



4th Primary Immunodeficiencies Forum
Primary Immunodeficiencies and Cross-Border
Healthcare

Report of the 4th PID Forum on
Primary Immunodeficiencies and Cross-Border Healthcare
29 January 2014
European Parliament, Brussels, Belgium
Hosted by Ms. Emer Costello MEP



INTERNATIONAL PATIENT ORGANISATION FOR PRIMARY IMMUNODEFICIENCIES

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1. Introduction

On Wednesday 29 January 2014, Ms. Emer Costello MEP (S&D, Ireland) hosted the fourth Primary Immunodeficiency Forum in the European Parliament in Brussels. The meeting was organised in collaboration with the International Patient Organisation for Primary Immunodeficiencies (IPOPI) and focused on primary immunodeficiencies and the Directive 2011/24/EU on the application of patients' rights cross-border healthcare.

Experts representing patients, academics, physicians and industry gathered with Members of the European Parliament and the European Commission to discuss the benefits afforded to patients by the Directive and the need for Member States to fully implement the legislative text. The implementation would include the establishment of adequate National Contact Points to inform patients about their treatment opportunities, and the development of European Reference Networks of centres of excellence, which is foreseen to particularly benefit patients with rare diseases. All participants in the meeting agreed that Member States should involve patients and their representatives when implementing the Directive to ensure that patients' needs and expertise would be taken into account.



2. Summary of discussions

Ms. Costello initiated the meeting by highlighting that the Cross-border Healthcare Directive is one of the most important and complex pieces of legislation adopted by the European Parliament and the Council of Ministers in the field of health. The Directive aims at guaranteeing that patients across Europe can have access to healthcare in a different Member State. Ms. Costello highlighted the benefits that this Directive could have for the life of patients with rare diseases, such as primary immunodeficiencies (PIDs). This is the case because for rare diseases medical expertise, diagnosis and treatment is not always available at home, hence the importance of the provisions facilitating that patients can seek treatment abroad. Ms. Costello furthermore highlighted that these rights afforded to patients need to be effectively enacted and called to Member States to fully implement the Directive.



Ms. Annika Nowak, Team Leader responsible for Cross-border Healthcare in the Health Systems Unit of DG SANCO, European Commission, presented the main provisions of the Directive, stressing that it increases and clarifies the patients' rights to choose to receive healthcare abroad and improves information to patients about their rights and the conditions under which they may seek



treatment abroad.

In addition, the Cross-border Healthcare Directive gives patients the right to receive information about the quality and availability of treatments for their conditions abroad (including treatments that are not available in their home country) so as to facilitate patients' informed choice. Furthermore, the Directive creates a minimum set of patient rights for all treatment delivered in the EU. She also insisted that it is also up to patients to use these provisions, especially by getting in touch with National Contact Points, which have now been set up in all Member States.

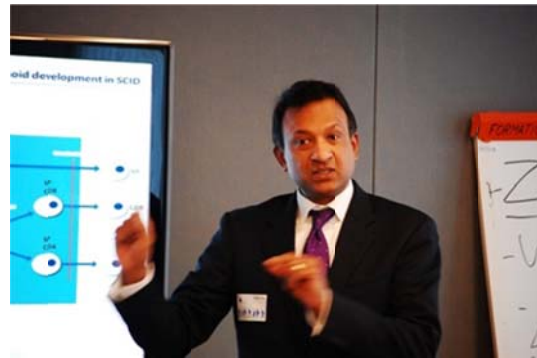
Specifically for rare diseases patients, she pointed out that many diagnostic services fall outside the scope of prior authorisation, which means that reimbursement is possible under the Directive, although most specialized services will require prior authorisation. The Directive is a major step forward for patients, as it allows comparison across Member States between entitlements, quality and safety standards, and treatments offered. On the European Reference Networks, Ms. Novak mentioned that the Commission is currently preparing an implementing act setting criteria for creation new infrastructures to improve access to diagnostics and highly specialised healthcare to patients with conditions requiring a particular concentration of expertise, such as is the case for rare diseases. The first call for expression of interest will be issued in 2015, and in the meantime, the Commission's focus will be on evaluating the implementation of the Directive.

