



Southeast Asia RARE DISEASE Summit 2025 *Special Edition*

United for Rare: Transforming Policies, Empowering Change in SEA

The 3rd Southeast Asia Rare Disease Summit 2025 Special Edition
19th February 2025 at Pimarnman Room, Anantara Siam Bangkok Hotel, Thailand

www.seararediseasesummit.org

PROGRAM BOOK



Organizers & Partners:



Southeast Asia Rare Disease Summit aims to bring together all stakeholders in the rare disease community: patient representatives, policymakers, clinicians, researchers, industry representatives, payers, and regulators to exchange invaluable knowledge. This platform aims to emphasize unified efforts to revolutionize policy frameworks and foster impactful change in the rare disease landscape of Southeast Asia (SEA) and reinforce commitment towards sustainable rare disease management and policies.
The 3rd Southeast Asia Rare Disease Summit, Special Edition is hosted by Takeda (Thailand) Limited in collaboration with Medical Association of Thailand (MAT), Thai Rare Disease Foundation (Thai RDF), and Medical Genetics and Genomics Association (MGGA) and The Allergy, Asthma, and Immunology Association of Thailand (AAIAT).

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C:ANPROM/TH/RDG/0066: Feb2025



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WELCOME MESSAGE

Dear Participants,

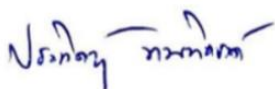
A warm welcome to the Third Southeast Asia R&D Summit, "United for Rare: Transforming Policies, Empowering Change in Southeast Asia."

This summit is a testament to the power of partnership in advancing the standard of care for the rare disease community. Together, we have the opportunity to drive meaningful change, shape policies, and strengthen advocacy efforts that will positively impact the lives of patients and families across the region.

We look forward to your active participation, insights, and collaboration in this important dialogue. Your contributions will play a crucial role in shaping the future of rare disease care and policy in Southeast Asia and beyond.

Let's unite for rare and make a difference!

Your Sincerely,



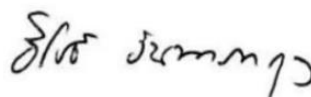
Prof. Dr. Prakitpunthu Tomtidchong, M.D.
President of Medical Association, Thailand



Prof. Duanrurdee Wattanasirichaigoon, M.D.
President of Thai Rare Disease Foundation



Prof. Thanyachai Sura, M.D.
President of Medical Genetics and
Genomics Association



Assistant Prof. Hiroshi Chantaphakul, M.D.
President of The Allergy, Asthma &
Immunology Association Thailand



Mr. Peter Streibl
General Manager of Takeda (Thailand) Limited

8:30 - 9:00	Registration
9:00 - 9:10	Welcome Remarks Dion Warren , Area Head of India & Southeast Asia (I-SEA), Takeda
9:10 - 9:20	Opening Ceremony Keynote Session Asst.Prof. Thanin Wechapinan, M.D. Deputy Director General, Department of Medical Services, Ministry of Public Health, Thailand
9:20 - 11:00	Evolving Rare Disease Policy in SEA
30 mins	Rare Disease Management in SEA: Current Policy Landscapes and Future Directions Dr.Khoo Yoong Khean Assistant Professor, The Centre of Regulatory Excellence (CoRE), SingHealth Duke-NUS Global Health Institute (SDGHI) and The Centre for Outbreak Preparedness (COP) at The Duke-NUS Medical School, Singapore
20 mins	Best Practices: National Rare Disease Funding Assoc.Prof. Cherdchai Nopmaneejumrulers Chairman Working Group on the Development of Rare Disease Healthcare Services (2023), NHSO Deputy Dean of Service Innovations and Corporate Value, Faculty of Medicine, Siriraj Hospital, Mahidol University
50 mins	Panel Discussion: Developing a Robust Policy Framework for Rare Diseases – The Critical Need for Specialized Policies Moderator: SG: Dr.Khoo Yoong Khean Assistant Professor, The Centre of Regulatory Excellence (CoRE), SingHealth Duke-NUS Global Health Institute (SDGHI) and The Centre for Outbreak Preparedness (COP) at The Duke-NUS Medical School, Singapore Panelists: MY: Dr.Tae Sok Kun Clinical Geneticist and Consultant Pediatrician, Genetic and Metabolism Unit, Head of Pediatric Unit, University of Malaya PH: Dr.Carmencita D. Padilla Emeritus Professor, University of the Philippines Manila Chair, Philippine Society for Orphan Disorders VN: Dr.Dang Viet Hung President of Hanoi University of Pharmacy TH: Prof.Duangrurdee Wattanasirichaigoon Professor, Division of Genetics, Department of Pediatric, Faculty of Medicine, Ramathibodi Hospital, Mahidol University and Vice President of Thai Rare Disease Foundation
11:00 - 11:15	Break
11:15 - 12:25	Advancing Partnerships for Sustainable Rare Disease Management
30 mins	Patient Advocacy for Policy Change: The ASEAN Rare Disease Consortium (ARdC) Roadmap & Outcomes Dr.Rajakanth Raman Programme Director, Rare Disorders Society (Singapore), Visiting Expert, Centre for Regulatory Excellence (CoRE) Duke-NUS Medical School
30 mins	Collaborative Achievements: Innovative Partnership for Rare Disease Impact Thai RD MOU; Prof.Thanyachai Sura President, Medical Genetics and Genomics Association (MGGA) Thai RD MOU; Assoc.Prof.Hiroshi Chantaphakul President of The Allergy, Asthma, and Immunology Association of Thailand (AAIT) Thai RD MOU; Preeya Singhnarula , President of Thai Rare Disease Foundation
10 mins	Empowering Patient Voice for Rare Disease Policy Reform Thitimon Burapa-arayahwong , President of HAE Thailand
12:25 - 12:30	Closing Remarks Peter Streibl , General Manager, Takeda Thailand

Invitation to attend the Third Southeast Asia Rare Disease Summit

On behalf of the organizing committee, you are cordially invited to attend the third Southeast Asia Rare Disease Summit on February 19, 2025, from 08:30 hrs to 12:30 hrs at the Anantara Siam Bangkok Hotel.

