National Institutes of Health (NIH)
Gene Therapy Policy Conference:
Prenatal Gene Transfer: Scientific, Medical, and Ethical Issues
January 7-8, 1999

Conclusions

This conference should not be considered as an endorsement by the NIH of prenatal gene transfer research. Rather, this conference was an initial step in an ongoing process of active public deliberation among scientists, clinicians, families, policy makers, individuals, and groups of concerned citizens to gather expert views and solicit public opinion regarding the substantive public policy issues raised by prenatal gene transfer research. It is anticipated that these deliberations will ultimately lead to the development of Federal policy in this arena. In doing so, the NIH and the Recombinant DNA Advisory Committee (RAC) continue to serve as a unique public forum for the discussion of science, safety, and ethics of recombinant DNA research.

At present, there is insufficient preclinical data to support the initiation of clinical trials involving prenatal gene transfer. A substantial number of critical scientific, safety, ethical, legal, and social issues must be addressed before clinical trials proceed in this arena. These issue include (but are not limited to):

1. Efficiency of gene transfer to target cells;

2. Specificity of delivery to target cells;

3. Level, duration, and regulation of gene expression;

4. Appropriate disease candidates;

5. Fetal immune response to transgene products and/or vectors;

6. Emergence of fetal immune tolerance;

7. Effects of gene transfer on pre- and post-natal development;

8. Possibility of generation and activation of transmissible vector or virus;

9. Possibility of initiating oncogenic or degenerative processes;

10. Limitations related to the accuracy of disease diagnosis;

11. Implications of diagnostic limitations on the design and conduct of clinical trials;

12. Elements of optimal clinical trial design and analysis;

13. Definition of clinical endpoints for the analysis of clinical outcomes;

14. Potential risk to the fetus and acceptable level of risk to the fetus in human experimentation;

15. Potential risk to the pregnant woman;

16. Inclusion and exclusion criteria for the pregnant woman;

17. Inclusion criteria for the fetus;

18. Pre- and post-pregnancy monitoring of the pregnant woman;

19. Pre-and post-partum monitoring of the fetus/child;

20. Detection and assessment of inadvertent germ-line transmission;

21. Ethical issues specific to the fetus;

22. Ethical issues specific to the pregnant woman;

23. Patient recruitment/enrollment processes;

24. Informed consent issues;

25. Societal issues; and

26. Legal issues

Next Steps

The RAC will continue to deliberate these issues at future meetings and is charged with the responsibility of recommending guidance on this topic in accordance with Section 301of the Public Health Service (PHS) Act, as amended (42 U.S.C. 241).