Southeast Asia
RARE DISEASE
Summit 2022

Bringing Rarity to Reality

PROGRAM BOOK

1st Southeast Asia Rare Disease Summit 2022
[Virtual Event]

January 20-21, 2022

13:00 – 16:30hrs Bangkok Local Time

C-ANPROM/TH/RDM/0016: Jan 2022

WELCOME MESSAGE

Southeast Asia RARE DISEASE Summit 2022

Dear Participants,

It is our absolutely pleasure to e-welcome you to the inauguration of the 1st Southeast Asia Rare Disease Summit, taking place on January 20-21, 2022, from 13:00 hrs – 16:30 hrs Bangkok Local Time GMT+7.

Bringing Rarity to Reality is chosen to be the theme of the summit to resonate our commitment in building a better future for rare disease patients and community. The programmes in the summit are designed to have something for everyone who stand in solidarity for rare disease communities across the region to share, engage, connect, further the discussion, or establish initiatives that to improve the healthcare ecosystem for people living with rare diseases.

As the event is completely virtual, we are confident that it will provide you the same vibrant programming, impactful networking, and opportunities to reflect and connect over the topics in our rare disease community.

You won't want to miss the opportunity to e-connect with many like-minded individuals who share a united goal in enhancing a dialogue and promote policy changes to support the building of sustainable journey from diagnosis to access for rare diseases. Please do take an opportunity to visit virtual exhibitions from the host and co-hosts to add to your conference learning experience.

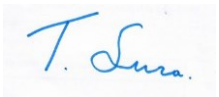
Fret not, if you are unable to join one of the sessions, the recordings of all sessions will be available for viewing until April 30, 2022, on the Southeast Area Rare Disease Summit 2022 platform. All event materials such as session recordings, virtual exhibitions, and publications will remain accessible to everyone registered for the event as well.

Many thanks go out to all participants who dial in regardless of your location, together we make new possibilities to rare disease communities.

Sincerely,

The Organizing Committee

The 1st Southeast Asia Rare Disease Summit



Associate Professor Thanyachai Sura M.D.,
President, Medical Genetics and Genomics Association

Associate Professor Dr. Budsaba Rerkamnuaychoke
President, Genetic Society of Thailand

Mrs. Preeya Singhnarula
President, Thai Rare Disease Foundation

Mr. Peter Streibl
General Manager of Takeda Thailand

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Southeast Asia RARE DISEASE Summit 2022

CONTENT

Southeast Asia RARE DISEASE Summit 2022

AGENDA

January 20, 2022

13:00 – 16:15hrs Bangkok Local Time

13.00 hrs – 13.20 hrs

Welcome Speeches
Peter Streibl
General Manager, Takeda Thailand
Prof. Dr. Thanyachai Sura
Co-host, President, APSHG, MGGA, Thailand

13.20 hrs – 13.40 hrs

Opening Remarks and Keynote Speech
Healthcare in Thailand: An Ambition to Achieve a Broader Medical Access for All
H.E. Mr. Anutin Charnvirakul
Deputy Prime Minister and Minister of Public Health, Thailand

13.40 hrs – 14.10 hrs

Plenary Session
The Exploratory Frontier for Diagnosis and the Innovative Future of Diagnostics
Sivasangaran Kumaran
Representative of Microsoft Corp and Rare Disease Advocate

14.10 hrs – 14.20 hrs

Break

14.20 hrs – 15.20 hrs

Panel Discussion 1
Redefining the Diagnosis
How Do Public Health Programs Successfully Support the Diagnosis of Rare Diseases?
Moderator:
Dr. Phichai Kanitcharaskul
Medical Affairs Head, Takeda Thailand

Panelists:
Prof. Dr. Kanya Suphapeetiporn
Chulalongkorn University, Thailand
Dr. Vu Chi Dung
Vietnam National Children's Hospital, Hanoi, Vietnam
Prof. Dr. dr. Damayanti Rusli Sjarif SpA(K)
Cipto Mangunkusumo National Referral Hospital, Indonesia
Prof. Yin-Hsiu Chien
National Taiwan University Hospital, Taiwan

15.20 hrs – 16.05 hrs

Panel Discussion 2
Empowering Patient Voices
Challenges in the Diagnosis Journey - Empowering Patients' Voices
Moderator:
Dr. Phichai Kanitcharaskul
Medical Affairs Head, Takeda Thailand

Panelists:
Preeya Singhnarula
President, Thai Rare Disease Foundation, Thailand
Raman Rajakanath
Programme Director, Rare Disorders Society, Singapore
Dr. Durhane Wong-Rieger
President, APARDO

16.05 hrs – 16.15 hrs

Summary and Closing of Day 1

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AGENDA

Southeast Asia RARE DISEASE Summit 2022

January 21, 2022

13:00 – 16:30hrs Bangkok Local Time

13.00 hrs – 13.10 hrs
Recap Session from Day 1

13.10 hrs – 13.20 hrs
Welcome Speech
Preeya Singhnarula
President, Thai Rare Disease Foundation, Thailand

13.20 hrs – 13.40 hrs
Plenary Session
APEC Action Plan on Rare Diseases: Focusing on New and Accessible Treatments
Prof. Matthew Bellgard
Chair of APEC Rare Disease Network

13.40 hrs – 14.00 hrs
Plenary Session
From Rare Disease Framework to Action: Thailand Perspective
Assoc. Prof. Dr. Cherdchai Nopmaneejumruslers
Chairman and Working Group Committee of Rare Disease, NHSO and Deputy Director, Siriraj Hospital, Thailand

14.00 hrs – 14.20 hrs
Plenary Session
Post-Pandemic Era: New Perspective in Rare Disease Access
Prof. Dr. Thong Meow-Keong
University of Malaya, Malaysia