The Third Southeast Asia Rare Disease Summit will spotlight unified efforts to transform policy frameworks and drive meaningful change in the rare disease landscape across the region. It underscores a steadfast commitment to sustainable rare disease management and policy development. This year's theme, "United for Rare: Transforming Policies, Empowering Change in Southeast Asia," highlights the power of partnership in elevating the standard of care for the rare disease community.

What you should expect by attending the summit:

Meaningful participation: All invitees are like-minded individuals with 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.

Experience from the experienced: You will have an opportunity to listen and understand the context and existing challenges of rare diseases in all aspects including diagnosis, treatment, and access. While the best-case sharing will inspire you to make new possibilities for rare disease communities.

Open new vistas: Expand networks, meet rare disease allies, and learn from people with a wide range of backgrounds and experiences.

We would be pleased if you accepted this invitation and joined us in harnessing positive empowerment for rare disease patients. Please kindly confirm your availability and interest by 31 January 2025 through the registration link <https://seararediseasesummit.org/registration/> Further details on the Summit arrangements are shared through the registration link.

Please do not hesitate to contact Dr Tangthai Saengtrakulcharoen, Senior Medical Advisor, Takeda Thailand tel.: +66 98 819 5848 email: Tangthai.Saengtrakulcharoen@takeda.com for any queries.

Your Sincerely,

We would be pleased if you accepted this invitation and joined us in harnessing positive empowerment for rare disease patients. Please kindly confirm your availability and interest by 31 January 2025 through the registration link <https://seararediseasesummit.org/registration/> Further details on the Summit arrangements are shared through the registration link.

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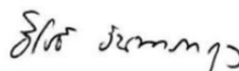
Prof. Dr. Prakitsuntho Tomtidchong, M.D.
President of Medical Association, Thailand



Prof. Duanrurdee Wattanasirichaigoon, M.D.
President of Thai Rare Disease Foundation



Prof. Thanyachai Sura, M.D.
President of Medical Genetics and
Genomics Association



Assistant Prof. Hiroshi Chantaphakul, M.D.
President of The Allergy, Asthma &
Immunology Association Thailand



Mr. Peter Streibl
General Manager of Takeda (Thailand) Limited

Rare diseases have characteristics that make them one of the significant health challenges of our time. Between 5,000 and 8,000 rare diseases have been identified (Rath & Janmaat, 2018). These diseases are uncommon individually, each presenting its own set of challenges and impacting the lives of those affected. Rare diseases are defined by their low prevalence, typically affecting a small number of individuals within the population. Despite their individual rarity, collectively, rare diseases impact a significant number of people globally, contributing to a broad spectrum of medical conditions and challenges. As a group they affect 6 to 8% of the global population (Barakat et al., 2014).

More than 80% of rare diseases are caused by genetic or congenital aberrations, and 75% present with a wide range of neurological symptoms and physical and intellectual disabilities (McClellan & King, 2010). Rare diseases mostly affect children or young adults, and several siblings can be affected in the same family. As such, these diseases come with substantial hardship for both parents and patients. Many rare diseases are fatal with no known treatment or cure—almost one-third of those born with a rare disease die before the age of five (Institute of Medicine, 2010). This “paradox of rarity” presents unique problems for not only the individuals living with rare diseases but for caregivers, researchers, policymakers, and industries as well (Schulenburg & Frank, 2015).

Given the diversity of rare diseases, treatment approaches vary significantly. Some conditions may have approved therapies, while others rely on supportive care and disease management. Furthermore, policy toward rare disease plays a crucial role in tailoring interventions to address the specific needs of individuals with rare diseases. Recognizing the significant impact of rare diseases on individuals and families, developing a comprehensive policy to address the unique needs of those affected is what we all need to work on. The policy will support to create a framework for improved diagnosis, treatment, research, and support for individuals living with rare diseases, leading to a better quality of life.

The Southeast Asia Rare Disease Summit is to shed light on the diversity, shared characteristics of rare disease community, and the collective efforts that each and every stakeholder is committed to those who are affected by rare diseases.

Through the unwavering commitment, the platform of Southeast Asia Rare Disease Summit was established to promote early and accurate diagnosis through increased awareness among relevant stakeholders and public. Ensure timely access to specialized care and treatments for individuals with rare diseases, recognizing the importance of a multidisciplinary approach. Beyond the discussion among rare disease experts, recognizing the role of patient advocacy groups and support organizations is a key driver to influence the policy changes where no one will be left behind.

Over the years, Takeda (Thailand) Limited together with Medical Association of Thailand, Thai Medical Genetics and Genomics Association, Genetic Society of Thailand, Hereditary Angioedema Thailand, The Allergy, Asthma, and Immunology Association of Thailand Rare Disease Foundation, Genetic LSD Foundation have entered the Rare Diseases Memorandum of Understanding, a first step to pave the way for the future of rare diseases and collaboration in long-term support to enable better quality of life for people living with rare disease.

The 1st Southeast Asia Rare Disease Summit was successfully held on 20-21 January 2022 with over 500 virtual attendees and 21 renowned speakers. The summit focused on the burden of delayed diagnosis and initiatives to address this challenge and discussed the importance of rare disease policy to improve access to the standard of care for rare diseases.



