### Preliminary Agenda

**DAY 0:** Friday, November 24 (4:30PM – 9:00PM MYT)

<table>
<thead>
<tr>
<th>Time</th>
<th>Program</th>
<th>Speaker(s)</th>
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<tbody>
<tr>
<td>4:30 – 5:30PM</td>
<td>APARDO Member Update Meeting</td>
<td>Dr. Durhane Wong-Rieger</td>
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<tr>
<td>6:00 – 9:00PM</td>
<td>Welcome Reception</td>
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**DAY 1:** Saturday, November 25 (8:00AM – 6:30PM MYT)

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<tr>
<th>Time</th>
<th>Program</th>
<th>Speaker(s)</th>
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<tr>
<td>8:00 – 9:00AM</td>
<td>Registration</td>
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<tr>
<td>8:30 – 8:45AM</td>
<td>Intention Setting and Daily Meditation</td>
<td>Malaysia Rare Disorders Society</td>
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<tr>
<td>9:00 – 9:15AM</td>
<td>Welcome Remarks Theme: The Rare Disease Patient Journey Empowering the Patient from Diagnosis to Policy</td>
<td>Dr. Durhane Wong-Rieger (APARDO)</td>
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<tr>
<td>9:15 – 9:30AM</td>
<td>Keynote Address: Advancing Rare Disease as a Key Priority in Universal Health Coverage in Malaysia</td>
<td>Dr. Zaliha Mustafa (Minister of Health, Malaysia)</td>
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<tr>
<td>9:30 – 9:40AM</td>
<td>Empowered Rare Disease Patient Advocacy</td>
<td>Ms. Nadiah Hanim Abdul Latif (Malaysia Rare Disorders Society)</td>
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<tr>
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| 9:40 – 10:45AM | Large Group (45 min)  
An Ideal-but-Realistic Shared Vision of Asia-Pacific Rare Disease Network — 7 Years from Today  
1. Why should governments invest in rare disease and what kinds of “Return on Investment” will be meaningful and be measured?  
2. How will rare diseases be diagnosed and how will this look across the region?  
3. How will optimized “shared-care” pathways be developed and put into practice across the region?  
4. What does “good” access to treatment look like? What and how should essential drugs be available? How should innovative drugs be accessible and affordable?  
5. What stakeholders will need to come together to build an AP Rare Disease Ecosystem and would effective partnerships look like?  
6. What does good support care for individuals and families look like?  
7. What are some “critical” rare disease policies to be adopted across the region?  
8. How will we know the Network Vision is Working?  
Panel & Participant Reflections (20 min)  
Should we (collectively) pursue this vision? Are these desired outcomes achievable? Who will benefit? What are facilitators and challenges? | Moderators:  
Ms. Monica Ferrie (Genetic Support Network of Victoria)  
Ms. Nadiah Hanim Abdul Latif (Malaysia Rare Disorders Society)  
Dr. Durhane Wong-Rieger (APARDO & Rare Disease International)  
Panelists:  
Galen Centre for Health (TBC)  
Dr. Saumya Shekhar Jamuar (SingHealth Duke-NUS Genomic Medicine Center)  
Dr. Duangrurdee Wattanasirichaigoon (Mahidol University & Thai Rare Disease Foundation)  
Ms. Preeya Singhnarula (Thai Rare Disease Foundation)  
Dr. Rachel Yang (China Alliance for Rare Diseases)  
Ms. Anne Sophie Chalandon (Sanofi)  
Ms. Alexandra Huember (RDI) |
| 10:45 – 11:15AM | Morning Tea Break & Media Briefing |                                                                                                                                                                                                               |
| 11:15AM – 1:00PM | Collaborative Approach to Shorten Diagnostic Odyssey  
Applying "Lessons Learned" to "Challenges" Along Diagnostic Journey (large groups) (60 min)  
• Family perspective –Lived experience of the journey to diagnosis – pain points along the way (10 min)  
• Lessons learned: awareness and education  
• Lessons learned: screening and diagnosis  
• Lessons learned: family counselling  
Applying Learnings: Simulation Case (Small groups) (30 min)  
• Changes in practice in getting to diagnosis  
• Changes patient/family participation in process of getting  
• Changes in healthcare provision  
Feedback (15 min) | Moderators:  
Ms. Nadiah Hanim Abdul Latif (MRDS)  
Malaysian Patient Family  
Mapping lessons learned along diagnostic pathway  
Dr. Yin-Hsiu Chien (HCP)  
Ms. Jasmine Goh Chew Yin (KK Women's and Children's Hospital Singapore)  
Ms. Sook Yee Yoon (Genetics Counsellor) |

