RVA Rare Disease Summit Program

‘A National Plan for Rare Diseases – Driving Collaboration, Driving Action’

The overarching goal of a National Plan is to drive coordinated and collaborative action to address important, common needs and concerns of people living with a rare disease.

The Rare Disease Summit brings together opinion leaders from patient, healthcare, research, government and industry organisations to share knowledge and insights for future action.

OBJECTIVES

1. Engage with opinion leaders to progress a National Plan for Rare Diseases. Achieve balanced participation from patient, healthcare, research, government and industry organisations.
2. Share knowledge and insights on important initiatives to achieve better health care for people living with a rare disease.
3. Create a national platform for ongoing dialogue and collaborative action on rare diseases.
4. Develop and endorse a Summit Communiqué to drive collaborative and coordinated action on rare disease and to progress a National Plan.

BACKGROUND AND SCOPE

RVA National Roadshow

Rare Voices Australia undertook a national Roadshow in 2014 to progress a national plan for rare diseases. The Roadshow involved round table discussions with patient, healthcare, research, government and industry stakeholders, with sessions in Queensland, Victoria, South Australia, New South Wales and Western Australia. The discussions focussed on the need, barriers and drivers for coordinated action on rare diseases and for a National Plan.

The key findings from the Roadshow, which will be presented at the Rare Disease Summit, have determined the Summit focus and themes.

Summit focus and themes

The focus is peoples’ experience of the health care system across Australia. The desire is for more equitable access to appropriate services, and to narrow the gap between how people with a rare disease experience the health system compared to people who also experience complex but more common health conditions.

Presentations and workshop sessions will cover four themes with a focus on principles and practical pathways to coordinate effort and collaborate, and the implications for a National Plan.

a) Data collection and use

i) What existing and emergent models and tools for data collection and use are meeting the particular needs of people living with a rare disease?
ii) What are the gaps in data collection and use for people with a rare disease compared to other complex, chronic conditions? What feasible approaches will narrow the gaps?
iii) What types of approaches would enable people living with a rare disease, clinicians, researchers, health service providers, government and industry to contribute to long term data collection on rare diseases, and derive benefits from its use?

iv) What principles and practical pathways will drive coordinated and collaborative action on data collection and use? What could people commit to?

b) Models of health care

i) What existing and emergent models of health care, and innovative approaches and tools, are meeting the particular needs of people living with a rare disease?

ii) What are the gaps between how people living with a rare disease experience the health system compared to others with complex, chronic conditions? What types of approaches will narrow the gaps?

iii) What principles and practical pathways will drive collaborative effort towards these models and approaches? What could people commit to?

c) Equitable access to diagnostics, therapeutics and services

i) What are the gaps experienced by people living with a rare disease compared to other complex, chronic conditions? What approaches will narrow the gaps?

ii) How should the Australian healthcare system assess, value and deliver therapies used to treat rare diseases?

iii) What diagnostic, therapeutic and service models are meeting the particular needs of people living with a rare disease? How could these models be extended?

iv) What principles and practical pathways will drive collaborative effort towards implementing these models and approaches? What could people commit to?

d) Working towards a National Plan

i) What principles, pathways and commitments will drive collaborative and coordinated action towards better health care for people living with a rare disease?

ii) What is the most appropriate national mechanism, or mechanisms to promote and implement the principles, pathways and commitments?

A Summit Communiqué will be developed to communicate key messages, pathways and commitments to drive collaborative and coordinated action on rare diseases. Summit participants and rare disease stakeholders will be invited to endorse the Communiqué.

APPROACH

Day one of the summit will set the context for participant deliberation on day two. Day one consists of short presentations on each theme to share knowledge and insights on principles, models and approaches, and opportunities for future coordinated and joint action.

Day two of the summit will bring together stakeholders in sector specific and cross-sector groups to consider principles, pathways and actions for each of the four themes. Structured approaches to group discussion will be used to draw out and test ideas, and identify areas of consensus and points of difference.

