



THE SECRETARY OF HEALTH AND HUMAN SERVICES  
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Committee Chairperson  
Discretionary Advisory Committee on Heritable Disorders  
in Newborns and Children  
Professor and Chairperson  
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Dear Dr. Bocchini:

As indicated in the January 27, 2014 letter from Secretary Sebelius, the Secretary's Discretionary Advisory Committee on Heritable Disorders in Newborns and Children (DACHDNC) recommendations regarding the addition of Pompe disease to the HHS Recommended Uniform Screening Panel (RUSP) were forwarded to the Interagency Coordinating Committee on Screening in Newborns and Children (ICC) for additional input regarding implementation.

The ICC reviewed the DACHDNC's recommendations as well as evidence from method evaluation studies, information on test quality, national guidance documents, and current state screening activities. In its report to me, the ICC noted challenges associated with the implementation of state newborn screening for Pompe disease including resource limitations for laboratory testing, management of late-onset cases, and increased burden on treatment and follow-up systems. However, the ICC emphasized that over time, adoption of this recommendation will help increase the number of newborns screened and decrease the morbidity and mortality of babies born with this disease.

I would like to commend the DACHDNC on their review and analysis of benefits and harms of newborn screening for Pompe disease and the ICC report that described the capability of state newborn screening programs to offer comprehensive testing and follow-up for the condition. The information from the objective evidence report, *Newborn Screening for Pompe Disease*, was taken into account as I reviewed the ICC's report.

Taking into consideration the information presented in these reports, I accept the DACHDNC recommendation to add Pompe disease to the RUSP. The Affordable Care Act requires that most health plans cover the evidence-informed preventive care and screenings provided for in the comprehensive guidelines supported by Health Resources and Service Administration (HRSA). Because the RUSP is a component of these guidelines, a condition added to the RUSP must be covered. It should be understood that addition of Pompe disease to the RUSP does not constitute a requirement for states to implement screening, only a recommendation. I recognize

the complex issues surrounding newborn screening for Pompe disease and encourage Federal agencies to support states as they build capacity and implement state-wide screening.

I appreciate the DACHDNC's dedication and continued hard work to improve the health of our nation's infants and children.

Sincerely,



Sylvia M. Burwell