The 2nd Southeast Asia Rare Disease Summit was held on 18 March 2024 in Bangkok, Thailand and featured a series of topics and panel discussions on future for national access policy, crucial role of patient organizations, sustainable diagnosis and access to innovative treatments, and patient centric access policy. Furthermore, several best practices sharing were able to engage the audience on the topic of optimal approaches on innovative funding and setting up for success in rare disease care. The summit has opened new vistas for all participants with many opportunities to meet in person with rare disease partners, alliances, and expand networks.



Rare Disease Management in SEA: Current Policy Landscapes and Future Directions

Khoo Yoong Khean, Assistant Professor, The Centre of Regulatory Excellence (CoRE), SingHealth Duke-NUS Global Health Institute (SDGHI) and The Centre for Outbreak Preparedness (COP) at The Duke-NUS Medical School, Singapore

Context: Inaugural session of Southeast Asia Rare Disease Summit

Focus: : Examine the current landscape of rare disease (RD) management policies in Southeast Asia, with a focus on detailed country-specific analysis. The session aims to identify existing policy gaps and outline future directions to address unmet needs in RD policies, thereby enhancing patient care and healthcare system sustainability.

Highlights:

- Elaborate on the current policies for rare disease management across Southeast Asia, highlighting both strengths and potential gaps.
- Share experiences and critical steps to advance RD policies, focusing on improved patient outcomes and sustainable healthcare systems.
- Propose actionable pathways for policy enhancement and implementation.

Best practices : National Rare Disease Funding

Assoc. Prof. Cherdchai Nopmaneejumrulers, Chairman Working Group on the Development of Rare Disease Healthcare Services, NHSO Deputy Dean of Service Innovations and Corporate Value, Faculty of Medicine, Siriraj Hospital, Mahidol University

Focus: Examine effective strategies and models for funding rare disease (RD) programs at the national level. Provide insights into how sustainable funding can support RD research, patient care, and healthcare system improvements.

Highlights: Despite the high-cost therapies, in 2020, National Health Security Office (NHSO) introduced the treatment for 24 rare diseases, known as inherited metabolic disorder or inborn errors of metabolism, into Universal Coverage Scheme (UCS)'s benefits package.

- Share successful national funding programs for rare diseases, highlighting key components that contribute to their effectiveness.
- Explore different funding mechanisms, including government grants, public-private partnerships, and philanthropic contributions.
- Showcase how targeted funding has led to advances in genetic research, early diagnosis, and comprehensive care for RD patients.
- Present case studies demonstrating the impact of robust funding on patient outcomes, healthcare services, and overall rare disease management.
- Encourage dialogue on best practices and innovative approaches for mobilizing resources and ensuring long-term sustainability of RD funding initiatives.

Panel Discussion: Developing a Robust Policy Framework for Rare Diseases - The Critical Need for Specialized Policies

Moderator: Khoo Yoong Khean, Assistant Professor, The Centre of Regulatory Excellence (CoRE), SingHealth Duke-NUS Global Health Institute (SDGHI) and The Centre for Outbreak Preparedness (COP) at The Duke-NUS Medical School, Singapore

Panelists:

- TH; **Prof. Duangrurdee Wattanasirichaigoon**, Professor, Division of Genetics, Department of Pediatric, Faculty of Medicine, Ramathibodi Hospital, Mahidol University and Vice President of Thai Rare Disease Foundation
- VT; **Dr. Dang Viet Hung**, President of Hanoi University of Pharmacy
- MY; **Dr. Tae Sok Kun**, Clinical Geneticist and Consultant Paediatrician, Genetic and Metabolism Unit, Head of Paediatric Unit, University of Malaya
- PH; **Dr. Carmencita D. Padilla**, Emeritus Professor, University of the Philippines Manila and Chair, Philippine Society for Orphan Disorders

Focus: Explore the necessity of developing a robust policy framework for rare diseases (RD) that is tailored to the unique contexts of Southeast Asia (SEA). Discuss the challenges and opportunities in creating specialized policies for RD management in the region.

Highlights:

- Discuss the role of government, healthcare institutions, and patient advocacy groups in advocating for and implementing specialized RD policies.
- Identify critical elements required for an effective RD policy framework in SEA, considering the diverse healthcare systems and resource availability.
- Provide insights into collaborative strategies to overcome common barriers in policy development and implementation.
- Foster a dialogue on innovative approaches and tools that can support the creation and sustainability of RD policies in SEA.

Patient Advocacy for Policy Change: the ASEAN Rare Disease Consortium (ARdC) roadmap & outcomes

Rajakanth Raman, Programme Director, Rare Disorders Society (Singapore), Visiting Expert, Centre for Regulatory Excellence (CoRE) Duke-NUS Medical School

Focus: Explore the role of patient advocacy in driving policy change for rare diseases within the ASEAN region through the initiatives led by the ASEAN Rare Disease Consortium (ARdC). Present the roadmap developed by ARdC to influence policy changes and improve outcomes for rare disease patients.

Highlights:

- Discuss the strategic goals and objectives of the ASEAN Rare Disease Consortium (ARdC) in advocating for rare disease policy changes.
- Share key milestones and achievements of the ARdC roadmap in promoting better healthcare policies for rare disease patients.
- Analyze the impact of patient advocacy efforts on policy development, highlighting successful case studies within ASEAN member countries.
- Delve into the collaborative efforts between patient advocacy groups, healthcare providers, and policymakers to enhance rare disease management.
- Outline future plans and initiatives by ARdC to continue driving policy changes and improving patient outcomes across the ASEAN region.

