NIH RAC to Discuss Proposed Human Gene Transfer Approaches to Treating Spinal Muscular Atrophy, Lymphedema, and Neuroblastoma – At its December 4-5 meeting, the NIH Recombinant DNA Advisory Committee (RAC) will review a protocol submitted by an investigator at the Research Institute at Nationwide Children’s Hospital that proposes to test a novel gene therapy approach for pediatric patients with the most severe form of spinal muscular atrophy, a leading genetic cause of infant death. The protocol proposes delivering intravenously a self-complementary adeno-associated serotype 9 vector carrying a survival motor neuron gene.

The RAC also will discuss the safety, tolerability, and preliminary efficacy of LX-1101, an adenoviral vector containing the transgene for vascular endothelial growth factor C (VEGF-C), in patients with secondary lymphedema associated with the treatment of breast cancer. VEGF-C has been shown in preclinical models to promote the growth of lymphatic vessels. The investigator proposes using LX-1101 to transduce an autologous flap of tissue that contains lymph nodes and then implant that tissue into the axilla of breast cancer patients with lymphedema. The goal is to induce the growth of lymphatic vessels to relieve the lymphedema and reduce complications associated with this condition.

Also on the RAC’s agenda is a study involving autologous activated T-cells transduced with a GD-2 chimeric antigen receptor and an iCaspase 9 safety switch, which investigators propose to administer to pediatric patients with relapsed or refractory neuroblastoma.

Additional protocols of interest to the scientific community will also be discussed. To learn more, consult the agenda, which is available on the OBA Web site. The meeting will be held at the NIH Campus, Building 31, 6th Floor, Conference Room 6, 9000 Rockville Pike, Rockville, 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.

OBA will be offering to those viewing the webcast the opportunity to submit comments via email, which will be read during the scheduled public comment periods. On the day of the meeting, please visit meetings page on the OBA Web site for information on how to provide comments.

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