



IPOPI 16th PID Forum

Vaccination and PIDs:

Focus on the added value of vaccination for primary immunodeficiency patients



 **16th PID Forum**
Vaccination and PIDs:
Focus on the added value of vaccination
for primary immunodeficiency patients

Hosted by:
MEP Maria da Graça Carvalho
(EPP, Portugal)

December 3, 2020, 11:00-12:30 CET
Virtual Forum



An IPOPI event

Thursday, 3 December 2020, 11:30 – 12:00 CET

Hosted by MEP Maria da Graça Carvalho (EPP, Portugal)

I. INTRODUCTION

On 3 December 2020, the International Patient Organisation for Primary Immunodeficiencies (IPOPI) organised virtually its 16th PID Forum entitled “**Vaccination and PIDs: Focus on the added value of vaccination for primary immunodeficiency patients**”. The online event was hosted by Member of the European Parliament (MEP), Ms. Maria da Graça Carvalho (EPP, Portugal), and moderated by Ms. Martine Pergent, President of IPOPI. Opening the event, **Martine Pergent** thanked all speakers and participants for taking part in this event. She introduced and thanked MEP Carvalho for graciously hosting this event.

1. Opening Remarks

Maria da Graça Carvalho (EPP, Portugal) expressed her delight in being the host of the 16th PID Forum, and thanked IPOPI for approaching her to discuss this topic as vaccination is a priority topic not only in the political agenda but in the mainstream media as well. Vaccine hesitancy was identified in the [2019 Companion Report of the State of Health in the EU](#) as one of the top trends in the transformation of health systems. She emphasised that European institutions have been working to tackle this even prior to the pandemic, for example through [the EU Global Vaccination Summit](#) of 2019. Ms Carvalho recognized that work in this area is beneficial to all but particularly to vulnerable populations such as PID patients. More recently, Ms Carvalho and her colleagues at the European Parliament approved €6.2 billion to tackle the COVID-19 crisis and speed up vaccine deployment.

MEP Carvalho explained participants how the EU is strengthening its health competences through building a European Health Union, and the European Biomedical Research Agency, which will also be established in 2021. This Agency is envisaged to support the EU’s response to cross-border health threats and emergencies but should go further than this and promote the cooperation in biomedical research. As the rapporteur for the EU Parliament report on the Strategic Agenda for the European Institute of Innovation and Technology (EIT) 2021-27, Ms Carvalho called for a EU prioritisation of Health and Digital policy. Technology is key to strengthen resilience of the EU and its population and can play a key role to improve the development, production, and deployment of relevant vaccines. The EU must actively support a better coordination and exchange of best practices in the field, and she is committed to supporting this. Mrs Carvalho stated that Portugal is a successful example of vaccination policy, as the country shows good vaccination rates often surpassing EU’s average. This can be attributed to vaccine’s accessibility, as they are (1) free and (2) can be provided in pharmacies as well.



Mrs Carvalho concluded by stating that scientific evidence is key to develop concrete policies to promote successful vaccination campaigns.

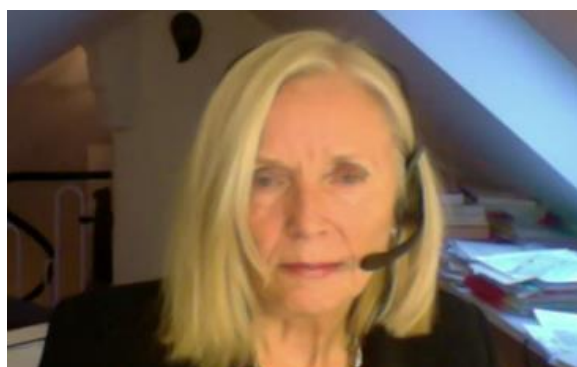
2. Why vaccines protect even those who cannot be vaccinated, the example of PIDs