Collaborative Achievements: Innovation Partnership for Rare Disease Impact

Thai RD MOU; **Prof.Thanyachai Sura**, President, Medical Genetics and Genomics Association (MGGA)

Thai RD MOU; **Assoc.Prof.Hiroshi Chantaphakul**, President of The Allergy, Asthma, and Immunology Association of Thailand (AAIAT)

Thai RD MOU; **Preeya Singhnarula**, President of Thai Rare Disease Foundation

Focus: Highlight the impact of the Thai RD Memorandum of Understanding (MOU) on fostering innovative partnerships and collaborations to address rare disease challenges. Discuss the achievements and outcomes resulting from these collaborative efforts in the realm of rare disease management and research.

Highlights:

- Showcase successful initiatives and projects undertaken as part of the Thai RD MOU that have led to tangible improvements in rare disease diagnosis, treatment, and patient care.
- Analyze the role of each participating organization in driving innovation and contributing to the overall success of the partnership.
- Present case studies that exemplify the benefits of interdisciplinary and cross-sector collaboration in rare disease research and management.
- Discuss the challenges faced in building and sustaining such collaborations and share strategies to overcome them.

Empowering patient voice for Rare disease policy reform

Thitimon Burapa-arayahwong, President of HAE
Thailand

Focus : Emphasize the importance of incorporating patient voices in the process of policy reform for rare diseases. Share the personal experiences and challenges faced by Rare Disease patients to underline the need for patient-centered policies.

Highlights:

- Share personal stories and experiences firsthand, illustrating the daily struggles and unique challenges of living with Gaucher Disease.
- Highlight the impact of existing policies on the quality of life for Gaucher Disease patients, and identify gaps that need addressing.
- Advocate for increased patient involvement in policy-making processes to ensure that policies are reflective of real patient needs and experiences.

Dr. Carmencita D. Padilla

Emeritus Professor, University of the Philippines Manila Chair, Philippine Society for Orphan Disorders

Assoc. Prof. Cherdchai Nopmaneejumrulers

Chairman, Working Group on the Development of Rare Disease Healthcare Services, NHSO
Deputy Dean of Service Innovations and Corporate Value, Faculty of Medicine,
Siriraj Hospital, Mahidol University

Dr. Dang Viet Hung

President of Hanoi University of Pharmacy

Dion Warren

Area Head of India & Southeast Asia (I-SEA), Takeda

Prof. Duangrurdee Wattanasirichaigoon

Professor, Division of Genetics, Department of Pediatrics, Faculty of Medicine, Ramathibodi Hospital, Mahidol University and Vice President of Thai Rare Disease Foundation

Assoc. Prof. Hiroshi Chantaphakul

President of The Allergy, Asthma, and Immunology Association of Thailand (AAIAT)

Dr. Khoo Yoong Khean

Assistant Professor, The Centre of Regulatory Excellence (CoRE), SingHealth Duke-NUS Global Health Institute (SDGHI) and The Centre for Outbreak Preparedness (COP) at The Duke-NUS Medical School, Singapore

Asst. Prof. Thanin Wechapinan, M.D.

Deputy Director General, Department of Medical Services, Ministry of Public Health, Thailand

Peter Streibl

General Manager, Takeda Thailand

Preeya Singhnarula

President of Thai Rare Disease Foundation

Dr. Rajakanth Raman

Programme Director, Rare Disorders Society (Singapore),
Visiting Expert, Centre for Regulatory Excellence (CORE), Duke-NUS Medical School

Dr. Tae Sok Kun

Clinical Geneticist and Consultant Pediatrician, Genetic and Metabolism Unit, Head of Pediatric Unit, University of Malaya

Prof. Thanyachai Sura

President, Medical Genetics and Genomics Association (MGGA)

Thitimon Burapa-arayahwong

President of HAE Thailand

Dr. Carmencita D. Padilla

Emeritus Professor, University of the Philippines Manila Chair,
Philippine Society for Orphan Disorders

Dr. Carmencita Padilla is an Emeritus Professor, College of Medicine, University of the Philippines Manila and National Scientist of the National Academy of Science and Technology. She is an international consultant on rare diseases and newborn screening (NBS).



Biography:

She is a pioneer in genetics in the Philippines and the Asia Pacific Region. She is a Commissioner of the Lancet Commission on Rare Diseases and a Member, Global Commission to End the Diagnostic Odyssey for Children with a Rare Disease. She initiated NBS for the country and has developed a sustainable NBS system that serves as a model for developing programs. She is responsible for the lobby efforts and passage of the Newborn Screening Act of 2004 (Republic Act [RA] No 9288) and the Rare Disease Act of 2016 (RA No 10747). She has received various awards for her lifetime works and has more than 180 publications.



Assoc. Prof. Cherdchai Nopmaneejumrulers

Chairman, Working Group on the Development of Rare Disease Healthcare Services, NHSO Deputy Dean of Service Innovations and Corporate Value, Faculty of Medicine, Siriraj Hospital, Mahidol University

Current position:

- Associate Professor in Respiratory Medicine
- Deputy Dean for Service Innovation and Organization Value

Biography:

Associate Professor Cherdchai Nopmaneejumrulers, MD received his training in medicine and Diplomate Thai-Board of Respiratory Medicine (2000) at Siriraj Hospital, Mahidol University, Bangkok Thailand and Research and Clinical Fellow in Respiriology 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 Nopmaneejumrulers, 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. Dang Viet Hung

President of Hanoi University of Pharmacy

Biography:

Dr. Hung is the President and Chairperson of the University Council at Hanoi University of Pharmacy in Hanoi, Vietnam. His previous roles include the General Director of the Department of Health Insurance as well as the General Director of the Department of International Cooperation at the Ministry of Health in Vietnam. With extensive experience in both academia and in the public sector, Dr Hung's research interests include health system efficiency, health system strengthening and health economics, among others.