14.20 hrs – 14.30 hrs
Break

14.30 hrs – 15.10 hrs
Panel Discussion 3
The Strategic Priorities to Improve Rare Disease Care
Shaping a Future With No One Left Behind: Advancing the Rare Disease Act
Moderator:
Assoc. Prof. Dr. Cherdchai Nopmaneejumruslers
Chairman and Working Group Committee of Rare Disease, NHSO and Deputy Director, Siriraj Hospital, Thailand

Panelists:
Dr. Carmencita D. Padilla, MAHPS
Chancellor, University of the Philippines Manila
Prof. Madhulika Kabra
All India Institute of Medical Sciences, India

15.10 hrs – 16.10 hrs
Panel Discussion 4
Innovative Approaches for Sustainable Access: The Theory vs the Real World
The Elephant in the Room: Gaps, Inequality, and the Way Forward in Funding and Financing
Moderator:
Assoc. Prof. Dr. Cherdchai Nopmaneejumruslers
Chairman and Working Group Committee of Rare Disease, NHSO and Deputy Director, Siriraj Hospital, Thailand

Panelists:
Prof. Dr. Duangrurdee Wattanasirichaigoon
Ramathibodi Hospital, Thailand
Dr. Nguyen Khanh Phuong
Vice-director of Health Strategy and Policy Institute, Ministry of Health, Vietnam
Dr. Carmencita D. Padilla, MAHPS
Chancellor, University of the Philippines Manila
Prof. Ming-Chin Yang
National Taiwan University, Taiwan

16.10 hrs – 16.20 hrs
Our Unwavering Commitment to Rare Disease Patients Across SEA
Thomas Willemsen
Area SVP Asia Pacific, Takeda

16.20 hrs – 16.30 hrs
Recap Session of Day 2

UNITING TO CREATE BRIGHTER FUTURES FOR PEOPLE WITH RARE DISEASES

C-ANPROM/TH/RDM/0016: Jan 2022

SYNOPSIS

DAY 1

Southeast Asia RARE DISEASE Summit 2022

How Do Public Health Programs Successfully Support the Diagnosis of Rare Diseases?

Moderator:
Dr. Phichai Kanitcharaskul, Medical Affairs Head, Takeda Thailand

Panelists:
Dr. Vu Chi Dung, National Pediatric Hospital, Vietnam

Synopsis:
Vietnam is the easternmost country on the Indochina Peninsula in Southeast Asia. With an estimated 94 million inhabitants as of 2016, it is the world's 13th-most-populous country, and the eighth-most-populous Asian country. Number of birth was about 1.5 million a year. Selected and pilot expanded screening for IEMs has been started in 2004 and 2014, respectively. The aim of this report is to highlight current status of screening for several IEMs in Vietnam and. The cooperation between doctors as promoter and advocacy groups, media and influences patients' lives in Vietnam will be discussed.

Prof. Dr. Damayanti Rusli Sjarif, SpA(K), Cipto Mangunkusumo National Referral Hospital, Indonesia

Synopsis:
What are the key barriers/ challenges for the diagnosis in rare disease?

- So far in Indonesia we do not have diagnostic lab for genetic diseases yet, Human Genetic Research Cluster in Faculty of Medicine Universitas Indonesia since 2017 develop the system to assist the clinical diagnosis from all over Indonesia online,
- We also help patients all over Indonesia to reach diagnosis and help them for financing the cost by crowd funding

Diagnosis rate in each in Indonesia (Human Genetic Research Cluster – IMERI FMUI)

- About minimally 4 patients clinically diagnosed every weeks and 2 patients confirmed laboratory
- Diagnosis improvement from newborn screening program
- So far on Indonesia only congenital hypothyroid screening recognized by ministry of health but not paid by government

SYNOPSIS

DAY 1

Southeast Asia RARE DISEASE Summit 2022

Challenges in Diagnosis Journey – Empowering Patients' Voices

Moderator:
Dr. Phichai Kanitcharaskul, Medical Affairs Head, Takeda Thailand

Panelists:
Preeya Singhnarula, President, Thai Rare Disease Foundation, Thailand

Synopsis:
Rare diseases as a word encompasses so many different types of diseases, each requiring different methods and urgency in dealing with it appropriately. Diagnosis of rare diseases in an accurate and timely manner is a matter of life and death for a plethora of rare diseases, especially for metabolic disease. It is important that hospitals and doctors are armed with the diagnostics tools and the knowledge of what diseases to test for; it is also essential for patients and their families to have access to information in order to pursue a diagnosis in an accurate and timely manner. There are still many challenges that remain in ensuring an accurate diagnosis; however, we have seen a shift in patient voices becoming stronger here in Thailand as rare disease receives the support of government agencies, pharmaceutical companies, media and patient organizations.

SYNOPSIS

DAY 1

Southeast Asia RARE DISEASE Summit 2022

Challenges in Diagnosis Journey – Empowering Patients' Voices

Moderator:
Dr. Phichai Kanitcharaskul, Medical Affairs Head, Takeda Thailand

Panelists:
Dr. Durhane Wong-Rieger, President APARDO

Synopsis:
Rare Disease—A Global Challenge
A rare disease affects few persons in one country but may be found in many countries. While 80% of rare diseases are caused by a genetic mutation, those affected may be unrelated in other ways. There are 6,000+ rare diseases, affecting over 300 million persons. We know the genetic cause of 4,000, but most persons remain undiagnosed and the typical diagnostic odyssey is seven or more years.

To solve challenges of rare diseases, we must act globally. Rare Diseases International is the global alliance of national and international rare disease patient organizations, bringing together all stakeholders, speaking with one voice, and representing the rare disease community with global entities including the United Nations, WHO, APEC, International Research Consortium for Rare Diseases, and the International Pharmaceutical Manufacturers Alliance.

Globally, RDI has succeeded in having "rare disease" designate a distinct underserved population in the UN Declaration on Universal Health Coverage, signed a memorandum of understanding with the WHO, and succeeded in UN passage of a Declaration addressing the challenges of rare diseases.

