Conclusion

The first realistic applications of human gene therapy will be closely scrutinized by both the public and the Federal Government. Civic, religious, scientific, and medical groups have all accepted, in principle, the appropriateness of gene therapy of somatic cells in humans for specific genetic diseases. Somatic cell gene therapy is seen as an extension of present methods of therapy that might be preferable to other technologies. Whether somatic cell gene therapy will become a practical medical technology will thus depend on its safety and efficacy, and the major question is when to begin clinical trials, not whether to begin them at all. The quality that distinguishes somatic cell gene therapy most strongly from other medical technologies is not technical, but rather the public attention that is likely to attend its commencement.

Federal oversight mechanisms for research and clinical application of *somatic* cell therapy are already in place, and enforcement of the mandated approval processes has already taken place in one instance, the breach of NIH guidelines perpetrated by Dr. Martin Cline. Committees exist at local institutions to monitor protocols for human subject protection, and all proposals for federally sponsored clinical trials should be referred to the RAC at NIH for approval, and may also be reviewed by FDA.

The consensus about the propriety of somatic cell therapy does not extend to treatment for traits that do not constitute severe genetic diseases, and does not encompass germ line gene therapy in humans. The question of whether germ line gene therapy should ever begin is now highly controversial. The risk to progeny, relative unreliability of the techniques for clinical use, and ethical questions about when to apply it remain unresolved. The question of whether and when to begin germ line gene therapy must therefore be decided in public debate informed by technological developments.

If gene therapy develops as a viable new medical technology, issues will emerge regarding who is to pay for it, how to assure equitable access to it, who is qualified to perform it, how to regulate its proper use, and which diseases merit its application. Many Federal agencies, including NIH, FDA, and health care payers, will be involved in such issues if the technology becomes part of standard medical practice.