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CLINICAL GENE TRANSFER: EDUCATION IS THE KEY

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Profound damage to public confidence in the discipline of gene therapy has been done in the past two years. The gene transfer incident at the University of Pennsylvania in 1999 and alleged financial conflicts associated with that trial, as well as subsequent revelations of massive underreporting of patient adverse events to the Recombinant DNA Advisory Committee (RAC) as required by the *NIH Guidelines on Recombinant DNA Research*, combined to cause much of this damage. In response to public concerns and congressional inquiries, relevant federal agencies, including the Food and Drug Administration (FDA), the Office of Biotechnology Activities (OBA) at the NIH, and the Department of Human and Health Services (DHHS), have tightened existing regulations and issued new rules and guidelines governing clinical gene transfer studies. One common feature among all of these federal activities is a call for education of investigators and team members to make them thoroughly familiar with all aspects of clinical gene transfer studies and to better protect patient safety.

In response to this challenge, the ASGT sponsored a comprehensive Clinical Gene Transfer Training Course over a two-day period immediately preceding the Society's 4th Annual Meeting in Seattle this past June. The training course was co-organized and cosponsored by the FDA, OBA, OHRP, and the Mount Sinai School of Medicine, and supported financially by only the OBA and the ASGT. Topics encompassed the following areas: 1. Planning for a Clinical Trial; 2. Preclinical Development of Gene Transfer Vectors; 3. Clinical Trial Design, Approval Process, and Trial Conduct; 4. Clinical Trial Compliance, Monitoring, and Oversight; and 5. Bioethics, Research Integrity, and Conflicts of Interest.

Training course objectives were to provide education to basic scientists and trainees on the principles of Good Laboratory Practice (GLP) in generating and analyzing preclinical efficacy and pharmacology/toxicity data to support clinical translational research; production facility directors and staff members on the principles of Good Manufacturing Practice (GMP) in manufacturing and testing of gene transfer products for human applications; and clinical investigators and team members on the principles of Good Clinical Practices (GCP) in accordance with the International Conference on Harmonization (ICH) in conducting clinical trials. In addition, all registrants were educated on the relevant federal regulations and guidelines governing clinical gene transfer studies. The training course was videotaped in its entirety, and complimentary copies will be sent to the deans at all medical schools in the United States for them to share with members of their Institutional Review Board (IRB), Institutional Biosafety Committee (IBC), and faculty and staff who are presently conducting or planning to conduct clinical gene transfer studies.

Although it was originally anticipated that the training course might attract up to 150 participants, 324 registered! Among the registrants were 20 faculty, including leaders from the ASGT, federal officials from the FDA, OBA, ORI, and OHRP, experienced investigators, monitors, and auditors from academia and industry, as well as officials from the relevant federal agencies. The course seemed to have wide appeal, as 33% and 22% of the registrants were non-ASGT members and from foreign countries, respectively. The composition of postgraduate degrees listed by the registrants was 47% Ph.D., 28% M.D., 20% M.D./Ph.D., and 5% others, suggesting that the training course attracted a good mix of basic and clinical scientists. The fact that 20% of the registrants were trainees also indicates that the field of clinical gene transfer is still attractive to young scientists.

Even more impressive than the number of registrants was the intensity they displayed throughout the training course. The lecture hall was jam-packed at all times and there were more questions from the audience than could be adequately handled by the faculty at the end of each session. In addition to receiving appropriate CME credits from the Mount Sinai School of Medicine, the qualified registrants also received a Certificate of Completion from the ASGT.

Such an overwhelming response to the training course from the scientific and clinical community reflects the fact that most practitioners of clinical gene transfer studies wish to be better informed in designing and conducting these studies. It also underlines their desire to be in full compliance with all existing and new federal regulations and guidelines while achieving maximal protection of patient safety at the same time. With this kind of community spirit and efforts, it can be expected that there will be visibly significant improvements in the scientific and ethical conducts of clinical gene transfer studies in the future. Actions always speak louder than words, and it is hoped that continuation of these types of activities will restore public confidence in our discipline.