RDI collaborates with regional alliances, including the Asia Pacific Alliance and ALIBER, the Iberoamerican Alliance and is supporting creation of an African Network.

RDI supports leadership training, capacity building and advocacy with national organizations.

RDI collaborates on access issues, including global access to rare disease medicine, timely diagnosis, and a global network of national and local specialty centres.

SYNOPSIS

DAY 2

Southeast Asia RARE DISEASE Summit 2022

APEC Action Plan on Rare Diseases: Focusing on New and Accessible Treatments

Speaker: Prof. Matthew Bellgard,
Director, eResearch, Queensland University of Technology, Australia and Chair of APEC Rare Disease Network

Synopsis:
Rare Diseases impacts approximately 200 million individuals with one of 7000 rare diseases within the 21 Asia Pacific Economic Cooperation (APEC) member economies. Rare diseases affect 35% of all deaths in the first life year, they can take approximately eight years to diagnose with two to three misdiagnoses along the way. Over 95% of rare disease have no treatment. In 2017, the APEC Life Science Innovation Forum established the APEC LISF Rare Disease Network (RDN) to address the barriers to rare disease diagnosis, treatment and care in the APEC region, as no economy can achieve the goals of Healthy Asia-Pacific 2020 if it does not first meet the needs of people living with a RD. Through the RDN, in 2018, the 21 APEC member economies endorsed the APEC Action Plan on Rare Diseases to facilitate greater alignment of domestic policies & regulations; support urgent implementation of proven best practices; promote multisectoral collaborations and patient partnership. The vision of the APEC RDN is that by 2025 all 21 APEC member economies have a domestic RD policy and/or plan in place. In this presentation I provide an overview of the APEC RDN, the APEC RD Action Plan focusing on new and accessible treatments.

SYNOPSIS

DAY 2

Southeast Asia RARE DISEASE Summit 2022

Post-Pandemic Era: New Perspective in Rare Disease Access

Speaker: Prof. Dr. Thong Meow-Keong, University of Malaya, Malaysia

Synopsis:
The COVID-19 pandemic has deeply impacted the medical and socio-economic world with short- and long-term consequences for patients with rare diseases (RD). The pandemic has significant effects on patient's health status, service use patterns, mental health, daily living activities, social life, financial status from a study in Hong Kong in 2020. An Asia-Pacific survey of genetic healthcare workers (HCW) performed in the early and mid-2021 reported the majority of workplace from middle-income countries were more likely converted to COVID-19 hospitals compared to high-income countries. There was interruption in patient care such as reduction of staff capacity, disruptions to diagnostic services and genetic test uptake and increased psychosocial issues faced by patients. Six months after the initiation of mass vaccination programs, the majority of countries have pivoted and implemented tele-genetic counselling in various forms. Countries continued to face major disruptions to their services. Hence, there is a need to increase public awareness on RD and working with support groups, setting up RD registries, engagement with policy-makers and pursue innovative funding models for RDs. The UN General Assembly has formally adopted the UN Resolution on Addressing the Challenges of Persons Living with a Rare Disease and their Families on the 16th December 2021. This is an impetus for governments in the Asia-Pacific region to re-focus their attention to the care of patients with rare diseases in the post-pandemic era

SYNOPSIS

DAY 2

Southeast Asia RARE DISEASE Summit 2022

Shaping a Future with No-one Left Behind: Advancing the Rare Disease Act

Moderator:
Assoc. Prof. Dr. Cherdchai Nopmaneejumruslers, Siriraj Hospital, Thailand

Panelists:
Dr. Carmencita D. Padilla, MAHPS, Chancellor, University of the Philippines Manila

Synopsis:
The first talk presents the experience of the Philippines with lobby efforts for the Rare Disease Act of 2016 or Republic Act of 10747. The rare disease law is comprehensive, sustainable and integrated with the public health delivery system. With the Department of Health (DOH) as lead agency, it provides a framework for access to health information and medical care; integrates educational and information campaigns into current DOH programs; creates a rare disease registry, and institutionalized a financial system for agencies. The Rare Disease Act of 2016 complements the Newborn Screening Act of 2004 which deals with the rare metabolic disorders. In 2021, the Department of Health launched the Integrated Rare Diseases Management Program which provides the strategic plan developed by stakeholders for 2022-2026 including the list of rare diseases in the Philippines across all medical specialties and subspecialties.

Prof. Madhulika Kabra, All India Institute of Medical Sciences, India

Synopsis:
Recently announced Rare Disease Policy in India, and how it is going to help the patients and families with rare diseases. The holistic directions proposed in the policy covering all aspects which include prevention, awareness, cost effective diagnostics, therapeutics and research.
The implementation plans and the challenges ahead Importance of collaboration for successful implementation of the policy and beyond
Efforts in the direction of collaborative work

SYNOPSIS

DAY 2

Southeast Asia RARE DISEASE Summit 2022

The Elephant in the Room: Gaps, Inequality and the Way Forward in Funding and Financing

Moderator: Assoc. Prof. Dr. Cherdchai Nopmaneejumruslers, Siriraj Hospital, Thailand

Panelists:

Prof. Dr. Duangrurdee Wattanasirichaigoon, Ramathibodi Hospital, Thailand

Synopsis:
Treatment for certain rare disease have been sustainably granted under Thai UC, including factor concentrate for hemophilia (2007) and enzyme replacement therapy for Gaucher disease (2013). Rare disease policy under Thai UC, formally launched in late 2019, contains 6 fundamental aspects: definition of rare disease, service management system, pharmaceutical management, HTA research, information management, and financing. Since then, an innovative management system of 24 rare inborn metabolic disorders (small molecules) has been successfully set up and expanded newborn screening (NBS) is about to be covered in 2022. There are still long-awaited medicine and necessary treatments for other rare disorders, especially for those expensive ones. Multi-stakeholder workshops held on the Rare Disease Day 2021, have delivered several important thoughts, ideas and suggestions with the hope for the improvement of Access to Medicine and Alternative Financial Management for rare diseases in Thailand. However, when it comes to pragmatism – lots of challenges have arisen. "where and how to start with, who would draw the conclusion, where to end, and so on" have led to an "unintentionally ignored" issue. With our great determination, learning from each other, brain storming, and innovative management, these challenged can be gradually resolved soon, I hope.

