

When Parents Request an Untested Experimental Therapy for Their Extremely Ill Child

The Gold Foundation *Ethics for Lunch* Seminar Series: A Difficult Case from the Morgan Stanley Children's Hospital of New York-Presbyterian Ethics Committee

John Lorenz, MD
Chair, Children's Hospital Ethics Committee
Thursday, February 16, 2006
12:00 noon – 1:30 pm
College of Physicians & Surgeons
P & S Amphitheater, First Floor
630 West 168th Street
New York, NY

Dr. John Lorenz, head of the Children's Hospital Ethics Committee, was the February speaker at the Gold Foundation *Ethics for Lunch* series. He presented to the audience, who were acting as Ethics Committee members, a case that illustrated the ethical dilemmas that arise when desperate parents request an as-yet untested experimental therapy for their extremely ill child. The infant was diagnosed with Spinal Muscular Atrophy-Type 1 (SMA), a progressive neurodegenerative disorder with no specific therapy where children become quadriplegic while their mental status remains completely normal. They usually die within the first year of respiratory failure. The parents had approached the doctors requesting consideration of a highly experimental stem cell procedure for their child. The bone marrow transplantation specialists requested an ethics consultation regarding whether it is ethical to offer an untested stem cell transplant to a baby with SMA-type 1. Without the treatment, the child's prognosis was very guarded. The basis for performing the stem cell transplant is purely theoretical, without any *in vitro*, human, or animal data. The audience and Dr. Lorenz engaged in a lively discussion about the relevant issues, and determined that the basis of the decision should first and foremost be the child's best interest.

The Hospital Ethics Committee, after much debate, recommended that with fully informed parental consent, stem cell transplantation was an ethically acceptable option in this case. Given the highly experimental nature of the procedure, care should be taken to learn as much as possible from this experience if it is undertaken.

After an hour of audience question and discussion, Dr. Lorenz revealed the actual outcome. The Director of the Bone Marrow Transplant Team related the issues and concerns that had been raised by the Ethics Committee to the parents. At that time, the parents decided against the stem cell transplantation. The child died several months later.

The Arnold P. Gold Foundation provided lunch for this event, which was attended by 102 people.