HB 111

Department of Legislative Services

Maryland General Assembly
2014 Session

FISCAL AND POLICY NOTE

House Bill 111 (Delegate Hubbard)
Health and Government Operations

Public Health - Newborn Screening Program - Lysosomal Storage Disorders

This bill requires the Department of Health and Mental Hygiene's (DHMH) Newborn Screening Program to include screening for the following lysosomal storage disorders: Krabbe leukodystrophy; Pompe disease; Gaucher disease; Neimann-Pick disease; Fabry disease; and Hurler syndrome.

Fiscal Summary

State Effect: General fund expenditures for the Laboratories Administration increase by $1.8 million in FY 2015 for additional staff and necessary equipment. Future years reflect elimination of one-time equipment costs, annualization of ongoing costs, and inflation. General fund revenues are not affected.

(in dollars)	FY 2015	FY 2016	FY 2017	FY 2018	FY 2019
Revenues	$0	$0	$0	$0	$0
GF Expenditure	1,803,400	452,200	464,500	477,200	490,400
Net Effect	($1,803,400)	($452,200)	($464,500)	($477,200)	($490,400)

Note:() = decrease; GF = general funds; FF = federal funds; SF = special funds; - = indeterminate effect

Local Effect: None.

Small Business Effect: None.

Analysis

Current Law: DHMH's Newborn Screening Program is a statewide system for screening all newborn infants in the State for certain hereditary and congenital disorders associated with severe problems of health or development (except when the parent or guardian of the newborn objects).

The DHMH Public Health Laboratory is the only laboratory in Maryland authorized to perform the screening tests. The system for newborn screening includes laboratory testing and reporting of test results and follow-up activities to facilitate the rapid identification and treatment of an affected child. DHMH establishes fees for newborn screening and follow-up costs; the current fee is $100 per newborn and an average of 75,000 newborns are screened annually. The laboratory is required to screen for 53 first-tier metabolic hereditary disorders on all screening specimens collected from a newborn infant. These disorders are all listed in the Code of Maryland Regulations 10.10.13.12. Second-tier tests can only be performed when requested by an individual authorized to request a medical laboratory test.

The State Advisory Council on Hereditary and Congenital Disorders gathers and disseminates information on the treatment of hereditary and congenital disorders in Maryland. The advisory council (1) continually evaluates the need for, and efficiency of, relevant State programs and (2) institutes and supervises education programs and counseling on heredity and congenital disorders.

Background: Lysosomal storage disorders are rare genetic diseases that generally affect newborns and very young children. As infants grow, symptoms develop and continue to progress until significant morbidity or mortality occurs. These disorders are not curable, but treatment can be provided to alleviate symptoms if the disorder can be clearly identified and a determination can be made that treatment is beneficial. In particular, the National Institutes of Health advises that effective enzyme replacement therapy is available for patients with type 1 Gaucher disease and some with type 3 Gaucher disease. Other treatments to alleviate symptoms include blood transfusions, spleen removal, and the use of prescription drugs like phenytoin and carbamazepine to treat bone pain for patients with Fabry disease.

The U.S. Department of Health and Human Services, Secretary of Health and Human Services' Discretionary Advisory Committee on Heritable Disorders in Newborns and Children (committee), was established by the federal Public Health Service Act, 42 U.S.C. 217a. The committee recommends that every state newborn screening program include a uniform screening panel that the committee establishes.

In 2008, the committee declined to nominate Niemann-Pick disease for several reasons including that (1) there is no population-based data available, which makes it difficult to discern between different types of the disease; (2) there are no published studies available to show the efficacy of treatment in humans for those most likely to benefit early in life, and there is no FDA approved treatment; and (3) there is a need for pilot studies of the newborn screening test and treatment protocols.

In 2008, the committee also declined to nominate Fabry disease even though it is detectable in a screening panel for lysosomal storage disorders. The committee declined for several reasons, including (1) it is unclear if those at highest risk of serious symptoms can be discerned in newborns; (2) there is a lack of published data on preventative treatment in early life; (3) and the need to demonstrate the benefit of newborn screening in terms of whether it is worth the therapeutic intervention.

In 2009, the committee evaluated whether to include Krabbe disease for inclusion on the committee's recommended uniform newborn screening panel for state newborn screening programs. The committee declined to add Krabbe disease because of "insufficient evidence of potential net benefit" to adding the disease. The committee also declined to add Krabbe disease because (1) there is no consensus on the definition of what constitutes Early Infantile Krabbe disease (EIKD); (2) the committee could not definitively recommend a standard method testing for EIKD; and (3) more information is needed regarding the benefits of treating patients with Hematopoietic Stem Cell Transplant.

In May 2013, the committee elected to include Pompe disease, which occurs in approximately 1 in 28,000 births. The committee chose to include Pompe disease because early detection and treatment with enzyme replacement therapy has been shown to modify the course of the disease.

The committee has not published responses to nominations to Gaucher disease or Hurler syndrome on its website.

In 2006, New York State began screening all newborns for Krabbe disease. Both the federal government and other states look to New York to provide a potential model for future legislation and to be a source of research and information on this disease.

The Maryland Advisory Council on Hereditary and Congenital Disorders advises and makes recommendations to the Secretary of Health and Mental Hygiene regarding Maryland's Newborn Screening Program. The council has considered recommendations to add Krabbe disease, along with other lysosomal storage disorders. However, the council declined to do so before further reviewing recommendations from the U.S. Department of Health and Human Services, Maryland State advocacy groups, and community stakeholders. Maryland does not currently screen for Pompe disease though it is included in the U.S. Department of Health and Human Services' Recommended Uniform Screening Panel.

State Revenues: The Laboratories Administration has regulatory authority under § 13-11 of the Health-General Article to establish fees for newborn screening that do not exceed the administrative, laboratory, and follow-up costs associated with newborn screening testing in the State. However, the bill does not require the administration to

increase the fee it charges, and the administration does not indicate that it has current plans to do so through regulations.

State Expenditures: General fund expenditures increase by $1,803,395 in fiscal 2015, which accounts for the bill's October 1, 2014 effective date. This estimate reflects the cost of hiring three full-time public health laboratory scientists to conduct additional testing for lysosomal storage disorders on approximately 75,000 newborns every year (the testing is done twice, with approximately 150,000 specimens tested). Additional equipment and supplies are also needed – specifically a mass spectrometer, integra liquid handling system, evaporator, and multichannel pipette set as well as reagents. The estimate includes salaries, fringe benefits, one-time start-up costs, and ongoing operating expenses.

Positions	3
Salaries and Fringe Benefits	$150,208
One-time Equipment Costs	1,564,372
Reagents and Related Supplies	61,500
Mass Spectrometer Maintenance Contract	20,250
Operating Expenses	7,065
Total FY 2015 State Expenditures	**$1,803,395**

Future years reflect full salaries with annual increases and employee turnover as well as annual increases in ongoing operating expenses.

Additional Information

Prior Introductions: None.

Cross File: SB 433 (Senator Dyson) - Finance.

Information Source(s): Department of Health and Mental Hygiene, National Institutes of Health, U.S. Department of Health and Human Services**,** Department of Legislative Services

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Analysis by: Kathleen P. Kennedy

Direct Inquiries to:
(410) 946-5510
(301) 970-5510