

The rare diseases agenda of the European Parliament

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1. Introduction

During the past years, rare diseases have been one of the main themes on the European Union's agenda. The reason for this theme being highlighted is the excellent exchange of knowledge between EU policy makers and the relevant patient communities. The former negligence has been caused by the apparent low patient numbers.

The definition of a rare disease in Europe contains, that there are less than five patients out of a total population of 10000 persons suffering from this disease. Taking the total of all patients, suffering from rare diseases, it becomes a different number. An estimated 30 million EU citizens are suffering from a rare disease. This tells us that rare diseases are actually not exactly rare.

Due to the great part of the population, which is affected, we have a social obligation to prioritise on this matter. Furthermore, the type of health conditions deserves a strong focus.

The European Union, and notably the European Parliament, has made significant progress towards the provision of a political framework, which would define rare diseases and provide incentives for the research and development of medicines.

The EU Orphan Medicines Regulation, adopted in 1999, was a political milestone in the prioritisation of rare diseases. It has provided the European Union with a clear competence in refining this topic. It was this legislation, which created incentives for medical manufacturers to develop products, furthermore, they generated a market exclusivity going beyond patent protection and a fee waiver for the evaluation and registration process of specialized products. The establishment of the European Medicines Evaluation Agency (EMA) in 1995, meant the foundation of a centralised body, which can appropriately deal with the necessary central registration of available and registered medicines. This has been a great success story.

Since then, 639 products have been registered with the European Medicines Agency. More importantly, the Agency transformed Europe to a leading region for research and development in this field.

Following this legislation, the European Parliament has repeatedly maintained a high priority on this topic both in political initiatives and public health programmes as well as with the appropriate legislation.

The Community Action Programme in Health (in the period from 2007 through 2013) adopted in 2005 reemphasised the prioritisation on rare diseases. This discussion is currently continued by building concrete steps for a European-wide Action Programme on Rare Diseases, discussed in the European Parliament during 2009.

The area of rare diseases is asking for innovation to be boosted. Innovative research leading to innovative therapies requires innovative legislation. The European Parliament has therefore strongly supported the innovation aspect when drafting and adopting the legislation on Advanced Therapies. I am personally very pleased that the contributions of my colleagues and myself to supporting highly innovative and creative solution providers stemming from Universities and research institutes and developing into small and medium sized manufacturers developing advanced therapies, have supported the content of this legislation.

In addition, the European Parliament has with the adoption of the Paediatric Medicines Regulation further boosted the sector by providing increased support and incentives for therapy developers in this field.

Given that about 80% of rare diseases are genetic diseases, the accompanying legislation on biotechnology, which has been put in place by the EU institutions, can also be considered as crucial. Especially the Directive on the protection of biotechnological inventions, providing patent protection for researchers, has been another milestone in the political framework.

The 7th Framework Programme on Research and Development has, following the good tradition of previous EU Framework Programmes, again strongly focused on funds, which have been made available for the research on rare diseases.

This demonstrates that the European Union and the European Parliament in particular, have well understood that tackling rare diseases demands a holistic approach with the involvement of various policy sectors. Beyond health policy, it clearly was a priority for the European Parliament's Research Committee, as well as the Legal Affairs Committee, providing legal protection for respective inventions.

2. Benefits from EU legislation for rare plasma disorders

It is the combination of a concrete rare diseases policy, the development of an orphan medicines legislation and a paediatric medicines regulation, that should be of direct benefit to patients suffering from plasma disorders.

Rare plasma disorders deserve, however, a special policy focus. There are many rare diseases to which there is no therapeutic solution, or only treatment with limited medical effect. We definitely need to make more progress on these diseases. This means also, however, that all needs to be done that patients, who suffer from treatable rare diseases, are appropriately diagnosed, treated and supported. Therapies have

well developed in the past decades and years, they enable persons, suffering from rare plasma disorders, such as primary immunodeficiencies or haemophilia, to live good lives when appropriately diagnosed and treated. With an adequate diagnosis and treatment such patients can today lead 'normal' lives. When diagnosed too late or with inappropriate treatment, in the case of primary immunodeficiencies, patients can die prematurely. This is an unacceptable situation, especially considering that such cases are preventable.

Rare plasma disorders are well placed on the political agenda in the European Parliament. I had the privilege to chair a meeting focusing on rare plasma disorders in the European Parliament in 2008. The outcomes of the meeting, especially the presented therapeutic developments, the fact that increasing numbers of patients are diagnosed in a more timely fashion, have been very encouraging. The enthusiasm of patient groups' representatives was inspiring. We, as policy makers, need now to create the framework within which persons suffering from rare plasma disorders and parents, uncertain about the health of their children, feel well taken care of. An important message of my recent meetings with representatives of the patient groups has been, that a great objective for these patients is to become contributors to society – rather than being considered a burden. Investing in the health and better care of persons with treatable rare diseases is an investment in society as a whole.

The debate was an excellent opportunity for stakeholders from all relevant fields such as politicians, physicians, industry and patients to discuss what the European Union and its Member States can do to tackle rare plasma disorders. The consensus points from the meeting were put into a Call for Action on Rare Plasma Disorders, which I have been encouraging my Parliamentary colleagues to sign to show their support. An encouraging number of MEPs from a range of backgrounds have signed so far, and I hope that this will continue in the Parliamentary term 2009–2014.

Currently, the European Union is working on crucial dossiers that should further lead to an improved care and hence improved quality of life for patients with rare disorders. The current discussion on cross-border healthcare should present best-practice in healthcare, best available treatment in various Member States and hence demonstrate best standards of care, that should then spread to other Member States. In addition, the improved information to patients should – in a new legislation – also strongly contribute to the patients' rights of accessing and receiving best available treatments.

Tremendous improvements have been achieved in the past years for patients suffering from rare plasma disorders. Nevertheless, diagnosis levels and care can still be strongly improved in order to support these patients being able to enjoy excellent quality of life. The European Parliament will therefore continue this prioritisation on rare diseases and ensure that the done progress will be continued.