

HUGO ETHICS COMMITTEE:

STATEMENT ON GENE THERAPY RESEARCH April 2001

Introduction

The advent of recombinant DNA technology and the publication of the draft sequence of the human genome have raised hopes that new genetic knowledge will lead to the increased possibility of treating diseases, including by gene therapy. Media interest has called these hopes to general attention.

For the purpose of this Statement, gene therapy refers to the correction or prevention of disease through the addition and expression of genetic material that reconstitutes or corrects missing or aberrant genetic functions or interferes with disease-causing processes. It has become standard among many researchers to use the term gene transfer instead of gene therapy, to refer to the fact that gene transfer may not always be intended to result in therapy. However, the term gene therapy remains in popular usage, so we have retained this term as a title to this statement. Genetic enhancement refers to a similar transfer of genetic material intended to modify non-pathological human traits. Inheritable genetic modification formerly known as germline gene therapy, as well as genetic enhancement is not addressed in this statement. This Statement is limited to somatic gene therapy, which does not affect biological descendants.

There has been a considerable change with respect to the focus of gene therapy research. Some 15 years ago, it was generally believed that the chief focus of gene therapy would be single gene diseases. Gene therapies for immune deficiencies, inherited anaemias and cystic fibrosis, for example, are the subjects of active research. Emphasis has shifted to attempts at experimental gene therapy for eventual use for common multigenic disorders, such as cancers and cardiovascular disease.

Although results justifying a cautious optimism are beginning to emerge from human gene therapy studies, all such studies remain experimental in nature. They carry risks, with benefits that are as yet uncertain.

Gene therapy is unusual in that the technology was preceded by extensive ethical debate. However, regulations on how to conduct gene therapy exist in a minority of countries. Moreover, conflicts of interest are a problem in this area. They arise in a number of ways. These conflicts compromise scientific objectivity, impair trust, and may endanger the welfare of research participants.

The existence and influence of ethical safeguards vary in different countries. In many, they do not exist, and where they do, they may not have a mandate to address gene therapy research.

The objectives of this Statement are to:

- Respond to public concerns about the ethical conduct, quality, and safety of somatic gene therapy research.
- Distinguish somatic therapy from germ-line therapy (inheritable genetic modification) and from genetic modification for enhancement.
- Stimulate discussion towards the adoption of international guidelines.
- Propose a framework for the public accountability of gene therapy research.

Common Principles

In its 1996 Statement on the Principled Conduct of Genetics Research, the HUGO Ethics Committee addressed concerns surrounding human genetic research involving individuals and populations. The four founding principles of the work of the Committee are relevant to the ethical responsibilities that researchers have in conducting gene therapy research, namely :

- Recognition that the human genome is part of the common heritage of humanity ;
- Adherence to international norms of human rights ;
- Respect for the values, traditions, culture, and integrity of participants ; and
- Acceptance and upholding of human dignity and freedom.

Recommendations

In view of the significant potential benefits of somatic gene therapy in treating disease, the HUGO Ethics Committee:

- Recognises the particular need for public oversight and ongoing review of somatic gene therapy research.
- Recommends that countries ensure that they have a national ethics body that includes in its mandate somatic gene therapy.

- Supports continuation of somatic gene therapy research that conforms to this Statement.
- Urges researchers, professional organisations, sponsors and governments to listen and respond to public concerns about the benefits, risks and ethical conduct of somatic gene therapy research.
- Recommends that all research be subject to stringent quality and safety controls and be in conformity with international ethical norms.
- Recommends that material conflicts of interest be identified, declared and addressed in the most transparent way possible, in particular to persons who are the subjects of research.
- Proposes that schemes of indemnity for harms directly incurred by participants and others as a consequence of research should be established.
- Recognises the important role of both researchers and the media in reporting on the developments in gene therapy research in a responsible and informative manner.
- Calls for widespread discussion on the appropriateness of the possible future use of gene transfer technology for enhancement and for germ-line interventions.

Note

It is the intention of the HUGO Ethics Committee that this Statement is to be read and implemented consistently with the principles outlined in its previous Statements.