill Hall MP (Newcastle) – Deputy Chair Standing Health Committee – update / bi partisan support for National Rare Disease Registry
- Hunter Medical Research Institute
- CEO- Medicare Locals Hunter Region
- Steve Waugh Foundation

### **ADVOCACY**

#### **Parliamentary Friends**

- late 2014 / early 2015 TBC

#### **Research Australia**

- Restating and Centralising the Special Conditions for Tax Concession Entities submission (joint submission)

#### **Research Australia / Philanthropic Australia Conference – Melbourne (18-19 August)**

- Invited to sit on a panel representing RVA

## **EVENTS**

### **National Clinicians Forum – NSW / Integrating Care 18 June**

- Megan attended

### **Consumer Health Forum – Health in a Time of Change – 18-19 June / Melbourne**

- Megan attend 18<sup>th</sup> June
- Jenny attend 19<sup>th</sup> June

### **Nepean Disability Expo – 12-13<sup>th</sup> Sep / Penrith Panthers**

- RVA sharing a stand with ARCAN

### **National Press Club – 19 March**

- Megan and Lesley attended (supported by Medicines Australia and GSK)
  - Dr Martin Cross; Medicines Australia Chairman
- 'Timely access to affordable medicines for all Australians - Certainty the key to the future.'

### **National Press Club - Simon McKeon review on Wednesday 11th June.**

- Rebecca and Megan were invited by Research Australia and sat on Ogilvy's table

### **Australian National University – Australian Phenomics Facility**

- Meeting with Director, Edward Bertram
- Presentation to Staff by MF
- Tour of mouse model facility
- Invitation to attend Symposium (10 yr Anniversary) at the Aust. Academy of Science, 20<sup>th</sup> Oct. Prof Bruce Beutler – (Nobel Prize in Physiology 2011). There will be funding for RVA and a cheque presented at this event.

### **Medicines Australia Ball**

Megan and Lesley attended (supported by Medicines Australia)

### **TGA Anniversary – Parliamentary Event**

Megan attended at Parliament House

### **Post Budget Breakfast seminar – Peter Dutton Minister of Health and Minister of Sport**

- Megan attended sitting at Ogilvy table

### **Lysosomal Diseases Weekend Conference – Sydney**

- Megan attended
- Supported by Genzyme Australia
- Attended full weekend conference and gala dinner

### **HGSA Workshop - National Health and Medical Research Council – 20 March 2014**

- Translation of Omics-Based Discoveries into Clinical Research and Practice
- Megan and Lesley attended

### **Combined RVA / AGSA / GSNV / GaRDN meeting**

- Hosted by RVA by TC – attended by AGSA, GaRDN – apologies from Louisa GSNV
- Talk about the need to support patients/families impacted by rare disease and how best to support them and refer to services.

### **Publications**

- RVA E News – Feb, March, May (will be bi-monthly rather than monthly – due to resourcing constraints)
- Health Matters – Consumer Health Forum (Booklet stories)
- Research Australia – Grass Roots Autumn edition (LM author)
- ACMSR – Australasian College of Medical Sciences and Research Newsletter
- Source Kids – double page colour article on Parliamentary event 2014
- Pharma Dispatch – RVA being recognized as National Rare Disease Alliance – Jill Hall MP / Hansard report 14 July 2014

### **Media**

See RVA website <https://www.rarevoices.org.au/> – too much to list.

### **Marketing / PR / 2015 Rare Disease Day Event**

University of Western Sydney - Dr Gwyneth Howell, Senior Lecturer, School of Humanities and Communication Arts – details forthcoming.

### **CRM – Salesforce**

- Rebecca trained
- Complete July

### **Rare Disease Registry – Wellcome Trust Pathway Award (Partners; RVA, Murdoch University, Shire Australia)**

- See attached document
- Steering Committee formed 17<sup>th</sup> July

### **Social Media Report - Amy**

#### **RVA Internal Policies**

- Ethical Collaboration Policy – Awaiting Board Approval
- Communication Policy
- Conflict of Interest Policy
- Membership Policy
- Privacy Policy
- RVA Internal Financial Policy
- RVA Travel & Subsistence Policy
- Social Media Policy
- Working with Pharma Industry Policy
- External fundraising policy

### **Strategy Day**

- Media Strategy
- Long term funding strategy
- Relationship Map
- Partnership Strategy
- Revise membership policy

### **2015 Focus / Projects - TBC**

1. Implementation Strategy – National Plan
2. Parliamentary Event (3)
3. Launch Parliamentary Friends – RVA
4. RVA Young Ambassador Program
5. Rare Friends (Facebook)
6. RVA Conference / Seminar for members / supporters – Sydney
7. LSDP Review
8. Website – personal stories / videos (Stage 3)
9. Rare Disease Day – Australia's response
10. National Rare Disease Registry – Pathway Award (Wellcome Trust), Murdoch Uni WA, Shire, RVA – partnership
11. Rare Memorial Garden, 2016+