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Orphan Drugs and Industrial Research (**)

The problem of orphan drugs is an issue of the utmost importance to mankind and has always been of great concern for our industry.

I would have been very glad to take advantage of this occasion and illustrate to you here the approach of our industry, but unfortunately there was no time to perform an enquiry among our members and to discuss with them the relevant aspects of a problem which requires a practical and pragmatic approach.

But, even if I am not here in the capacity of a speaker of the whole Italian industry, I am, all the same, a qualified member of this important sector.

After having been introduced, nearly forty years ago by my teacher, Prof. Marini-Bettolo, into the world of chemistry, I have spent a professional lifetime in pharmaceuticals, first in research and in production, then in the management of the international affairs of our industry.

Industry will consider with great attention the invitation to join in a program which intends to find practical solutions of this problem, but the practical aspects and the tasks to be assumed by the participants will have to be discussed with the individual industries.

We may, however, try to outline a workable overall scheme of action.

All of you know well that ours is one of the few actually advanced and truly research-based industries and that our innovation is almost completely based on industrial funding.

This condition has allowed our industry to operate with great flexibility and independence in the choice of targets and to adapt its scientific strategy to the staggering speed of evolution of the scientific background.

In a very short lapse of time, less than two generations, we have realized breakthroughs and achieved goals which have completely modified centuries of pathology and reversed the prognoses of quite a number of terrible scourges.

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I do not wish to repeat here facts known to all of you, but I would like to draw your attention to a serious drawback in this otherwise outstanding situation: the cost of research.

Science has always been a tremendously expensive item and had it not been for its unbelievable return — return in the broadest meaning of the term, like a logarithmic growth of our scientific knowledge, the dramatic improvement of our health and socio-economic conditions — science and research could have been considered a hobby of the affluent world.

But this high level of cost imposes also very severe limitations on industry.

According to figures disclosed recently in the European Parliament, the development of a new drug requires today something like 50 to 100 million ECU, corresponding to 30-60 million dollars, and an average of 10 to 12 years for research, development, clinical trials and market approval.

This astronomic amount of investments in terms of capital, manpower and time can be recovered only by commercially successful drugs.

Today there is not any industry able to afford such a challenge as the research of drugs with a limited or no market at all.

This is a diagnosis, a matter of fact. But this does not mean that there are no solutions; such a conclusion would be a tragic mistake.

There are many orphan drugs; some of them, like those needed by the developing world, have actually a huge market, but this market could not afford their economic cost. A similar problem exists also in the industrialized countries. Here even an expensive drug would do, but often the actual cost would all the same be much too high.

The problem of orphan drugs is a very complex one and there are no simple ways for a satisfactory solution because too many divergent exigencies are involved and a workable approach must give an acceptable reply to most of them.

We may consider this problem from several points of view: first of all the social aspects and those of public health. These are political problems and the responsibility for a solution belongs to the national authorities and to the international organizations which should mobilize the resources of the affluent nations to the benefit of the deprived ones.

From the point of view of the industry, the orphan drugs represent an exciting challenge for reasons of scientific prestige and, why not, an occasion to improve its reputation and its public image.

The industry is already engaged in quite a number of absolutely non-profit initiatives, which require an enormous scientific and technical skill, and many industries market drugs which have a very restricted market and are manufactured at a loss, but are still available. These are certainly true orphan drugs. There is of course a return in these operations in terms of prestige, of human solidarity and reliability, but obviously there is a limit to these activities because the costs must be recovered from the profits realized by other, commercially more successful products.

These considerations introduce us to the economic point of view, which probably is the most important one because economic requirements restrict heavily all nonprofit operations of the industry.

Any industrial activity, like the production of goods and of services, is possible only if there is a return on the investment, which allows to recover the costs and to realize a certain profit margin. This is a universal law, valid in the capitalistic as well as in the socialist world. In fact, not even the industries of the socialist countries, whose political reasons often prevail over the economic ones, have been able to give a significant contribution in the field of orphan drugs.

There are also other problems, like legal aspects and scientific and technological problems. The most important legal problem is certainly a clear definition of the basic item: the orphan drug itself and its legal status, so as to avoid an undue exploitation of the facilitations granted this particular category of products.

Another important problem is the rights of intellectual and industrial property. There is a considerable amount of ingenuity and know-how involved, and the rights of those who have contributed to the development should be duly protected.

Then we have the problem of toxicological, pharmacological and clinical trials and the procedures for market approval, which certainly will be somewhat different from those for ordinary drugs.

The "Orphan Drug Act", approved in 1983 by the U.S., represents a first step in this direction and could be an important model.

The scientific and technological problems concern the scientific community as well as the industry. You know that in a development of a new drug, basic research and technology are interconnected in an indissoluble way.

We are here to discuss possible solutions for a difficult and complicated problem and I would be glad to offer even a small contribution. I would suggest that you consider the approach proposed by Farmindustria to overcome the critical health conditions in the developing world. It was illustrated by me in our magazine "L'Industria dei Farmaci", issue 7-8 of 1984. The basic idea was to mobilize the resources, the efforts, the skill and the experience of the interested parties, namely, the governmental authorities of the developing world, the World Health Organization and other international bodies and the pharmaceutical industry, for a responsible cooperation on the health problems of the developing world.

Because the actual cost of drugs and health assistance often exceeds the available resources, we suggested that a quota of the funds appropriated for assistance to the development of Third World countries, should be allocated for purposes of health assistance in the realization of specific projects in cooperation with the industry. These funds could fill the gap between the actual cost of the project and the available resources. Such a system would also prevent misuse and dispersion of valuable efforts and resources.

I wonder if a similar approach could be useful in the case of orphan drugs. The funds allocated by governments and other sponsors could be utilized for grants destined for individual research projects to be realized in cooperation with the industry. This is just an idea; there may be other solutions and we would be glad to discuss them with you.

I hope that my remarks have been of some interest for you and I thank you for your attention.