genzyme

Contribution to DG Enterprise, European Commission
Public Consultation on a Legal Proposal on Information to Patients

We welcome the European Commission's initiative to issue a public consultation on a potential legal proposal on Information to Patients.

The objective of the legislative proposal to harmonise possibilities to access information should iron out differences that arise due to the geographical location of the person in question (caused by differences between national legislation) and segments of the population according to other criteria (e.g., Internet access, languages spoken, etc.), as well as difference caused by the prevalence of the disease.

We support the objective of the proposal and would like to make the following suggestions / comments regarding the consultation paper, with the objective of ensuring that the final proposal does indeed contribute to the objectives for all patients.

The biopharmaceutical and pharmaceutical trade associations have compiled comprehensive input for the consultation on behalf of their members, therefore, we do not intend to go into detail that repeats positions expressed in those papers. We support the proposals contained therein: careful definitions of what constitutes "information" and education on diseases / conditions vs. advertising, a continued ban on advertising of prescription medicinal products and effective, transparent and trusted monitoring mechanisms.

However, **in the field of rare diseases there probably are compelling reasons to allow for more information towards patients and the public in general** since more and better awareness about rare diseases and treatment options is a public health goal in the EU. If this is addressed, it may also substantially help to shorten the time to a confirmed diagnosis, which is one of the major difficulties for patients affected with rare diseases.

Our submission intends to highlight the specific needs and considerations around orphan diseases / conditions and Orphan Medicinal Products, as defined in EU Regulation 141/2000, and to make suggestions to ensure that such specificities are taken into consideration during the legislative drafting.

Rare Diseases – Information need even greater

As mentioned above, one of the key issues facing rare disease patients in Europe is a general lack of awareness of rare diseases. This is the case in the wider general public but also, in many cases, amongst the medical community as well. Given that there are an estimated 5,000-7,000 rare diseases, most physicians will never see a case of a specific rare disease during their whole career.

Many patients with a rare disease, therefore, unfortunately experience a "diagnostic odyssey" and may visit numerous doctors over many years (e.g. >15 years for Fabry

disease) before the correct diagnosis is confirmed. This is also illustrated by examples of patients who "diagnosed themselves" thanks to certain disease and product websites. Those without such information access are cut off from such opportunities. In addition, often we all hear from patients and their associations that a key wish (apart from access to treatment) is more information about their disease and treatment options.

The companies that are developing, or have developed, treatments for rare diseases or conditions have a wealth of information about such diseases, which builds up with each year of a treatment being available.

We, therefore, believe that there is no reason why these companies – which, in many cases may be the ones that have the most information about a product or the rare disease for which it is intended – should be excluded from playing a key role in providing information.

Of course we understand that this role must be subject to proper regulations and (self)-control mechanisms.

However, the **nature of Orphan Medicinal Products and the rarity of the diseases** and the activity in the field of research and development (or, rather, lack of activity in the field of research and development) **may create certain specific situations which should be considered during the development of the legal proposal.**

1. Channels & content for "push" tools for information on rare diseases & potential treatments (Information passively received by citizens: 3.3.1 – page 6)

Given that the objective of the legislation is to iron out differences between access to information, as many channels as possible should be used to **proactively push information on rare diseases / conditions (not products specifically)** to patients. It should be possible to provide unsolicited access to information on rare diseases and treatment options, but not on products.

This information access could be created and organised by a single company or multiple companies working together. However, given that there should be **no proactive push of specific products or one product over another**, where reference is made to treatment options, the different specific treatment options should be genericised ("oral tablets", "subcutaneous injection") and then either the material should either:

(i) Include a list of ***all*** the authorised medicinal products in the EU, irrespective of which company made the informational material available and links to the information for all those authorised medicinal products in an identical way for all of them; or

(ii) Direct the reader to official listings of approved and authorised treatments e.g., the EU's register of products with a European Marketing Authorisation and the list of national registers of approved products.

The existing www.orpha.net database is an excellent resource for information on products in development, clinical trials and other information on rare diseases as well as authorised products and treatment options. It should be further supported.

