

PPTA Position Paper

European Commission's Public Consultation: The EU role in global health

The Plasma Protein Therapeutics Association (PPTA) welcomes the Commission consultation on the EU Role in Global Health and is pleased to provide the following contribution in order to help defining the rationale and strategic objectives for this important role.

PPTA is the primary advocate for the world's leading producers of plasma-derived and recombinant analogue medicinal products. The medicines produced by PPTA members are used to treat patients suffering from rare life-threatening and / or life-impairing disorders and serious medical conditions including bleeding disorders (e.g. Haemophilia), immune system deficiencies (e.g. Primary Immunodeficiencies), autoimmune diseases, burns and shock. The rarity of some of these disorders often means that access to treatment, proper diagnosis and treatment levels is not optimal and varies greatly from region to region, and from country to country.

Question 1: In your opinion, does the proposed concept 'global health' cover the most relevant dimensions? If not, which other essential factors would you suggest?

The definition of global health should also include the European Union's role and contribution to the improvement of health both inside and outside of the Union. Based on our experience, we know that patients who are affected by rare plasma related disorders treated with plasma protein therapies in the EU can experience difficulties accessing proper treatment inside the Union depending which member state they live in. It seems therefore that the role of the EU within the Union should be incorporated in the definition of the concept of global health. This would reflect and strengthen the number of positive actions the EU has taken in recent years on health with initiatives such as those on patient mobility, patient information and rare diseases to name a few. The European industry's leading role in research and development of innovative treatments and the EU's impact in helping developing countries to help improve their health should also be considered.

Question 2: Are the effects of globalisation on health, on the spread of diseases (whether communicable or life-style non-communicable) and on equitable access to health care sufficiently described?

Global pandemics provide a useful study on the effects of globalisation on health, but the situation must be viewed more broadly. The equitable access to healthcare by people around the world is sometimes blocked by technical Non Tariff Barriers to trade, and the EU should pursue the freer trade of medicines to ensure the positive benefits of globalisation are felt by those who need it most. The plasma protein therapeutics industry is amongst the most regulated industries in the world. The need for global regulatory harmonization in our field has been a topic of discussion for years and disharmonized regulatory measures both inside and outside the EU do have a negative impact on equitable access to care. The need to ensure that European legislation is properly implemented at Member State level would be a first step in ensuring more equitable access to treatment. This aspect should therefore not be ignored when considering globalisation on health. The recent efforts between the EU and the United States of America in harmonizing orphan drug regulations provide a helpful example of the positive role the EU can play in this area.

Question 4: In your opinion, which are the main strengths and weaknesses of the current EU policy on health and development cooperation, and which dimensions should be given greater attention in order to face the challenges ahead?

It is important that the European Union continues to address health challenges through global fora but direct action that tangibly affects people's lives for the better should always be the priority. The European Commission should create closer links between its public health and development policies in order to strengthen its approach, and create synergies between what the EU is doing in public health and how it can support developing countries outside.

Question 5: Could you identify health problems that have been neglected by the EU and international health research agenda and propose the best means to support innovation to address them, especially in low- and middle-income countries? and Question 8: In the context of aid effectiveness and alignment of financing to national priorities, what can be done to make sure that adequate attention is paid to health priorities and to strengthening health systems?

The European Union has made significant achievements in tackling common and fatal diseases such as TB and Malaria in low and middle income countries. However, supporting the development of infrastructures in such countries (with both finance and advice) that deal with rarer, but treatable, diseases should also be pursued. For example, Haemophilia can be treated with Factor VIII, and Primary Immunodeficiencies

with immunoglobulin therapies which are on the WHO List of Essential Medicines, yet still many patients lack access to these life-saving therapies.

Question 9: What are your suggestions for striking the right balance between addressing health priorities and providing support for developing health systems? and Question 19: How do you think national capacity and local scientists in low-income countries could be empowered to conduct research relevant to their countries' priorities?

In the field of rare plasma related disorders, the development of networks of reference centres has provided valuable support to countries lagging behind in terms of diagnosis rates and treatment levels. By linking emerging and established hemophilia organizations and treatment centres, the Twinning Program put in place by the World Federation of Hemophilia provides an example which has improved treatment and care for people with hemophilia around the world. Another excellent example on a global scale are the networks of reference centres in the field of Primary Immunodeficiencies put in place by the Jeffrey Modell Foundation. These initiatives fit with the rare diseases vision which the EU is promoting and are concrete examples of what kind of support is needed to help developing health systems and address important health priorities in the field of rare diseases.

