

RAC STATEMENT

March 11, 1999

The RAC continues to explore the issues raised by the potential of *in utero* gene transfer research. However, at present, the members unanimously agree that it is premature to undertake any human *in utero* gene transfer experiment.

Rationale:

Significant additional preclinical and clinical studies addressing vector transduction efficacy, biodistribution, and toxicity are required before a human *in utero* gene transfer protocol should proceed. In addition, a more thorough understanding of the ontogeny of human organ systems, such as the immune and nervous systems, is needed to better define the potential efficacy and risks of human *in utero* gene transfer.

Prerequisites for considering any specific human *in utero* gene transfer procedure include an understanding of the pathophysiology of the candidate disease and a demonstrable advantage to the *in utero* approach. Once the above criteria are met, the committee would be willing to consider well rationalized *in utero* gene transfer protocols.