This kind of information on rare diseases should be able to be provided via any communication channels, including print, radio, television and mass communications. **Internet sites alone are not enough for informing about rare diseases, because significant portions of the EU population do not have access** to this for one reason or another, including language. TV, radio and other mass communication channels might not be desirable for more prevalent / well-known conditions, but for rare diseases, we believe that there is a case for using all available channels and appropriate tools possible to inform about the diseases and treatment options.

In the cases of rare diseases, there may only be one approved treatment (if any) available in the EU. The drafting of the legislation on information to patients should take into account this possibility when dealing with Orphan Medicinal Products, as defined in the EU Regulation 141/2000. It is possible that for a disease / condition awareness campaign, when making reference to "treatment options", there might be only one treatment option approved, due to the rarity of the disease. Having only one treatment available should, however, not prevent disease awareness campaigns because this would discriminate against patients suffering from certain rare diseases, who would be denied information just because there is only one treatment developed and available for their condition. The necessity to await a second treatment option to reach market should not prevent a patient from receiving valuable health information.

2. Content of information for the channels (Scope, content and general principles of the new legal provisions: 3.2 – page 6)

Unsolicited, "push" tools – as detailed above – **should only contain information on the disease / condition as well as the listings of any treatment options, as explained above**.

"Pull" tools, such as websites, as well as any information "pushed" to patients who, for example, sign up to electronic mailing lists should only contain product information based on the SmPC and the PIL or other scientifically validated data.

In all cases, the **information should be factual and unbiased**. This can be achieved by both **content** and **language used**, which should avoid value-based adjectives (e.g., "revolutionary").

Where possible, information on a medicine should be based on validated information such as that used in the legally approved Patient Information Leaflet (PIL) or the SmPC or scientifically verifiable data. Other types of information for rare diseases that could fall under "other limited medicine-related information" (section 3.2) could include mechanism of action, production process and information on clinical trials results of a therapy for a rare disease under development in line with those required in other public databases. This last type of information is very often requested by rare disease patients, especially those who have no treatment option available yet. It would be helpful to have clear guidance on how companies should handle such types of request and we believe that this proposal would provide the opportunity to do so in such cases.

It could also be useful to include information that could support diagnosis (e.g., reference centres) and other relevant information, including any company-provided support for reimbursement.

Proposed routes / channels for dissemination of information on rare, "Orphan" diseases as defined in EU Regulation 141/2000 and treatments covered by this Regulation			
Type of information	How get to patient	What is in it	Channels / routes
"Push" information	Unsolicited	Disease information / education only. With either links to all approved medicinal products for that condition or to EU / national listings of approved products	TV, radio, Internet, all available channels
"Pull" information	Sought out	Information on products – factual and scientifically substantiated	Only websites / help-lines or direct contact with company (no TV / radio mentioning products)

With regard to the source of information, it should be the content of a message (e.g., the information about a disease or product) that needs to be assessed vis-à-vis the rules and not the originator of the message. If good, well-regulated information comes from a company, it should be treated the same as information coming from a physicians' organisation. Likewise, information which does not meet certain quality criteria should not be distributed by a (bio)pharmaceutical company or by anyone else.

3. Monitoring information requested by patients (Answering requests from citizens: 3.3.3 – page 7)

Since replies by industry to enquiries from citizens will be monitored based on complaints, there should be a clear requirement on companies to establish methodologies to monitor such complaints, including guidelines and methodologies on how they are logged, maintained, tracked and responded to.

Evaluations of the implementation should be included in company regulatory audits as part of good operating procedure evaluations. This will ensure proper corrective action can be undertaken should the case arise.

4. Quality Criteria (Section 4, page 7)

Section 4 (page 7) refers to "Quality Criteria", including the statement that "Comparisons between medicinal products should not be allowed".

Under the EU's Orphan Medicinal Product Regulation 141/2000, referring to a period of Market Exclusivity:

"*the Community and the Member States shall not…accept another application for a marketing authorisation, or grant a marketing authorisation…for the same therapeutic indication, in respect of a similar medicinal product*" [Article 8(1) of 141/2000], but "*by way of derogation…a marketing authorisation may be granted, for the same therapeutic indication, to a similar medicinal product if: … (c) the second applicant can establish in the application that the second medicinal product, although similar to the orphan medicinal product already authorised, is safer, more effective or otherwise clinically superior*" [Article 8(3) of 141/2000].