Prof Alain Fischer, immunologist from the Necker-Enfants Malades Hospital (France), emphasised the importance of vaccination for the wider population. Vaccination is lifesaving and had led to the eradication of a number of diseases such as smallpox. Patients with primary Immunodeficiencies (PIDs) have partially non-functioning immune systems, which often leads to the misconception that these patients cannot receive vaccinations. Most types of PIDs involve partial problems of branches of their immune

system rather than the entire immune system, meaning they are still able to benefit from receiving vaccines. The recommendations for PID patients need to be adapted on a disease-by-disease basis, as for instance some PID patients need to receive vaccines more often than the general population, while others may need to avoid live vaccines (i.e. vaccines prepared from living micro-organisms). Patients who undergo haematopoietic stem cell transplantation will need to begin the vaccination programme from the start. It is also crucial to vaccinate the people around PID patients, to decrease even further the risk of infection, which is an extension of the concept of herd immunity. Prof Fischer also mentioned that misinformation leads to a harmful movement of vaccine hesitation. He urged patients and participants to follow the indications of their clinicians, and to look for reliable sources of information.

Prof Ann Gardulf, Board Member of the International Nursing Group for Immunodeficiencies (INGID), contributed to the discussion by presenting the practical considerations for PID patients when seeking vaccination. She referred to her work in immunology and vaccination, particularly her own vaccination clinic. The technique to administer vaccines does not differ to the general population, but special considerations for PIDs are nonetheless needed. It is not appropriate, for instance, for adults and children with PIDs to come into contact with many people in waiting rooms or in the clinics. Especially in cases of mass vaccination, individual appointments are needed to protect these patients. A concern within the healthcare professionals' community is a lack of nurses. The WHO has declared this year to be the Year of Nurses and Midwives, and sadly, the [report](#) on the global situation points at lack of nurses. **This has resulted in decreased rates of vaccination, which may put patients with PIDs at increased risks of preventable diseases.** As highlighted by Prof. Fischer, Prof. Gardulf underlined that an important action is to encourage the closest people to the PID patient to also get vaccinated to reduce the risk of infection. It is also key to increase awareness on the need to obtain or maintain a high vaccine coverage.



3. Regulator's perspective

Dr Manuela Mura, Scientific Officer in the Biological Health Threats and Vaccines Strategy office at the European Medicines Agency (EMA), provided an overview of the regulatory approval of COVID-19 vaccines at the EMA. The agency adapted processes to support vaccine development in order to accelerate development without compromising quality, efficacy and safety. The agency has a [Health Threat Plan](#) adopted in 2013 with internal guidance. They also established an EMA Pandemic Task Force (ETF) whose mandate was expanded to cope with COVID-19 and includes key representatives of several stakeholders including patient organisations. The EMA introduced accelerated procedures to ensure rapid approval of COVID-19 vaccines, including development and evaluation support. The traditional approval process can take 15 years or longer from design and exploratory studies until large-scale production and distribution. The SARS-CoV-2 vaccine development scenario can last from 10 months until 1.5 years total. This is possible by overlapping clinical phases, and through adapting designs from similar vaccine procedures, such as for Middle Eastern Respiratory Syndrome (MERS). When the minimum amount of data required to define the effectiveness and safety of a vaccine is achieved, a conditional marketing authorisation can be granted rapidly. After this authorisation, trials continue to submit the final data and any other relevant data. At the time of the Forum, 25 scientific advice procedures had been finalised, 4 were currently ongoing, and 4 had an ongoing rolling review. COVID-19 vaccines will only be approved upon compelling demonstration of efficacy and safety, and marketing authorisation holders will still have post-authorisation obligations.



4. Vaccines in Europe

The landscape of vaccines in Europe involves many different moving parts. This panel included the interventions from **Dr Maria Syrochkina**, IDM Medical Lead Vaccines Eurasia/Baltics, Pfizer, Vaccines Europe to represent the industry perspective, and **Ms Leire Solis**, Health Policy and Advocacy Senior Manager, IPOPI to represent the patient view.