The summit is very action focused and registered attendees MUST attend both days.
STEERING COMMITTEE

Rare Disease Summit 2015 – Steering Committee

Cameron Milliner – Shire Australia
Shelley Evans – Genzyme, A Sanofi Company
Professor Jack Goldblatt – Genetic Services, WA
Professor Jeff Szer – Melbourne Health
Nicole Millis – MPS & Related Diseases Society
Jenny Sturrock – PNH Support Association Australia
Megan Fookes – Rare Voices Australia
Rebecca Novacek – Rare Voices Australia
Lisa Adams – Independent Facilitator

SPONSORS

Rare Voices Australia would like to formally acknowledge and thank the following companies who have equally contributed as the Rare Disease Summit 2015 sponsors.

Alexion Australasia, Amicus Therapeutics, BioMarin, Genzyme (a Sanofi Company), Janssen, Pfizer, Shire Australia, Vertex.
### PROGRAM DAY ONE

<table>
<thead>
<tr>
<th>Item</th>
<th>Who</th>
<th>Time Allocated</th>
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<tbody>
<tr>
<td>Registrations Open:</td>
<td>Joanne / Rebecca</td>
<td>8:00am - 8:45am</td>
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<tr>
<td>Summit Open:</td>
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<tr>
<td>- Welcome /Introduce speaker</td>
<td>Megan Fookes</td>
<td>9:00am - 9:05am</td>
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<tr>
<td>- Guest speaker opening address</td>
<td>Senator Richard Di Natale</td>
<td>9:05am - 9:15am</td>
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<tr>
<td>Summit Overview</td>
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<tr>
<td>- Background on the National Plan and Summit objectives, approach</td>
<td>Jenny Sturrock</td>
<td>9:15am - 9:25am</td>
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<tr>
<td>and program</td>
<td>Lisa Adams</td>
<td>9:25am - 9:30am</td>
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<tr>
<td>SESSION 1: Insights on progressing a National Rare Diseases Plan</td>
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<tr>
<td>Session Chair: Alan Bittles</td>
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<tr>
<td>Drivers, Barriers and Opportunities</td>
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<tr>
<td>- RVA National Roadshow key findings (RVA)</td>
<td>Megan Fookes</td>
<td>9:30am - 9:45am</td>
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<tr>
<td>- Australian Rare Disease Survey – Children (Research)</td>
<td>Yvonne Zurynski</td>
<td>9:45am - 10:00am</td>
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<tr>
<td>- Australian Rare Disease Survey – Adults (Dept of Health)</td>
<td>Caron Molster</td>
<td>10:00am - 10:15am</td>
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<td>- McKell Institute ‘Funding Rare Disease Therapies in Australia’ Report</td>
<td>Sam Crosby</td>
<td>10:15am - 10:30am</td>
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<tr>
<td>Question and Answer session to Panel of speakers</td>
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<tr>
<td>Focus: Implications and opportunities for a National Plan</td>
<td>Alan Bittles</td>
<td>10:30am - 10:45am</td>
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<tr>
<td>MORNING TEA</td>
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<tr>
<td>SESSION 2: Models for data collection and use. How to better meet</td>
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<tr>
<td>rare disease patient needs.</td>
<td>Shelley Evans</td>
<td>11:15am - 12:15pm</td>
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<tr>
<td>Session Chair: Shelley Evans</td>
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<tr>
<td>Gaps, Needs and Opportunities</td>
<td>Michele Adair</td>
<td>11:15am - 11:30am</td>
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<tr>
<td>- Patient Perspective – Cystic Fibrosis Registry</td>
<td>Jeff Szer</td>
<td>11:30am - 11:45am</td>
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<tr>
<td>- Clinician Perspective –</td>
<td>Andreas Zankl</td>
<td>11:45am - 12:00pm</td>
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<tr>
<td>- International Perspective -</td>
<td>Marcus Klein</td>
<td>12:00 - 12:15pm</td>
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<tr>
<td>Question and Answer session to Panel of Speakers</td>
<td>Shelley Evans</td>
<td>12:15 - 12:45pm</td>
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<td>Focus: Models for data collection and use. How to better meet rare</td>
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<td>disease patient needs.</td>
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<tr>
<td>LUNCH</td>
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<td>12:45 - 1:20pm</td>
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### SESSION 3: Models of Care to better meet patient needs
**Session Chair:** Cameron Milliner

