Conclusions

The Accademia delle Scienze detta dei XL organized in March 8-9th an International Meeting on "New Strategies for Orphan Drugs" sponsored by the Directorate General for Science Research and Development of the EEC and with the collaboration of the "Società Italiana di Medicina Tropicale" and the "Società Italiana di Scienze Farmaceutiche".

The participants discussed several aspects of the orphan drugs problem, in particular those concerning the developing countries, and reached the following conclusions:

The development and production of "Orphan Drugs" are important for both their social impact and the international responsibilities towards the needs of developing countries.

Orphan Drugs may be considered drugs (i.e., pharmaceutical and biological products including vaccines) of limited commercial value, thus preventing substantial investment needed for their development.

Under this definition, which applies to a worldwide approach to the problem and not the health policies of single countries, we may consider two main categories:

1 - Drugs for rare diseases;

2 - Drugs for developing countries, and in particular new drugs for tropical diseases.

From this point of view the participants discussed three main items:

a) the identification of the targets and their possible priorities;

b) the methods to promote research;

c) the possible way to remove the economic and regulatory barriers which hinder the research and development of these drugs.

Generally, orphan drugs are *new* drugs to be discovered, studied and developed, but there are also examples of highly efficient drugs already known and not on the market because of their negative economic balance. It was agreed that a different approach to the development of new orphan drugs should be envisaged for rare diseases as differentiated from those of developing nations.

For rare diseases (talassemia, Alzheimer, idatitosio, etc.) a programme of incentive — including grants from private and public institutions — is recommended.

For developing nations the identification of specific diseases — such as filiariasis, leishmaniasis, trypanosomiasis, malaria, schistosomiasis, leprosy, etc. — is

the necessary first step to focusing global research capacities, including biotechnologies, for the control and eventual eradication of these diseases. It has been stressed that in developing countries new drugs would not be sufficient for the control of the diseases unless accompanied by a vector control — in respect to the environment — and by a hygienic and socio-economic education.

A realistic approach for obtaining new drugs for the above purposes should be based on international collaboration between academia, research institutes and industry.

Owing to the high cost of a new drug (about 50-60 million dollars) and the need of substantial investments, manpower and scientific effort, it is necessary to establish at world level a program of research and development. This implies the choice of priorities and coordination of the programs as well as the division of tasks. Also drugs for animal diseases should be considered in this regard.

The development of new drugs generally requires basic research on the etiology of the diseases, and in the case of communicable diseases, on the mode of transmission and on the biology of the pathogenic agent. Fundamental research should take place mainly in the research centres of developing countries which since the beginning of this century have greatly contributed to the advancement of knowledge in this field.

The development of new drugs based on the above results should be undertaken in cooperation with the scientists of developing countries.

The possibility of manufacturing these drugs in developing countries should be considered: the positive results obtained in these countries in the production of important vaccines and medicinal products should be further developed.

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The promotion of research on orphan drugs for rare diseases may be achieved through private and public financing and at the national and international levels.

In the case of new drugs for developing countries, the importance of financing the fundamental research carried out in these nations and the need to support their research centres should be stressed.

Collaboration between developing countries should be encouraged and also the North-South cooperation, especially in the training and in the use of highly specialized technologies, should be supported.

Financing of these programs could be made by allocating to this purpose a percent, even small, of the funds assigned to development by the various nations.

International centres of research and formation, such as those on biotechnologies of Trieste and New Delhi, could be of great importance for promoting specific research in this field and for the formation of the operators.

It is known that public and private funds of many countries are available, which could be utilized for this purpose.

National and International Academies of Science (the Academy of Science of the Third World, the Latin-American Academy of Science) could cooperate in

this effort, with national and international organizations to establish priorities and programs for developing new drugs. These are urgently needed in order to control endemic diseases which at the present moment *constitute the main negative factor hindering development.* Also for orphan diseases it is important in a social context to promote research for new drugs.

The participants in the meeting, representing academia, industry and research centres of many countries, suggest that this document should be submitted to the General Directorate for Science, Research and Development of the Commission of the European Communities, sponsor of the present meeting.

They also propose that this document, through adequate channels, should be brought to the attention of the Governments and of the International and National Agencies. It is important to stress the need for prompt action in promoting research, development and manufacture of orphan drugs, mainly those for tropical diseases.

This document should also be sent by the Accademia delle Scienze to the United Nations and their Agencies, in order to give the broadest support to this initiative.