

Medicines For The Few - How Can Society Better Understand The Reality Of Medicines For Rare Diseases?

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One in 33 babies is born with a rare and serious genetic disease - most of which have no treatment(1). Yet the burden of rare diseases is immense and affects millions of individuals across Europe(2). This week more than 120 stakeholders from patients' organisations, academia and the biotechnology and pharmaceutical industries came together for the 8th EPPOSI Workshop on Partnering for Rare Disease Therapy & Development in the Danish Parliament, in the presence of HRH Crown Princess Mary of Denmark, to assess how European society can better understand the reality of rare diseases in Europe.

During the workshop, participants identified the following key areas that need to be addressed to tackle the continuing issue of rare disease treatments and their availability to patients.

1. An orphan medicine is defined as a treatment for a life-threatening or seriously debilitating disease, for which no other alternative therapy exists, or if it does, has a significant benefit over it.
2. Several factors need to be solved simultaneously in order to ensure rare disease patients have access to treatments. That's why cooperative dialogue such as this is key.
3. Access to treatments remains a political decision. Health technology assessments can be used to inform these decisions, but they remain policy decisions to be based on societal values and preferences.
4. It is reasonable to ensure that we only pay for treatments that work, but mechanisms are needed to ensure that this does not delay or prevent access while the evaluation is happening.
5. Suitable models do exist in several member states to make sure that patients do get access. These could serve as examples in other countries.
6. Access to rare disease treatments needs public acceptance, support and solidarity. We all need to have faith in our partners. Transparency and trust is vital another reason that we all need to work together.
7. The EU's orphan Regulation is a success. It is anticipated that, within the next 5 years, Europe will have authorised between 85 to 105 new treatments, thanks to the Regulation. A third of them are currently developed by Small & Medium-sized Enterprises (SMEs).
8. Predictions of an avalanche of treatments eating into healthcare budgets are unfounded. The evidence shows that there is a gap between theoretical numbers of patients and those actually treated. Prevalence may be over-estimated at the time of designation, not all patients are eligible for treatment, not all eligible patients have access to treatment at country level, and not all patients are diagnosed in a timely enough manner to allow them to be treated. While it is clear we do not have this data, information on the real situation should be gathered to allow us to identify potential solutions.
9. Registries should continue to be established to gather real-life evidence of the effect of available treatments through coordinated efforts at European or even global level. The data should be made available to all researchers and external audiences.
10. The European Clinical Trials Directive has a negative effect on clinical research and development of treatments in the field of rare diseases. It raises costs and increases complexity.
11. Communication with the general public and within the field is essential to find consensus on issues. To engage personally is essential for all stakeholders.

Torben Grønnebæk, chairman of Rare Disorders Denmark and member of the European Organisation EURORDIS, knows the difficulties of suffering from a rare disease. He suffers from Wilson's syndrome, which threatened his physical mobility until he was finally diagnosed and received the appropriate treatment; commenting on the recommendations, he stated:

Rare diseases are often overlooked, and because of this the first obstacle is get the proper diagnosis. Then it becomes a matter of treatment - does a treatment form actually exist? It is not very lucrative to develop and produce medicine for treating rare diseases, which is why we need to create a better platform for research and the development of this type of medicine. We cannot allow ourselves to

ignore the sufferings of people afflicted by rare diseases simply because there are a few of them.

EPPOSI aims to build dialogue and foster understanding between all parties with an interest in human healthcare. The joint recommendations developed in Copenhagen will be shared with European experts and decision-makers at all levels.

- EPPOSI is a patient-led, not-for-profit organization, founded in 1994 for the exchange of information and discussion of EU policies in human healthcare between patients, industry, academic science and regulators.

- EPPOSI aims to positively work on policies in human healthcare in Europe by facilitating discussion and identifying consensus between its stakeholders. Any joint views developed by its stakeholders as consensus recommendations are shared with European decision-makers at all levels.

- EPPOSI Website: <http://www.epposi.org>

- EU Regulation 141/2000 - the European Orphan Medicinal Products Regulation, which creates a framework for incentivising research and development into rare diseases - came into force in 2000. ([Link here](#))

- Since 2000, more than 470 products have been registered in the EU as potential treatments for rare conditions, of which more than 30 are already allowed on the European market, listed on the official EU Register of Orphan Medicinal Products. ([Link here](#))

- For more information on the estimated 7,000 rare diseases, please see the [Orphanet](#) website.

Reference notes

1. [Jeans for Genes Appeal](#) website
2. [European Organisation for Rare Diseases \(EURORDIS\)](#) website
3. Rare Disorders Denmark - Sjældne Diagnoser.

<http://www.epposi.org>

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