<table>
<thead>
<tr>
<th>Gaps, Needs and Opportunities</th>
<th>Christine Jeffery</th>
<th>1:20pm - 1:35pm</th>
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<tbody>
<tr>
<td>Patient Perspective – IDFA Model for patients</td>
<td>Carolyn Ellaway</td>
<td>1:35pm - 1:50pm</td>
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<tr>
<td>Clinician Perspective – Lysosomal care Westmead</td>
<td>TBC</td>
<td>1:50pm - 2:05pm</td>
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<tr>
<td>Department of Health Perspective - Industry Perspective –</td>
<td>David Kwasha</td>
<td>2:05pm - 2:20pm</td>
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**Question and Answer Session to Panel of Speakers**
*Focus: Models of Care to better meet patient needs*

| Cameron Milliner | 2:20pm - 2:50pm |

**Afternoon Tea**

| 2:50pm - 3:15pm |

### SESSION 4: Equitable Access to diagnostics, therapeutics and services.
**Session Chair:** Jeff Szer

<table>
<thead>
<tr>
<th>Gaps, Needs and Opportunities</th>
<th>Kathryn North</th>
<th>3:15 - 3:30pm</th>
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<tbody>
<tr>
<td>International Perspective – Murdoch Institute - Melb</td>
<td>Nicole Millis</td>
<td>3:30pm - 3:45pm</td>
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<tr>
<td>Patient Perspective - MPS Society access to treatments</td>
<td>Jack Goldblatt</td>
<td>3:45pm - 4:00pm</td>
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<tr>
<td>Clinician Perspective – Genetic Services, WA</td>
<td>Carol Wicking</td>
<td>4:00pm - 4:15pm</td>
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<tr>
<td>Researcher Perspective – Qld Rare Disease Research Unit</td>
<td>Tim James</td>
<td>4:15pm - 4:30pm</td>
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<tr>
<td>Industry Perspective – Medicines Australia</td>
<td>Jeff Szer</td>
<td>4:30pm - 4:50pm</td>
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**Question and Answer session**
*Focus: Equitable Access to diagnostics, therapeutics and services*

| Facilitator – Lisa Adams | 4:50pm - 5:00pm |

**Preparations for Day 2**
Briefing to prepare for work streams on day two

| TBC | 5:15pm - 6:30pm |

### Summit Networking Event
**Guest Speaker:** Minister or Shadow Minister of Health

**PROGRAM DAY TWO**

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<thead>
<tr>
<th>Item</th>
<th>Who</th>
<th>Time Allocated</th>
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<tbody>
<tr>
<td>Day Two Open: Chair: Jack Goldblatt</td>
<td>Thomas Lönngren (Former CEO of European Medicines Agency EMA)</td>
<td>9:30am - 9:40am</td>
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<tr>
<td>Opening Address – Guest Speaker Thomas Lönngren</td>
<td>9:40am - 10:00am</td>
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<tr>
<td>Overview of work plan for day two</td>
<td>Facilitator (L Adams)</td>
<td>10:00am - 10:10am</td>
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First Concurrent Session:

i. Approaches to data collection and use that better meets the needs of people living with a rare disease

ii. Approaches to coordinated care that better meet the needs of people living with a rare disease

iii. More equitable access to diagnostics, treatments and services across states and territories

Working groups will review identified gaps and case studies and identify practical pathways to drive coordinated and collaborative action in each of the following areas

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<tr>
<th>All</th>
<th>10:15am - 11:00am</th>
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Morning Tea

11:00am - 11:25am

Second Concurrent Session:

i. Approaches to data collection and use that better meets the needs of people living with a rare disease

ii. Approaches to coordinated care that better meet the needs of people living with a rare disease

iii. More equitable access to diagnostics, treatments and services across states and territories

• Formulating key messages and presentations

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<tr>
<th>All</th>
<th>11:30am - 12:30pm</th>
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Concurrent Session Reporting and Briefing

• Presentations from concurrent sessions

• Key messages / actions / commitments

Facilitator (L Adams) 12:30pm - 1:00pm

Lunch

1:00pm - 1:45pm

Session Three: Summit Communique

• Presentation and review

Facilitator (L Adams) 1:45pm - 2:30pm

Next Steps and Summit Close

• Outline process to progress issues;

• Action items and work parties to lead;

• Close summit

Jeff Szer 2:30pm - 3:00pm

3:00pm

** Disclaimer – the program is subject to change.**