Dr Syrochkina explained how the industry collaborates closely with regulators and healthcare providers across all countries. Dr Syrochkina noted that Vaccines Europe is committed to expanding access to vaccines and are involved in 40 partnerships for this purpose. In terms of development, it takes on average between 10 to 15 years to research and develop a vaccine so it can undergo thorough research. This research is not finalised when the vaccine enters the market, but the vaccine continues to be evaluated for safety and

effectiveness reasons. Dr. Syrochkina explained that clinical trials process is composed of three phases, followed by pharmacovigilance throughout the entire life of the vaccine. Regarding COVID-19, there were at the time of the Forum over 164 confirmed vaccine projects being developed globally with 48 ongoing clinical trials. This was only possible through strong collaboration between all relevant

stakeholders. Vaccines Europe members signed a pledge to uphold the integrity of the scientific and regulatory process in the work towards the first COVID-19 vaccines. Ms Syrochkina showed that there are at least 5 types of vaccines being developed for COVID-19, most being protein-based, followed by viral vector vaccines, nucleic acid, as well as virus inactivated and virus weakened, and lastly other types of vaccines. Dr Syrochkina also emphasised that access to vaccines is crucial and ended up calling for collaboration between the patient community, healthcare professionals, industry, regulators, researchers, and the media to tackle vaccine hesitancy.

Ms Solis showcased that accessibility is an issue of concern in the EU. The [Pharmaceutical Strategy for Europe](#) stated that access is essential to wellbeing and good health, and that sadly many patients do not benefit from innovation due to lack of access. With this in mind, Ms Solis explored the status of accessibility of vaccines in the current EU landscape on the basis of IPOPI's [PID Life Index](#). The Index pools together information on how life with a PID is like across the globe, focussing on 5 principles of care (diagnosis, treatment, universal health coverage, specialised centres, patient organisations and registries). The map shows that Europe has a mostly even landscape in regard to availability of vaccines. Ms. Solis underlined that availability, however, does not equate accessibility, meaning that even if a vaccine is available, access by patients to these may be difficult. A study by the Spanish Ministry of Health on unmet health care needs across Europe shows that several countries show an increase in barriers to healthcare access from 2008 to 2018. The EU landscape on financial coverage of vaccines by national governments varies between countries, regions, and even depend on the specific vaccine in question. Through the PID Life Index, many of IPOPI's national member organisations showcased also that there is a disparity between legislation and implementation. IPOPI welcomes EU initiatives that promote vaccine research and development and support patient accessibility to these innovations.

5. Open Floor for Discussion

Johan Prevot, Executive Director at IPOPI, commented that it was great to hear about Portugal's success in regard to vaccination coverage. He asked Mrs Carvalho whether there are any key takeaways that could be implemented in a broader sense. **Mrs Carvalho** replied that in her view, improving vaccination coverage depends on three considerations: (1) the trust in the scientific evidence, (2) the trust in the healthcare system, and the (3) political landscape. Portugal values science and has developed a scientific community that is involved in policymaking. Furthermore, the public trusts academics and researchers.

Mr Prevot also commented that there are several vaccines against COVID-19 being developed and asked what would be the guideline for PID patients in terms of knowing which type of vaccine they should get or not, also considering the different types of PIDs. **Prof Gardulf** responded that it is still somewhat premature to fully answer that question. Access to the vaccine will be one aspect to this answer, but more will be learned once these are deployed.

Margo Dona, member from patient organisation Stichting Voor Afweerstoornissen (SAS), asked panellists whether a vaccine could be administered the same day as an infusion of immunoglobulin whether it would be better to have some time in between (before/after). **Dr Nizar Mahlaoui**, from the Necker-Enfants Malades Hospital in France, explained that around 50% of patients with PIDs will be under immunoglobulin replacement therapy as a long-term treatment. There is some data to suggest that a response to the vaccine will form on the same day as an intravenous infusion, albeit lower than a vaccine injected in between two intravenous immunoglobulin courses. The evidence is however limited to a degree. There is no specific recommendation against taking a vaccine on the same day as infusion treatment.



