Asia Pacific Alliance of Rare Disease Organisations

RARE DISEASES INTERNATIONAL

APARDO Virtual Conference 2021

Treating Rare Disease As Priority

DAY 1 : Tuesday, December 14 (9:00AM – 12:30PM SGT)

Time SGT	Program	Speaker
0900 – 0915	Welcome and Introduction	
Session 1 : Rare Disease Ecosystem from 2019 to 2021 and What Next?		Moderator/Chair: Durhane Wong-Rieger
0915 – 0945	Panel Discussion • How prepared are health, social, economic and political systems to support access to advanced diagnostics and therapy? • What was the status of system readiness and access up to 2019? • What changed as a result of the pandemic? • What needs to be done in 2022 and beyond to optimize access for all patients? • Patients perspective — how prepared are they?	Panellists: Yann LeCam (EURORDIS) (TBC) Lucia Monaco (IRDiRC) (TBC) Scott Williams (Sanofi Genzyme) (TBC) Rachel Yang (China Alliance) (TBC)
Session 2: Global Access to Essential Medicines for Rare Diseases: Implications for APAC		Moderator/Chair: Durhane Wong-Rieger
0945 – 1045	Panel Discussion • Upon validation of the essential rare disease medicines list by appropriate healthcare and patient experts, how could the list be used by healthcare professional and patient advocacy groups? • Only 25 of the 204 orphan and rare disease drugs are also on the WHO list of Essential Medicines. WHO recommends high-income countries make all essential medicines available to all citizens and middle-income countries work toward that goal. How much time and effort should (we) invest to getting these drugs on the WHO Essential Medicines list? • Not included in this list are the new cellular and gene therapies that require only a single administration to provide "long-lasting", durable, or even curative effect. However, they can only be administered and managed in certain sites with advanced technological capabilities; they also tend to be very expensive. Should these cell and gene therapies be considered essential therapies and, if so, for which patients? • What are the key challenges and barriers to access to essential orphan and rare disease therapies in your country? What are barriers to equitable "global" access, especially in LMICs? • What are initiatives, programs, and/or strategies for advancing global access to medicines, especially in LMICs?	Presenter: Durhane Wong-Rieger (President of APARDO) Panellists: William Gahl (NIH and Co-Chair Treatment Access WG) Choo Beng Goh (Takeda) Salome Mekhuzla (WFH Director of Global Development) Harpeet Ram (EVR Consulting)
1045– 1100	Break	

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Session 3: Leveraging Universal Healthcare to Advance Rare Disease Care in Asia-Pacific		Moderator: Durhane Wong-Rieger
1100 – 1200	Panel Discussion • How is healthcare financed in your country (taxation schemes, national health insurance, "sin" taxes) and how many persons are covered? • How sufficient are your health systems in terms of facilities, medicines, data systems, staff and volunteers? Which services (health promotion, prevention, rehabilitation, care) are delivered and to which communities? • Which stakeholders are engaged in health systems governance: (1) government; (2) public and private health service providers and professional associations; (3) civil society (patient associations, community service organizations, nongovernmental organizations, social protection agencies, and grassroots advocates)? • How does your country measure on three dimensions of UHC: who is covered; which services are covered; and what proportion of health costs are covered? • To what degree is there equity in health services (equal and fair access)? To what degree are "so-called" essential health services (main health needs of population) covered? Does "cost-effectiveness" (greatest good for the greatest number) used to determine health funding allocation? • How are rare diseases explicitly or implicitly included in all aspects of UHC? are there specific provisions for populations that are marginalized, disadvantaged, or excluded?	Presenter: Durhane Wong-Rieger (President of APARDO) Panellists: Monica Ferrie (Genetic Support Network of Victoria, Australia) Dr. Carmencita Padilla (University of the Philippines, Manila) Prof. Cherdchai Nopmaneejumruslers (Siriraj Hospital, Thailand) Dr. Phuoc Huy Do (Vietnamese Organization for Rare Diseases, Vietnam) Eric Obscherning (APEC Rare Disease Network) Yukiko Nishinura (ASrid, Japan) Karin Jaeger (Takeda)
1200 – 1215	Q&A	
1215 – 1225	Wrap Up	Durhane Wong-Rieger (President of APARDO)

