

# From GMP to GBP\*

## Fostering Good Bioethics Practices Among the European Biotechnology Industry

### Gene and Cell Therapies

#### Project Summary

The project: "From GMP to GBP – From Good Manufacturing Practices To Good Bioethical Practices" is a Specific Targeted Research Project funded by the European Commission, Sixth Framework Programme.

The project aims at integrating bioethical practices as an intrinsic part of industrial standards, just as Good Clinical Practices (GCP) or Good Manufacturing Practices (GMP) are. These standards of Good Bioethical Practices (GBP) would allow the industry to define its own issues and recommendations inform to European Union legislators and relevant authorities. The project also aims to improve understanding of bioethical issues, based on the current practices of biotech companies, as well as to elaborate clear and independent positions on bioethics, based on regularly updated scientific and technological data. The final step will involve disseminating this information to the industry and to society.

The project is piloted by the French biotechnology association, France Biotech, in partnership with a consortium comprising the European Association of Bioindustries, EuropaBio, and national biotechnology associations from Estonia (the Estonian Biotechnology Association), France (France Biotech), Hungary (the Hungarian Biotechnology Association), Sweden (SwedenBio) and Spain (ASEBIO), as well as the French Institute for Health and Medical Research (INSERM U558).

#### Issues

The lack of an EU-wide regulatory framework in the past led to divergent national approaches which hindered patient access to products, hampered the growth of this emerging industry, and ultimately affected EU competitiveness in a key biotech area. Recently, however, EU Institutions agreed on a Regulation on Advanced Therapies (ATMPs, see note) to ensure the free movement of advanced therapy products within Europe, to facilitate access to the EU market and to foster the competitiveness of European companies in the field, while guaranteeing the highest level of health protection for patients.

Gene and cell therapies and tissue engineering share several key scientific, regulatory and economic features:

- They are based on complex, highly innovative manufacturing processes. The specificity of the product lies precisely in the process.
- Traceability from donor to patient, long-term patient follow-up and a thorough post-authorisation risk management strategy are crucial aspects to be addressed when evaluating advanced therapies.
- Advanced therapy products are subject to rapid and often radical innovation.

A main issue affecting the Regulation on Advanced Therapies Medicinal Products is that there is no consensus among Member States as to which harmonised decisions should be taken on the use or prohibition of ATMPs containing certain types of cells, such as embryonic stem cells. A central issue affecting all cell and gene therapies is the potential impact on biodiversity.

*Note: The term "Advanced Therapy Medicinal Products" regroups new medicinal products based on genes (gene therapy), cells (cell therapy) and tissue (tissue engineering). These advanced therapies herald revolutionary treatments for a number of diseases or injuries, such as skin in burn victims, Alzheimer, cancer, or muscular dystrophy, and are expected to have a major impact on public health.*

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# From GMP to GBP: Gene and Cell Therapy

## Recommendations

### Advanced Therapies Medicinal Products Regulation

The recommendations of the consortium concerning the Advanced Therapies Medicinal Products Regulation are:

- 1▶ There should be no room for national interpretation as Member States apply the Advanced Therapies Medicinal Products Regulation.
- 2▶ It is important that the expertise within the Committee on Advanced Therapies (CAT) is a true pooling and sharing of expertise within the EMEA's CAT.
- 3▶ The role of the CAT as leader in the assessment of marketing authorisations should be clarified, and its productive interactions with other EMEA Committees should be ensured.
- 4▶ The discussions related to the ATMPs Regulation in the European Parliament were in part overloaded by ethical concerns. It would be more helpful if ethical questions could be discussed separately for more technical regulations, or implementation of guidelines, in order to achieve a true EU-wide harmonization.

### Impact on Biodiversity

In some cases participants in gene or cell therapy trials are not the only people at risk. Germline gene therapy can also affect the genetic heritage of future generations with hazardous consequences.

- 5▶ The consortium recommends that germline gene therapy should be strictly forbidden.

### Informing the general public

- 6▶ The consortium stressed the need for greater information for the general public regarding the potential benefits of Advanced Therapies Medicinal Products, scope and advantages of new advanced therapies, and a raising of civic awareness.
- 7▶ It is agreed that there is a serious need to achieve greater dissemination of results and to undertake tasks aimed at informing society about new biomedical advances. Furthermore, it would be appropriate to take the opinion of the general public into account in such paramount issues and ones where the outcome may affect everyone.

### Informed consent of donors

Generally, the standards for consent are negotiated according to the type of biological element and the ethics committee concerned. The majority of emerging ethical questions are related to the use of cell lines (whether they are embryonic or not), to questions concerning the embryo, to marketing, and to the original properties of the cells.

- 8▶ The consortium recommends that the consent form should give a choice to donors as to what uses they agree upon.

### Donor Compensation

Where compensation and/or reimbursement for donation is concerned, the group came to consensus. The general agreement was that a donor should receive compensation/ reimbursement for time spent even if there are no expected benefits or a potential health risk.

- 9▶ The consortium recommends support for compensation for human tissue donors, but only for their expenses and inconvenience, when appropriate.