Mr. Johan Prevot, Executive Director of IPOPI, highlighted that the implementation of the Directive is very important for PID patients, as it allows them the possibility to make an informed choice on their treatment. Furthermore, the Directive aims to improve access to diagnosis and treatment and to facilitate patient mobility. Given the complexity of the legislation, Mr. Prevot also highlighted some potential risks in the transposition of the Directive. As a first example, he highlighted the potential danger of requesting an up-front payment from patients, as in many cases, it may render the diagnosis or treatment abroad unaffordable. To emphasise the need to implement the Directive towards the Member States, Mr. Prevot requested the present MEPs to table a Parliamentary Question to the Council to which there was broad support from the MEPs.



Mr. Prevot also expressed concern about patients' need to obtain prior authorisation for certain procedures and the bureaucratic burden linked to the procedure. This requirement could create a barrier to access to treatment in time for the most urgent situations if not dealt within a reasonable timeframe. A good example of this is the case of bone marrow transplantation for patients with Severe Combined Immunodeficiency (SCID), where the treatment implies hospitalisation of the child and follow-up after the surgery. Finally, he expressed the hope that cooperation on Health Technology Assessments (HTA) between Member States and with key stakeholders could focus on establishing an appropriate approach for rare diseases therapies (i.e. immunoglobulin replacement therapy) to ensure that important elements, such as improvement in the quality of life and societal benefits are taken into consideration in the appraisal.

Prof. Bobby Gaspar, UCL Institute of Child Health/Great Ormond Street NHS Trust, presented a practical example of the potential of Cross-Border Healthcare to improve treatment for rare diseases. Prof. Gaspar provided an overview of his experience in treating children with SCID and explained how innovative procedures such as somatic gene therapy which is currently only available at Great Ormond Street Hospital in London could allow significant financial savings to Member States healthcare budgets by providing access to specialized cross-border healthcare that can cure the children.



Prof. Gaspar was supportive of the Cross-border Healthcare Directive as it would not only facilitate this type of research, but could also foster cooperation between Member States on developing the best possible treatments for rare diseases by sharing the expertise available within the EU.

Ms. Yvonne Rooney, from the Irish Primary Immunodeficiencies Association, provided the patients perspective on the importance of the Directive for PID patients. Ms Rooney highlighted that many patients affected by PIDs and their families are challenged by the



lack of knowledge and information about the treatments available in their home country. She described it as particularly frustrating when she, as a concerned parent taking her child to the GP, experienced that she knew more about the disease than the doctor did. For a patient, the Directive is very complex, and in Ms. Rooney's view, its beneficial effects will depend on how countries translate it into their national laws.

The Directive has many important provisions, which, she hopes, will improve the level of information among patients and physicians, and provide increased rates of diagnosis and treatments. Finally, she stressed that it is very important that national authorities consult with patients when implementing the Directive, as patients are the ones with the first-hand experience. She greatly welcomed the opportunities offered by the Directive of seeking medical expertise abroad, but saw the real strength to be the improved exchange of knowledge between countries as the first choice of treatment would be in the patient's own Member State.

She finalised her presentation by stating that although she was aware that there was a National Contact Point in Ireland for Cross-border Healthcare, she had not been aware of this in advance of researching her presentation for this meeting. Ms. Rooney also indicated that more needed to be done in order to ensure that patients and patient organisations are aware of their National Contact Point.

3. Discussion

After the presentations, there was a lively debate, which touched on several very important subjects.



A question was raised regarding the issue of the prior authorisation of treatment with a reference to Prof. Gaspar's trial in the United Kingdom on whether the trial would indeed not be possible without the Cross-border Healthcare Directive. Ms. Nowak and Prof. Gaspar both confirmed that the trial was possible under the current legislation, but that the intention of the new Directive was to facilitate these types of exchanges, and to improve patients' knowledge of the opportunities to seek treatment abroad.

