EURORDIS response to the European Commission Public Consultation "Legal proposal on Information to patients"

EURORDIS - the European Organisation for Rare Diseases - represents more than 320 rare disease organisations from 37 countries, 24 of which are EU member states, and thereby reflects the voice of an estimated 29 million patients affected by rare diseases in the European Union.

In response to the Commission's Public Consultation "Legal proposal on Information to patients", EURORDIS is pleased to send its comments from the rare disease patients' perspective. Part of this document is based on a previous paper elaborated by EURORDIS in June 2007 in response to the Commission's Draft Report on current practice with regard to the provision of information to patients on medicinal products.

EURORDIS welcomes a Commission's initiative in this field but has serious reservations on the current proposal as it stands. In addition to the important issues already expressed by EURORDIS in June 2007 (that will be re-iterated here below), EURORDIS feels the necessity to emphasize the four following issues as major requirements within the current reflection process on information to patients: the recognition that "patient" and "consumer" are not synonyms and do not cover the same realities; the need to ensure efficacious protection against promotional information to patients; the need to combine new rights for pharmaceutical companies together with improved obligations; and finally, the identification of the risks entailed by the legislative frame as proposed by the Commission in its consultation document.

EURORDIS acknowledges that nobody knows the product better than the pharmaceutical company producing it. Companies also know the off label use which is made of the product, and which is very widespread for rare diseases patients. EURORDIS is therefore in favour of the establishment of mechanism at EU level allowing companies to give accurate information on their products to patients needing them, within regulatory boundaries which ensure the prohibition of active promotional information to patients. EURORDIS also believes than any form of *ex ante* validation is unrealistic and too complex to be applied effectively, and that therefore, only product information already validated through the EPARs should be made available on the Internet. As long as the product information provided does not exceed the scope of the EPARs, it should be deemed accurate and reliable.

1. The words "patient" and "consumer" are not synonyms:

All patients are also consumers - but all consumers are not patients, especially not patients affected by severe, highly-debilitating and life-threatening rare diseases for which very little information is available.

The main differences between rare diseases patients and consumers lay in both the motivation and operation of the purchase.

- Motivation of the purchase: rare diseases patients do not wish to buy medicinal products; they need to buy medicinal products for their survival.

- Operation of the purchase: medicinal products needed by rare diseases patients have to be first prescribed by a doctor and must be then reimbursed by a third party, namely the health insurance scheme, whether it is public or private. Two additional parties, external to the buyer/consumer relationship, are involved in the purchase of medicinal products. This is not the case for "consumers"[1]. Therefore, both the motivation and the *modus operandi* of purchasing prescription medicines are very different than when purchasing other consumer goods.

The legal proposal of the Commission under discussion does address the issue of information to patients on prescription medicines. Therefore the most legitimate stakeholder here are the patients - within the specific situation of being forced to buy drugs that must be reimbursed.

2. Need to ensure efficacious protection against promotional information to patients

EURORDIS strongly opposes the legalisation of the so-called push information disseminated by the industry on prescription medicines, through TV, radio or any other mass media. In our perspective, these activities do not differ from promotional information (advertising) to patients.

Even if the information provided in this way would be objective and of good quality, the company disseminating this push information would *de facto* be in a promotional situation compared to a competitor that will not have the resources to disseminate information on its product. In order to ensure the objective, clearly stated in different documents of the Commission, to keep a total ban on advertisement to public, this push information must also be banned.

3. Combination of rights and duties:

An increasing number of pharmaceutical companies are willing to take seriously their responsibilities in terms of Public Health actors. So, EURORDIS believes that as counterparts of increased rights for pharmaceutical companies on the issue of provision of information to patients, companies should easily accept to have increased duties. In the context of information to patients, this means that within the information made publicly available and hence disclosed to patients should also be included the following elements:

- The results, both negative and positive, of clinical trials, when published and also results of unpublished clinical trails. In the absence of publication in a peer review journal or of a presentation at a conference, a summary of the results should be provided;
- Complete information on the Marketing Authorisation: in which countries the product is marketed, which is the distribution circuit, what is the reimbursement rate and how much is left to the patient to pay from his pocket, which is the number of patients treated with the drug, etc.
- The uses made of the drug beyond the indication for which the Marketing Authorisation was granted, when this off label use exists.

