

## IPOPI National Clinical Care Symposium and 2<sup>nd</sup> IPOPI National PID Patients & Doctors Meeting Hanoi, Vietnam, 8-9 December 2017



IPOPI organised two meetings in Hanoi, Vietnam on December 8-9, 2017, in close collaboration with the National Children's Hospital and the Paediatric Association of Vietnam. The first meeting took place in the National Children's Hospital in Hanoi and aimed at educating healthcare professionals and medical students about primary immunodeficiencies (PIDs). The second meeting was aimed at PID patients from all over Vietnam with a programme consisting of educational lectures and discussions.

### Country update:

- Number of PID patients: 200 (information from PID centres)
- PID registry in National Children's Hospital, Hanoi
- Immunoglobulins (IVIG) are reimbursed for children under 6 years
- Children over 6 years of age: 80-95% of the cost of Ig is covered by the government, depending on financial status
- Adults: 30-95% of the cost of Ig is covered by the government, depending on financial status
- BMT/HSCT for PIDs is available in one hospital in Hanoi, 50% covered by the government for the recipient
- Subcutaneous Ig has been approved but not yet reimbursed by the government

## 1 PID Clinical Care Symposium, 8 December

The first meeting, PID Clinical Care Symposium was successful in gathering 120 healthcare professionals and medical students from Hanoi and the region, together with 18 satellite hospitals connected virtually, to listen to lectures on the latest updates in the field of PIDs. Several international and local key opinion leaders with the focus on PIDs came to share their knowledge with the future and present physicians of the country. The meeting was the first of its kind in Vietnam and had a wide audience which will help to raise awareness of PIDs in the country.

The welcome remarks (**1<sup>st</sup> Session**) of the meeting were given by the Director of the National Children's Hospital in Hanoi, **Prof Le Thanh Ha** followed by a few welcome words from IPOPI's President, **Ms Jose Drabwell**. They both extended a warm welcome to the participants expressing their satisfaction with the great number of listeners and thanking each other for the close collaboration in making the meeting possible.

### **2<sup>nd</sup> Session: Diagnosis and management of PIDs**

The first lecture of the day was given by **Dr Teresa Espanol**, the honorary vice-chair of the IPOPI Medical Advisory Panel from Barcelona, with a presentation on how to suspect and diagnose PIDs with non-expensive tests. She briefly explained the functions and components of the immune system and went over the most classical diagnostic tests and approaches. She proceeded to give case examples on different manifestations of PIDs such as combined immunodeficiencies, hypogammaglobulinemia, CVID and CGD. Finally, she highlighted that the knowledge on the PID types and checking the family history is very important in the detection of PIDs.

The second lecture was provided by **Dr Wim Dik** from Erasmus Hospital, Rotterdam with a presentation on PIDs and vaccinations. He introduced the audience to a 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, after the introduction of the national vaccination program. Since the implementation of 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.

**Dr Nizar Mahlaoui**, the chair of IPOPI Medical Advisory Panel from Necker Hospital, Paris gave a presentation on "Management of PID registries – ESID experience". He went over the features and the different levels of the ESID registry and showed some examples of studies using data from the registry. He gave some advice based on his experience on how the registry could be managed in low income countries, what to bear in mind and what kind of challenges could be faced. Finally, he highlighted the importance of the existence of international registries for getting data on the basic statistics of PIDs, e.g. prevalence/incidence, delay on diagnosis, access to treatments and quality of health care. All these are important data for research but also for national advocacy purposes.

**Prof Martin van Hagen** the vice-chair of IPOPI Medical Advisory Panel from Erasmus Hospital, Rotterdam provided a presentation on primary immunodeficiencies in various medical disciplines. In his lecture he went over the "triple A" related to PIDs: autoinflammatory, autoimmune, and allergic diseases. He highlighted that PID patients often suffer from organ damage due to the triple A diseases which can lower the quality of life for the patient. Finally, he presented some case examples of these diseases and stressed the importance of taking into consideration the related gene and co-morbidities to be able to offer more precise medication for the patients.

The final lecture of the session was given by **Prof Lennart Hammarstrom** from Karolinska Institute Sweden. His presentation provided an overview of PIDs and genetics - a multi-omic approach to primary immunodeficiency disorders. He went over some common types of PIDs and detected genes related to

them. He highlighted the importance of detection of the defected gene in PID diagnosis and treatment. He gave the example of common variable immunodeficiency (CVID) where many different genes are involved, and more are found each year. He introduced whole exome sequencing (WES) and whole genome sequencing (WGS) and their pros and cons in gene detection. Finally, he highlighted that without the genetic diagnosis patients may receive the wrong type of treatment.

