



NATIONAL HEMOPHILIA FOUNDATION

for all bleeding disorders

MASAC Document #160

MASAC RECOMMENDATION ON THE NEED FOR LONG-TERM FOLLOW-UP OF HEMOPHILIC DOGS IN GENE TRANSFER STUDIES

The following recommendations were approved by the Medical and Scientific Advisory Council (MASAC) of the National Hemophilia Foundation on February 12, 2005, and adopted by the NHF Board of Directors on March 12, 2005.

A major issue in safety of gene transfer has been assessment of long-term risks, including risks related to insertional mutagenesis and/or to unanticipated consequences, of long-term expression of the donated gene. FDA is currently recommending follow-up of at least 15 years for human subjects enrolled in gene transfer studies, and all gene transfer consent forms state that an autopsy will be requested at the time of the subject's death.

Hemophilic dogs have proven an excellent model of the human disease, and they have been used extensively to assess safety and efficacy of virtually all currently used products for treatment of hemophilia. More recently, hemophilic dogs have been used successfully to assess safety and efficacy of a variety of gene transfer methods for hemophilia. Because hemophilic dogs have accurately modeled many aspects of gene transfer in human subjects, and in some cases have been cured, the dogs constitute an important source of long-term safety data that can be used to address issues of safety in humans.

MASAC urges that funds be made available for long-term (lifelong) follow-up of gene therapy-treated hemophilic dogs or other large animal models and for comprehensive necropsy studies at the time of death.

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