During the period of Market Exclusivity, in some cases, sponsors might be required to perform comparative trials to prove clinical superiority over another product before being granted a Marketing Authorisation in that period. It could even be referred to in the documentation around the Marketing Authorisation.

In other cases, the mere granting of a second Marketing Authorisation during the same period will indicate that the authorities believe in one way or another that the second product is in some way "clinically superior".

The fact that a company may have provided information to prove clinical superiority in order to be granted Marketing Authorisation (which may be listed in the SmPC / EPAR / PIL) should not, if a link is provided to these documents, be considered as comparison.

5. Oversight, Monitoring & Penalties (5. "Proposed structure for monitoring and sanctions" – page 7)

We are convinced that regulating the dissemination of disease- and product-information, towards the public or patients according to the different channels and with different content / approaches detailed above, is not only justified, but highly desirable.

Our preference is to apply self- or co-regulation among the various stakeholders in the field of rare diseases particularly, since we believe that such a system has the potential to balance the need for effective "policing" and avoid overly bureaucratic systems.

However, we believe that the proposal to have information regulated by **a series of national bodies could potentially undermine the overall value of creating one set of rules, especially for rare diseases.** If there were a series of national approval bodies, this could lead to a continuation of the country-by-country approach, which is detrimental to the field of rare diseases because of their rarity. This will undermine the objective of facilitating harmonisation of access to information. Patients in larger countries could continue to benefit but maybe at the expense of smaller countries. Additionally, the creation of multiple layers of oversight could risk creating a burdensome and overly bureaucratic process, which might deter provision of information for small patient populations which are, arguably, the most in need of information.

If we take a website as an example, in an ideal world, the text of the website would be developed based on validated information such as the SmPC and the PIL to create on text, and then translated into the official languages of the EU, accessible to all. If that

draft text had to be reviewed by 27 different national bodies, there would be the possibility of it ending up as 27 different sets of text, to meet the differing national codes and/or to meet the points of view of the 27 different national bodies. The smaller the company and the smaller the patient population, the more burdensome would this become, with the need to monitor and comply with 27 different codes, to ensure that the information remained compliant with potentially changing codes.

For rare diseases, there should be **one set of information that is valid throughout Europe**, which is **overseen by a single European Committee**. This could be an amalgamation of the self-regulatory approach proposed via association membership and the Advisory Committee with no Comitology powers, chaired by the Commission, as proposed on page 8 (section 5 c). If such a self-regulatory Committee of members would be chaired by the European Commission and would have a seat for an EMEA representative, it would increase the perception and validity of the self-regulatory approach.

Conclusion

We welcome the opportunity to support the contribution of the trade associations of which we are members and, also, to give this **additional input for consideration when dealing with rare diseases and Orphan Medicinal Products** in the new legislative proposal.

Since its foundation in 1981, Genzyme has been focussed on researching and developing treatments for rare diseases. In 2001, Genzyme received the first Marketing Authorisation in the EU for an Orphan Medicinal Product, and currently has several orphan medicines on the market, as well as several development projects with designated orphan status.

Since then, Genzyme has also diversified into many fields where it can apply the most advanced technologies to making a major positive impact on the lives of people with serious diseases.

However, **our 26 years' experience** in the research, development and treatment of rare inherited disorders – including of the EU system following the creation of the Orphan Medicinal Products Regulation, means that we have heard **first-hand from many patients** what they would like from us and **what they believe we should provide to them as information.**

We see the European Commission's current initiative as a huge opportunity to do the right thing for Europe's rare disease communities alongside doing the same for those with more prevalent or well-known conditions.

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Thank you for giving us the opportunity to input into this process.
We hope you find our contribution useful.
However, should you have any questions or require any additional information, please do not hesitate to let us know. We would be happy to provide further details:
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