### **3<sup>rd</sup> session: Case Studies**

The session kick-started with a lecture by **Dr Nizar Mahlaoui** about PID transition care. He started his presentation with giving some survival improvements in some diseases in the last decade through newborn screening. He stressed that since PIDs are often diagnosed at the paediatric stage, these patients will need to be transferred to adult care when the time comes. He gave some definitions of transition care and timelines when it is usually introduced and implemented, and highlighted the importance of the broad network and collaboration of disciplines to make the transition possible. Finally, he stated some key points to take into consideration in order to make the transition as comfortable and successful as possible for the patient.

The second lecture was given by **Dr Teresa Espanol** with a presentation on “Experience on PID diagnosis and therapy: case study examples”. She further explored her previous topic by giving several case study examples which steps have led to PID diagnosis and appropriate therapy from her own experience in Spain, ranging from ‘easy’ cases to diagnosing more complicated cases including defects of the innate system. She highlighted the guidelines on the careful use of different types of vaccines in PID patients. Finally, she briefly went over the improvements of PID treatment in Spain over the last 10 years and the increasing use of gene therapy to treat PIDs.

**Prof Martin van Hagen** gave a lecture on the immune system and auto-inflammation. In his presentation he first introduced the audience to the acute phase proteins, C-reactive proteins and the concept of autoinflammation. He then went over examples of different types of autoinflammatory syndromes such as monogenetic syndromes, polygenetic syndromes and other rare and common diseases. He concluded that autoinflammatory diseases are rare and predominately monogenetic diseases, whereas inflammatory diseases are mostly combined autoimmune-autoinflammatory diseases. He stated that many metabolic diseases may have an autoinflammatory component and that through autoinflammation mechanisms we can learn more about inflammatory diseases and even generate new therapies and extend the use of available therapies.

The last lecture of the session was given by **Dr Virgil Dalm** from Erasmus Hospital, Rotterdam. His presentation touched the topic of “Novel treatment strategies for primary immunodeficiencies”. He went over the currently available treatment options for PIDs and highlighted the benefits of identifying the defected 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 and improved the quality of life for the patient.

### **4<sup>th</sup> session: Regional updates on PIDs**

The first presentation of the session was given by **Dr Narissara Suratannon** from Chulalongkorn University Hospital, Bangkok. She gave an overview of PID management in South East Asia from the SEAPID perspective. 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 reach the appropriate diagnosis or care. 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 second presentation was provided by **Prof Le Thi Minh Huong** from National Children's Hospital (NCH), on the topic of PID management in Hanoi. She went over the historical development since the first PID case was diagnosed in 2008: so far there are over 100 diagnosed PID cases in Hanoi according to the registry of the NCH. The hospital is the only one in the country that performs HSCT (first time in 2014) and they have also started to provide genetic diagnosis for the patients. She mentioned the support received from centres in Japan, Netherlands, USA and Hongkong towards the department that has made the progress possible. She was also pleased to announce that the first PID patient was recently successfully transferred from paediatric to adult services. Finally, she went over the current priorities of the immunology department in terms of PIDs which included training of medical staff, availability of SCIG, speciality tests to better diagnose patients, national PID registry, community awareness and eventually newborn screening for SCID.

The last presentation of the day was given by **Dr Nguyen Minh Tuan** from Children's Hospital 1. He gave an overview of the PID management in Ho Chi Minh City. Currently the hospital has diagnosed 70 patients with a PID using the basic diagnostic tests available. He stated that because of the very high cost of the tests, if families are not certified as "poor" or lack a health insurance, they cannot afford to get affirmative genetic diagnosis (which is self-covered in Vietnam) and treatment. In Ho Chi Minh City, currently there is no immunology department and PIDs are treated in haematology, but there are hopes to establish such a department in the future. He stressed the importance of comprehensive care and education of medical staff, nurses and PID patients and their families to ensure successful and compliance of treatment. Finally, he went over the challenges for PID treatment in Vietnam and highlighted that through collaboration, e.g. with SEAPID, it is possible to improve the diagnosis and management of PID diagnosis on the national level.

Finally, there was a Q&A session followed by a discussion. The meeting was closed by **Prof Le Thi Minh Huong** who thanked all the speakers, organisers and participants who made the meeting possible.

## 2 The Second National Patients & Doctors Meeting, 9 December 2017



The Second National Patients & Doctors meeting was a half day meeting and brought together more than 100 participants including patients and their family members, nurses and PID specialists from different regions of Vietnam. The meeting consisted of educational lectures about the immune system, updates on PID care in Vietnam, plasma collection and manufacturing as well as a presentation from IPOPI and two other patient organisations, MyPOPI (Malaysia) and the Vietnam Haemophilia Association.

