

December 21, 2009

Dear Dr. Lloyd-Puryear,

We have completed the final revision of the evidentiary review of Krabbe disease. The revised final report includes several changes resulting from the discussion at the September 24-25<sup>th</sup>, 2009, ACHDNC meeting. We sought evidence regarding the following inquiries:

- Patients' pretransplant GALC levels and genotypes for those transplanted presymptomatically and postsymptomatically.
- Additional information on the follow-up of high-, moderate-and low-risk infants identified by New York State Krabbe disease pilot screening program.
- More information on quality of life and parents' experiences, especially in the case of the screen positive infants who have not shown any symptoms.
- The number of laboratories in the United States that offer GALC enzyme testing.

We were not able to obtain information regarding all of these items, as noted in the report. In addition, the report has been revised to improve readability.

Sincerely,

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