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DAY 2 : Wednesday, December 15 (9:00AM – 12:30PM SGT)

Time SGT	Program	Speaker
0900 – 0915	Welcome and Review of Day 1	
Session 4 : Thriving with Rare Disease: From Emotional Stress to Emotional Well-Being		Moderator: Monica Ferrie (Genetic Support Network of Victoria, Australia)
0915 – 0955	Panel Discussion • What is your experience with rare diseases and how do you feel living with a rare disease impacts on patients' and caregivers' mental health? • What professional and other support services are available to patients and caregivers? Do you feel there is sufficient focus on mental health and well-being? • What are specific barriers to dealing with mental health in Asia-Pacific cultures? What are ways in which mental health can be successfully addressed? • What is the experience of mental health in your community or across rare disease communities? What are the key issues? • How has "research" on these issues and the data helped to engage other stakeholders? • Are there some quintessential "Asia-Pacific" ways of defining and responding to mental health challenges? • What are programs or strategies that increase collaboration in other disease communities or countries? Case Study: Ågrenska: National Centre for Rare Diseases (Sweden) • The Ågrenska Centre in Gothenburg, Sweden, provides support services to children with disabilities and their families; these services include a unique programme of family activities, respite services, education, information projects, and research with the aim to contribute to people´s coping with everyday life and empower them to become as independent as possible. • The benefits of the Ågrenska approach extend to healthcare savings, nearly a three-fold decrease in direct and indirect healthcare costs versus routine support services.	Panellists: Yap Sook Yee (We Care Journey, Malaysia) Yukiko Nishimura (Asrid, Japan) Jaime Christmas (New Zealand Amyloidosis) Christine Cockburn (Rare Cancers Australia) Presentater: Anders Olauson (Ågrenska, Sweden)
0955 –1005	Q&A	

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Session 5: Patient Voice in Drug Development and Access		Moderator:/Chair: Eileen Li (CORD)
1005 – 1045	Panel Discussion • How valuable is it to directly engage patients in drug research and development process? • What are challenges and opportunities for including patients in R&D in Asia-Pacific? • What is the importance of the support of medical professionals/ academia to achieve the ideal R&D opportunities for patient engagement? • Are there examples or best practices of patient engagement across the spectrum of R&D and access in AP region (or elsewhere)?	Presenter: Safiyya Gassman (Pfizer) Katherine Beaverson (Pfizer) Panellists: Ruth Chen (Taiwan Foundation for RD) Lisa Foster (Rare Disorders New Zealand) Fiona Wardman (Australasian Hereditary Angioedema) K.P. Tsang (RD Hong Kong) Jill Morjaria (IQVIA Asia Pacific) Eileen Li (Biogen)
1045 – 1055	Q&A	
1055 – 1110	Break	
Session 6: How Genomics is Transforming Rare Disease Diagnosis and Treatment		Moderator: Monica Ferrie (Genetic Support Network of Victoria, Australia)
1110 – 1155	Current landscape and future of genetic testing, diagnosis and treatment Panel Discussion • Advances in genetics and genomics as applied to diagnosis in rare diseases • Next-Generation genomic sequencing in rare diseases • Genetic-based therapies (gene replacement, gene manipulation, gene editing)	Presentation: Prof. Thong Meow Keong (University Malaya Specialist Centre, Malaysia) Panellists: Prof. Dr. Duangrurdee Wattanasirichaigoon (Ramathibodi Hospital, Thailand) Dr. Koh Ai Ling (KK Women's and Children's Hospital, Singapore) Edmund Lim (WeCare Journey, Malaysia) Dr. Melanie Alcausin (Institute of Human Genetics Philippines)
1155 – 1210	Q&A	
1210 – 1230	Wrap Up	Durhane Wong-Rieger (President of APARDO)

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