Natalie Helena, Events Manager at IPOPI, raised the question about whether the EMA would consider this accelerated process for future vaccines and medicines to be authorised in a similar short time scale. **Ms Mura** explained that accelerated process would be considered if needed, and this is also what happened to Remdesivir for COVID-19. The rolling review, which is when the data is reviewed on a rolling basis, has already previously been applied to, for example, the 2009 pandemic influenza and during the ebola outbreak along with the conditional marketing authorisation. This is a tool employed during emergencies. However, it is not used in non-emergency times as it is resource intensive.

Jose Drabwell, board member of IPOPI, asked whether more vaccines will be developed based on the DNA and RNA approach. **Ms Syrochkina** replied that there are many DNA and RNA based vaccines under development at the moment. Further research on their real-life effects is needed to fully reply to this question, as well on how the regulatory landscape adapts to these.

Jose Drabwell enquired what could the EU do to combat misinformation about vaccines. **Ms Carvalho** stated that Members of the European Parliament, as well as the European Commission, can spread information on the scientific basis of vaccination, emphasising their safety and benefits. Several campaigns focusing on communication can bridge this gap, such as through the intergroups in the European Parliament. **Ms Mura** added to this by emphasising the [Vaccination Portal](#), which was launched by the European Commission, the European Center for Disease Prevention and Control (ECDC) and the EMA in order to fight misinformation. Ms Mura explained that the EMA will dedicate part of its efforts to showcase the benefit-risk balance of vaccination through a regulatory perspective, regarding the approval process.

6. Concluding remarks



Ms Pergent thanked all speakers and participants for the valuable contributions they made to the 16th PID Forum in order to emphasise the benefit and added value of vaccination across the EU, particularly for PID patients. It was agreed by the speakers that vaccine hesitancy is a major challenge across Europe. Interestingly, COVID-19 shed a new light to this situation due to the lack of available treatment, particularly to concepts such as herd immunity, risk groups, vaccination, new

vaccine types – which are already familiar concepts in the PID community. The pandemic has also provided citizens with the unique opportunity to witness science in the making. Ms Pergent concluded by highlighting that once approved, vaccines needed to be made available to patients, without causing any financial hardship to vulnerable patients.

II. LIST OF PARTICIPANTS

Panellists

- Maria da Graça Carvalho, Member of the European Parliament (EPP, Portugal)
- Martine Pergent, International Patient Organisation for Primary Immunodeficiencies (IPOPI)
- Prof Alain Fischer, Necker-Enfants Malades Hospital, France
- Prof Ann Gardulf, International Nursing Group for Immunodeficiencies (INGID), Karolinska Institutet (Sweden), and Inland Norway University of Applied Sciences (Norway)
- Manuela Mura, European Medicines Agency (EMA)
- Maria Syrochkina, Pfizer, Vaccines Europe
- Leire Solis, International Patient Organisation for Primary Immunodeficiencies (IPOPI)
- Nizar Mahlaoui, Necker-Enfants Malades Hospital, France