Dr. Nguyen Khanh Phuong, Vice -director of Health Strategy and Policy Institute, Ministry of Health, Vietnam

Synopsis:
Current funding and financing landscape

- Shaping access landscape
- HTA perspective
- Role of Collaboration among multi stakeholders to accelerate patient access to innovative treatment
- Funding and alternative source of fund for sustainability

SYNOPSIS

DAY 2

Southeast Asia RARE DISEASE Summit 2022

The Elephant in the Room: Gaps, Inequality and the Way Forward in Funding and Financing

Moderator: Assoc. Prof. Dr. Cherdchai Nopmaneejumruslers, Siriraj Hospital, Thailand

Panelists:

Dr. Carmencita D. Padilla, MAHPS, Chancellor, University of the Philippines Manila

Synopsis:
The second talk presents the gaps, the hope and the challenges of rare diseases in the Philippines.

Prof. Ming-Chin Yang, National Taiwan University, Taiwan

Synopsis:
Taiwan is the world's 5th country to pass rare disease legislation and to safeguard rare disease patients by assisting them to obtain medication and subsidies
With this rare disease specialized rare disease regulation will be able to help those rare diseases patients access to medical services easily.
However, recently more and more challenges come from the government tend to balance the costs and medical needs, and impact on patient access to innovative drugs.

Assoc. Prof. Dr. Cherdchai Nopmaneejumruslers
Organization/Institute: Siriraj Hospital, Mahidol University
Country: Thailand

Biography:

Associate Professor Cherdchai Nopmaneejumruslers, MD received his training in medicine and Diplomat Thai-Board of Respiratory Medicine (2000) at Siriraj Hospital, Mahidol University, Bangkok Thailand and Research and clinical Fellow in Respirology at Toronto General Hospital, Ontario Canada (2001-2003) and Certificate Fellowship in Sleep Medicine, Toronto General Hospital: Sep 2003 – Jan 2005 and Executive MBA Sasin Graduate Institute of Business Administration of Chulalongkorn University 2014-2016

Associate Professor Cherdchai Nopmaneejumruslers, MD is the Vice Hospital Director at the first and biggest hospital in the country with 2,200 beds, Siriraj Hospital, Bangkok Thailand. He has more than 20 years in quality improvement and promotes learning organization experience, through supporting and facilitating lean, routine to research (R2R) methodology, and health economic and decision model analysis. He has been certified Lean Six Sigma Green belt since 2010 and TQA (Thailand Quality Award) assessor of Thailand productivity institute (from 2011-2019). He was also assigned as Asian Productivity Organization (APO) Technical Expert Service (TES) on Lean management in Hospitals and Healthcare Systems, in Oct 2011 . He is a course coordinator Health economic analysis for the Master of Science (M.Sc.) in Clinical Epidemiology Thai Cert and has been working as a project leader in R2R Thailand project to promote research conduction to improve hospital services, In 2016 he was appointed as a co-Learning council members of Center for Healthcare Innovation (CHI), Singapore and the Foundation Committee for The Health Intervention and Technology Assessment Program (HITAP), Thailand and The Chair of the Academic Working Group selects topics for evaluation to develop a set of benefits in the National Health Security System, and Working Group on Public Health Economics. To define the type and scope of public health services. Recently in 2020, He was appointed as the Chairman Working Group on the Development of Rare Disease Healthcare Services, Thailand.

Dr. Carmencita D. Padilla, MAHPS
Organization/Institute: The University of the Philippines Manila
Country: Philippines

Biography:

Dr. Padilla is Professor of Pediatrics at the College of Medicine and currently Chancellor of the University of the Philippines Manila. She is the Founding Director of the Institute of Human Genetics and the Newborn Screening Reference Center at the National Institutes of Health. Recognizing her varied contributions to the academic growth of genetics in the Philippines, she was conferred Academician of the National Academy of Science and Technology (NAST) in 2008. Dr. Padilla is a pioneer in genetics in the Philippines and the Asia Pacific region. In the Philippines, she is responsible for setting up the clinical genetic services at the Philippine General Hospital in 1990 and the various genetic laboratories now housed at the Institute of Human Genetics National Institutes of Health (www.ihg.upm.edu.ph). She is also responsible for setting up of national newborn screening services in the Philippines, currently available in 7200+ health facilities in the country and being served by 6 newborn screening laboratories and 14 continuity clinics that monitor the long term care of the patients. In the Asia Pacific region, she is part of the pioneering group that established the Asia Pacific Society for Human Genetics and served as president in 2008-2010. Dr. Padilla is Council member of the Human Genome Organization, an international organization of scientists from 69 countries (www.hugo-international.org). In 2010, she was appointed country representative of NAST to the InterAcademy Partnership for Health, a global network of more than 150 academies in the world (www.interacademies.org).

Dr. Padilla has been a recipient of international and national awards. In 2017, she received the much coveted International Society for New Born Screening (ISNS)/ Robert Guthrie Award.

Dr. Padilla has more than 120 publications. In the area of policy making, she is responsible for the Newborn Screening Act of 2004 (Republic Act 9288) and the Rare Disease Act (Republic Act 10747).

