RAC to Review a Human Gene Transfer Study Involving Patients with Mucopolysaccharidosis (MPS) – At its March 12 meeting, the NIH Recombinant DNA Advisory Committee (RAC) will review a Phase I/II trial involving the intravenous administration of an AAV9 vector to a pediatric population with MPS IIIB, also known as Sanfilippo syndrome. The disease causes neurological and other symptoms within the first years of life, but unlike some other lysosomal diseases, survival may extend into young adulthood. This is the first trial to use systemic intravenous administration of AAV9; previous protocols proposed isolated limb infusion or intrathecal delivery.

The RAC will also review a Phase I study aimed at determining the effects of intraperitoneal delivery of allogeneic mesenchymal stem cells transduced with a plasmid expressing interferon β in research participants with advanced refractory epithelial ovarian cancer.

The full agenda is now available on the OBA Web site. The meeting will be held at 6701 Rockledge Drive, Room 9100, Bethesda, MD 20852. All portions of the meeting are open to the public and will be webcast. Please visit the meetings page of the OBA Web site to access the link to the webcast.

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