Asia Pacific Alliance of Rare Disease Organisations

Malaysian Rare Disorders Society

APARDO CONFERENCE

MALAYSIA

24-26 NOVEMBER, 2023

Venue: Pullman Kuala Lumpur City Centre, Malaysia

Preliminary Agenda

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

Time MYT	Program	Speaker(s)
4:30 – 5:30PM	APARDO Member Update Meeting	Dr. Durhane Wong-Rieger
6 :00– 9:00PM	Welcome Reception	

DAY 1 : Saturday, November 25 (8:00AM – 6:30PM MYT)

Time MYT	Program	Speaker(s)
8:00 – 9:00AM	Registration	
8:30 – 8:45AM	Intention Setting and Daily Meditation	Malaysia Rare Disorders Society
9:00 – 9:15AM	Welcome Remarks Theme: The Rare Disease Patient Journey Empowering the Patient from Diagnosis to Policy	Dr. Durhane Wong-Rieger (APARDO)
9:15 – 9:30AM	Keynote Address: Advancing Rare Disease as a Key Priority in Universal Health Coverage in Malaysia	Dr. Zaliha Mustafa (Minister of Health, Malaysia)
9:30 – 9:40AM	Empowered Rare Disease Patient Advocacy	Ms. Nadiah Hanim Abdul Latif (Malaysia Rare Disorders Society)

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Time MYT	Program	Speaker(s)
9:40 – 10:30AM	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? Discussion (10 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. Carmencita Padilla (TBC) Dr. Duangrurdee Wattanasirichaigoon (Mahidol University & Thai Rare Disease Foundation) Dr. Fuu-Jen Tsai (Taiwan Human Genetics Society)(TBC) Dr. Thong Meow Keong (Pediatrics and Clinical Genetics, University of Malaya Medical Center, Kuala Lumpur, Malaysia) Ms. Preeya Singhnarula (Thai Rare Disease Foundation) Ms. Cynthia Magdaraog / Ms. Jane Francisco Kochis (Philippine Society for Orphan Disorders) Korean Organization for Rare Diseases (TBC) Dr. Rachel Yang (China Alliance for Rare Diseases) Ms. Anne Sophie Chalandon (Sanofi) Ms. Alexandra Huember (RDI)
10:30 – 11:00AM	Morning Tea Break & Media Briefing	
11:00AM – 12:00PM	Collaborative Approach to Shorten Diagnostic Odyssey Applying "Lessons Learned" to "Challenges" Along Diagnostic Journey (60 min) • Family perspective –Lived experience of the journey to diagnosis – pain points along the way (10 min) • Network for Newborn Screening across 7,000 islands (15 min) • From Diagnosis to Specialized Care and Treatment (15 min) • Supporting the Family with Diagnosis (15 min) Discussion: How will AP Network share lessons learned?	Moderators: Ms. Nadiah Hanim Abdul Latif (MRDS) Malaysian Patient Family Dr. Carmencita Padilla (MAHPS Professor and Chancellor, University of the Philippines Manila; Founding Chairman, Philippine Society for Orphan Disorders) Ms. Jasmine Goh Chew Yin (KK Women's and Children's Hospital Singapore) Ms. Sook Yee Yoon (Genetics Counsellor)

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Time MYT	Program	Speaker(s)
12:00PM – 1:00PM	<u>Diagnosis</u> Presentations: What are the tools and technologies that improve pathways to diagnosis? Screening and Testing – Updates in Practice • Newborn Screening Innovations across APAC • Partnerships in practice: Newborn Screening in SMA , Carrier Screening • Next Generation testing: Genomic Sequencing Discussion: How will AP Network promote diagnosis across the region?	Moderator Ms. Monica Ferrie (GSNV) Mr. Will Greene (Roche) Ms. Tara Pensa (Novartis) Dr. Alison Archibald (Mackenzie's Mission) Dr. Claudia CY Chung (Hong Kong Genome Institute)
1:00 – 2:00PM	Lunch and Networking	
2:00 – 3:30PM	<u>Patient Journey: Pathways to Diagnosis and Beyond</u> Workshop: Patients and Health Professionals – Applying learnings with "deep dive" application to different conditions (3 rotations x 20 minutes) • PKU (NBS, Diet & their therapeutic interventions • Prader Willi: Later childhood diagnosis, therapeutic interventions, progressive neurological and physical, behavioural issues • SMA: Fast progression, therapies but costly and not easily administered • Lysosomal Storage Diseases: Varied age/stage of diagnosis, treatments available but costly • Hemophilia: Early diagnosis, treatment covered • Cystic Fibrosis: NBS, onerous management regimen, treatments but costly • Mayer-Rokitansky-Küster-Hauser (MRKH) syndrome, Kleinfelter syndrome: Invisible condition; later diagnosis affect reproduction • Amyloidosis: Adult onset, often misdiagnosed, some treatments, varied access What are learnings on accelerating pathways to accurate diagnosis to care and treatment?	Moderator Dr. Durhane Wong-Rieger (APARDO & RDI) Ms. Nadiah Hanim Abdul Latif (MRDS) Health Professional Leads Patient Support Group Leads

