



IPOPI 20th PID Forum **Anti-Microbial Resistance and the PID Perspective**

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and the PID Perspective**

Virtual Event
17th May 2022 15:30-17:00 CET
Co-hosted by MEPs Juozas Olekas (S&D, Lithuania) &
Sarah Wiener (Greens/EFA, Austria)

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Introduction

On 17 May 2022, the International Patient Organisation for Primary Immunodeficiencies (IPOPI) organised its 20th PID Forum entitled “**Anti-Microbial Resistance and the PID Perspective**”. The online event was co-hosted by Members of the European Parliament (MEPs) **Juozas Olekas (S&D, Lithuania)** and **Sarah Wiener (Greens/EFA, Austria)**, and moderated by **Mr Johan Prevot**, IPOPI Executive Director, together with **Ms Martine Pergent**, President of the board of IPOPI.

During the event, participants analysed the challenges anti-microbial resistance (AMR) poses for primary immunodeficiency (PID) patients. Participants were informed about the recent developments the EU has undertaken when it comes to AMR and the impact these actions have on PID patients. The aim was to explain how AMR affects PID patients, encourage policymakers to consider vulnerable populations in their work and support research and development on antibiotic research to ensure there are medicines available for the patients that need them.

The event was marked by MEPs Juozas Olekas (S&D, Lithuania) and Sarah Wiener (Greens/EFA, Austria) confirming their political support to the PID community when it comes to promoting their needs regarding AMR.

Welcome & Opening Remarks

Opening the 20th PID Forum, MEP Sarah Wiener (Greens/EFA, Austria) welcomed all speakers and participants and expressed her delight to be able to support the PID patient community and speak about a topic that is close to her heart as Chair of the MEP Interest group on AMR. She explained that antimicrobial resistance (AMR) is the ability of microorganisms to resist antimicrobial treatments, especially antibiotics, and that AMR is responsible for an estimated 33,000 deaths per year in the EU. She highlighted that AMR is particularly important for primary immunodeficiency (PID) patients, many of whom are treated with prophylactic, long-term antibiotics. Patients thus need novel therapeutics to be developed to replace existing medicines - this is of paramount importance for those patients who have resistant bacterial infections. She outlined that in response to the crisis, which will affect all European citizens in the coming years, the European Commission has made tackling AMR one of its priorities. This is taking place within the framework of the EU One Health Action Plan against AMR.



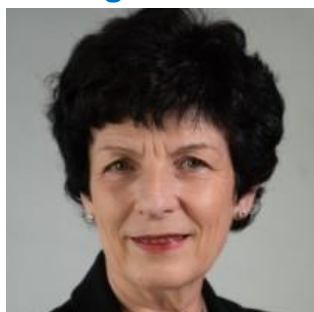
Ms. Wiener expressed that the initiatives of the Commission and Parliament are a step in the right direction. However, there is still much work to be done, which is why awareness of AMR must be improved both among healthcare professionals and the general population, particularly for rare conditions such as PIDs. To ensure patients' lifelong access to antibiotics, it is necessary that resistance building is minimised as much as possible. As a result, she agreed that there should be increased guidance and regulation from EU legislators on antibiotics usage – such that the general population takes antibiotics only when needed and as prescribed by their healthcare professional. She also stated that it is crucial for the PID population that research and innovation into novel therapies are supported. In the expected case of many antibiotics losing their efficacy, PID patients will require immediate, safe and effective replacements.

She concluded by saying that revising existing policies to stimulate innovation in areas of unmet antibiotic needs is only the first step to ensuring that PID patients can benefit from a better quality of life. The next step is for the pharmaceutical innovation to become more patient-centred, health system-oriented and address the multi-disciplinary requirements which PID patients need.

Co-host MEP Juozas Olekas (S&D, Lithuania) followed MEP Wiener's introduction by additionally expressing his support for the event and the PID patient community before introducing the speakers and agenda for the Forum.



Setting the Scene: PIDs and AMR



Ms Martine Pergent, President of IPOPI, began by thanking the MEPs for their political support, as well as IPOPI's partners who made this event possible. She introduced IPOPI and elaborated on the difficulties AMR represents for patients with PIDs and why targeted action on this topic is so important. AMR is a growing concern, with the EU taking – and planning for – a host of responses. Addressing AMR is particularly important for the PID patient community for a multitude of reasons, including long-term

antibiotic use or in a prophylactic manner as well as increased risk of infection.