The meeting was opened with remarks by **Prof Nguyen Gia Khanh**, the President of the Vietnam Paediatric Association. He extended his appreciation to IPOPI and Kedrion for making this important meeting possible. He stated that through meetings like this, the challenges regarding PID diagnosis and treatment can be discussed and addressed in Vietnam. Finally, he welcomed all the participants and invited the first speaker to the podium.

The first lecture of the day was provided by **Dr Teresa Espanol** from Barcelona. She introduced the patients to the functions of the immune system and primary immunodeficiencies. She explained the purpose of T-cells, B-cells and their interaction. She proceeded to explain the function of immunoglobulins and the immunological memory. Finally, she gave an overview of the most common manifestations of PIDs and available treatment options to the most common PIDs.

The second presentation was provided by **Ms Jose Drabwell**, Chair of IPOPI. Her presentation gave examples on the achievements of national patient groups on a global level and their role in advocating, raising awareness and educating the patient community. She gave examples of advocacy campaigns implemented successfully and gave an overview of IPOPI support and tools towards its membership. Finally, she concluded that it is important to work together with many stakeholders to reach best outcomes and expressed her hopes to reach a consensus of the next steps for VietPIPS at the end of the meeting.

The next lecture was given by **Dr Thuc Thanh Huyen** from the National Children's Hospital in Hanoi. She provided an update on PID care in Vietnam for the patient audience. She highlighted the fact that even with some improvements, there is still a lot of late and misdiagnosis in PIDs which lead to high mortality of the patients. She stated that based on the current international data, in Vietnam there should be around 75 000 PID patients relative to the 200 currently diagnosed. She went over the PID diagnosis

process and the available tests and treatments. She stressed some common challenges that PID patients face in Vietnam, such as long distances to treatment centres and the economical losses due to time taken off from work and school. Finally, she gave an overview of the latest achievements of VietPIPS and the future key issues to address through the patient group.

The next speech was provided by **Ms Bui Huong Giang** from the Vietnamese PID patient organisation, VietPIPS. She highlighted the fact that the time spent at the hospital for infusions is a time where the parents usually get to discuss and share experiences of their children with PID. She stated that this is very important, but over time she has noticed how it has become more and more important to expand the activities of the patient community to regular meetings to update each other on advances made and to exchange ideas on what more could be done to help the patients. Finally, she highlighted the continuous and vital support from the physicians towards the patient community.

**Mr Bruce Lim**, an IPOPI board member, gave an overview of the achievements of the Malaysian PID patient organisation, MyPOPI. He presented the short but impressive history of the organisation, with many successful campaigns each year. He explained that the first MyPOPI campaigns were aimed to raise public awareness of PIDs and now as the organisation developed more, they have started to work on advocacy as well. He introduced their newly published white paper which they had just recently presented to the Ministry of Health. Lastly, he summarised the importance of patient organisations in being the national PID experts, peer supporters and in spreading awareness and organising advocacy campaigns to reach better quality of life for PID patients and their families.

The next presentation was given by **Dr Nguyen Thi Mai**, the Director of the Vietnam Haemophilia Association with the topic of “Experience from Vietnamese Haemophilia Organisation”. She gave an overview of the advocacy campaigns and fundraising activities the organisation has implemented. She highlighted that it is of utmost importance that there is a well-functioning national patient organisation that is ready to take the challenge and work for the greater good to benefit the patient community. Finally, she stated that it is great to see Ms Giang’s enthusiasm to lead the PID patient group and that with her help VietPIPS will go a long way.

The last lecture of the day was provided by **Mr Johan Prevot**, IPOPI Executive Director. He gave a lecture on “What are Plasma-Derived Medicinal Products?” In his presentation he answered the question of “what is plasma and where does it come from?”. He explained how the manufacturing of immunoglobulin is dependent on both blood and plasma collections. Plasma is categorised as source plasma if it is collected from plasma donors whilst recovered plasma refers to plasma which has been retrieved from a blood collection. Source plasma represents 80% of the annual global plasma supply. He went over the safety issues of plasma products and proceeded to introduce the stages of various plasmapheresis processes. Finally, he went over the most important plasma derived products and the growing trend in the global demand of immunoglobulins and other plasma products.

The meeting was successful in empowering more patients to volunteer in the IPOPI National Member Organisation (NMO) VietPIPS and in identifying the most pressing issues for PID patients in the country.

#### The next steps agreed for VietPIPS:

- SCIG reimbursed by government
- Identify specific roles for the new volunteers
- Start being more active on social media
- Plan a meeting/campaign in 2018

#### The key challenges for the patients:

- The long distances to travel to receive treatment
- The economic disadvantage caused by receiving treatment
- Late diagnosis and lack of centres
- Difficulties to meet with other parents due to long distances



IPOPI wishes to thank Kedrion for their support towards this meeting.

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