

## “Improving Care for Rare Plasma Related Disorders”

**2 December 2008, 12.30-14.30**

**European Parliament, Brussels, MEP Salon**

With Rare Diseases at the top of the French EU Council Presidency Agency, European patients have high expectations of what the EU can do for them at this time. It is in this light that a lunch debate was held in the European Parliament on 2 December 2008, hosted by Jorgo Chatzimarkakis MEP and Miroslav Mikolasik MEP. The debate was related to Rare Diseases, but specifically to rare plasma related disorders, which include rare chronic life-threatening disorders such as Haemophilia and Primary Immunodeficiencies amongst others.

Both host MEPs have been keen campaigners for patients with rare plasma disorders in the past. For example Miroslav Mikolasik hosted a lunch in January 2008 at the European Parliament on plasma protein therapies which are biological medicinal products that have undergone a sophisticated production process to treat these disorders. Both MEPs therefore were especially enthusiastic to support the patients affected by these life-threatening disorders by bringing the debate to the European Parliament at this time where Rare Diseases are foremost on the EU health agenda. Jorgo Chatzimarkakis said on opening the event that “it is vital that the EU and its Member States listen to all relevant voices and I think that today’s meeting represents an excellent opportunity to do this”. The appeal of the subject was also proven by the attendance of other MEPs such as Adamos Adamou, the rapporteur on the recent European Parliament organ donation report, and influential health MEP Frieda Brepoels.

Nick Fahy, Head of Unit for Health Information at DG SANCO, presented the European Commission’s proposal for a Council Recommendation with great flair and enthusiasm. Mr. Fahy said that the public consultation for this dossier received a record number of responses, 584, and that DG SANCO therefore realised that all stakeholders were highly anticipating the proposals. He identified the areas where the European Commission feels the EU can encourage and add value to Member State actions. This includes improving the recognition and visibility of rare diseases leading to better diagnosis, supporting policies on rare diseases in the Member States and developing European cooperation, coordination, and pooling of expertise. In order to do this, the European Commission’s proposals (released in November 2008) suggest a number of courses of action that the EU and its Member States should take to improve the situation for patients:

- Member States should all introduce a dedicated Rare Diseases Action Plan.
- Creating a co-ordination of existing reference networks, which valuably support patients with Rare Diseases and the healthcare professionals diagnosing and treating them..
- Provision of funding for Rare Disease research, supporting orphan drugs and backing non-governmental actions that support patients.

Brian O'Mahony, dedicated campaigner for people with Haemophilia in Ireland through the Irish Haemophilia Society, spoke next on behalf of 'PLUS' (the Platform of Plasma Protein Users) a recently created group representing the interests of patients affected by rare plasma disorders which include over 200 conditions.. Mr. O'Mahony knows only too well the issues facing people with rare plasma disorders, as he has Haemophilia himself. Mr O'Mahony together with Mr David Watters from the International Patient Organisation for Primary Immunodeficiencies (IPOPI) recently met with Health Commissioner Androulla Vassiliou to discuss how the EU can support people with these conditions. He outlined patient requirements from EU actions in the following key areas:

- Good accurate data is needed on the diseases to improve awareness of the conditions and diagnosis rates – national registries are therefore vital and must be encouraged.
- Reference Centres for treatment of plasma protein disorders are crucial to ensure access to appropriate treatment due to lack of knowledge in standard healthcare setting, given the rarity of the diseases. This is particularly important given that most rare plasma protein disorders can be successfully treated by plasma protein therapies which are medicinal products produced from human plasma, thereby enabling these patients to live normal and productive lives.
- Consensus treatment protocols.
- Patient involvement in policy process will be key to ensuring community actions deliver real benefits for patients at both EU and Member State level, through representation by patient organizations.
- Access to high quality and effective plasma protein therapies is the number 1 priority for patients with rare plasma disorders. Plasma protein therapies are unique medicinal products that differ from traditional pharmaceuticals given their biological origin and their unique production and cost structure
- Plasma and Blood, and the medicines that are derived from them, are intrinsically different. They should be considered on a separate basis in EU legislation and actions to ensure patients can optimally access life-saving plasma protein therapies

Mr. O'Mahony was generally in favour of the DG SANCO proposals, and felt that they were all the better for the proper consultation and discourse with patients groups. He encouraged Mr. Fahy that this consultation procedure should continue, and could even be more formalised.

Representing the physician, who obviously has a significant role to play in tackling Rare Diseases, was Professor Lennart Hammarström from the Karolinska Institut in Stockholm. Professor Hammarström has spent his working life helping people with Primary Immunodeficiencies, which are a group of rare diseases affecting the immune system, making people vulnerable to illnesses and infections.

Professor Hammarström echoed the statement of Brian O'Mahony that reference centres for treatment and patient registries are vitally important to support patients with Rare Diseases. He also stated that availability of plasma, which is obtained through a process known as plasmapheresis or recovered from blood donations, must be assured as it is from this plasma that the vital life saving therapies the patients need

can be made. In most cases the therapy not only saves the patient's life, but can also make life approach normality. The Professor also encouraged Member States to provide high levels of treatment by economic incentive; provision of treatment makes a benefit of €60 000 per year of "full" quality of life as the person can work and contribute to society. Brian O'Mahony vehemently backed these statements from the patient's point of view.

Former MEP and European Parliament representative on the EMEA (European Medicines Evaluation Agency) Management Board Prof. José Luis Valverde chose this occasion to launch a new book that he has piloted and edited on this topic, called "Focus on Immunodeficiency". The book focuses on the policy and legal background of Immunodeficiencies, and features a number of chapters from European level actors such as Miroslav Mikolasik MEP.

Following on from the presentations, a lively debate took place which demonstrated the importance of the topic and the high level of interest from the attendants. This included patients' groups, Permanent Representations from Member States, industry and scientific experts. The consensus was that the European Union has a significant role to play, echoing the statement of Commissioner Vassiliou that the area of Rare Diseases represents the field of healthcare where it is most obvious that the European institutions can add value.

Participants unanimously agreed to launch a "Call for Action" outlining the necessary actions and steps forward identified during the meeting which must be encouraged at both EU and Member State level. The call for action will be shortly released officially.