Memo

Date: December 6, 2017

To: Commissioner Ed Ehlinger

Through: Paul Allwood, Assistant Commissioner, Health Protection Bureau
Joanne Bartkus, Public Health Laboratory Division, Director

From: Jan Larson, Advisory Committee for Heritable and Congenital Disorders Chairman
Mark McCann, Newborn Screening Section Manager, Public Health Laboratory Division,
Maggie Dreon, Newborn Screening Genetic Counselor, Advisory Committee Coordinator
Sondra Rosendahl, Newborn Screening Genetic Counselor, Advisory Committee Coordinator

Subject: Request for Response to the Recommendations made by the Advisory Committee on Heritable and Congenital Disorders

The Minnesota Advisory Committee on Heritable and Congenital Disorders would like you to consider the expansion of the Minnesota newborn screening panel to include spinal muscular atrophy (SMA). The Minnesota Department of Health Newborn Screening Program supports this recommendation. The mechanism for revising the list is described in Minnesota Statute, 144.125 "Tests of infants for heritable and congenital disorders," as follows:

"The list of tests to be performed may be revised if the changes are recommended by the advisory committee established under section 144.1255, if approved by the commissioner, and published in the State Register. The revision is exempt from the rulemaking requirements in chapter 14, and sections 14.385 and 14.386 do not apply."

Expansion is being sought because the Committee believes that Minnesota is capable of initiating screening, the treatment benefit is obvious, early detection is critical, and that the information made available supports the addition of SMA to the Minnesota panel at this time.

> Be it resolved that the Minnesota Department of Health's Advisory Committee on Heritable and Congenital Disorders recommends to the Commissioner of Health that testing for Spinal Muscular Atrophy be added to the Minnesota newborn screening panel without waiting for the Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC) to recommend it for the recommended uniform screening panel (RUSP).
> **Motion carries – 14 ayes, 1 nay**

We hope you find this package helpful as you thoughtfully consider this recommendation.

Request for Response
We ask you to review this package and the Committee's recommendation and provide us with a response at your earliest convenience. For your awareness, the next Committee meeting will be held on April 17th, 2018.

November 1, 2017

Dear Commissioner Ehlinger,

Over the course of the past year, the Committee on Heritable and Congenital Disorders has been reviewing spinal muscular atrophy (SMA) as a candidate condition for addition to the Minnesota newborn screening panel. We have had three committee meetings where we listened to presentations from the newborn screening lab, local specialists, and advocates. During the Committee's October 10th, 2017 meeting, we discussed SMA readiness in Minnesota utilizing the 'Guidelines for Determining Appropriate Target Conditions for Newborn Screening' tool, previously developed and adopted by this committee to establish a mechanism for the evaluation of disorders being considered for addition to the Minnesota panel.

Based on these deliberations, multiple presentations from experts, and public comment, the Committee voted on the following:

> Be it resolved that the Minnesota Department of Health's Advisory Committee on Heritable and Congenital Disorders recommends to the Commissioner of Health that testing for Spinal Muscular Atrophy be added to the Minnesota newborn screening panel without waiting for the Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC) to recommend it for the recommended uniform screening panel (RUSP).
> **Motion carries – 14 ayes, 1 nay**

Spinal muscular atrophy is a neuromuscular disease caused by a deficiency of a motor neuron protein called SMN from mutations in the *SMN1* gene. SMA affects the part of the nervous system that controls voluntary muscle movement. There are four types of SMA and the degree of severity correlates with the age of onset. When detection is obtained prior to onset, long term survival and treatment options increase. Without newborn screening, most children with SMA are identified after significant morbidity due to the loss of lower motor neurons; neurons which cannot be replaced or redeveloped after they are lost. The evidence is clear that the earlier the treatment is initiated, the better the outcomes for these children. Children with SMA type 1 who received treatment while asymptomatic, who typically never sit unassisted or learn to walk, crawl, or stand in the absence of treatment, are reaching all their developmental milestones by age 1 when treatment is initiated before symptoms become obvious.

We, as a committee, had a robust discussion on the merits of SMA in the context of being added to Minnesota's newborn screening panel. A full summary of the discussion is included in this package for your review, however, a few key elements of the discussion bear acknowledgment. The obvious and stark benefits offered by the treatment made a compelling case, specifically to those children who are able to initiate treatment before the damage and loss of milestones occurs. By screening newborns for SMA, we will be able to protect Minnesota's youngest citizens from irreparable damage. We also debated the potential 5% false negative rate for SMA screening based on the currently chosen method. Some advisors voiced concern, while other advisors articulated that current disorders on the Minnesota panel had similar or worse 'miss rates' and that we should not seek perfection when we can do a lot of good for the 95% of children who would be detected. Additionally, we were reassured that the decisions made today would not impede MDH's ability to adopt a new testing method, should one ever become available in the future. Thirdly, we discussed our adherence or independence from the ACHDNC. One advisor articulated an opinion that States should not act independent of the federal advisory group and their activities, which resulted in a nay vote. While other advisors acknowledged the great work that the ACHDNC does in reviewing conditions, it was not clear what additional information would be forthcoming from the ACHDNC that would merit delaying a vote. Additionally, many advisors felt it was the role and responsibility of this committee to act in the interests of Minnesota and

articulated that no guiding documents, rules, or statutes exist acceding the authority of this committee to the federal committee. We ultimately felt that we should not limit this committee's ability to help Minnesotans, as that is our charge.

Following these deliberations, we elected to proceed with a vote and subsequent recommendation. Although SMA is in evidence review with the ACHDNC currently, it is a lengthy process (a 9-month process followed by an additional 120 days should they recommend it to the Secretary of DHHS). Absent the likelihood of any compelling information forthcoming from the ACHDNC, our decision to not wait for the ACHDNC was based upon our assessment of the unique readiness of Minnesota's Newborn Screening Program, the readiness of Minnesota's treating clinicians, and the importance of timeliness for Minnesota families and newborns.

Thank you for your consideration.

/s/

Sincerely,
Jan Larson, J.D.
Chairperson

Cc: Joanne Bartkus - Public Health Laboratory Division Director
Myra Kunas - Public Health Laboratory Assistant Division Director
Mark McCann - Newborn Screening Program Manager
Janet Olstad - Community and Family Health Interim Division Director, Assistant Division Director
Barb Dalbec - Children and Youth with Special Health Needs Manager