AMR as a Challenge for PID patients

Mr David Catena is a patient living with a PID. He was diagnosed with a primary immunodeficiency called agammaglobulinemia before the age of one. Due to his specific condition, he has been receiving immunoglobulin replacement therapy regularly since the diagnosis and have been prescribed antibiotics in addition to his regular treatment. For the past year, when he contracted COVID-19, his doctor has prescribed him antibiotics on a prophylactic manner. This long-term course of antibiotics has some inconveniences implied, such as intestinal problems. Still, Mr Catena considered that antibiotics are key to keep infection abide and to ensure him and many other patients with PIDs can have a healthy life.



Dr Nizar Mahlaoui (Unité d'Immuno-Hématologie & Rhumatologie pédiatrique, Hôpital Universitaire Necker-Enfants Malades, Assistance Publique-Hôpitaux de Paris, France) provided a medical presentation explaining the use of antibiotic therapies in the treatment of patients with PIDs. Dr Mahlaoui explained that there are many treatments available for patients with PIDs and that a great proportion of patients will require immunoglobulin replacement therapy. Antibiotic prophylaxis, such as with azithromycin, showed in clinical trials how patients receiving it would have lower risk for exacerbations, lower risk for hospitalisation, lower need for additional antibiotic courses and improved quality of life, amongst others. He finished his presentation by calling for a cautious use of antibiotics only as prescribed by medical doctors as a way of avoiding increasing antimicrobial resistance.



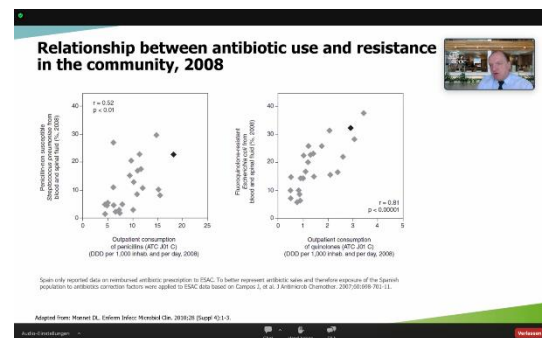
The Need for Research and Innovation

Ms Kristine Peers (representative of the European Federation of Pharmaceutical Industries and Associations (EFPIA)) shared the perspective of the pharmaceutical sector, describing how prevalent AMR remains as a healthcare challenge and thus which efforts are being undertaken by the industry to address AMR, including the AMR Action Fund, AMR Industry Alliance, and Innovative Medicines Initiative. She highlighted the risks for industry in developing antibiotics, as few projects result in novel, successful antibiotics. Antibiotic development and discovery has been in decline since the 1950s. Ms Peers emphasised that the antimicrobial market is not functioning and therefore new funding models are needed that account for the risks and difficulties of antibiotic research to ensure a healthy and sustainable antimicrobial pipeline and continued access. She explained this requires initiatives funding early R&D ('push funding'), but also 'pull funding' for later and costly stages of drug development. A pull incentive in the form of transferrable exclusivities would be a powerful incentive that would be most effective and feasible in the EU.



ECDC Activities on AMR

Dr Dominique L. Monnet, Head of Section, AMR and Healthcare-Associated Infections at the European Centre for Disease Prevention and Control (ECDC) described the actions that the ECDC has taken to

