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FBG views on
The Future of Pharmaceuticals for Human Use in Europe

Recently, the European Commission (EC) posted a questionnaire on the topic of "The future of pharmaceuticals for human use in Europe". They requested as many stakeholders as possible to give their views on ways to both strengthen the competitiveness of European Companies and patient safety.
The 'Forum Biotechnologie en Genetica' (Forum on Biotechnology and Genetics, FBG) is the national Dutch multistakeholder discussion platform chaired by former health minister Dick Dees. It is supported by contributions in kind from its stakeholders and further financed by the Dutch Health Ministry (VWS). Its aim is, by fostering debate amongst stakeholders and by providing VWS with advice via e.g. issue papers, to provide patients as quickly as possible with the therapeutic improvements emerging from the application of biotechnologies in healthcare.

The FBG is happy to present its views. It will not do so along the lines of the key questions as listed in the EC paper, but rather by providing some observations on several issues relevant to those questions.

Role of patients

Although touched on in the document, the European Commission might place more emphasis on the role patients can play in the further regulatory landscaping in the EU. Their practical approach has proven a welcome addition in many discussion fora. For instance, they have their own views on the balance between protecting patient safety and promoting development of innovations. Sometimes authorities place emphasis on the former to the detriment of the latter. However, in some cases patients would take a less risk-averse view. A middle road should be found here.
Finally, a fresh look should be taken at the balance between protection of privacy and the delaying effect the privacy legislation can have on medicinal product development in cases where cooperation with patients is of crucial importance.

Small and Medium Enterprises (SME)

Although important for larger companies as well, SME would very much appreciate a more proactive approach of regulatory authorities in guiding them through the maze of the regulations that govern the R&D and production process. The EMEA has in recent years pioneered activities in the area of training and guiding small companies that have to use these regulations but do not have the specialized personnel to adequately interpret and apply the regulations. Generally, an extension of activities along the lines of consultation, as a companion to regulation and enforcement, would be welcomed. On the other hand, in some areas EMEA personnel is still cautious in giving informal advice, as they fear it will be used against them as if it had been a formal guidance. Some solution will have to be found here.

R&D and production sites

The choice for R&D and production sites is interlinked. Mostly, production goes to areas where the balance between availability of trained personnel on the one hand and operating and salary costs is optimal. In recent years, however, it has also been noticed that R&D facilities tend to relocate to the vicinity of the production sites. The EC cannot influence the operational costs in the EU, but it can perhaps stimulate the availability of sufficient amounts of trained personnel. Not only the 7th Framework Program would be instrumental here, but also a rapid implementation of the Innovative Medicines Initiative. Another initiative that would position the EU as being more attractive is one that promotes creating a level playing field regarding production licenses in the individual member states. Too much inter Member State variation in this area (as in other ones) impacts negatively on the EU's attractiveness.

Access to treatment

The EU should make equal access of citizens to all available treatments a top priority. Between many member states, large differences still exist in purchasing power, whereas even within member states, financial constraints lead to unequal distribution of treatments over patients. All efforts by the EU to improve Europe as a location for research, development and production of adequate treatments, would be useless from a patient perspective if it would not lead to accessibility, within a reasonable timeframe and at reasonable cost in terms of insurance coverage or out-of pocket payments, for all those who would benefit from that treatment.
Even though reimbursement decisions and lag time between registration and actual availability are a national responsibility, the EU might find means to stimulate shortening of the timelines involved.

Counterfeit, parallel trade and access

The growing threat of counterfeit medicines to the European patient necessitates creative measures by the European Commission. Counterfeit is greatly facilitated by a flourishing parallel trade. It is accepted that parallel trade is necessary for the functioning of the Union. Still, the European Commission as well as the European Court of Justice have realized that in order to protect the patient, parallel trade must sometimes be curbed. Moreover, parallel trade is the greatest threat to a policy of tiered pricing, adopted by the pharmaceutical industry to solve the problem of access to e.g effective HIV medication in underdeveloped countries
The customs controls to detect counterfeit have become more effective. Yet within many member states, the manpower that Inspections devote to detecting counterfeit is insufficient. Surprisingly, both inspection activity and fines for breach of marketing codes[1] are very high. This should be matched by corresponding penalties at least as high, for an activity that is potentially much more detrimental to patient health.

[1] "….a fine not exceeding 5% of the Community-wide turnover in the preceding business year. It may also impose a daily penalty not exceeding 2,5% of the MAH's average daily Community-wide turnover in the preceding business year."

