

Mr. Spencer O. Perlman
Legislative Affairs Coordinator
Families of Spinal Muscular Atrophy
1025 Connecticut Ave., NW, Suite #216
Washington, DC 20036

Dear Mr. Perlman:

The Advisory Committee on Heritable Disorders in Newborns and Children (Committee)'s Internal Nomination and Prioritization Workgroup (Workgroup) conducted the review of Spinal Muscle Atrophy (SMA) on October 20, 2008 and presented the report to the Committee on November 24, 2008. The report was based on the nomination package you submitted (your nomination form, submitted references) and other publicly available materials. The consensus of the Workgroup was that at this time, the addition of SMA to the uniform screening panel is premature for evaluation by the Committee's evidence review workgroup based on the submitted evidence. The Workgroup recommends: 1) no evidence review at this time; and 2) the implementation of prospective pilot studies of the screening method by one or more traditional public health laboratories to test the reproducibility of the preliminary findings by Dr. Prior's laboratory. This time frame also could allow for an assessment of potential therapies of drugs and other treatment benefits rather than just relying on the nutritional support and respiratory care options at this time.

The Committee unanimously concurred with the Workgroup that the evidence review would not be appropriate this time. The Committee encourages the nominators to proceed with further recommendations of the Workgroup, and noted that SMA would be a very important addition to the newborn screening panel. The Committee will reconsider the nomination after new evidence is made available for evaluation.

Sincerely yours,

/s/

R. Rodney Howell, M.D.
Chairperson

Enclosures