EURORDIS believes that patients affected by any chronic or severe disease, frequent or rare, would expect to find all of the above listed information on the website of each pharmaceutical company for their products.

[1] This is usually the case, with the exception of preventive treatments, such as vaccines or quinine against malaria. In these cases, consumers are being prescribed a vaccine or a treatment that is often reimbursed; still they are not patients as they don't suffer from a disease. Nevertheless, prevention therapies represent a marginal issue in the mass of medicinal products that are indeed mainly used to fight diseases. The scope of the current proposal on information mainly addresses patients' needs.

The obligation to submit trials data for a marketing authorisation application is already part of the legislation: article 8(3) of Directive 2001/83/CE. The point is that this obligation is often theoretical. With EudraCT, the authorities will have increased abilities to chase non submitted information if the trials are conducted in the EU. But for trials not conducted in the EU, European authorities are missing the adequate tools to ensure all results of all clinical trials are part of the application. Before considering how to communicate proprietary information to the patients, the legislation and the Commission should maybe first consider how to enforce the full respect of article 8(3) of Directive 2001/83/CE.

4. Risks for the healthcare budgets of Member States

EURORDIS would like to draw the attention to the following risk for access to medicinal products for patients: the legislative framework conceived by the Commission and proposed in the Consultation Paper will *de facto* invite the pharmaceutical industry to allocate increased resources to promote their products through a variety of media channels (the push information). Because of competition, companies will not have other choices than allocating more spending to marketing, sales and product promotion. These budgets for advertising agencies or media coverage will increase the expenditures of pharmaceutical companies, and therefore will increase the price of medicinal products promoted. These increasing prices will add to the cost of healthcare. This will have as an indirect consequence to reduce the access to more expensive and innovative treatments for patients.

What is the public health benefit of having national health care systems paying for more promotional activities? What is the benefit for companies? Nothing guarantees that this will improve their profit margin or their creation of value for their shareholders. Is this an additional burden that society is ready to bear, at the expenses of costs dedicated to a real improvement of the healthcare systems?

Patient needs market rules pushing for more innovation, better access and better healthcare, not to pay for products' promotional activities at the expenses of access to care.

Furthermore, EURORDIS also wishes to re-iterate the main issues expressed in June 2007:

Absolute right to information

EURORDIS advocates in favour of the absolute right to information for patients, within the wider context of patients' rights. When information relates to medicinal products, access to information becomes a vital right. This information includes information on the disease itself, ways of preventing it, existing treatment options, including medicinal products and their availability, research on and development of new drugs.

It is important to underline that patient' representatives advocate in favour of information to patients on medicinal products with the aim of improving a "good use" of these products. When patients are well informed, it has positive consequences in terms of patient comfort and contribution to patient care, better efficacy of the medicinal product, minimisation of adverse effects (and other safety issues), better adherence to the treatment, patient's empowerment.

This educational and empowering effect is incremental and cumulative, having both short and long term benefits for chronic diseases, with long term care and use of medicines.

Role of patient organisations

EURORDIS wishes to underline the importance of the role of patient organisations in the delivery and process of information on medicinal products to their patients. Patient organisations have a pivotal role to play as legitimate and trusted source of information, beside healthcare professionals and industry:

- patient organisations may act as relay of information;

- they can have an active vulgarisation role by performing explanatory work towards lay persons;
- in practice, they do often complete the information provided by the doctors who usually have limited time to explain the disease, as well as the long term physical, psychological and social implications of both the condition and the treatment;
- most patient organisations have developed a variety of supports that can be used to disseminate information to their members (leaflets, brochures, mailing lists, on line forums, meetings, help lines, etc.) and also have a proven availability to the services of mutual aid;
- patient organisations can also act as emitter of relevant information, when the information is considered to be potentially beneficial for their constituencies (patients, families and carers).

Responsibility and means

The responsibility for patient organisations to serve as a reliable partner in regulating medicinal products, and also gathering, processing and disseminating information has been acknowledged and conferred to patient representatives in different EU legislative texts. The logic counterpart of any recognised responsibility is to be given the appropriate means to be able to carry out such responsibilities.