Dion Warren

Area Head of India & Southeast Asia (I-SEA), Takeda

Biography:

Dion Warren is the Head for the I-SEA region for the Growth & Emerging Markets Business Unit (GEM BU), responsible for Takeda's India and Southeast Asia markets. He was previously Head of the US Oncology Business Unit (US OBU) at Takeda Pharmaceuticals and served as a member of the Global Oncology Leadership Team.

In that role, Dion led Takeda's efforts in the US to accelerate our capabilities, culture and commercialization of our medicines with life-transforming potential. During his 20 years at Takeda, Dion has held several global and diverse roles with increasing responsibility and was a key leader in the acquisition of Millennium. Prior to becoming Head of the US OBU, Dion was Head of the Europe and Canada OBU based in Zurich, where he played a leading role in successfully globalizing Takeda Oncology.

Dion also served as Head of Oncology for Emerging Markets based in Singapore, where he built the oncology team and business across approximately 50 countries and helped develop Takeda's industry-leading Access to Medicines program. Beyond these leadership positions, Dion has also worked in various critical roles across Takeda Corporate Strategy, Business Development, Marketing, Investor Relations, and Financial Planning & Analysis. Dion holds Bachelor of Science degree in Finance and Real Estate from Florida State University.



Prof. Duangrurdee Wattanasirichaigoon

Professor, Division of Genetics, Department of Pediatrics,
Faculty of Medicine, Ramathibodi Hospital, Mahidol University
and Vice President of Thai Rare Disease Foundation

Biography:

Duangrurdee Wattanasirichaigoon, is currently a Professor of Pediatrics of the Division of Medical Genetics, Department of Pediatrics, Faculty of Medicine Ramathibodi Hospital, Mahidol University.

She is a co-founder and currently the Vice President of Thai Rare Disease Foundation (ThaiRDF),

Vice President of Thai Medical Genetics and Genomics Association (TMGGA).

Prof. Duang obtained her medical degree from Faculty of Medicine, Khon Kaen University; Board of Pediatrics from Ramathibodi Hospital; Diploma of the American Board of Medical Genetics in Clinical Genetics and Clinical Molecular Genetics, from the Boston Children's Hospital, Harvard University, MA (1999). Her researches involved clinical and molecular aspects of various rare genetic diseases; inborn metabolic disorders including lysosomal storage diseases, especially Gaucher disease, and citrin deficiency; genetics of congenital hearing loss. Prof. Duang has been actively involved in rare disease policy advocacy in Thailand since 2011 and in promoting/supporting several patient organizations.



Assoc. Prof. Hiroshi Chantaphakul

President of The Allergy, Asthma, and Immunology Association of Thailand (AAIAT)

Biography:

Dr. Hiroshi Chantaphakul, M.D., FAAAAI, is the President of the Allergy, Asthma, and Immunology Association of Thailand (AAIAT) and leads the Division of Allergy and Clinical Immunology at Chulalongkorn University. He completed his medical education at Chulalongkorn University and postgraduate training in the U.S., earning board certifications in Allergy/Immunology, Rheumatology, and Internal Medicine. Actively involved in clinical research, policy development, and international collaborations, he has contributed to key guidelines and publications in allergy and immunology while serving in national and global organizations such as WAO.



Khoo Yoong Khean,

Assistant Professor, The Centre of Regulatory Excellence (CoRE), SingHealth Duke-NUS Global Health Institute (SDGHI) and The Centre for Outbreak Preparedness (COP) at The Duke-NUS Medical School, Singapore

Biography:

Dr. Khoo Yoong Khean is a medical doctor by training with more than 15 years of clinical and public health experience. He started his career as a physician in Malaysia in primary healthcare, emergency and trauma medicine, nephrology, cardiology and infectious diseases.

He is currently an Assistant Professor at the Centre of Regulatory Excellence (CoRE), SingHealth Duke-NUS Global Health Institute (SDGHI) and the Centre for Outbreak Preparedness (COP) at the Duke-NUS Medical School, Singapore. He is also an advisory board member of the Asia Pacific Oncology Alliance. He obtained his Master of Business Administration in 2022.

Yoong Khean focuses on global health policy and health systems strengthening through policy development and capacity-building efforts. His areas of research and interests also include patient engagement in health systems, access to medicine and healthcare services and regulatory systems strengthening.



Asst. Prof. Thanin Wechapinan, M.D.

Deputy Director General, Department of Medical Services,
Ministry of Public Health, Thailand

Biography:

Asst.Prof. Thanin Wechapinan is the Deputy General Director of the Department of Medical Services at the Ministry of Public Health and The Former Director of the Neurological Institute of Thailand. A distinguished neurologist and academician, he has made significant contributions to healthcare education, research, and public health. Asst.Prof. Thanin has played a pivotal role in advancing neurological care, including spearheading collaborations with leading institutions such as Mahidol University and B. Braun (Thailand). He has co-authored research on rare neurological conditions, such as Schwartz-Jampel Syndrome, and is an advocate for public health, particularly addressing the impacts of air pollution on children's health. Through his leadership, clinical expertise, and dedication to improving healthcare systems, he continues to drive progress in neurology and public health in Thailand.



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 leadership as a General Manager at Takeda Thailand, he has been an integral part in forging public-private partnership towards driving equitable access and raising standard of care in rare diseases and beyond.

He is also committed to contribute to the Thai healthcare industry with a strong support to solidify its position as a leading global medical hub.