Following up on the presentation by Ms. Rooney, several participants expressed their agreement about the lack of information and communication to patients and their organisations about their possibilities and entitlements to seek information, diagnosis and treatment from experts abroad. On this topic, Ms. Novak noted the lack of information and indicated that Member States should inform the Commission on what they have done to implement the Directive provisions on information to patients.



Ms. Mairead McGuinness MEP (EPP, Ireland) intervened expressing her support for patients with rare diseases, and stressing that the Directive is an important step in improving patient information, and therefore in ensuring better results for them.

4. Conclusions

Ms. Emer Costello MEP concluded the debate by thanking all participants for the interesting presentations and discussions. She stressed that the meeting has highlighted the importance of the Cross-Border Healthcare Directive in the field of rare diseases, and that the concrete examples provided in the meeting further underline the importance of properly implementing the Directive.

It is essential, she added, that patients' organisations actively get involved in the implementation process and that decision-makers, both European and national level, take their views into account.

Several actions have been planned to follow up on the recommendations that were made during the meeting which are outlined below.



5. Recommendations

- The European Union must continue its work in supporting its citizens that suffer from rare diseases including primary immunodeficiencies (PIDs) an important subgroup of 250 rare disorders.
- The Cross-border healthcare Directive lays the ground for improving patients' rights and access to diagnosis, treatment and care.
- Patient organisations and healthcare professionals should be involved in the process so as to have their first-hand experience.
- Member States can improve diagnosis rates by improving patients' access to medical expertise abroad. Bureaucratic requirements should not hinder access to diagnosis and care.
- The EU should encourage Member States to provide timely access to lifesaving treatments such as bone marrow transplants and immunoglobulin therapies for patients with PIDs.
- Access to diagnosis/treatment/care should not be limited by prize (pay up-front).
- The availability of specialist treatments in another Member State should not incentivize Member State of affiliation to discontinue or not increase its level of care.
- Cooperation on HTA between Member States and with key stakeholders should focus on establishing an appropriate approach for rare diseases therapies (i.e. immunoglobulin replacement therapy) to ensure enhanced access to care for PID patients.
- Member States that have not yet implemented the Cross-border healthcare Directive should do as soon as possible, with the "spirit" of the Directive in mind.



6. List of participants

Ms. Emer Costello, Member of the European Parliament, host of the PID Forum
Mr. Pat the Cope Gallagher, Member of the European Parliament
Ms. Mairead McGuinness, Member of the European Parliament
Mr. Sean Kelly, Member of the European Parliament
Ms. Amanda Bok, European Haemophilia Consortium
Prof. Bobby Gaspar, UCL Institute of Child Health
Mr. Rüdiger Gatermann, CSL Behring
Ms. Verena Jirgal, Rohde Public Policy
Dr. Mary Keogan, Beaumont Hospital
Mr. Cristian Lutan, Rohde Public Policy
Dr. Nizar Mahlaoui, CEREDIH (Le Centre de Référence Déficiés Immunitaires Héréditaires)
Ms. Maria Michelfelder, IPOPI
Ms. Lucy Moylan, Parliamentary Assistant to Emer Costello MEP
Ms. Annika Nowak, Team Leader, Health Systems Unit, DG SANCO, European Commission
Ms. Deirdre O'Hea, Parliamentary Assistant to Mairead McGuinness MEP
Mr. Rune Orloff Pedersen, Rohde Public Policy
Ms. Martine Pergent, IPOPI
Mr. Johan Prevot, IPOPI
Ms. Yvonne Rooney, IPIA
Ms. Laura Savini, European Haemophilia Consortium
Ms. Leire Solis, IPOPI
Dr. Paul Strengers, IPFA
Mr. Jean-Marie Vlassembrouck, PPTA
Mr. Charles Waller, Rohde Public Policy