

**GENERAL ASSEMBLY OF NORTH CAROLINA
SESSION 2017**

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HOUSE BILL 270*

Short Title: The Haley Hayes Newborn Screening Bill. (Public)

Sponsors: Representatives Lambeth, Adcock, Murphy, and White (Primary Sponsors).
For a complete list of sponsors, refer to the North Carolina General Assembly web site.

Referred to: Health, if favorable, Finance, if favorable, Appropriations

March 8, 2017

A BILL TO BE ENTITLED

1
2 AN ACT DIRECTING THE COMMISSION FOR PUBLIC HEALTH TO ADOPT RULES TO
3 ADD A SCREENING TEST FOR POMPE DISEASE, MUCOPOLYSACCHARIDOSIS
4 TYPE I (MPS I), AND X-LINKED ADRENOLEUKODYSTROPHY (X-ALD) TO THE
5 NEWBORN SCREENING PROGRAM; INCREASING THE FEE FOR NEWBORN
6 SCREENING TESTS; AND APPROPRIATING FUNDS TO THE DEPARTMENT OF
7 HEALTH AND HUMAN SERVICES, DIVISION OF PUBLIC HEALTH, TO PURCHASE
8 NECESSARY EQUIPMENT AND UPGRADES AT THE STATE LABORATORY OF
9 PUBLIC HEALTH FOR NEWBORN SCREENING AND ALL OTHER LABORATORY
10 OPERATIONS.

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

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

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

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

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

33 Whereas, symptoms usually emerge between the ages of 2.5 and 10 years and include
34 behavioral changes, poor memory, seizures, poor coordination, difficulty swallowing, impaired



1 hearing and vision, progressive dementia, and death within one to 10 years after symptom onset;
2 and

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

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

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

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

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

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

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

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

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

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

33 The General Assembly of North Carolina enacts:

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

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

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

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

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