Peter is also serving as the Vice President of the Pharmaceutical Research and Manufacturers Association (PREMA) of Thailand and with its 30 innovative bio pharmaceutical member companies to advocate for better access to innovative medicines.

In 2019, a dedicated Rare Disease Memorandum of Understanding, a 5-year framework to raise the standard of care in rare diseases, where 8 prominent partners have entered into this partnership alongside Takeda Thailand. Through the efforts from all partners, the 4 centre of excellence sites were implemented for Hereditary Angioedema (HAE) diagnosis with C1-INH testing facilities and screening, representing a significant step forward in our commitment to improving the lives of individuals affected by HAE.

Peter holds a Master's Degree in Business Administration from the Vienna University of Economics and Business Administration.



Preeya Singhnarula

Foundation/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.



Dr. Rajakanth Raman

Programme Director, Rare Disorders Society (Singapore),
Visiting Expert, Centre for Regulatory Excellence (CORE),
Duke-NUS Medical School

Biography:

Raj has 30 years of experience in healthcare advocacy, policy and governance in the Southeast Asian region. He has created and led specific illness patient access programmes, and initiated and built extensive patient care networks comprising policymakers, industry players, healthcare professionals and patients. Raj's expertise also includes creating solutions for multiple stakeholders in economically and culturally diverse countries in the Asia Pacific region



DR TAE SOK KUN

Clinical Geneticist and Consultant Paediatrician
Genetic and Metabolism Unit, Service
Department of Paediatrics, Faculty of Medicine
University of Malaya

Biography:

Dr Tae Sok-Kun is a clinical geneticist, consultant pediatrician, and senior lecturer at Genetic and metabolism unit, Department of Paediatrics, University of Malaya and currently heading the unit. She studied medicine at University of Malaya and obtained her postgraduate qualification from Royal College of Paediatric and Child Health (RCPCH), United Kingdom. She received genetic training and completed her fellowship in Clinical Genetics at the Victorian Clinical Genetics Service (VCGS) in Melbourne, Australia.

Dr Tae is involved in diagnosing and managing patients with rare diseases which include gene therapy and genetic counselling for patients. She is currently embarking on several research projects as principal/co-investigator for a few research grants. She took part in a few clinical trials for genetic diseases. She has published a few peer-reviewed papers on rare genetic conditions and co-authored a book chapter. She presented several posters and oral presentations at national and international scientific meetings. She is involved in both undergraduate and postgraduate training in the field of medicine, paediatric and genetic counselling.

Her major area of interests includes preventive and curative strategies for genetic disorders, genetic counselling, health economy in genomic medicine, medical education and access to genetics and genomics services in developing countries.



Prof. Thanyachai Sura

Country : Thailand

President, Medical Genetics and Genomics Association (MGGA)

Biography:

Current Address and appointment

Medical Genetics and Genomic Medicine, Department of Medicine, Ramathibodi Hospital, Mahidol University, Rama 6 road Bangkok 10400 Thailand

President of Medical Genetics and Genomics Association (Thailand)

President of Ambulatory Medicine Association (Thailand)

Immediate Past President of Asia Pacific Society of Medical Genetics (APSHG)

Subcommittee of Medical Council of Thailand

Foreign subcommittee, Royal College of Physicians of Thailand (RCPT)

Education:

1988 Diplomat of the Thai Board of Internal Medicine, Faculty of Medicine Ramathibodi Hospital Mahidol University

1989 Fellowship of the Royal College of Physicians of Thailand

1991 Certificate in Medical Genetics and Molecular Medicine, Oxford University, England

2019 Fellowship of the Royal College of Physicians and Surgeons of Edinburgh (FCPE)

Research interest

Dr. Sura's main research interest is about using NGS for diagnosis in rare and undiagnosed diseases from his patients and families in medical genetic clinics. He has published over 70 articles in national and international peer-reviewed medical journals, including Human Genetics, Science, Nature, Journal of Clinical Neuromuscular Disease, Journal of Human Genetics, and PLoS One.

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.

Working experience

Head of Medical Genetics and Molecular Medicine, Ramathibodi Hospital 1995-2015

Committee of the Royal College of Physicians of Thailand (RCPT) (since 2016)

Subcommittee of the Thai Medical Council postgraduate training (since 2016)

Cofounders of the Medical Genetics training in Thailand in 2019

Cofounders of Genetic Counselling for healthcare personnel in 2020.



Thitimon Burapa-arayahwong
President of HAE Thailand

Biography:

A highly motivated and dedicated individual with a Bachelor's Degree in Liberal Arts from Thammasat University. Possessing a strong passion for patient advocacy, I serve as the President of the Hereditary Angioedema (HAE) Patient Club in Thailand, where I also act as the National Coordinator for the HAE disease network. My experience as an HAE patient and advocate has equipped me with a deep understanding of the challenges faced by individuals living with rare diseases. I am committed to raising awareness, fostering support, and improving the lives of HAE patients in Thailand and beyond.

Education

Bachelor of Arts (Liberal Arts), Thammasat University

Experience

President, Hereditary Angioedema (HAE) Patient Club, Thailand (2021 – Present)

Lead and manage the HAE Patient Club, overseeing all activities and initiatives.

Serve as the primary point of contact for HAE patients and their families in Thailand.

Collaborate with healthcare professionals, researchers, and policymakers to raise awareness and improve access to care for HAE patients.

Organize and participate in events and campaigns to educate the public about HAE.

Represent the HAE Patient Club at national and international conferences and meetings.

National Coordinator, HAE Disease Network, Thailand

Coordinate and facilitate communication and collaboration among HAE patients, healthcare providers, and other stakeholders across Thailand.

