2nd PID FORUM on Health Technology Assessment Hosted by Mrs. Glenis Willmott, MEP Report

Introduction

On Tuesday 6th December 2011, Mrs. Willmott MEP (S&D, United Kingdom) hosted the second Primary Immunodeficiency Forum in the European Parliament. The meeting was organised in collaboration with the International Patient Organisation for Primary Immunodeficiencies (IPOPI) and focussed on health technology assessments (HTAs). Experts representing patients, academics, physicians and industry discussed with several Members of the European Parliament the need to involve patients in HTA appraisals as to ensure these are sustainable and rooted in reality.

Summary of discussions

Mrs Willmott set the scene by reminding that health technology assessment is a multidisciplinary process summarising medical, social, economic and ethical information related to the use of a health technology. The Directive 2011/24/EU on patients' right in Cross border healthcare promotes exchange of best practices in regard and recognises the need for "appropriate consultation of stakeholders" (art.15). MEP welcomed participants' calls for an increased involvement of stakeholders and experts throughout the process. They considered that this would help in capturing the real value of decisions and their impact on people's lives, especially for rare diseases such as SCID and other PIDs.

Mrs Willmott explained that several physicians had reported that HTA procedures in their countries were under pressure to limit the prescription of life-saving treatments to patients suffering from a PID. If patients suffering from a rare condition were not able to access the therapy and care they needed, efforts at EU and national level to develop supportive rare diseases policies would be seriously undermined. Mrs Willmott reminded the audience that HTA can play a valuable role in health-care decision-making provided that the process is transparent, timely, relevant, in-depth and usable.

Dr Teresa Español, Emeritus specialist in immunology, presented on the HTAs procedures for assessing therapies for PIDs. In her presentation, Dr Español stated that if patients suffering from PID were not treated, they would have a short life-span due to repeated serious infections, such as secondary bronchiectases or lymphomas. The treatment received by PID patients should be sufficient, adapted to the normal Intravenous Immunoglobulin (7 to 16 gr/L). This is important, as only a sufficient and best available treatment will allow PID patients to become healthier, happier and more

active members of society.

Mr. Brian O'Mahony, Chief Executive of the Irish Haemophilia Society, developed on patients' involvement in HTA. In some cases, patients' organisations may submit data and make representations - but this not consistently requested by HTAs boards and practices varies widely across the EU. By involving the patients, national authorities would have "real experts" in the condition, as patients are the ones living with it. In this sense, patients could work together with doctors to provide evidence based data. Participants agreed with this point and added that patients involved in HTA would not only provide experience but also expertise i.e. two key elements to an assessment rooted in reality. Participants supported this argument and considered that the use of HTAs were not an issue per se. Experts carrying these should nonetheless be educated about the consequences of their decisions on patients' lives – ad factor in the costs that society would bore if a treatment were not provided.

Mr. Johan Prevot, Executive Director of IPOPI, provided the view of PID patients regarding HTAs. He started by explaining that government and national agencies were increasingly implementing cost containment mechanisms on healthcare budgets to respond to rising costs of healthcare. The assessment of therapies for rare diseases are more complicated than the ones for common diseases due to the lack of available information, also due to the difficulty to perform large randomized clinical trials or pharmaco-economic data. HTA models applied to common conditions are often not applicable to seemingly "costly" rare diseases therapies.

Mr. Prevot pointed out that in case of PIDs it is extremely important to consider the impact of the therapy on the life expectancy and the quality of life of the patients. It is also essential to factor the macro-economic impact of the therapy, by including the costs induced to society related to the inability to work and lead a productive life without the treatment. HTAs authorities should also consider the impact of a restricted access to the appropriate therapy and the subsequent medical costs associated to the symptoms treatment rather than to the symptoms cause. The EU is leading the way in terms of promoting the involvement of patients into the HTA process through a series of initiatives: the EUnetHTA, the implementation of the Cross-border healthcare directive of the Health for growth programme proposal 2014-2020. Mrs Nessa Childers MEP agreed with Mr Prevot and considered essential to factor micro- and macro-economic aspects in the HTA procedures.

During the debate, Dr Español explained that the diagnosis of PIDs was difficult and that immunology was a new field that still needed to be further developed and recognised. Mrs Willmott MEP completed the picture by citing the example of patients' families from the East-Midlands, who had to face similarly dramatic situations. Other participants indicated that given the scarcity of expertise on rare diseases, it was essential to involve specialists in HTA appraisals and any decision regarding these diseases.

List of participants

Mrs. Glenis Willmott, Member of the European Parliament, host of the PID Forum

Mrs. Nessa Childers, Member of the European Parliament

Mrs. Linda McAvan, Member of the European Parliament

Mr. Bill Newton-Dunn, Member of the European Parliament

Ms. Edwina Hanbidge, office of Mrs. Marian Harkin, Member of the European Parliament

Mr. Mark Taylor, office of Mrs. Mairead McGuinness, Member of the European Parliament

Ms. Celia Fontaine, office of Mrs. Michèle Rivasi, Member of the European Parliament

Ms. Catherine Conway, office of Mr. Proinsias de Rossa, Member of the European Parliament

Mr. Andrea Zanaglio, office of Mr Carlo Fidanza, Member of the European Parliament

Mr. Johan Prevot, IPOPI

Mrs. Jose Drabwell-Wuite, IPOPI

Mrs. Martine Pergent, IPOPI

Dr. Teresa Español, IPOPI

Mr. Brian O'Mahony, EHC

Mr. Alain Weil, EHC

Prof. Jose-Louis Valverde, Granada University

Mr. Ruediger Gatermann, CSL Behring

Mr Jan Maarten Ten Brundel, Baxter

Mrs. Louise Marland, Baxter

Mr. Tim Ruebesam, Grifols

Mr. Charles Waller, PPTA

Mr. Albert Farrugia, PPTA

Mr. Sebastian Rohde, Rohde Public Policy

Mr. Giovanni Asta, Rohde Public Policy

Ms. Malvika Vyas, Rohde Public Policy

Ms. Leire Solis Garate, Rohde Public Policy