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Time MYT	Program	Speaker(s)
3:30 – 4:30PM	Feedback and Large Group Discussion Speed: How can AP Network shorten diagnostic journey with lessons learned? Scale: How can AP Network facilitate spread of shared learnings to other disease areas and countries where not yet available? 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?	Moderator: Dr. Durhane Wong-Rieger (APARDO & RDI) Ms. Monica Ferrie (GSNV) Panelists: Ms. Mary Wang (RDI) Dr. Damayanti Rusli Sjarif (Cipto Mangunkusumo National Referral Hospital, Indonesia) Dr. Ngu Lock Hock (Clinical Genetics Consultant Kuala Lumpur Hospital) Korean Organization for Rare Diseases (TBC) Ms. Doreen Tan (Sanofi) Ms. Nadiah Hanim Abdul Latif (MRDS)
4:30 – 5:00PM	Networking & Tea	
6:30PM	Reception	

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DAY 2 : Sunday, November 26 (8:30AM – 3:30PM MYT)

Time MYT	Program	Speakers
8:30 – 8:45AM	Intention Setting and Daily meditation	MRDS
9:00 – 9:15AM	Highlights and Key Learnings from Day 1	

9:15 – 10:30AM	Presentation: Innovative data platforms for patient powered databases and patient data platforms
	• Benefits and Risks of using real-world data & real-world evidence to optimize patient outcomes
	• Introduction to patient registries
	  • Japanese registries
	  • MATRIX Patient Platform
	
	Discussion: Issues & Q&A
	• Ownership, safety, and privacy protection
	• Registry to Natural History Mapping
	• Managing, accessing, and using data to monitor therapeutic interventions
		Moderator:
		Ms. Monica Ferrie (GSNV)
		
		Dr. Durhane Wong-Rieger (APARDO & RDI)
		Mr. Jason Colquitt
		Ms. Andrea Rogers
		Dr. Rachel Yang (CHARD)
		
		Discussion/Q&A

10:30 – 11:00AM	Break	
11:00 – 12:00PM	Access to Treatment – What are Rare Therapies? What are essential medicines for rare and orphan indications? What are emerging innovative therapies? • Essential medicines: IRDiRC/RDI list and WHO List of Essential Medicines • Cell and Gene therapies and other Advanced Therapeutic Products	Moderator: Ms. Mary Wang (RDI) Ms. Alexandra Huember (RDI) Pfizer/Alliance for Regenerative Medicine (TBC)
12:00 – 1:00PM	Access to Treatment – Options for Funding What are Financing Options for Timely, Affordable Sustainable Access? Presentations: • Patient Engagement in Healthcare Decision-Making across AP region: HTA and Beyond (15 min) Panel Discussion: • What are Innovation Opportunities for Improving Access to Rare Disease Treatments	Dr. Durhane Wong-Rieger (APARDO & RDI) Alison Keetley (Janssen) Harpreet Ram (GARDaccess) Dr. Ratna Devi (DakshamA Health) Dr. Rachel Yang (CHARD)
1:00 – 2:00PM	Lunch Break	

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Time MYT	Program	Speakers
2:00 – 3:15PM	Connecting to Care • Hong Kong Impact of RD on Patients and Families • Role of Caregivers - Families, caregivers, healthcare professionals, community-based support • Rare Disease and Stigma • Role of RD Nursess • 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) Dr. Claudia CY Chung (Hong Kong Genome Institute) Ms. Jasmine Goh Chew Yin (KK Women's and Children's Hospital Singapore) Dr. Ritu Jain (DEBRA International & APARDO) Dr. Dong Dong (Illness Challenge Foundation) Wira Sudepta - Dihan (Malaysia)
3:15 – 3:30PM	Conference Summary Next Steps & Closing Remarks	APARDO

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