Work to strengthen the HAE care network and ensure that patients receive timely and appropriate care.

HAE Patient (Diagnosed in 2020)

Actively involved in the HAE Patient Club since diagnosis.

Participated in various activities and initiatives aimed at supporting HAE patients and raising awareness about the condition.

Conference Participation

HAEI Global Conference, Sweden (October 2024)

Participated in the conference organized by HAEI Global to share experiences and best practices with HAE patient groups from around the world.

Gained valuable insights into the challenges and opportunities in HAE care and advocacy.

Co-Hosts



Partners





Medical Association of Thailand under His Majesty the king's Patronage

Organization Profile:

The Medical Association of Thailand under the Royal Patronage symbolizes the collaboration and cooperation of every Physician to intertwine their contribution into 'oneness' in order to carry out the constructive activities that surveillance all physicians to practice under the ethical code.

"The Medical Association of Thailand under the Royal Patronage" has been function to promote and develop issues concerning medical studies, researches, including promoting moral and medical ethics among the member physicians. Moreover, this body also has a close collaboration with governmental sectors and medical organizations nationally and internationally. This is to scale up medical knowledge and practices of the members to international standard at present time and in the future.

The Medical Association of Siam was first initiated on the 25th October 1921 by being registered as an association. The temporary office of the organization at that time was located at the Administration Building of Chulalongkorn Hospital Bangkok Thailand. There were 10 senior physicians who had involved in the setting up of this association. Their names were as follows

Naval Colonel M.J.Thavornmongkonwong Chaiyata: Senior Naval Medical Officer, who became a Naval General later on
Colonel Phrayavibul-Ayuravej: Senior Army Medical Officer, Name before royal appointment was Sekh Thamsaroch
Colonel PhraSakda Pholrak: Director of Chulalongkorn Hospital Name before royal appointment was Chuen Phutiphat, later on was Army General and royal appointment was Phraya Damrong Phatta Phattayakhun
Ammart Tho Luang Ayurapattis: Director of Siriraj Hospital, Named before royal appointment was Sai Khojaseni,
Ammart Thri Luang Upphantraphathpisan: Named before royal appointment was Kamchon Bhalangkool, later was Royalty appointed as Phra Upphantraphathpisan
Ammart Thri Luang Vaitayesarangkool: Named before royal appointment was Cheuch Israngkool Na Ayuthaya
Dr. M.E. Barns
Ammart Ek Phraya Vechsithpilas: Dean of Faculty of Medicine, Chulalongkorn University
Colonel M.J. Wallapakorn Worawan
Dr. Leopold Roberte

Contact Information:

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Thai Rare Disease Foundation

Organization Profile:

Why must it be established?

In total, 5 percent of the population will be found. That means there are 3.5 million Thai people with rare diseases. However, in reality, there are no statistics compiled on how many people with rare diseases in Thailand. Therefore, it is a problem in organizing policies and care systems for patients with rare diseases.

In addition, they do not receive funding for medical benefits. Society lacks understanding of disease, shortage of disease experts. Information and knowledge are not widespread. Access to appropriate drug treatment False delayed diagnosis As for the family Patients who lack psychological care and support, patients and their families do not know who to consult or exchange information about the disease or where to find support groups.

"There are 3.5 million Thai people with rare diseases, of which there are more than 7,000 rare diseases."

The challenge is there are more than 7,000 rare diseases, each with its own set of problems and symptoms. For some diseases, there may be only 1-2 cases in the country. It is difficult to create patient groups for each disease. Therefore, grouping all rare diseases under one big umbrella is the reason for the establishment of "Thai Rare Disease Foundation"

Contact Information:

Website: www.thairdf.org

Address: 39/279 Arun Niwet Village, Nawamin 163 Yaek 11, Nuanchan, Bueng Kum, Bangkok, 10230

Tel: +6695 397 5331 , +6685 326 4282

Email: thairaredisease.f@gmail.com



Medical Genetics and Genomics Association (MGGA)

Organization 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..

Promote education, research, and innovation related to medical genetics and medical genomics. and disseminate knowledge about genetics and medical genomics to the public. In order to gain knowledge and understanding of care and prevent genetic diseases appropriately.

Objective:

Promote education, research, and innovation related to medical genetics and medical genomics.

Promote the study of social, ethical and legal impacts related to the development of medical genetics and medical genomics.

Support the development of policies and guidelines to have appropriate advances in medical practice in the field of medical genetics and medical genomics. Taking into account social and ethical impacts

Promote academic work and exchange knowledge between medical genetics doctors, medical personnel and public health officials involved in the Thai context and support increasing the production of practitioners in the fields of medical genetics and medical genomics.

Support activities and cooperation with government agencies and various organizations both inside and outside the country related to medical genetics and medical genomics.

Spread knowledge about genetics and medical genomics to the public. To be aware of the effects of genetic factors on health and gain knowledge and understanding of care and prevent genetic diseases appropriately

Carry out charity work and organize campaign activities to support the dissemination of academic knowledge in medical genetics and medical genomics. for patients, families and society.

Promote, support, and cooperate with other charitable organizations for public benefit

The association does not deal with politics in any way.

Contact Information:

Website : www.tmgga.or

Address : 148/856, Ramkhamhaeng 190, Min Buri, Min Buri, Bangkok, 10150

Email : contact@tmgga.org



The Allergy, Asthma and Immunology Association of Thailand

Organization Profile:

In 1977, the Allergy, Asthma and Immunology Association of Thailand (AAIAT) was founded as a club of allergists and immunologists to meet as a social group with common interests in the field of allergy and immunology in Thailand. Then it became a society and on April 4, 1984, it was legally registered as "The Allergy and Immunology Society of Thailand" (AIST). Since then, we have been publishing "Asian Pacific Journal of Allergy and Immunology", our society's official journal.