Prof. Dr. dr Damayanti Rusli Sjarif, SpA(K)
Organization/Institute: Cipto Mangunkusumo National Referral Hospital Jakarta
Country: Indonesia

Biography:

Prof Dr dr Damayanti Rusli Sjarif SpA(K)
Pediatrician, consultant in pediatric nutrition and metabolic diseases

Education
- Pediatrician from Faculty of Medicine Universitas Indonesia
- Clinical training in metabolic Diseases in Wilhelmina Children Hospital and Medical Genetics In Clinical Genetic Center Utrecht, The Netherlands
- PhD in Utrecht Universiteit The Netherlands

Position
- Professor in Pediatrics in Faculty of Medicine Universitas Indonesia
- Chair of Human Genetic Research Cluster IMERI Faculty of Medicine Universitas Indonesia
- Chair of Rare Diseases National Center of Referral – Cipto Mangunkusumo National Referral Hospital Jakarta Indonesia

Prof. Dr. Duangrurdee Wattanasirichaigoon
Organization/Institute: Mahidol University, Faculty of Medicine Ramathibodi Hospital
Country: Thailand

Biography:

Duangrurdee Wattanasirichaigoon, is currently a Professor of Pediatrics and Chief of the Division of Medical Genetics, Dpt. of Pediatrics, Faculty of Medicine Ramathibodi Hospital, Mahidol University. She is the Chair of the Medical Genetics Network, Genetics Society of Thailand; Vice President of Thai Medical Genetics and Genomics Association (TMGGA); and a board member of the Asia Pacific Society of Human Genetics.

Prof. Duang obtained her MD from Faculty of Medicine, Khon Kaen University; Board of Pediatrics from Ramathibodi Hospital; Diploma of the American Board of Medical Genetics (Clinical Genetics and Clinical Molecular Genetics), from the Boston Children's Hospital, Harvard University, MA, in 1999. Her researches involve clinical and molecular aspects of various genetic diseases; inborn metabolic disorders of amino acids and organic acids, lysosomal storage diseases; metabolic liver diseases and citrin deficiency; molecular genetics of congenital hearing loss and other rare disease.

Prof. Duang has been leading Rare Disease Day campaign and rare disease advocate in Thailand since 2011. She has worked with multistakeholders including National Health Security Office (NHSO, or UHC), the Ministry of Public Health, National List of Essential Medicine (NLEM), Thai Rare Disease Foundation (ThaiRDF), other patient organizations to promote benefit package and innovative managements for rare diseases and orphan drugs in Thailand. She has long involved in patient support group empowerment and endorsed professional education on rare diseases. Prof.Duang is also a co-founder of Prader-Willi syndrome Association of Thailand (2003), and ThaiRDF (2016).

Dr. Durhane Wong-Rieger
Organization/Institute: Rare Diseases International
Country: Canada

Biography:

Durhane Wong-rieger, PhD is Chair of Rare Disease International, Chair of Asia Pacific Rare Disease International, Treasurer of United Nations Nongovernmental Organization for Rare Diseases. Chair of Patient Advocates Constituency Committee of the International Rare Disease Research Consortium, Patient Advisor to the APEC Rare Disease Network, member of the Editorial Board of The Patient- Patient Centred Outcomes Research, member of the Global Commission to End the Diagnostic Odyssey for Rare Diseases and member of Health Technology Assessment International Patient /Citizen Involvement Interest Group

In Canada, she is President & CEO of the Canadian Organization for Rare Disorders, Chair of the Consumer Advocare Network, President & CEO of the Institute for Optimizing Health Outcomes and Chair of Canadian Heart Patient Alliance. She is a certified Health Coach.

Dr. Wong-Rieger has served on numerous health policy advisory committees and panels and is a member of Ontario's Rare Disease Implementation Working Group and member of Genome Canada Steering Committee for the Rare Disease Precision Health Initiative.

Durhane has a PhD in psychology from McGill University and was professor at the University of Windsor, Canada. She is a trainer and frequent lecturer and author of three books and many articles.

Prof. Dr. Kanya Suphapeetiporn
Organization/Institute: Faculty of Medicine, Chulalongkorn University
Country: Thailand

Biography:

PRESENT ACADEMIC POSITION
- Professor of Pediatrics
- Head of Division of Medical Genetics and Metabolism, Department of Pediatrics, Faculty of Medicine, Chulalongkorn University
- Director of Excellence Center for Genomics and Precision Medicine, King Chulalongkorn Memorial Hospital

BOARD CERTIFICATION
- American Board of Pediatrics, 2005
- American Board of Clinical Molecular Genetics, 2005
- Thai Board of Pediatrics, 2006

MEDICAL LICENSURE
Medical License of Thailand, License #20315, April 1995

HONORS AND AWARDS
- TRF-CHE-Scopus Young Researcher Award for distinguished research, TRF-CHE-Scopus, 2009
- Award for Distinguished Research Article from National Research Council of Thailand, 2010
- Award for Distinguished Research Article from National Research Council of Thailand, 2012

PROFESSIONAL AND SOCIETY MEMBERSHIPS
- Member of the American College of Medical Genetics
- Member of the American Academy of Pediatrics
- Member of the Royal College of Pediatricians of Thailand
- Member of the Medical Council of Thailand
- Member of Chulalongkorn Medical School Alumni Association
- Member of Yale Alumni Association

Prof. Madhulika Kabra
Organization/Institute: All India Institute of Medical Sciences, New Delhi
Country: India

Biography:

Professor of Pediatrics , In-charge of Division of Genetics at the All India Institute of Medical Sciences, New Delhi. Working in the specialty of Medical Genetics for almost 25 years.

