GENERAL ASSEMBLY OF NORTH CAROLINA
SESSION 2017

S **1**

SENATE BILL 190*

Short Title:	The Haley Hayes Newborn Screening Bill.	(Public)
Sponsors:	Senators D. Davis, Barefoot, Krawiec (Primary Sponsors); and Waddell.	
Referred to:	Rules and Operations of the Senate	

March 7, 2017

A BILL TO BE ENTITLED

AN ACT DIRECTING THE COMMISSION FOR PUBLIC HEALTH TO ADOPT RULES TO ADD A SCREENING TEST FOR POMPE DISEASE, MUCOPOLYSACCHARIDOSIS TYPE I (MPS I), AND X-LINKED ADRENOLEUKODYSTROPHY (X-ALD) TO THE NEWBORN SCREENING PROGRAM; INCREASING THE FEE FOR NEWBORN SCREENING TESTS; AND APPROPRIATING FUNDS TO THE DEPARTMENT OF HEALTH AND HUMAN SERVICES, DIVISION OF PUBLIC HEALTH, TO PURCHASE NECESSARY EQUIPMENT AND UPGRADES AT THE STATE LABORATORY OF PUBLIC HEALTH FOR NEWBORN SCREENING AND ALL OTHER LABORATORY OPERATIONS.

Whereas, Pompe disease is a rare, heritable disorder that causes progressive muscle weakness and is one of several lysosomal storage disorders (LSDs) that affect lysosome function in cells; and those diagnosed with infantile onset may die in early childhood, while those with late onset suffer progressive muscle weakness into adulthood as glycogen accumulates in the muscle cells; and

Whereas, without early identification and treatment for Pompe disease, infants may suffer from a buildup of glycogen in the heart muscles, causing muscle damage or early mortality, but in some, early intervention may prevent irreversible muscle damage and available options for treatment, including the use of enzyme replacement therapy, may slow the disease progression; and

Whereas, Mucopolysaccharidosis Type I (MPS I) is an inherited condition known as an autosomal recessive LSD that can affect many parts of the body, and patients with this disorder have difficulty breaking down certain types of complex sugars, which causes harmful substances to build up, become toxic, and damage cells and organs; and

Whereas, patients with the severe and most dominant form of MPS I, known as Hurler syndrome, tend to develop symptoms in the first or second year of life and often die before the age of 10; and

Whereas, X-Linked Adrenoleukodystrophy (X-ALD) is a rare, heritable disorder that causes progressive damage to the kidneys, brain, and spinal cord and is one of a group of genetic disorders called leukodystrophies that primarily affects males and causes the accumulation of high levels of saturated very long chain fatty acids in the kidneys and the deterioration of insulating layers surrounding nerve cells throughout the brain and spinal cord; and

Whereas, symptoms usually emerge between the ages of 2.5 and 10 years and include behavioral changes, poor memory, seizures, poor coordination, difficulty swallowing, impaired hearing and vision, progressive dementia, and death within one to 10 years after symptom onset; and

Whereas, delaying diagnosis and treatment of X-ALD can lead to irreversible muscular and organ damage; and

Whereas, treatments such as the use of adrenal hormones, physical therapy, and special education can lead to longer life and less invasive disease management but must be administered before symptoms occur; and

Whereas, early recognition of Pompe disease, MPS I, and X-ALD through newborn screening is critical to successful management of patients; and

Whereas, 10-year-old Haley Hayes was born prematurely with Pompe disease and received her first treatment at seven months of age; she is wheelchair bound and unable to stand without assistance; but if Haley had received diagnosis at the time of birth, her current mobility would more than likely be greater; and

Whereas, development and implementation of screening tests for iduronate sulfatase, alpha-glucosidase, and C26:0 lysophosphatidylcholine have been accomplished, which led to the unanimous recommendations by the United States Secretary of Health and Human Service's Advisory Committee on Heritable Disorders of Newborns and Children in 2015 to add MPS I and Pompe disease and in 2016 to add X-ALD to the list of conditions routinely screened at birth; and

Whereas, as of February 2017, four states conduct newborn screening for Pompe disease, three states conduct newborn screening for MPS I, and one state conducts newborn screening for X-ALD; and

Whereas, one of the leading centers for treatment of Pompe disease, MPS I, and X-ALD in the United States is located in North Carolina at the Duke University Medical Center (where Haley Hayes receives treatment); and

Whereas, early diagnosis of these conditions through newborn screening and treatment with bone marrow transplant or enzyme replacement therapy or both prevents death or lifelong disability; and

Whereas, for Pompe disease, early diagnosis and treatment results in 100% survival at five years but untreated MPS I or X-ALD leads to permanent lifelong disability or death in childhood; and

Whereas, there are reliable screening tests for these disorders via bloodspots; Now, therefore,

The General Assembly of North Carolina enacts:

SECTION 1. G.S. 130A-125(c) reads as rewritten:

"(c) A fee of ~~forty-four dollars ($44.00)~~ fifty-five dollars ($55.00) applies to a laboratory test performed by the State Laboratory of Public Health pursuant to this section. The fee for a laboratory test is a departmental receipt of the Department and shall be used to offset the cost of the Newborn Screening Program."

SECTION 2. The Commission for Public Health shall amend the rules adopted pursuant to G.S. 130A-125 to implement the Newborn Screening Program established under G.S. 130A-125 to add to the newborn screening panel a screening test for Pompe disease, Mucopolysaccharidosis Type I (MPS I), and X-Linked Adrenoleukodystrophy (X-ALD).

SECTION 3. There is appropriated from the General Fund to the Department of Health and Human Services, Division of Public Health, the sum of two million seven hundred thousand dollars ($2,700,000) for the 2017-2018 fiscal year. These funds shall be used to purchase laboratory instrumentation and upgrades to the existing North Carolina State Laboratory of Public Health (NCSLPH) Laboratory Information System in order to provide the required informatics capabilities for newborn screening and all other laboratory operations at the NCSLPH.

SECTION 4. This act becomes effective July 1, 2017, and Section 1 of this act applies to laboratory tests conducted on and after July 1, 2017.