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<td>1:00 – 2:00PM</td>
<td>Lunch and Networking</td>
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<td>2:00 – 3:00PM</td>
<td><strong>Diagnosis</strong>&lt;br&gt;Presentations: What are the tools and technologies that improve pathways to diagnosis?&lt;br&gt;&lt;br&gt;Screening and Testing – Updates in Practice&lt;br&gt;• Newborn Screening Innovations across APAC&lt;br&gt;• Carrier Screening&lt;br&gt;• Genetic and Genomic Testing&lt;br&gt;• Support along the way&lt;br&gt;&lt;br&gt;Panel: How will AP Network foster spread of lessons learned into emerging areas?&lt;br&gt;&lt;br&gt;<strong>Patient Journey: Pathways to Diagnosis and Beyond</strong>&lt;br&gt;Workshop: Patients and Health Professionals – Applying learnings with “deep dive” application to different conditions (3 rotations x 20 minutes)&lt;br&gt;• PKU (NBS, Diet &amp; their therapeutic interventions&lt;br&gt;• Prader Willi: Later childhood diagnosis, therapeutic interventions, progressive neurological and physical, behavioural issues&lt;br&gt;• SMA: Fast progression, therapies but costly and not easily administered&lt;br&gt;• Lysosomal Storage Diseases: Varied age/stage of diagnosis, treatments available but costly&lt;br&gt;• Hemophilia: Early diagnosis, treatment covered&lt;br&gt;• Cystic Fibrosis: NBS, onerous management regimen, treatments but costly&lt;br&gt;• Mayer-Rokitansky-Küster-Hauser (MRKH) syndrome, Kleinfelter syndrome: Invisible condition; later diagnosis affect reproduction&lt;br&gt;• Amyloidosis: Adult onset, often misdiagnosed, some treatments, varied access&lt;br&gt;&lt;br&gt;What are learnings on accelerating pathways to accurate diagnosis to care and treatment?</td>
<td>Moderator&lt;br&gt;Ms. Monica Ferrie (GSNV)&lt;br&gt;Mr. Will Greene (Roche)&lt;br&gt;Dr. Alison Archibald (Carrier Screening, Mackenzie's Mission)&lt;br&gt;Dr. Carmencita Padilla (MAHPS Professor and Chancellor, University of the Philippines Manila; Founding Chairman, Philippine Society for Orphan Disorders)&lt;br&gt;Philippines Patient Lead&lt;br&gt;<strong>Health Professional Leads</strong>&lt;br&gt;<strong>Patient Support Group Leads</strong></td>
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<td>4:00 – 4:15PM</td>
<td>Networking &amp; Tea Break</td>
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![Novartis](image2.png)
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<td>Feedback and Large Group Discussion</td>
<td>Moderator: Dr. Rachel Yang (CHARD)</td>
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<td>Questions: Why AP RD Network?</td>
<td>Panelists:</td>
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<td>Speed: How can AP Network shorten diagnostic journey with lessons learned?</td>
<td>Ms. Mary Wang (RDI)</td>
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<td>Scale: How can AP Network facilitate spread of shared learnings to other disease areas and countries where not yet available?</td>
<td>Dr. Damayanti Rusli Sjarif (SpA(K), Cipto Mangunkusumo National Referral Hospital, Indonesia)</td>
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<td>Sustainability: How can AP Network address physical, knowledge, and psychological barriers among HCPs and patients; how can AP Network improve government support and pharmaceutical investment?</td>
<td>Dr. Ngu Lock Hock (Clinical Genetics Consultant Kuala Lumpur Hospital)</td>
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<td>Ms. Doreen Tan (Sanofi)</td>
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<td>Ms. Nadiah Hanim Abdul Latif (MRDS)</td>
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<td>6:30PM</td>
<td>Reception: Networking with Sponsors and Supporters</td>
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### DAY 2: Sunday, November 26 (8:30AM – 3:30PM MYT)

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<td>8:30 – 8:45AM</td>
<td>Intention Setting and Daily meditation</td>
<td>MRDS</td>
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<tr>
<td>9:00 – 9:15AM</td>
<td>Highlights and Key Learnings from Day 1</td>
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| 9:15 – 10:30AM| Presentation: Innovative data platforms for patient powered databases and patient data platforms  
• Registries and platforms for submitting and collecting patient data  
• Japanese registries  
• MATRIX Patient Platform  
• Ownership, safety, and privacy protection  
• Registry to Natural History Mapping  
• Managing, accessing, and using data to monitor therapeutic interventions  
Discussion/Q&A | Moderator:  
Ms. Monica Ferrie (GSNV)  
Presentations (20 minutes each):  
Ms. Yukiko Nishimura (ASRiD)  
Mr. Jason Colquitt  
Ms. Andrea Rogers  
Dr. Rachel Yang (TBC)  
Discussion/Q&A |
| 10:30 – 11:00AM| Break                                                                   |                                                                         |
| 11:00 – 12:00PM| Access to Treatment – What are Rare Therapies?                          | Moderator:  
Ms. Mary Wang (RDI)  
Ms. Alexandra Huember (RDI)  
Dr. Rachel Yang (TBC)  
Pfizer/Alliance for Regenerative Medicine (TBC)  
Dr. Sophelia Chan (Hong Kong Children’s Hospital) |
| 12:00 – 1:00PM| Access to Treatment – Options for Funding                              | Dr. Durhane Wong-Rieger (APARDO & RDI)  
Panel Discussion  
Ms. Ratna Devi (DakshamA Health)  
Dr. Rachel Yang (CHARD)  
Harpreet Ram (Partnership for Quality Medical Donations)  
Fiona Tsai (Janssen) (TBC)  
Regulator/HTA Perspective (TBC) |

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![AstraZeneca](image1.png)  
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## Time MYT

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<tr>
<td>1:00 – 2:00 PM</td>
<td>Lunch Break</td>
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| 2:00 – 3:15 PM | Connecting to Care  
- Assessing socioeconomic impact of rare disease and value of diagnosis and treatment  
- Hong Kong Impact of RD on Patients and Families  
- IFPMA/RDI SE impact in LMICs  
- Role of Caregivers - Families, caregivers, healthcare professionals, community-based support  
- Role of RD Nurses  
- Patient Support Networks Mutual support models for building mental health, compassion, competence, confidence, and resilience in individuals and families | Moderator:  
Ms. Nadiah Hanim Abdul Latif (MRDS)  
Ms. Anne Sophie Chalandon (Sanofi)  
Ms. Jasmine Goh Chew Yin (KK Women’s and Children’s Hospital Singapore)  
Dr. Ritu Jain (DEBRA International & APARDO)  
Dr. Claudia CY Chung (Hong Kong Genome Institute) (TBC)  
Wira Sudepta - Dihan (Malaysia) |
| 3:15 – 3:30 PM | Conference Summary  
Next Steps & Closing Remarks | APARDO |