



AS EXECUTED

Asia-Pacific
Economic Cooperation

APEC Policy Dialogue on Rare Diseases

6-7 June 2018 | Beijing, China

Da Xue Tang Conference Center, The Lakeview Hotel, Peking University

Day 1 Wednesday, 6 June	
07:30-08:30	Registration
08:30-09:00	<p>Welcome and Opening Remarks</p> <ul style="list-style-type: none">• Prof. Zhouyi WANG, Deputy Director, Office of Humanities and Social Sciences, Peking University, China• Ministry of Education, China <p><i>Introductions</i></p> <ul style="list-style-type: none">• Prof. Matthew BELLGARD, Chair, APEC Life Sciences Innovation Forum - Rare Disease Network and Director of eResearch and Commercialisation, Queensland University of Technology, Australia• Dr. Li HE, Vice President, Chinese Pharmaceutical Association, China• Prof. Xiaoying ZHENG, Director, APEC Health Science Academy, Peking University, China
09:00-10:30	<p>Panel I: Setting the scene for Rare Disease issues in APEC Economies</p> <p><i>How can economies adopt an inclusive approach to “Healthy Asia Pacific 2020” implementation by addressing barriers to healthcare services for populations with rare diseases? How can APEC help facilitate greater alignment of domestic policies, implementation of best practices, and multi-sectoral collaborations?</i></p> <ul style="list-style-type: none">• Dr. Jakris EU-AHSUNTHORNWATTANA, MD, PhD, Lecturer, Department of Community Medicine, Faculty of Medicine Ramathibodi Hospital, Mahidol University, Thailand• Mr. Cameron MILLINER, Industry Co-Chair, APEC Rare Disease Network and Head Public Affairs and Patient Advocacy – Asia Pacific, Shire• Prof. Xinming SONG, Senior Advisor, APEC Health Science Academy, Peking University, China• Moderator: Jean-Luc LOWINSKI, PhD, MBA, Head of Emerging Markets, Sanofi Genzyme
10:30-10:45	Break
10:45-12:15	<p>Panel II: Determining and implementing a Rare Disease definition</p> <p><i>How can economies implement a definition with a clear objective criteria and quantifiable prevalence for what constitutes a rare disease, is not too restrictive on small populations, and remains in line with international standards?</i></p>



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	<ul style="list-style-type: none">• Dr. Durhane WONG-RIEGER, Chair, Rare Diseases International and President & CEO, Canadian Organization for Rare Disorders• Mr. Andrew MARTIN, Vice President, Marketing - Rare Disease: Latin America, Asia-Pacific, Africa/Middle East, Pfizer• Moderator: Prof. Matthew BELLGARD, Chair, APEC Life Sciences Innovation Forum - Rare Disease Network and Director of eResearch and Commercialisation, Queensland University of Technology, Australia
12:15-13:40	Lunch
13:40-14:00	<p>Day 1 Keynote Presentation – “Construction of the Clinical and Research System for Rare Diseases in China: Experience from Peking Union Medical College Hospital and National Rare Disease Registry System”</p> <ul style="list-style-type: none">• Dr. Mengchun GONG, Executive Director, National Rare Disease Registry System of China, China
14:00-15:30	<p>Panel III: Health system design to address the needs of the Rare Disease Community <i>How can economies integrate rare disease management into existing healthcare systems to ensure access to diagnosis and treatment for patients living with a rare disease. How should healthcare systems provide services and how should they be structured? How can economies establish centers of excellence that provide specialized care for rare diseases?</i></p> <ul style="list-style-type: none">• Mr. Luke Elias, Director, Business Analytics & Performance, Blacktown and Mount Druitt Hospitals, Western Sydney Local Health District, Australia• Mr. Hyun Min SHIN, Chairman, Korean Organisation for Rare Diseases, Korea• Mr. Kevin Huang, President, Founder & President, Chinese Organization for Rare Disorders, China• Moderator: Mr. Peter Fang, Head of Asia Pacific, Shire
15:30-15:45	Break
15:45-17:15	<p>Panel IV: Diagnosing Rare Diseases early, accurately, and systematically <i>What are APEC economies doing to improve diagnosis of rare diseases? What infrastructure needs to be in place to allow for genetic testing and diagnosis? What about newborn screening programs?</i></p> <ul style="list-style-type: none">• Dr. Duangrurdee WATTANASIRICHAIGOON, Professor of Pediatrics and Medical Genetics and the Chief of Division of Medical Genetics, Department of Pediatrics, Faculty



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	<p>of Medicine of the Ramathibodi Hospital, Mahidol University, Thailand</p> <ul style="list-style-type: none">• Dr. Eva Maria C. CUTIONGCO-DELA PAZ, Vice Chancellor for Research and Executive Director, National Institutes of Health (NIH), University of the Philippines - Manila, Philippines• Dr. Yin-Hsiu CHIEN, Department of Pediatrics and Medical Genetics, Taiwan University Hospital, Chinese Taipei• Mr. Andrew MARTIN, Vice President, Marketing - Rare Disease: Latin America, Asia-Pacific, Africa/Middle East, Pfizer• Moderator: Dr. Joan KEUTZER, Vice President and Head, Integrated Solutions, Rare Diseases Franchise, Sanofi Genzyme, Sanofi
17:15-17:30	Wrap-Up of Day 1
17:45-18:45	Reception



