"Plasma products in the treatment of rare diseases"

A lunch discussion hosted by
Miroslav Mikolasik MEP
European Parliament, Brussels
Wednesday 23 January 2008

MEPs attending:
Miroslav Mikolasik (EPP-ED, SLOVAKIA), Adamos Adamou (GUE/NGL, CYPRUS), Jorgo Chatzimarkakis (ALDE, GERMANY), Milan Gal'a (EPP-ED, SLOVAKIA), Richard Howitt (PES, UK), Peter Liese (EPP-ED, GERMANY), Frédérique Ries (ALDE, BELGIUM)

On Wednesday 23 January 2008, I was delighted to host a lunch meeting in the European Parliament on the topic of "Plasma Products in the Treatment of Rare Diseases". The lunch was an excellent opportunity for representatives of the European Parliament and European Commission, patients' groups, experts and industry to pool their considerable experience and opinions. The esteemed panel of guests received three presentations on the topic:

David Watters represented the **Plasma Protein Users Group (PPUG)**, and outlined a number of the most common plasma protein disorders such as haemophilia and primary immunodeficiency, and what daily life is like when you suffer from them. Mr. Watters emphasised the life-transforming nature of the treatments available, which allow the majority of those suffering from plasma protein disorders to live happy and productive lives.

Johan Prevot, **Plasma Protein Therapeutics Association (PPTA)**, informed us how plasma protein therapies are developed, manufactured and used to improve the lives of patients with rare and chronic disorders. Mr. Prevot also emphasised a number of beneficial actions for patients treated with plasma protein therapies and for the industry which are being taken at EU level, such as in the areas of patient information, health services, rare diseases and telemedicine.

Antoni Montserrat of **DG SANCO (Health Information Unit)** presented the European Commission's viewpoint on rare diseases. A summary of actions taken previously to address rare diseases at EU level was made, such as the European Commission Taskforce on Rare Diseases. Mr.Montserrat recognised the need for a Commission communication on rare diseases, and announced that this will be published in May 2008, following the review of responses received in the open consultation. He was able to give a preview of some the proposals that DG SANCO want to make such as ensuring that Member States have national action plans, the creation of an EU Working Group of rare diseases as advisory group for WHO and the development of national/regional centres of reference and the establishment of EU reference networks.

After the presentations, a most interesting and informative discussion took place between the presenters, MEPs and Mr. Montserrat representing the Commission. Whilst a number of differing views were expressed, some common themes emerged from all sides of the debate:

- **The benefits of patient groups**
  Patient groups can share info and expertise, increase visibility and can benefit from the synergy of combining their efforts. The Plasma Protein Users Group is a great example.

- **Diagnosis must be improved**
  More effective and widely available diagnosis for chronic, rare diseases such as plasma protein related disorders is needed. The EU must work to reduce inequalities of diagnosis levels between Member States.

- **Better and more equal treatment**
  Patients have the right to better information about their conditions and the treatment options available to them.

  Treatment levels for plasma protein deficiencies vary greatly depending on the Member State. Member States should take measures to ensure optimal access to treatment. Plasma protein therapies are unique therapies which differ from traditional pharmaceuticals, for a number of reasons linked to the biological origin of their raw material (Human plasma). Recognition of their unique nature needs to be taken into account in national health policies to ensure appropriate access to treatment for patients whose life and quality of life depend on these important therapies.

- **DG SANCO proposals on rare diseases signal 2008 as a vital year for rare diseases**
  A more effective co-ordination of Member State activities on rare diseases is essential, so that patients, healthcare professionals and healthcare providers know where they stand.

  MEPs are keen to see how the Commission intends to add real value in the area of rare diseases.

- **National action plans are vital to success in tackling rare diseases**
  Many EU Member States do not have a national action plan on rare diseases, which suggests that not enough emphasis is being given to helping those with rare disorders. The EU's proposals will focus on encouraging Member States to buy-in to the benefits of action plans, with a greater level of co-ordination at EU level.

I present this summary and conclusions document as a record of an interesting and productive lunch discussion on a most important topic.

With kind regards

Dr. Miroslav Mikolasik MEP