Research funding and regulatory compliance

The administrative burden accompanying the application procedures for the 7th European Framework is sometimes too high for SME's. Occasionally, they even prefer to do business with partners outside the EU rather than within.
Corresponding movements may be the result of the burden of regulatory compliance. It is not so much the lack of speedy response from regulatory authorities that dogs both small and large companies, but the lack of predictability of regulatory behavior and the long time periods needed to create or update European Guidelines. This results in postponement of business decisions, sometimes prompting companies to contract out or relocate to outside the EU.

Animal experimentation

A major threat to the development of new therapies for EU patients is the overoptimistic notion, promoted by the anti-animal experimentation lobby, that total replacement of animal experiments by in vitro methods is easy and achievable today, provided that enough funds are available. The truth is that concerted action by all stakeholders concerned certainly has resulted and will result in the use of lesser and lesser animals to solve a given research question. Nevertheless, continued investment by the EU in alternatives for animal experiments is recommended. If however, the EU would act precipitately in severely restricting or banning animal experimentation on the basis of unproven assumptions, the competitiveness of the industry would suffer. On a global scale, the amount of animal studies would not decrease, but would be contracted out on a larger scale to e.g. Asia. Moreover, the use of animals to prove the effect of new drugs surely needed to combat cancer and contagious diseases like HIV, will remain necessary in the foreseeable future.

Information to patients

Patients should be more adequately informed about treatment options for their diseases, including lifestyle and preventative measures. The national and perhaps also the European authorities should take the lead here, given the fact that single stakeholder communication might be unbalanced.
But patients should be adequately informed about *all* treatment options for a given disease. Several ideas and plans have been presented on this topic by e.g. EFPIA and the Europeanl Patients' Forum.
To extend these thoughts, the creation of a European-wide Center of Expertise for specific diseases, with national satellites, might be an option. The Centers would be charged with compiling expert knowledge from all relevant stakeholders. This should be used to communicate to the general public the direction and focus of current R&D and clinical research, emphasizing the meaning of this research for the short and long term prospects of the patients. Moreover, the Centers would be an ideal repository of the experiences of patients with existing treatments against their disease. These data could be used to supplement meta-analyses with patient preference and -experience. Finally, the Centers could offer a platform, not only for healthcare professionals. They could also be used to facilitate patients to communicate with each other on drugs and (future) treatments. As such, the centers could function as an extension of existing patient organizations. Finally, the Centers could communicate about the interpretation of safety problems of drugs and their practical relevance to patients. The organization and management of these centers should be driven by a collaborative effort of medical professions and patient representatives, and financed wit fund deriving from a EU framework-like construction.

Those managing such centers should actively collect relevant information from all stakeholders, including health researchers, fundamental scientists, patients and industry. This approach might be instrumental in solving the current deadlock on the information to patients issue.

Pharmacovigilance

The current approach to pharmacovigilance by the EMEA is adequate. More rules and regulations will most probably not result in a corresponding increase in patient safety. However, the communication to the public of the outcome of regulatory investigations about e.g. unexpected effects of medicinal products is inadequate. In a number of cases, the compounds in question have been highlighted in the press as being dangerous, but corrective coverage has rarely occurred after the compound was partly or completely cleared of suspicion by the EMEA. The EMEA may not be well placed to communicate to the public about this. Perhaps a body like a Center of Expertise may be of help here.

Emerging technologies

The European regulatory system will be able to accommodate future developments in regenerative medicine, nanotechnology etc. For a flexible approach to Advanced Therapies, some guidance is already in place.. The creation of the Advanced Therapy regulation is a good example: a system is created to adequately deal with emerging technology as knowledge and experience expand. The provision however is that regulation follows the establishment of best practices and not the other way round. Therefore, actual requirements need to be formulated as soon as knowledge accumulates.

Medicinal products for special groups

More research into the development of new drugs for special and sometimes neglected groups such as children, sufferers from rare diseases and the elderly is needed. These topics are part of the philosophy of "Priority Medicines", and attention is also given in FP7 context. For instance, management of polypharmacy in the elderly and the creation of new and better administration forms to improve compliance fit well in the European context of scientific capability. A point for concern is that both for medicinal products for children and orphan diseases, it is the in particular the more rare instances that need EU attention for two reasons: even with stimulating legislation, they will still be unattractive commercially. In the second place, the EU could play a important role in aggregating rare expertise on a European level, as these things are difficult to achieve at member state level.