IMPORTANT RECOGNITIONS & HONORS

- Genetics Division is a WHO CC for training in Clinical and Laboratory Genetics in Developing Countries
- Chairperson of the Center of excellence for Rare Diseases Committee (AIIMS, New Delhi designated as one of the 8 COE for Rare Diseases)
- Dr IC Verma Lifetime Achievement Award of ISHG -2016
- Dr IC Verma SIAMG Outstanding Scientist Award -2017
- Past President Society for Indian Academy of Medical Genetics
- Member Central Technical Committee for Rare Diseases (Government of India)
- Member of "National Apex Committee" by the Ministry of Health & Family Welfare for planning a route map and monitoring of activities of the "National Consortium for Research and Development on therapeutics for Rare Diseases"
- Nominated as a Vice-Chairperson of the new committee of ICMR Task Force on Rare diseases

Fields of Interest: Rare diseases, Intellectual disability, Dysmorphology, Storage disorders, Newborn screening

Prof. Matthew Bellgard
Organization/Institute: eRsearch Office, Queensland University of Technology
Country: Australia

Biography:

A polished, strategic, academic leader, with extensive experience leveraging the latest in technological innovation and digital transformation in order to ensure excellence in research and development wonderful experience for researchers and educators. Areas of expertise cover a wide scope, including genomics, bioinformatics, health informatics, AI, machine learning, biosecurity, eResearch, remote sensing, and radio astronomy. Long track-record of delivering major projects to completion across government, industry and academia. Demonstrated ability in establishing sound professional networks locally, nationally, and globally, encouraging a thriving learning community. Extremely detailed, methodically organized, and a proven problem solver.

Prof. Dr. Thong Meow-Keong
Organization/Institute: University of Malaysia
Country: Malaysia

Biography:

Dr. Thong Meow-Keong is a Professor of Paediatrics and Consultant Clinical Geneticist at the University of Malaya Medical Centre. He was a Fulbright Scholar and an Australian board-certified clinical geneticist and established the first Genetics Clinic in Malaysia in 1995. He is the current President of the College of Paediatrics, Academy of Medicine of Malaysia; Vice-President of the Medical Genetics Society of Malaysia, Trustee of the Rare Disease Alliance Foundation Malaysia, appointed member of the Malaysia Medical Council (Education) and Advisor to the Malaysian Rare Disorders Society. He was the Head, Department of Paediatrics, University of Malaya and past President, Asia-Pacific Society of Human Genetics.

His clinical practice and research are focused on rare diseases, genomic medicine, inborn errors of metabolism and genetic counselling. He has published extensively in the field of paediatrics and the practice of genetic medicine in low-resource settings. He has authored/co-authored over 100 WOS/ISI journal publications, 3 books, 18 book chapters including the Oxford Monograph in Medical Genetics and an IDEAS White Paper entitled "Rare Diseases in Malaysia". He was consulted by the World Health Organization and the Ministry of Health Malaysia on various technical issues and clinical practice guidelines. He was active in developing undergraduate and postgraduate paediatric training curriculum programs and promoted advocacy issues affecting children and individuals with rare diseases. He has won major research awards and research grants and has collaboration with major universities globally. He was elected a Fellow of the Academy of Sciences Malaysia, Academy of Medicine of Malaysia and Academy of Medicine Singapore.

Prof. Ming-Chin Yang
Organization/Institute: College of Public Health, National Taiwan University
Country: Taiwan

Biography:

Ming-Chin Yang is an experienced professor with a demonstrated history of working in the higher education and planning and evaluation of the National Health Insurance system. Also involved in Taiwan national health insurance system planning and with comprehensive knowledge of policy transformation from the very beginning. Skilled in Public Health program evaluation, pharmacoeconomic evaluation, and Qualitative Research. Strong professional with a MPH and DrPH focused in Health Services Organization from the University of Texas, Health Science Center at Houston, USA.

Dr. Nguyen Khanh Phuong
Organization/Institute: Health Strategy and Policy Institute
Country: Vietnam

Biography:

Dr. Nguyen Khanh Phuong is currently Vice-Director of the Health Strategy and Policy Institute, Ministry of Health. She is a leading health economist with more than 20 years of experience in health financing, health economic and health system reform in Vietnam. She also has a strong background on policy evaluation and monitoring, research proposal design and development, provider payment methods, hospital services cost, health technology assessment and project evaluation. Dr. Nguyen Khanh Phuong has extensive experience in the healthcare sector, having held many positions such as coordinator, senior technical officer, principal investigator and national consultant. She has involved in several studies which provided evidence for developing important health policies such as health insurance, health financing reform... She has been served as technical advisor for some initiatives in the area of health financing such as provider payment method reform, procurement and price negotiation etc... She is currently a member of HTAsiaLink Scientific Board, HTAi Asia Policy Forum Organizing Committee, Joint Learning Network...

Peter Streibl
Organization/Institute: General Manager, Takeda Thailand
Country: Thailand

Biography:

Peter is an accomplished international leader with years of experience in healthcare, inspired by the mission to bring better health and brighter future to patients. Upon his appointment as a General Manager at Takeda Thailand in 2019, he has actively collaborated with public and private stakeholders in Thailand towards driving innovation and patient access. Under his leadership, he is committed to contribute to the health of Thai patients through Takeda's innovative treatments in Gastroenterology, Rare Genetics & Haematology, and Oncology which will transform and improve patients' lives and ultimately to support the country's initiative to become a leading medical hub.

Peter is a strong advocate for patients with neglected non communicable diseases. In 2019, a dedicated Rare Diseases Memorandum of Understanding, a 5-year framework to raise the standard of care in rare diseases, where 8 prominent partners have entered the MOU alongside Takeda Thailand. Within the framework of this MOU, outlines its support of Hereditary Angioedema (HAE) diagnosis with C1-INH testing facilities and screening at 4 centre of excellence sites. Beyond that, the action plan of this MOU will further support to raise the public awareness and improve the diagnostic journey of rare diseases in Thailand.

Peter earns an MBA from the Vienna University of Economics and Business Administration.

Preeya Singhnarula
Organization/Institute: Thai Rare Disease Foundation (ThaiRDF)
Country: Thailand

Biography:

My name is Preeya Singhnarula and I am the President of Thai Rare Disease Foundation. I have an MBA from Sasin University Chulalongkorn and in a former life worked as a Senior HR consultant for AON Hewitt. For the last 8 years I have been a full-time parent to my children since my son was born with a rare disease in 2013, working as a parent, cook, nurse, driver, caregiver, therapist, advocate and any other role that has been required to he can live his life in the best possible way. I hope to see a future for rare disease families that are better supported with policies and funding that no longer requires the immense sacrifices families have to make in order to provide for their child/children.

