Meeting summary

On May 25th, Primär immunbristorganisationen, PIO, hosted a workshop on the supply of immunoglobulins in Stockholm. The workshop was part of the Nordic Meeting for Primary Immunodeficiency, NMPI 2019, and brought together representatives from the Nordic patient organizations for primary immunodeficiencies, from IPOPI, the medical industry and PTTA as well as doctors and nurses working with patients with primary immunodeficiencies. Through lectures and workshop discussions we shared and gained knowledge and insights with the goal to find tools to secure the availability of immunoglobulins.

Anneli Larsson, Executive director of PIO, Primär immunbristorganisationen, started the day with welcoming all participants (7 guest speakers, 14 patient representatives, and 8 representatives from medical companies) and emphasized that this is an important day that deals with life and death.

Alexa Wetzel, Director, Source Europe, PPTA, then went on to give us a picture of the Ig use in relation to source plasma collected, within Europe with reference to the European Plasma Alliance but also worldwide. She also described what plasma is and how the process of plasma collection differs from whole blood donations. Her presentation concluded with the need for regulatory and political steps as well as increased awareness (e.g. through the current campaign "How is Your Day?") and a positive experience for donors to create a motivation for more people to donate plasma. *(For details, please see links to Power Point files from the meeting at p. 4.)*

Julie Birkofer, Senior Vice President,
North America & Global Health Policy, PPTA, and
Alexa Wetzel, Director, Source Europe, PPTA

Whereas Alexa's presentation focused on the donation side, *Julie Birkofer, Senior Vice President, North America & Global Health Policy, PPTA,* in her presentation turned to the manufacturing side and frequently returned to another very important message of the day: the quality and, above all, safety of plasma products. We received a comprehensive account of the time and money invested in the production of plasma protein therapies to gain an understanding of the major costs for building new collection centres and manufacturing facilities and the extended time for approvals etc. before the actual start-up, which shows the dedication of the member companies of the PPTA that focus on therapies for rare diseases in a highly regulated and highly technical industry. She also stressed the importance of access to several brands, since plasma products are not generics. Each company has scientists working on constantly evolving products.

In connection with these initial presentations there was a discussion about pros and cons of compensating donors, where Julie and Alexa stressed that donor compensation aims to compensate for necessary travels and the time spent, and should not be confused with actually paying someone for the donation. They expressed that PPTA recognizes the lifesaving contributions of all plasma donors. The ban for compensation was a result of the fear of HIV infections in the 1980's. But the extensive checks that donors and the collected plasma go through today make it impossible for unhealthy donors to donate.

Jose Drabwell from IPOPI described the effect that a limited supply or changes in access can have on patients, resulting in feelings of anxiety, disappointment and frustration, e.g. due to changes with short notice with prolonged treatment time due to a product switch or an enforced switch from SCIG to IVIG or vice versa. But she also stressed the importance of keeping a positive attitude and, above all, the need for data. In order for patients to be successful lobbyists and to be able to influence government decisions, we need statistical data on the number of patients, how much Ig is used etc. Patient organizations can for example offer their knowledge to national agencies responsible for health technology assessments (HTA) to spread awareness about primary immunodeficiencies. In this kind of advocacy work it is also beneficial to join forces with other patient groups that use plasma-derived medicinal products (PDMP). On an international level, IPOPI works together with PLUS (Platform of Plasma Protein Users) and key statements have been drawn up by IPOPI and PLUS regarding the importance of ensuring access to Ig therapy and strategies to encourage blood and plasma donations that can be found on the IPOPI website. Jose also raised the concern with 80 % of the global collection of plasma taking place in the US, which could be a problem if the export was to be limited or stopped.

Ramona Fust from Linköping University Hospital and *Carina Hagstedt from Ryhov Hospital, Jönköping,* were invited to give the nurses' perspective and explained how they base their treatment decisions on the Swedish physicians' guidelines for diagnosis and treatment of PID and the nurses' treatment guidelines. They described delivery problems that have occurred and how this not only makes the patients uneasy, but also means more work for the nurses when they have to calm patients, stay in contact with the pharmacy and reschedule patient visits. From their point of view, the situation could be improved by:

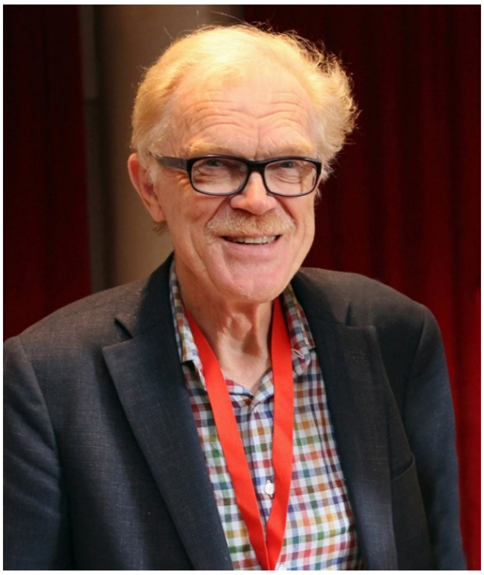
- closer contacts with the manufacturing companies to assure supply.
- delivery to the hospital pharmacy.
- agreements 9 months in advance.
- setting up a priority list within the hospital together with all clinics that use Ig.

Jose Drabwell, IPOPI

Carina Hagstedt, Ryhov Hospital, Jönköping and Ramona Fust, Linköping University Hospital

The invited doctors, *Børre Fevang, MD, PhD, Senior Consultant, Oslo University Hospital,* and *Anders Fasth, Professor of Pediatric Immunology, Dept of Pediatrics, University of Gothenburg,* both described a stable supply at the moment and functioning tender processes regarding IVIG, but they also recognized the need for better knowledge about the use of Ig in other departments and requested a dialogue within the hospital in order to set up common priorities. Neurology was mentioned as a department that uses a large amount of Ig to achieve an immune modulating effect and not as a replacement therapy as in PID.

As an example, Anders described the situation at Sahlgrenska University hospital, of which Queen Silvia Children´s Hospital in Gothenburg is a part, where annually approximately 1 500 000 doses of IVIg are prescribed by the neurology departments compared to only 90 000 doses by the Children's hospital (for children with PID or autoimmune diseases). The increasing number of individuals diagnosed with PID around the world is also a factor that must be considered when discussing a growing demand for Ig.



Anders Fasth, Professor of Pediatric Immunology, Dept of Pediatrics, University of Gothenburg

Børre Fevang, MD, PhD, Senior Consultant, Oslo University Hospital

The day concluded with *workshops in smaller groups* to discuss activities to secure the supply of immunoglobulins. In the long term, the supply of Ig must increase which implies both an increase in the number of donors as well as an increased production on behalf of the medical companies.

Since all lobbying work needs to be based on data, is it important to summarize the number of patients, e.g. in patient registries, and the amount of Ig used and needed. Consumption statistics are known but is the need greater?

The work to raise awareness about the importance of Ig treatment must be carried out on multiple levels; regionally, nationally, and on an EU level. Within this area, cooperation with other patient groups was highlighted in order to align the message towards payers and decision makers. One issue that was raised was the need to stop the waste of plasma from donated blood, as in Norway and Iceland where this plasma is simply discarded of. Suggestions on how to raise awareness included a Nordic version of the statement papers from IPOPI and PLUS, a local adaptation of the campaign "How is Your Day?" and the International Plasma Awareness Week.

Organizations involved in plasma and blood collection could be contacted to help raise awareness and increase the number of plasma donors. For example on the Swedish blood bank information site Geblod.nu, the information about plasma needs to be updated.

To make the supply more predictable, clearer tendering processes was requested with information about volume. Today, tenders in Sweden only stipulate price and not volume. With regards to production, it is also important to spread knowledge about the time needed for production of Ig products and that it takes time to make up for shortages in the global market.

In the short term, there is a need for a dialogue and cooperation between clinics within the hospitals and between various expertise groups in order to collect common statistics and data about the actual usage and priority lists should be put into place in the case of shortage (the National Demand Management Programme for Immunoglobulin from the NHS was mentioned as an example). To save Ig, alternative treatments for autoimmune diseases could be discussed.

In the discussions during the day, it was clear that all stakeholders share the same worries and also the responsibility for the situation. The industry must secure the production and supply and patients have an important role to play in spreading awareness about how important Ig treatment is. As it was repeated frequently during the day: immunoglobulin treatment is not only a question of quality of life but a matter of life and death.

Summarized by
Maria Michelfelder for PIO

Links to the Power Point presentations held at the Nordic meeting:

http://pio.nu/wp-content/uploads/NMPI/Alexa-Wetzel-slides-Nordic-meeting-May-25_2019.pdf

http://pio.nu/wp-content/uploads/NMPI/Julie-Birkofer-slides_Nordic-meeting-May-25_2019.pdf

http://pio.nu/wp-content/uploads/NMPI/Jose-Drabwell-slides-Nordic-meeting-May-25_2019.pdf

http://pio.nu/wp-content/uploads/NMPI/Borre-Fevang-slides-Nordic-meeting-May-25_2019.pdf