EURORDIS considers that the financial means needed to perform these numerous new and growing tasks expected from - and legally requested to – patients' representatives must come from the public sector. Funding from the public sector is the only way to guarantee independence and to expect capacity to deliver and accountability.

Patient representatives are more and more involved in different steps of the drug development process, e.g. orphan drug designation, scientific advice, active participation in clinical trials, compassionate use, risk/benefit evaluation, post marketing surveillance, etc. This involvement does increase the quantity and quality of information that patient representatives hold on relevant medicinal products. It is important for the rare diseases community to give the means to patient organisations to appropriately communicate on these products.

Validation of information and good practice guidelines

For rare diseases in particular, validated information does not always exist. Still, patients need information that is often investigational or not evidence based. In the absence of a formal and rigorous validation process, EURORDIS calls for the definition of good practices guidelines on how to establish control procedures in order to disseminate valid – if not formally validated – information, e.g. consultation with experts in the field and regulatory authorities prior to disseminate the information. For validated information, EURORDIS proposes to expand the training programme on where to find validated medical information on the Internet to more patients and patient organisations[2].

In the area of rare diseases where information is so scarce, it is particularly difficult to justify that important information has not been disseminated to patients until a complete and formal validation has been achieved. It is therefore necessary to encourage rare diseases patient organisations to discuss extensively with healthcare professionals and regulatory bodies the validity of the information before releasing it to their patients.

For information on a specific drug, precautionary measures have to be taken by consulting with the relevant "experts" on the drug, such as the persons responsible for its assessment in the evaluation agencies, the health professionals in the Centres of Expertise when they exist, and also the researchers from the pharmaceutical company producing the drug. We believe that the trust relationship that links the patients and their representatives would be respected

[2] EURORDIS training programme started in 2006.

if the patient organisation follows the good practice[3] of consulting all the "most informed people", which include the pharmaceutical company holding the MA.

No advertising to patients

It is fundamental to underline that EURORDIS - while being in favour of freedom of information and of wide access to information for patients - is firmly against the drift towards a USA-like system where advertising to patients is allowed. In fact, retention of relevant information that could be beneficial for rare diseases patients would be unethical, but misleading and inaccurate information could be dangerous and therefore also unethical.

It is worth-while noticing that the current EU legislative framework doesn't neither reflect nor respond to the reality, and creates further inequalities. In fact, patients and carers can easily have access to information through the web, including information from the marketing authorisation holders, as this is allowed in the US. Therefore, European citizens who have access to internet, speak English and are knowledgeable on how to search information on the Web are "privileged" compared to citizens having less access to electronic media.

Conclusions on the European Information System:

EURORDIS believes it is the responsibility of the European Community to elaborate and implement a system that will strike the right balance between access to reliable and up-to-date information and prohibition of direct or indirect (such as through disease-awareness campaigns) advertising to patients.

Important elements of the European Information System should be:

1. Definition of good practice guidelines for elaboration, validation and dissemination of information;

2. These good practice guidelines should include information control procedures with healthcare professionals and with patient representatives;

3. Training sessions for patient representatives to build their capacities as producers, disseminators and users of information;

4. Control by EU competent Health Authority should be *a posteriori* and not *a priori*: *a priori* control would create a bottleneck, delay in access to information and administrative burden. This *a posteriori* control would cover proper implementation of good practice guidelines and quality assessment of information. On this issue the Commission's proposal to create control bodies at national and European levels seems very unclear. In fact, as it stands the respective roles of these two levels' bodies seem to be too vague. In particular, the suggested co-regulation system with sanctions only for "repeated and severe cases of non-compliance is absolutely unclear and those not provide legal certainty. On the contrary, it leaves the door wide open to endless interpretation discussions on when non-compliance is "severe" or not, how many times should it be "repeated" before taking actions, and in the end may lead to abuses of all kinds;

5. Precautionary measures taken by patients organisations when important information has not been formally validated yet;

6. Firm commitment from all involved parties not to perform any kind of commercial promotion of medicinal products towards patients.

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[3] For more information concerning the delivery of information on rare diseases: http://ec.europa.eu/health/ph_projects/2002/rare_diseases/fp_raredis_2002_a5_05_en.pdf