





MGH Center for Child and Adolescent Health Policy

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Maternal and Child Health Bureau 5600 Fishers Lane. Rm 18-A-19 Rockville, MD 20857

Dear Dr. Lloyd-Puryear,

Following the February 26-27, 2009, presentation of the evidence review report for Severe Combined Immunodeficiency (SCID) and the Committee discussion, we made a few minor changes to the written report to address concerns raised at the meeting.

As you are aware, there was discussion regarding the degree of evidence supporting the benefits of early transplant for SCID. In order to ensure that the level of evidence is not mis-represented, where early treatment is discussed, we have removed adjectives that explicitly denoted the evidence as limited. Additionally, we have added some information from the European experience which corroborates the benefit of early treatment described in other studies.

The committee and meeting attendees also discussed the risks associated with the use of live vaccines in a population which has not been screened for SCID. We searched the literature for studies investigating this concern. We found no systematic studies of risks of vaccine use in undiagnosed children with SCID, although there are multiple individual case reports including:

- 1. Several cases of disseminated BCG
- 2. One case of polio infection from the strain associated with the oral vaccine
- 3. One report of varicella hepatitis following vaccination
- 4. One case of disseminated measles.

Because these were all single case reports and therefore did not meet the criteria for inclusion in the evidence review, these data were not added to the report.

We thank the committee for the opportunity to submit this slightly-revised final report.

Sincerely,

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Director, Division of General Pediatrics
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Cc: Evidence Review Workgroup Members