

Senator Monnes Anderson,

You asked me about SB 284 the lysosomal storage disorders.

I have seen this particular disease be raised by advocate families around the country for several years. It is a heart breaking story for this family (I saw their blog) and all who are touched by a congenital condition and all who love children. I am attaching the link to the HHS decision not to include this condition in the recommended uniform panel of newborn screens. It does not mean it will never be included but as of yet the experts report that there is no good screening method for the condition, no FDA approved lab testing for this at this time. Each state would have to develop their own way to validate a test and no good treatment for the condition as yet. Mike Skeels, Ph.D. is the Director of the Oregon Public Health Laboratory and newborn screening is done through his department. He is also a member of the advisory panel to the Secretary of HHS on Heritable Disorders in Newborns and Children. He would certainly know a lot more about the disease condition and the recommendation to not include these disorders to the recommended screening panel than I. Also, in Oregon Bob, Steiner, MD at OHSU would be a good source of information on the condition. I am attaching a commentary he wrote.

Of the specific LSD mentioned in SB 284 three have been reviewed and rejected by the Secretary's Advisory Committee on Heritable Disorders in Newborns and Children: Krabbe, Neimann-Pick and Pompe's disease. They did not meet the criteria for the recommended uniform panel of screens. Again, this is not to say they won't when more research is done, better tests and treatments are discovered. I know states are asked to screen for quite a number of conditions. The Secretary's Advisory committee was established to keep the integrity of recommending new screens when there is sufficient evidence to do so.

<http://www.hrsa.gov/advisorycommittees/mchbadvisory/heritabledisorders/nominatecondition/reviews/krabbedecision.pdf>

New screens are added to this recommended uniform panel as evidenced by CCHD being added. There is another condition that has been recently added-- SCID. With SCID there is now not only a treatment but a cure.

Not sure if this helps or not?

Best regards,
Joanne