

Secretary's Advisory Committee on Heritable
Disorders in Newborns and Children
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February 21, 2012

Lois Johnson, M.D., F.A.A.P.
Pennsylvania Center for Kernicterus
4411 Osage Avenue
Philadelphia, PA 19104

Dear Dr. Johnson:

The Secretary of Health and Human Services' Advisory Committee on Heritable Disorders in Newborns and Children (Committee) appreciates your nomination of Universal Pre-Discharge Bilirubin Screening Assay (Hyperbilirubinemia/Kernicterus) for inclusion in the Committee's recommended uniform newborn screening panel for state newborn screening programs. As part of the review process, the Committee requested a formal review of the scientific evidence by the External Evidence Review Workgroup regarding screening, diagnosis, and follow-up care for Hyperbilirubinemia/Kernicterus, using both published and unpublished data. As you know, the decision to go forward with the review reflects positively on the importance of the condition and on the status of test development and treatment evaluation.

The findings from the comprehensive evidence review was presented and discussed by the Committee at the January, 2012 meeting. The Committee unanimously voted to recommend not adding Hyperbilirubinemia/Kernicterus to the Committee's recommended uniform newborn screening panel at this time. Specifically, the Committee identified the following issues:

1. **Screening clinical validity should be improved.**

There is insufficient evidence to support the link between newborns with increased transcutaneous bilirubin screening levels and those who experience an increase in acute clinical manifestations.

2. **There are significant gaps in the evidence.**

- No clear connection between specific bilirubin levels and chronic bilirubin encephalopathy (kernicterus).
- No clear evidence that treating clinically significant neonatal hyperbilirubinemia prevents chronic bilirubin encephalopathy.
 - a. The number of chronic bilirubin encephalopathy cases that could be prevented by universal screening is unknown.
 - b. No evidence regarding universal pre-discharge bilirubin newborn screening logistics and large-scale screening impact.

3. **More data are required about the cost effectiveness for the Recommended Uniform Newborn Screening Panel (RUSP) addition, as opposed to current practices and standards of care.**

There is lack of data to support the addition to the RUSP in terms of cost effectiveness or public health impact.

The Committee recommended that additional studies, including a population-based pilot study to address many of the knowledge gaps identified by the Evidence Review Workgroup, be conducted before the condition can be re-nominated for consideration.

After new evidence addressing the above issues is made available for evaluation, the Committee is willing to reconsider whether the new data provides sufficient support to recommend adding Hyperbilirubinemia/Kernicterus to the Committee's recommended uniform newborn screening panel.

Sincerely yours,

/s/

Joseph A. Bocchini Jr., M.D.
Chairperson