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Day 2 Thursday, 7 June	
08:30-08:40	<p>Recap of Day 1 and setting the scene for Day 2</p> <p>Prof. Matthew BELLGARD, Chair, APEC Life Sciences Innovation Forum - Rare Disease Network and Director of eResearch and Commercialisation, Queensland University of Technology, Australia</p> <ul style="list-style-type: none">• Prof. Xiaoying ZHENG, APEC Health Sciences Academy, Peking University, China
08:40-09:00	<p>Day 2 Keynote Presentation</p> <ul style="list-style-type: none">• Dr. Jie Ding, Chairman of the Rare Disease Branch of the Beijing Medical Association, Member of the National Committee of the Chinese People's Political Consultative Conference, and Professor in the Department of Paediatrics, Peking University First Hospital, China
9:00-10:15	<p>Panel V: Innovative financing for Rare Disease research, diagnosis, and treatment</p> <ul style="list-style-type: none">• Mr. Prasanna SHIROL, Co- Founder and Executive Director, Organization for Rare Disease India, India• Dr. Dino SEPÚLVEDA, Head of the Department of Evaluation of Health Technologies and Health Based on Evidence Division of Health Planning, Ministry of Health, Chile• Moderator: Dr. Vinciane PIRARD, Co-Chair, Joint Task Force on Rare Diseases and Orphan Medicinal Products, European Federation of Pharmaceutical Industries and Associations (EFPIA)
10:15-10:30	Break
10:30-12:00	<p>Panel VI: Accelerated regulatory pathways and pre-regulatory early access for Orphan Medicinal Products (OMPs)</p> <p><i>How can economies implement pre-regulatory early access programs and/or accelerated regulatory processes with clear eligibility requirements, applicability to all OMPs, and assurance of coverage support to fund medicines for patients?</i></p> <ul style="list-style-type: none">• Prof. John CW LIM, APEC LSIF Board Member, Executive Director, Duke-NUS Centre of Regulatory Excellence & Chairman, Singapore Clinical Research Institute, Singapore• Dr. Lahouari BELGHARBI, Director General, Centre of Excellence on Regulatory Sciences and Good Regulatory Practices, Federal Commission for the Protection against Sanitary Risk (COFEPRIS), Mexico• Kyu-Been SOHN, Asia Regional Regulatory Strategist, Pfizer



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	<ul style="list-style-type: none">• Moderator: Ms. Eileen ANG, Head of Regulatory Affairs – APAC, Shire
12:00-13:15	Lunch
13:15-14:30	<p>Panel VII: Value assessment process and funding for Orphan Medicinal Products (OMPs) <i>How can economies develop a flexible value assessment process to exempt OMPs from strict cost effectiveness analysis within certain thresholds, and ensure adequate funding for OMPs through clear and transparent reimbursement criteria?</i></p> <ul style="list-style-type: none">• Dr. Elena NICOD, PhD, Senior Consultant, Dolon Ltd; Co-Leader of IMPACT-HTA Work Package 10 on Appraisal of Orphan Medical Products, Bocconi University, Italy• Dr. Camila QUIRLAND LAZO, PharmD, MSc, Health Technology Assessment Unit Chief, Oncology Institute, Arturo López Perez Foundation Santiago, Chile• Ms. Julie KIM, Global Franchise Head, Hematology, Shire• Moderator: Prof. John CW LIM, APEC LSIF Board Member, Executive Director, Duke-NUS Centre of Regulatory Excellence & Chairman, Singapore Clinical Research Institute, Singapore
14:30-14:45	Break
14:45-16:00	<p>Panel VIII: Leveraging public-private partnerships for Rare Disease patient registries <i>How can economies implement centralized, digital disease registries that allow for better management of rare diseases in a larger population by gathering as much patient info as possible and linking together different regional or local databases?</i></p> <ul style="list-style-type: none">• Prof. Matthew BELLGARD, Chair, APEC Life Sciences Innovation Forum - Rare Disease Network and Director of eResearch and Commercialisation, Queensland University of Technology, Australia• Dr. Mengchun GONG, Executive Director, National Rare Disease Registry System of China, China• Dr. Joan KEUTZER, Vice President and Head, Integrated Solutions, Rare Diseases Franchise, Sanofi Genzyme, Sanofi• Moderator: Dr. Durhane WONG-RIEGER, Chair, Rare Diseases International and President & CEO, Canadian Organization for Rare Disorders
16:00-17:15	<p>Panel IX: APEC economy plans and strategies for Rare Diseases <i>How can economies develop comprehensive domestic plans on rare diseases that incorporate actionable strategies for policy development, prioritize key geographies and populations, and support implementation with financing provisions?</i></p>



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17:15-17:30	<p>Closing Remarks</p> <ul style="list-style-type: none">• Prof. Xiaoying ZHENG, APEC Health Sciences Academy, Peking University, China• Prof. Matthew BELLGARD, Chair, APEC Life Sciences Innovation Forum - Rare Disease Network and Director of eResearch and Commercialisation, Queensland University of Technology, Australia