Raman Rajakanath
Organization/Institute: Rare Disorders Society Singapore
Country: Singapore

Biography:

Raj has 20 years of experience in healthcare advocacy, policy and governance. As Executive Director of Rainbow Across Borders, he has created and led specific illness patient access programmes and campaigns. He has also initiated and built an extensive network comprising policy makers, industry players, healthcare professionals and patients with regional and local platforms.

As Principal Consultant at Manifeste LLP, Raj has been creating solutions for multiple stakeholders in economically and culturally diverse countries in the Asia Pacific region. He has been able to revitalise strategies to overcome a multitude of challenges in the healthcare sector.

Raj strongly believes in the power of the patient voice and the need for a patient-centric community. Over the years, he has been actively supporting the patient community by creating opportunities for patient support groups to train, connect and collaborate in the Asia Pacific region.

He also works closely with rare diseases patients and their families as Programme Director of Rare Disorder's Society (Singapore)

Sivasangaran Kumaran
Organization/Institute: Microsoft
Country: Malaysia

Biography:

Siva is a caregiver of Swathi Nisha, his 4 years old daughter, diagnosed with Infantile Pompe. As a rare caregiver, Siva strive to discover and bring together rare parents and drive awareness on rare disease in aim to save and improve quality of life of rare disease patients, especially of children's. Then Malaysia Prime Minister, YAB Tun Dr. Mahathir in 2018, launched Siva's website www.rarediseasemalaysia.com, which has state of Malaysia rare disease, statistics, stories and blogs. The website has it's content in 4 major languages to help reach mass public from various ethnics. Siva climbed Mount Kinabalu to raise fund and awareness for rare disease children's. This effort was supported by Ministry of Health (MOH), Malaysia. Siva have contributed to IDEAS whitepaper on Malaysia Rare Disease (2019), which is a key input to MOH's National Rare Disease draft blueprint. Siva primarily advocates on rare disease awareness and national rare disease framework. This is achieved via multi-stakeholders collaboration and close partnership with Ministry of Health Malaysia, Rare Disease NGO's, think tanks (IDEAS), corporates, and public. Siva advocates and speaks at events, appears in news and TV media, drive rare disease activities, as well as digital advocacy. Siva also collaborates closely with medical professionals from Hospital Kuala Lumpur, University Malaya, MMA NS, and doctors from various hospitals and clinics. Siva partner with many other Malaysia rare disease NGO's such as to promote their rare disease agenda, events and patient support groups. At regional level, Siva collaborates closely with Asia Pacific Alliance of Rare Disease Organisations (APARDO), Rainbow Across Borders, and Rare Disorder Society Singapore. Siva is an active contributor to Global Rare Disease Commission, a framework jointly setup by Takeda, EURODIS and Microsoft.

In effort to empower and create young rare disease advocates, Siva partnered with National Organisation of Rare Disease (NORD US) and successfully nominated Malaysia's 1st student to NORD Summit 2019. The student from Brickfields Asia College have also initiated their Rare Disease Student Club, the 1st known in Malaysia private education institute.

Prof. Dr. Thanyachai Sura
Organization/Institute: Medical Genetics and Genomics Association
Country: Thailand

Biography:

Thanyachai Sura MD FRCP
Professor of Medical Genetics
Medical Genetics, Department of Medicine, Ramathibodi Hospital Bangkok Thailand
President of Medical Genetics and Genomics Association (Thailand)
President of Asia Pacific Society of Medical Genetics (APSHG)

Doctor Sura is currently the President of Medical Genetics and Genomics Society of Thailand and President of Asia Pacific Society of Medical Genetics (APSHG). He became a diplomate of the Thai Board of Internal Medicine, Faculty of Medicine Ramathibodi Hospital Mahidol University in 1987.

Dr. Sura received further training in molecular medicine at the John Radcliffe Hospital in Headington, Oxford University, England. He also received a certificate in Medical Genetics and Molecular Medicine from the Oxford University in 1990. He then became a member of the Royal College of Physicians in London in 1992.

Dr. Sura's main research interest is in molecular medicine and clinical genetics, and has published over 70 articles in national and international peer-reviewed medical journals, including Human Genetics, Science,Nature,Journalof ClinicalNeuromuscularDisease,Journalof HumanGenetics,andPLoSOne. His published research papers cover a range of topics, among which include molecular studies on the abnormal hemoglobin, descriptive studies on fragile X syndrome, Duchene muscular dystrophy, Hereditary of spinocerebellar ataxia, familial cancers and several papers on genetic susceptibility to tropical infections.

Dr.Sura has been the subcommittee of the Thai Medical Council postgraduate training as well as the committee of the Royal College of Physicians of Thailand since 2016 and initiated the Medical Genetics training in Thailand in 2019 as well as a short course training of Genetic Counselling for healthcare personnels in 2020.

Dr. Vu Chi Dung
Organization/Institute: National Children's Hospital
Country: Vietnam

Biography:

Dr. Vu, Chi Dung is the Head of Department of Medical Genetics, Metabolism and Endocrinology and Director of Center for Rare Disease and Newborn Screening, Vietnam National Children's Hospital which is one of the best pediatric hospitals in Vietnam. He has extensive clinical experience in pediatric endocrinology, medical genetics and inherited metabolic disease. He trained at the Hospital Pediatrics of St. Antony, Catholic University of Lille, France and worked as research fellow at the Department of Pediatrics, Saint Louis University, USA and as a fellow at Royal children's Hospital, Melbourne, Australia. He has received several awards including the Pfizer Overseas Fellowship, Asian Investigator Award in Japan, ICIEM award in USA and Award for Excellent Study in Korea.