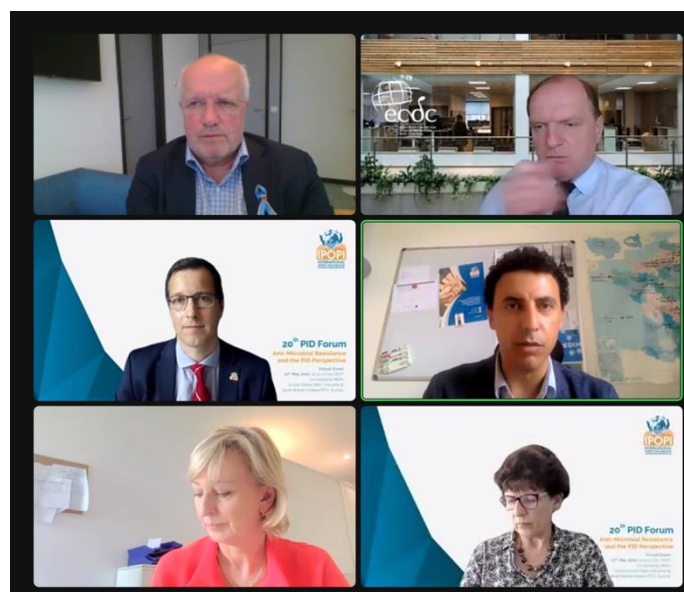


address AMR, including sharing patient stories as well as awareness campaigns. He described the burden of infections with antibiotic-resistant bacteria, demonstrating how significant it is, as well as the main actions being taken to prevent and control AMR. Namely, these are prudent use of antibiotics, the prevention of cross-transmission of antibiotic-resistant bacteria, and the development of new antibiotics. Each year, the ECDC hosts the European Antibiotic Awareness Day, a European health initiative to raise awareness about prudent use of antibiotics, which provides different toolkits and communications materials for stakeholders. In 2022, the ECDC plans to update its annual data on surveillance and health burden of AMR as well as continue to contribute to risk assessments of emerging AMR threats to health.

Panel Discussion and Q&A on AMR and PID Medical Care

All speakers came together for a panel discussion following the presentations, during which they shared their perspectives on how PIDs and the PID patient community can be a part of the AMR conversation. Specifically, they addressed topics including how to effectively communicate with patients as a healthcare provider, how to prudently utilise antibiotics and recognise patient’s needs, as well as how to incentivise best practices and research collaborations.

The need for innovative treatments and new models for developing antibiotics was discussed from both a research and industry perspective, with the important pinpoint that the patient community be continually involved. All speakers recognised the importance of acknowledging the different perspectives of patients and how the PID patient community plays a unique role in the AMR conversation due to their specific needs for antibiotic treatment.



Closing Statements & Next Steps

MEP Sarah Wiener began her closing remarks by stating that no EU citizen should ever be left behind because of lack of access to a treatment which is tailored to their needs. Alternatives need to be found to the current options that are on the market to ensure that patients benefit from efficacious novel treatment and pharmaceutical innovation that is patient centred is direly needed. She added that legislation must support patients and their needs, keeping in mind that this means a variety of different things for different individuals and conditions. Eliminating antibiotic usage is not an option nor the end goal – effective and long-term accessible usage is.

MEP Juozas Olekas emphasised that AMR is one of the key challenges of our time. The event's experts clearly described the lack of awareness amongst the general population and healthcare professionals, particularly in regard to the connection between AMR and PID patients, and how this can lead to errors in treatments. As many patients are at high risk of infection or experience recurrent infections, they may be on long-term antibiotic treatment.

Mr Olekas underpinned that awareness should be raised on the challenges faced by PID patients who need to have access to antibiotics in a prophylactic manner and are at higher risk of infections and poor infection outcomes – and an important step towards highlighting these issues is through events like this Forum. He reiterated the words of Ms. Peers, highlighting that there is a necessity to invest in the development of novel antimicrobials or alternatives to ensure continuity of care. There have been examples of successful models to incentivise new antibiotics, as well as best practice initiatives, and National Health Authorities should take these as examples to increase the quality of life of PID patients, while EU policymakers need to keep them in mind as they assess upcoming legislation, such as the revision of the general pharmaceutical legislation.

Mr. Olekas stated that it is crucial that throughout all of the upcoming legislative actions patient communities or associations are consulted, as the patients' voice should always be heard when taking policy action at the EU or national level. He concluded by reiterating that no EU citizen should be left behind because of a lack of access to a treatment which is tailored to their needs. Europe needs to be at the heart of the solution when it comes to the fight against AMR, and this starts with being aware of the challenges AMR represents for patients, such as those with primary immunodeficiencies. He closed the meeting by thanking all the participants, speakers and organisers for sharing this space and furthering the discussion on AMR and primary immunodeficiencies.

Mr Johan Prevot provided the final remarks of the event and likewise thanked all speakers and participants, particularly those representing PID patients, for taking part in the 20th PID Forum. He emphasised that IPOPI is excited to build on the awareness raised at the Forum to ensure future action and is ready to act as the go-to patient community for policymakers and other stakeholders to assist them in pushing for the following:

- Emphasising the importance of revising existing policies to stimulate innovation in areas of unmet antibiotic needs, including the inclusion of PID patient needs in the upcoming Council Recommendations on AMR
- Investing in the development of novel antimicrobials or alternatives following a patient-centred approach.
- Raising awareness of the challenges faced by PID patients who need to have access to antibiotics in a prophylactic manner and are at higher risk of infections and poor infection outcomes.