Our association, along with other Asian national societies, founded the first Asia regional society, the "Asian Pacific Federation of Allergy and Immunology Society," in 1989, and conducted its first meeting on October 26-29, 1989 in Bali, Indonesia.

Prof. Montri Tuchinda, AIST president, proposed that the federation be expanded to include more members from Southeast Asia and South Asia countries, and that the federation's name be changed to "Asia Pacific Association of Allergy and Clinical Immunology" (APAACI) for the first time in 1992, with Prof. Montri Tuchinda elected as the first president of APAACI. The first congress was held in Bangkok from November 22 to November 26, 1992.

In 1991, AIST collaborated with other immunology societies in Asia to form the "Federation of the Immunological Societies of Asia-Oceania" (FIMSA), with Prof. Dr. Stitaya Sirisinha of Thailand serving as the founding committee and executive committee of FIMSA from 1991 to 1994. FIMSA is also a member of the International Union of Immunological Societies (IUIS). Later in the year, on January 23-27, 2000, AIST convened the second FIMSA Congress in Bangkok, where Prof. Stitaya Sirisinha of Thailand was elected President of FIMSA Councils.

Since then, AIST has expanded its membership to include allergists, immunologists, clinical immunologists, and other medical professionals with an interest in allergy and immunology.

AIST was renamed "The Allergy, Asthma and Immunology Association of Thailand" (AAIAT) in 2010 to reflect its expanded scope of interests, which included allergy, asthma, and immunology at the time.

Vision:

The Allergy, Asthma, and Immunology Association of Thailand will be the nation's most trusted professional and public resource.

Mission:

The Allergy, Asthma and Immunology Association of Thailand will be the National Center for disseminating knowledge, stimulating research and providing standard cares for people and society in the areas of allergy, asthma, and immunology.

AAIAT has a national academic meeting twice a year: in the early spring for the annual meeting and in the early fall for the semi-annual meeting.

AAIAT has hosted several international immunology conferences, including

FIMSA Congresses in 2000 (the 2nd) and 2018 (the most recent) (the 7th)

The 6th FIMSA Advanced Training Course: Molecular Mechanisms of Infection and Immunity in

Phra Nakorn Si Ayutthaya, Thailand, in 2002

The 12th FIMSA Advanced Immunology Training Course: Innate Immunity in Chiang Mai, Thailand, in 2013

Since 2002, AAIAT has organized numerous activities to raise public awareness about primary immunodeficiency diseases, including Global Day of Immunology, as well as assisting in the formation of the Thai Patient Organization of Primary Immunodeficiency (ThaiPOPI), which was founded in 2014.

Contact Information:

Website : <https://allergy.or.th/2016/>

Address : The Allergy, Asthma, and Immunology Association of Thailand, Division of Pediatric Allergy & Immunology, Department of Pediatrics, Faculty of Medicine Siriraj Hospital, Mahidol University

2 Wang Lang Road, Bangkok Noi District, Bangkok 10700, Thailand

Phone : +66 65-608-2983



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.

Contact Information:

Website: www.takeda.co.th

Address: Takeda (Thailand), Ltd.

57 Park Ventures Ecoplex Building, 15th Floor

Wireless Road, Lumpini, Patumwan

Bangkok 10330 Thailand



Pharmaceutical Research & Manufacturers Association

Organization Profile:

A non-profit organization representing members who are research based pharmaceutical companies innovating medicines to combat the previously incurable and to improve on existing treatments.

PReMA works to encourage the Thai people

to become a part of the international pharmaceutical research and development process creating innovation not only for our own enhanced social well-being and national economic benefits but also to the benefit of the world community.

Vision / Mission

"Innovative Medicines. Healthier Life." The Pharmaceutical Research and Manufacturers Association (PReMA) is a non-profit organization representing members who are research based pharmaceutical companies innovating medicines to combat the previously incurable and to improve on existing treatments.

In order that Thai people may benefit from the scientific and technological advancements of innovative medicines equally with the rest of the world, PReMA is devoted ourselves to the support of R&D initiatives which will lead to better healthcare and greater quality of life. In turn the social well-being and economy of the nation will be enhanced.

Contact Information:

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Fax : +662-619-0080
Email : office@prema.or.th
Facebook : PReMA Thailand



Rare Disorders Society Singapore

Organization Profile:

Rare Disorders Society (Singapore) also known as RDSS, is a non-profit organisation initiated by parents of children with LSD (Lysosomal Storage Disorder). Established in 2011, it aims to create awareness about various life threatening rare diseases (RD). RDSS is currently the only organisation focusing and supporting only on rare disease patients in Singapore. Unlike other charities that focus on specific disease group or made rare genetic disease as one of the supported group, RDSS accepts and supports ALL patients currently diagnosed with rare diseases, including those undiagnosed cases.

Through increased awareness of rare diseases among various stakeholders, RDSS aspires to promptly facilitate patients with rare diseases to receive timely intervention, thereby enhancing their quality of life.

We are committed to improving the lives of patients with rare diseases and their families.

Our Mission:

To improve the lives of patients living with rare diseases, and their families. We embrace equity and inclusivity to empower them to live a good quality life.

Our Vision:

To be an empathetic and effective Patient Organisation. Currently, we care for more than 180 beneficiaries and those living with them, impacting more than 700 lives.

Contact Information:

Website : <https://www.rdss.org.sg/>

Phone : +65 6402 2898

Email : contact@rdss.org.sg

Facebook : Rare Disorders Society (Singapore)