

## The Second Primary Immunodeficiency Meeting in Thailand: From Bench to Bedside 4<sup>th</sup> December, Bangkok, Thailand



### Report

IPOPI, Care4Rare, Chulalongkorn University Hospital and Erasmus Hospital co-organised a meeting in Bangkok on the 4th December 2017. The meeting was held to spread knowledge about the latest updates on PID diagnostics and treatments. It brought together 80 healthcare professionals from different regions of Thailand together with 10 doctors from Indonesia who virtually took part in the lectures through a video conference system.

The lectures were provided by several international PID experts from Europe and South-East Asia, touching upon topics such as haematopoietic stem cell transplantation, SCID newborn screening, PID registries, international collaboration in research and legal perspectives to international data sharing.

The opening remarks of the meeting were given by the Dean of the faculty of medicine at Chulalongkorn University, **Prof Suttipong Wacharasindhu**, the Head of the Paediatrics Department, **Prof Siriwan Wanankul** and the President of the Allergy, Asthma and Immunology Society of Thailand, **Prof Charelat Direkwattanachai**. The opening remarks highlighted the goal to have early diagnosis and appropriate treatment for all PID patients across the country by the year 2020. Since the partnership with Erasmus, Care4Rare and IPOPI, Chulalongkorn Hospital has become one of the leading hospitals in South East Asia. Prof Wacharasindhu, Prof Wanankul and Prof Direkwattanachai all expressed their hopes that the meeting will help update the knowledge of the Thai specialists on PIDs and further improve the quality of life of PID patients in Thailand. Finally, they all thanked the meeting organisers and the speakers and welcomed the audience.

The first lecture of the day was given by **Prof Martin van Hagen** from Erasmus University, Rotterdam, about PID, allergy and autoimmune diseases: different sides of the same coin. He went over the most common signs and characteristics of the diseases and some of the known gene mutations. He introduced the audience to the closely PID related concept of “triple A”, which include the autoinflammatory,

autoimmune, and allergic diseases. Finally, he presented some case examples of the diseases and stressed the importance of identification of the related gene and comorbidities to be able to offer more precise medication for the patients.

The next presentation was by **Dr Wim Dik** from Erasmus Hospital, who gave an overview of specific antibody testing for primary immunodeficiency. He introduced the audience to the bead-based multiplex immunoassay (MIA) and went over the most used vaccination responses and the challenges in using this method for PID diagnosis. The currently used criteria have huge challenges, since the introduction of the national vaccination program. Following the program, sometimes the vaccination responses do not meet the currently used criteria, even for completely healthy patients. Dr Dik concluded that when conducting antibody testing it is important to check the patient's vaccination status.

The next presentation, "Newborn screening for severe combined immunodeficiency (SCID)" was given by **Dr Fabian Hauck** from Ludwig-Maximilians-University of Munich. He went over the definition and different manifestations of the SCID and showed the road map leading to diagnosis of SCID in Germany. His presentation was followed by **Mr Johan Prevot** from IPOPI, who gave an overview of Newborn screening program from IPOPI perspective. Mr Prevot gave examples of some of IPOPI's SCID NBS Campaigns and the progress made over time. He highlighted that SCID is a paediatric emergency that can be diagnosed, treated and even cured in most cases. He concluded that IPOPI would be happy to support any advocacy efforts aiming at implementing NBS for SCID in South East Asia, necessitating that the treatment (BMT) for the disease is in place.

**Dr Narissara Suratannon** from Chulalongkorn University, Bangkok gave a presentation with an update on managing a PID registry in South East Asia. She went over the biggest challenges in terms of PIDs for SEA countries: the limited diagnostic facilities, access to treatment and lack of specialists. She stated that despite the challenges, given that the collaboration in the area is close, it is feasible for patients and samples to move from country to country to get the diagnosis or care they need. She went over the SEAPID network's mission and gave examples of the collaboration between the national patient groups. Finally, she gave an overview of the ongoing collaborative studies in SEA and gave examples of the regional specificities of PIDs.

The first presentation of the second half of the meeting was given by **Dr Nicole Kien** from Erasmus Hospital, Rotterdam on the topic of "International Patient Data Traffic: The Legal Perspective". She gave an overview of the EU regulation that can be applicable in other places as well, and should be when handling data of patients from EU. She explained the difference between anonymized and pseudomized data, the rights of the subject and the responsibilities of the data controller and processor. She concluded that a solution for data transferring between countries that have different data protection laws could be Binding Corporate Rules (BCR).

The next presentation, "Optimizing treatment outcomes: hematopoietic stem cell transplantation for PID", was provided by **Prof Michael Albert**, Ludwig-Maximilians-University of Munich. He went briefly over the transplant history in Europe and stated that to this day there have been over 5000 transplants conducted in Europe. He stressed that since better conditioning and new post-transplant medication, the complications for matched unrelated donor transplants have reduced significantly and the outcomes of the transplants have improved drastically. He touched upon the ESID guidelines for HSCT for PID and highlighted that with new techniques and new data the transplants can be tailored by disease and alternative donors can be used successfully.

**Prof Suradej Hongeng** gave a presentation on "Experiences on hematopoietic stem cell transplantation for PID in Thailand". In his presentation he mentioned that for Thalassemia, the survival rate after transplant is 95 % in Thailand, but that for SCID, there have been only 8 transplants conducted, of which two have been successful. He went over treatment regimens in Thailand and showed some case study

examples. Finally, he mentioned that in Thailand, there is a donor registry managed by the Red Cross and stressed that HSCT guidelines for PID are urgently needed for Thailand.

**Dr Virgil Dalm** from Erasmus Hospital, Rotterdam gave a presentation on gene-targeted therapy in PID. In his presentation he highlighted the benefits of identifying the gene for PID treatment. Through gene identification, the patients can avoid serious organ damage and other long-term harm that cannot be treated with available medicines. He stated that scientists are acquiring more knowledge on underlying genetic defects in PID, which may provide a basis for gene-targeted or pathway-targeted treatment approach. Finally, he gave a case example on how, by identifying the gene defect of a CVID patient, the therapy was adjusted which lead to improved quality of life for the patient.

The last presentation of the day was given by **Dr Nipan Israsena** from Chulalongkorn University, Bangkok. He gave an overview on primary immunodeficiency modelling with induced pluripotent stem cells. He went over a recent study conducted in Chulalongkorn University Hospital about using induced pluripotent stem cells in gene correction to treat Wiscott-Aldrich syndrome (WAS). He concluded that this procedure could be a solution for patients without HLA-matched donors since nonidentical bone marrow transplantation has been shown to be associated with significant comorbidity.

After the presentations there were discussions between the participants. The meeting was well received and successful in updating the knowledge of PID specialists in Thailand and in Indonesia. During the meeting, the hospital held a press conference on the current situation of PID patients in Thailand. After the meeting, the medical societies stated their hopes to initiate a SCID newborn screening campaign in Thailand, to which IPOPI offered their support.



## Symposium for Physicians & Healthcare Professionals



December 4<sup>th</sup>, 2017 ; 08.15 – 16.30; Venue: Mongkolnavin Conference Room, 10<sup>th</sup> Floor

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