Question 12: What impact will the global crisis (climate change, food prices and economic downturn) have on global health and what could be done to help mitigate their ill effects?

At a recent meeting organized by the European Platform for Patients' Organisations, Science and Industry the impact of the global financial crisis on the rare diseases field was addressed. EPPOSI subsequently called for policy continuity and continued research to ensure best quality healthcare in Europe. EPPOSI also concluded that action is needed to secure funding for research, timely diagnosis, equitable treatment and better care to patients and their families. Rare diseases are a crucial field of research and a precursor of future developments in the healthcare field, making it a "societal laboratory" for new healthcare systems such as personalized medicine and sustainable innovation-based and patient centred healthcare

The excellent work of the EU on rare diseases and the call for action at Member State level should therefore not be slowed down by the global crisis. Patient access to life saving rare diseases therapies which represent only a modest amount of national healthcare budgets should be prioritized in national healthcare budgets particularly in time of financial crisis to ensure that European efforts in this field are not wasted and continue to pay for rare diseases patients.

Question 14: Which action do you think the EU should take to stem the brain drain of health workers, while respecting their freedom of movement?

It is important that the balance is kept between free movement of workers and supporting all European Union member states to improve the quality of healthcare available to their citizens. In the field of Rare Diseases, due to the lack of prevalence of the conditions, expertise is often spread thinly. Physicians may move to countries that are able to support their work with more resources, and potentially better personal financial terms. Whilst it is difficult to avoid a trend such as this due to disparities in economic strength, the European Union could facilitate a greater exchange of best practice between Member States in order to support less rich countries. In addition, EU funding for research could be specifically targeted at countries that are suffering badly from the 'brain drain' phenomenon.

Question 15: What role do you see for new technologies (including telemedicine) in enabling developing countries to provide access to care even in remote areas and to allow better sharing of knowledge and expertise between health professionals, and how can the EU support this?

The further development of new web based technologies is highly anticipated in the Rare Disease community. Because, as mentioned above, expertise on conditions is often thinly spread patients often have to travel long distances to receive care or settle for lower quality care nearby. The possibility for experts on a condition to address a patient remotely, even from thousands of miles away, could dramatically improve the standards of diagnosis and management of rare conditions. Such technology could also improve the exchange of information between reference centres and the running of EU registries. For example, the European Society of Immunodeficiencies (ESID) has put in place an EU wide online registry for Primary Immunodeficiencies, through which centres from all over Europe specialising in PID are collaborating to document their patients into a single database available online. This is a great initiative that could be looked at as a best practice example. The European Union should increase its support for research into telemedicine, and ensure that compatibility of systems within the EU and also outside to maximise the potential benefits for patients.

Question 23: Do you think a definition of a universal minimum health service package would facilitate a rights approach and progress towards more equitable coverage of services? If so, how could such a universal minimum standard be defined?

The definition of a universal minimum health service package sounds like a fast-track attempt at ensuring equitable healthcare between Member States. However, the fact is that Member States do have differing levels of resources, and also variable public

health challenges. A definition of a universal minimum health service package must not interfere with a country's ability to address its own specific health needs.

In terms of Rare Plasma disorders, the levels of treatment vary wildly between EU Member States. A person with primary immunodeficiency in France has a significantly better chance of being diagnosed and treated than a person with the same disease in Bulgaria, and will also receive superior treatment afterwards. Generally there are more tangible and realistic ways to support countries in developing their healthcare systems than by imposing minimum standards on them; as these standards could only really apply to poorer countries as most likely the richer ones are already over and above what could be set as a challenging target. However it has to be recognized that in poorer developing countries particularly outside the European Union the lack of resources may necessitate some guidance on essential health needs. The WHO List of Essential Medicines provides such a guidance tool.

Question 27: What, in your view, is the main added value offered by the EU in the field of global health?

The main added value offered by the EU in the field of global health is hard to define, considering that the effectiveness of its actions is not easily measured. However, the EU is globally recognised for its actions in the health field. Ensuring better health within the EU, supporting and adding to Member State actions, of course contributes to global health. In addition, EU support for developing countries in terms of finance, expertise and infrastructure is also widely recognised.

Last but not least the EU has taken an increasingly strong role on Rare Diseases in recent years, which is an area in which EU action is most logical. The EU's recent actions in promoting awareness of rare diseases and in helping define the scope of national rare diseases policies are having a concrete, positive impact on the health of rare diseases patients at national level. This experience should be shared with countries outside the EU who could benefit from the EU's experience in this field.