Congress Reports on Gene Therapy

The first authorized attempts at correcting genetic defects by use of human gene therapy are expected to take place sometime in 1985, assuming that approval from the National Institutes of Health (NIH) and Food and Drug Administration (FDA) is forthcoming. An experimental protocol for using genetically-engineered organisms in patients with Lesch-Nyhan syndrome has already been approved by the local Institutional Review Board (IRB) at the University of California at San Diego. A proposal to try human gene therapy in the treatment of adenosine deaminase (ADA) deficiency, the immune disorder that afflicts so-called bubble children, is also expected to be among the first to be passed up to NIH and FDA for approval at the national level.

Anticipating the advent of human gene therapy, Representative Albert Gore, Jr. (D–Tenn.), asked the congressional Office of Technology Assessment (OTA) to conduct an analysis of the scientific and ethical issues inherent in medicine's advance into the treatment of certain generally rare diseases by the repair or replacement of defective genes. The OTA has just released its report, a remarkably lucid document that clearly distinguishes between gene therapy for somatic or body cells that will only affect the patient being treated and germ line therapy that would lead to heritable changes.

The report, which has been endorsed by Gore, who won a seat in the Senate in the recent election, sees no unique ethical obstacles to somatic cell therapy, provided that considerations of safety and some reasonable expectation of efficacy are met, as they must be for any new experimental medical procedure. According to the report, "Because cells that are used in reproduction are not involved, gene therapy [in somatic cells] is quite similar to other kinds of medial therapy, and does not pose new kinds of risks. When considering gene therapy that does not result in inherited change, the factor that most distinguishes it from other medical technologies is its conspicuousness in the public eye; otherwise it can be viewed as simply another tool to help individuals overcome an illness."

On the subject of germ line experimentation, the OTA report is much more cautious. "There is," it says, ". . . no agreement about the need, technical feasibility, or ethical acceptability of gene therapy that leads to inherited changes. [Therefore, it] should not proceed without substantial further evaluation and public discussion."

The OTA report was based on material gathered from hearings Gore has held on human applications of genetic technology, as well as conclusions from a specially convened advisory panel comprised of scientists, attorneys, representatives of groups of patients with genetic diseases, religious leaders and others. It was chaired by LeRoy Walters of the Kennedy Institute of Ethics at Georgetown University. The OTA evaluation, which includes extensive discussion of the real and perceived moral questions that surround biotechnology, is noteworthy in its support of somatic cell therapy for the treatment of appropriate diseases.

—BARBARA J. CULLITON

Another Round in Rifkin Versus Gene Splicing

The National Institutes of Health (NIH) and activist Jeremy Rifkin clashed again recently in federal court over the issue of releasing genetically modified organisms into the environment. If the judges' remarks in court and their past rulings are any indication, NIH may be required to produce a full-scale environmental impact statement before approving any more so-called deliberate release experiments. However, the actual ruling from the U.S. Court of Appeals for the District of Columbia, which heard the case on 5 December, is not expected for months.

NIH and the University of California are appealing a decision handed down in May by federal district judge John Sirica that, in effect, put a moratorium on all field tests of genetically altered organisms by academic and industry researchers. (Private companies are not legally bound to refrain from field testing since Sirica only halted only federally supported research, but so far companies have chosen not to go forward.) Three tests had been planned when Sirica ruled in May (*Science*, 1 June, p. 962).

Rifkin had filed suit against NIH, claiming that it had broken the law by failing to conduct a proper analysis of the environmental impact of deliberate release experiments. Sirica's ruling stopped NIH from approving any more of these experiments until Rifkin's suit is disposed of. It also halted a University of California field test of bacteria modified to prevent frost formation on potato plants, a decision that the university appealed at the same hearing.

At issue is whether NIH should have analyzed in depth a change in its policy in 1978 that allowed deliberate release experiments on a case-by-case basis. Specifically, did the policy revision oblige NIH to conduct an environmental impact statement as defined under the National Environmental Policy Act?

The Justice Department, which is representing NIH, argues that a single impact statement, which is a comprehensive analysis, of deliberate release experiments is not possible because individual experiments vary too widely to be considered generically. NIH argues that the proper analysis must take place on a case-by-case basis.

At the appeals hearing, the three-judge panel asked Justice Department lawyer J. Carol Williams by what criteria NIH evaluates the environmental impact of the field tests. "There is no particular checklist that RAC uses," said Williams. (RAC stands for the recombinant DNA advisory committee, which reviews researchers' proposals submitted to NIH regarding gene-splicing experiments.) Williams reiterated that NIH review procedures are the equivalent of an environmental evaluation. When pressed again to describe specific standards, Williams said, "There are not definitive standards. These kinds of experiments don't permit standards." Responded one judge, "There doesn't seem to be an effort [by NIH] to develop meaningful measuring sticks [to assess the environmental risk]."

Just before the December appeals hearing, NIH conceded that it would write a simpler kind of environmental impact report of the three approved