Attendees

- Ahmed Seri Ibrahim Mohamed, Sudan organization of patients with Primary immunodeficiencies (SOPPI)
- Alberto Casaca, Associação Portuguesa de Doentes com Imunodeficiências Primárias (APDIP)
- Alecsandra Filip, Romanian Association for Patients with Primary Immunodeficiencies (ARPID)
- Amanda Bok, European Haemophilia Consortium (EHC)
- Ana Posea, Romanian Association for Patients with Primary Immunodeficiencies (ARPID)
- Andrea Gressani, Associazione Immunodeficienze Primitive odv
- Anna Swierczyna, Vaccines Europe
- Anneli Larsson, Sweden Primär Immunbrist Organisationen (PIO)
- Anzelika Chomiciene, Vilnius University Hospital
- Argyro Panayiotou, Cyprus Alliance for rare disorders
- Bénédicte Faure, World PI Week
- Bianca Oiantanida Pizzera, Associazione Immunodeficienze Primitive (AIP)
- Birgit Schlennert, Deutsche Selbsthilfe Angeborene Immundefekte (DSAI) e.V.
- Carla Morgado, International Patient Organisation for Primary Immunodeficiencies (IPOPI)
- Christiana Demetriou, The Association of People with Primary Immunodeficiency and Friends (Cyprus)
- Christoph Geier, Immunologische Tagesklinik
- Clare Glynn, International Patient Organisation for Primary Immunodeficiencies (IPOPI)
- Eva Brox, Norwegian Immunodeficiency Organisation
- Eva Varga, Hungarian Organization for Patients with Immunodeficiencies (HOPI)
- Janika Sundström, Imppu ry (Finland)
- Joelle Khraiche, CSL Behring
- Johan Prevot, International Patient Organisation for Primary Immunodeficiencies (IPOPI)
- Jose Drabwell, International Patient Organisation for Primary Immunodeficiencies (IPOPI)
- Julia Nordin, International Patient Organisation for Primary Immunodeficiencies (IPOPI)

- Kersti Urbala, Estonian Patient Society for Immunodeficiencies
- Kotryna Linauskiene Kotryna, Vilnius University Hospital
- Kristina Skeries, Na
- Laima Aleksandraviciute, Vilnius University Hospital Santaros Klinikos (VUL SK)
- Lotte Denning, Aarhuslægerne
- Lynne Hyman, International Diabetes Federation (IDF)
- Maarit Syväluoma, Immuunipuutospotilaiden yhdistys ry
- Magda Lourenço, International Patient Organisation for Primary Immunodeficiencies (IPOPI)
- Magdalena de Azero, Vaccines Europe
- Margo Dona, Stichting Voor Afweerstoornissen (SAS)
- Maria Ascenso, International Patient Organisation for Primary Immunodeficiencies (IPOPI)
- Maria Charalambous, The Association of People with Primary Immunodeficiency and Friends
- Maria Kanariou, IASO Children's, Attica, Greece
- Mihaela Gunescu, Romanian Association for Patients with Primary Immunodeficiencies (ARPID)
- Mihaela Bataneant, University of Medicine and Pharmacy Timisoara, Romania
- Natalie Helena, International Patient Organisation for Primary Immunodeficiencies (IPOPI)
- Nedelcho Hadzhinikolov, Association of People with Congenital Immunodeficiency – Bulgaria
- Otilia Stanga, Romanian Association for Patients with Primary Immunodeficiencies (ARPID)
- Patricia Urtila, University of Medicine and Pharmacy V Babeş, Children's Emergency Hospital Louis Turcanu Timisoara
- Rachel Elizabeth Morse, Chronic Disease Coalition
- Ruediger Gatermann, CSL Behring
- Sally-Ann Malone, European Parliament
- Sandie Stegenberg, Sweden Primär Immunbrist Organisationen (PIO)
- Sergio Vicentini, Associazione Immunodeficienze Primitive (AIP)
- Sian Van den Bogaerd-Rance, Stichting Voor Afweerstoornissen (SAS)
- Siddig Abdallah, Sudanese Patients Organization for Primary Immunodeficiency (SOPPI)
- Sofia Tantou, Department of Immunology, "Aghia Sophia" Childrens Hospital, Athens, Greece
- Stoian Nicoleta, Romanian Association for Patients with Primary Immunodeficiencies (ARPID)
- Súsanna Antonsdóttir, Lind Iceland
- Susanne Larsson, Sweden Primär Immunbrist Organisationen (PIO)