Prof. Dr. Yin-Hsiu Chien
Organization/Institute: National Taiwan University Hospital
Country: Taiwan

Biography:

Dr Yin-Hsiu Chien is Clinical Professor at the Department of Pediatrics at the National Taiwan University in Taipei, Taiwan, and Attending Physician of the Department of Medical Genetics and Pediatrics at the National Taiwan University Hospital. She undertook pediatric residency training at National Taiwan University Hospital, and completed her fellowship in Pediatric Allergy, Immunology & Rheumatology before then completing her fellowship in Medical Genetics and Metabolism, both at National Taiwan University Hospital. Dr. Chien has made diverse contributions in the fields of inborn errors of metabolism and primary immunodeficiencies. She is the director of the newborn screening center at National Taiwan University Hospital, which routinely screens around one third of newborn infants in Taiwan. Dr. Chien and her team have played an important role in making Taiwan's newborn screening programs into one of the most developed newborn screening programs in the world. She is currently focusing on Pompe disease, specifically on early diagnosis and improvement of treatment, as well as on early diagnosis of AADC deficiency, Spinal muscular atrophy, and other LSDs.

CO-HOSTS

PARTNERS

Thai Rare Disease Foundation

Company Profile:
Leading patient organization driven to advocate for the needs of rare disease patients through working with public and private organizations.

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MGGA สมาคมเวชพันธุศาสตร์และจีโนมิกส์ทางการแพทย์
MEDICAL GENETICS AND GENOMICS ASSOCIATION

Medical Genetics and Genomics Association (MGGA)

Company Profile:
Promote knowledge exchange between doctors of genetic medicine, and public health officials to support the development of policies and guidelines, considering social and ethical implications..

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Asia Pacific Alliance of Rare Disease Organisations (APARDO)

Company Profile:
The Asia Pacific Alliance for Rare Disease Organisations (APARDO) is a non-profit organisation legally registered in Singapore with 8 Board of Directors from across the region. We are patient advocate leaders from across the Asia Pacific region representing rare diseases and rare cancers bound together with the goals of not only providing a forum for sharing experiences and learning but also increasing rare disease patients' voice and addressing priority issues. Some of our members are representatives of disease-specific groups; others were from societies for rare diseases or rare cancers in general; and others representing patient group alliances or networks.

The formation of the APARDO marks the opportunity for patient groups representing rare diseases and rare cancers to work together on common goals, facilitating research in the region, sharing resources and best practices, and collaborating on joint initiatives.

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สมาคมพันธุศาสตร์แห่งประเทศไทย
Genetics Society of Thailand

Medical Genetics Network, Genetics Society of Thailand

Company Profile:
The Genetics Society of Thailand (GST) was firstly conceptualized from the landmark conference on "Genetics and Country Development" organized by Mahidol University on the November 27th – December 1st, 1978, at the Faculty of Medicine Siriraj Hospital. As the chair of the conference organizing committee, Professor Supa Na-Nakorn, M.D., the Head of Division of Hematology, Department of Medicine, Faculty of Medicine Siriraj Hospital, invited more than 50 Thai geneticists and academics from the universities and institutes all over Thailand to participate in this conference. After extensive discussion, sharing and exchange of idea, information, and knowledge on the topic regarding how to implement and apply genetics for the country (Thailand) development, on the closing date of the conference (December 1st, 1978), the 'Genetics Club of Thailand' was established and Professor Supa Na-Nakorn was elected by the conference participants to take the position as the First President of the Club for the period of two years (1978-1980).

At that time, many academics and geneticists, who participated in the conference, volunteered to be members of the executive committee of the Club, including Professor Khunying Soodsarkorn Tuchinda, M.D., who was the first Treasurer of the Club. After the first term, the Second Elected President was Associate Professor Arpornrat Rattanatarot from the Department of Botany, Faculty of Sciences, Chulalongkorn University, who took the presidential position for two terms in 1980 – 1982 and 1982 – 1984. After this initiation and operation as the Genetics Club of Thailand for seven years, the key executive committee member of the Club – Assistant Professor Orasri Romyanan, M.D., from the Department of Anatomy, Faculty of Medicine, Chulalongkorn University, took the important step in leveraging the Club to register it as 'The Genetics Society of Thailand (GST)' to the Office of the National Culture Commission on the November 16th, 1984. The late Professor Sumin Smutkupt, Ph.D., from the Department of Biology, Faculty of Sciences, Kasetsart University, was the First President of GST, which, since then, the office is located at the Botany Building, Faculty of Sciences, Chulalongkorn University, Phayathai Road, Bangkok.

GST is regarded as an academic society. One of the main activities of GST is the co-organizer of the National Genetics Conference every two years. With the collaboration between GST and the universities in Thailand, each university rotates to be the co-organizer with GST, with the tradition of an alteration of the co-organizers, every two years, between the universities located in the central part and those located in the other parts of Thailand. GST also actively organizes meetings, trainings, seminars, workshops and conferences related to genetics and genetic research and education. GST is also active in publishing Thai Journal of Genetics and books related to genetics. Currently, GST has more than 600 members from all over Thailand.

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Takeda Thailand

Company Profile:
Takeda is a patient-focused, values-based, R&D-driven global biopharmaceutical company committed to achieving "better health for people, brighter future for the world". Our vision to discover and deliver life-transforming treatments is guided by our commitment to patients, our people and the planet. Our employees are committed to improving the quality of life for patients and to working with our partners in health care in approximately 80 countries.

Serving patients and customers in Thailand for over 50 years, the company is focused on value enhancement and the sustainable delivery of innovative treatments research and developing of biopharmaceuticals to promote the better health of Thai people as well as changing the lives of patients and being patient centric. Aligned with Takeda's global strategy, we are committed to bringing life-changing therapies to patients in the core areas of: Gastroenterology (GI), Oncology, Rare Genetics & Hematology, and preparing for the anticipated launch of Vaccines.

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