DREXEL UNIVERSITY
School of
Public Health

Department of Community Health and Prevention

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Chair
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Dear Representative Cruz and Committee Members:

It is a great honor and pleasure for me to be here today as the Vice-Chair of the Pennsylvania Newborn Screening Technical Advisory Board to discuss newborn screening, a critical public health program that saves and improves the lives of Pennsylvania babies and their families each year. Professionally, I am also Chair and Associate Professor of the Department of Community Health and Prevention at the Dornsife School of Public Health at Drexel University in Philadelphia. I have been on the faculty at Drexel and a proud resident of the City of Philadelphia since 2004. Before that I received my PhD and MPH from Columbia University in New York City. My training and expertise are in the history and ethics of public health, and I conduct research currently on the ethics of autism research, the history of autism, the challenges of studying human populations by race, and the history of scientific racism and genetics. I am also the author and editor of three books, including two books on the human genome project, and research articles on public health and ethics. My work is situated at the crossroads of public health, ethics, and history, and, as such, I am dedicated to the just and ethical treatment of babies and families who are screened at birth in the Pennsylvania Newborn Screening Program.

As my colleague Dr. Jerry Vockley, the Chair of the Newborn Screening Technical Advisory Board (TAB) has stated in his submitted written testimony, the TAB was constituted by law in 1980 to provide advice regarding the Newborn Screening Program to the Secretary of Health. We do not make or enforce policy. Instead, the committee's collective expertise, drawn from a distinguished group of clinicians, scientists, and service providers, makes recommendations to the Secretary based on our understanding and analysis of the latest science. We make decisions based on specific criteria that include whether testing has a significant impact on babies, whether a sensitive and specific screening test is available, whether early treatment of the disease affects its outcome, and whether the resources exist to pay for screening, following up, and support of infants who test positive for a particular disease.

As an ethicist, my role on the committee is to help the group consider the ethical dimensions of new tests recommended for mandated testing, whether and how we are adequately educating the public about the Program, and the ethics of any potential expansion to the Program.

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For example, at last week's quarterly TAB meeting, we examined the testing outcomes for Pompe Disease, an autosomal recessive lysosomal storage disorder, over the period from February 2016 to February 2017. Of the 9 babies with Pompe identified by the Program during this time, six of the babies will have late onset disease, which can appear from childhood through adulthood. While there is no reason to change the current testing recommendation for Pompe, the fact that many of the babies tested will have late onset disease does force us to consider the potential impact of sharing this type of information with parents and its impact on the children tested. The nature of the Program's opt-out consent is based on identifying diseases that require treatment at or shortly after birth. Should that calculus change if we are telling parents that their child's condition might not appear until adolescence or later? Are we interfering with a family's autonomy by conducting such tests? How might the child's identity be altered under such circumstances? Might that child and their family face stigma or discrimination based on a diagnosis that could be far in the future? And might a family seek to take an action contrary to the child's interest because of a late onset diagnosis?

The Newborn Screening Program does not bare the responsibility of addressing all of these questions and concerns. But as testing technology advances in the future, and it surely will, Newborn Screening Programs around the country, including ours, will certainly have to evolve and adapt to be able to provide resources parents will need to answer such questions. To do this, it is essential that the Program a) have adequate resources to be able to make recommendations to add and remove tests from the screening panel to keep up with the latest science, and b) ensures that parents are educated on the nature of the Program and its potential impact on their babies.

The TAB has also recommended expanding the length of time blood spots are stored on filter paper by the state. Currently, the storage of newborn's filter paper is limited to one year. The TAB recommended last year to increase storage of filter paper to five years. Increased duration of filter paper storage would a) be used for quality control; b) be used to demonstrate the utility of the blood spots for other potential program efforts; c) could be used to retrospectively test spots when a condition is added to mandated tests; d) could be used as part of research to develop novel screening markers for new tests. The Program is currently unable to do this, however, out of funding concerns.

For the Program to be able to successfully educate the public about newborn screening, for the Program to adapt to a rapidly changing technological terrain and the expansion of testing, and for the Program to have successful quality control and scientific reliability, the

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Program must sit on sound financial footing. This is why, and I speak for both Dr. Vockley and myself, we are excited about House Bill 1081, which seeks to institute a newborn screening fee. Such a fee would go a long way towards providing a stable source of revenue to fund the Program and its projects, particularly as there are increased mandates for screening and the Program is forced to adapt to our rapidly changing technological age.

Finally, we appreciate the language in the Bill deferring to the expertise of the TAB in adding and subtracting diseases. However, our committee is concerned about the addition of specific diseases to the list of mandated screens, an approach which would circumvent the TAB and sets a precedent that could have a significant impact on the Program. This approach, while driven by deep compassion for families, can contradict the best clinical evidence. The lysosomal storage disorder Krabbe Disease is among several new diseases currently listed as mandated tests in House Bill 1081. I would echo Dr. Vockley's statement from his written testimony that there is still insufficient medical evidence to yet change the TABs previous recommendation on this and several other new diseases listed in the Bill. We look forward to the day when successful interventions justify adding these conditions to the mandated tests. Thus, we advocate continuing to follow the recommendations of the RUSP and the best medical evidence as we make our recommendations to the Secretary.

We look forward to continuing to work with you, the Committee, and the Secretary of Health to strengthen this essential public health program that protects the babies born in Pennsylvania.

Should you have any questions, please contact me at 267.359.6084 or at myudell@drexel.edu.

Sincerely,

Michael Yudell, PhD, MPH
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