

## HOUSE OF REPRESENTATIVES STAFF ANALYSIS

**BILL #:** CS/HB 403 Newborn Adrenoleukodystrophy Screening

**SPONSOR(S):** Health Quality Subcommittee; La Rosa

**TIED BILLS:** **IDEN./SIM. BILLS:**

REFERENCE	ACTION	ANALYST	STAFF DIRECTOR or BUDGET/POLICY CHIEF
1) Health Quality Subcommittee	12 Y, 0 N, As CS	Langston	O'Callaghan
2) Health Care Appropriations Subcommittee			
3) Health & Human Services Committee			

### SUMMARY ANALYSIS

Newborn screening is a preventive public health program that is provided in every state in the United States to identify, diagnose, and manage newborns at risk for selected disorders that, without detection and treatment, can lead to permanent developmental and physical damage or death. The United States Department of Health and Human Services (HHS) Discretionary Advisory Committee on Heritable Disorders in Newborns and Children (DACHDNC) advises HHS on the most appropriate application of universal newborn screening tests, technologies, policies, guidelines and standards. DACHDNC establishes the heritable disorders listed on the federal Recommended Uniform Screening Panel (RUSP).

In Florida, the Department of Health (DOH) is responsible for administering the statewide Newborn Screening Program (NSP), which conducts screenings for 53 disorders. Once a heritable disorder is added to the RUSP, it is reviewed by the DOH Newborn Screening Advisory Council, which recommends to DOH whether the disorder should be added to the NSP panel of disorders to be screened for in Florida.

Adrenoleukodystrophy (ALD) is a genetically determined neurological disorder that affects one in every 17,900 boys worldwide. ALD strips away of the fatty coating that keeps nerve pulses confined and maintains the integrity of nerve signals. This process causes neurological deficits, including visual disturbances, auditory discrimination, impaired coordination, dementia, and seizures. To date, ALD has not been added to the RUSP.

Currently, ALD is not on the NSP's panel of disorders that are required for newborn screening. The bill amends s. 383.14, F.S., to direct DOH to expand statewide newborn screening to include screening for ALD when ALD is added to the RUSP.

The bill will have a significant negative fiscal impact on DOH and AHCA and no fiscal impact on local government.

The bill provides an effective date of July 1, 2015.

# FULL ANALYSIS

## I. SUBSTANTIVE ANALYSIS

### A. EFFECT OF PROPOSED CHANGES:

#### Current Situation

##### Federal Recommendations for Newborn Screening

The United States Department of Health and Human Services (HHS) Discretionary Advisory Committee on Heritable Disorders in Newborns and Children (DACHDNC), under the Public Health Service Act,<sup>1</sup> fulfills the functions previously undertaken by the former Secretary's Advisory Committee on Heritable Disorders and Children (SACHDNC),<sup>2</sup> to reduce morbidity and mortality in newborns and children who have, or are at risk for, heritable disorders.<sup>3</sup> To that end, the DACHDNC advises the Secretary of HHS the most appropriate application of universal newborn and child screening tests and technical information for the development of policies and priorities that will enhance the ability of state and local health agencies to provide for screening, counseling, and health care services for newborns and children having, or at risk for, heritable disorders.<sup>4</sup>

As part of this process, DACHDNC establishes the heritable disorders listed on the federal Recommended Uniform Screening Panel (RUSP). The RUSP currently provides 31 core conditions and 26 secondary conditions.<sup>5</sup>

##### Florida Newborn Screening Program

Section 383.14(5), F.S., establishes the Florida Genetics and Newborn Screening Advisory Council with the purpose to advise the Department of Health (DOH) about which disorders should be screened for under the Newborn Screening Program (NSP) and the procedures for collection and transmission of specimens. Florida's NSP currently screens for all disorders that are included on the RUSP.<sup>6</sup>

The intent of the NSP is to screen all newborns for hearing impairment and to identify, diagnose, and manage newborns at risk for selected disorders that, without detection and treatment, can lead to permanent developmental and physical damage or death.<sup>7</sup> The NSP is a comprehensive system involving coordination among several entities, including the Bureau of Laboratories Newborn Screening Laboratory in Jacksonville, Children's Medical Services (CMS) Newborn Screening Follow-up Program in Tallahassee, and referral centers throughout the state. The NSP screens for 31 core conditions and 22 secondary conditions (a total of 53 conditions); this includes all of the core conditions recommended by the RUSP and 50 of their recommendations overall.<sup>8</sup>

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<sup>1</sup> 42 U.S.C. 217a: Advisory councils or committees (2014).

<sup>2</sup> U.S. Department of Health and Human Services, *Discretionary Advisory Committee on Heritable Disorders in Newborns and Children*, <http://www.hrsa.gov/advisorycommittees/mchbadvisory/heritabledisorders/index.html> (last visited March 13, 2015)

<sup>3</sup> Id.

<sup>4</sup> Secretary of Health and Human Services, *Charter Discretionary Advisory Committee on Heritable Disorders in Newborns and Children*, April 24, 2013, available at

<http://www.hrsa.gov/advisorycommittees/mchbadvisory/heritabledisorders/about/charterdachdnc.pdf>

<sup>5</sup> Discretionary Advisory Committee on Heritable Disorders in Newborns and Children, *Recommended Uniform Screening Panel (as of April 2013)*, available at

<http://www.hrsa.gov/advisorycommittees/mchbadvisory/heritabledisorders/recommendedpanel/uniformscreeningpanel.pdf> (last visited March 18, 2015).

<sup>6</sup> Id.

<sup>7</sup> Florida Department of Health, *2014 Agency Legislative Bill Analysis HB 591*, January 14, 2014 (on file with Health Quality Subcommittee).

<sup>8</sup> Florida Department of Health, *Disorder List*, available at [http://www.floridahealth.gov/programs-and-services/childrens-health/newborn-screening/\\_documents/newborn-screening-disorders.pdf](http://www.floridahealth.gov/programs-and-services/childrens-health/newborn-screening/_documents/newborn-screening-disorders.pdf); this list is also maintained by DOH in Rule Rule 64C-7.002, F.A.C.

In Florida, the specimen card is sent to the DOH Newborn Screening Laboratory (DOH State Laboratory) in Jacksonville for testing. The DOH State Laboratory receives about 250,000 specimens annually from babies born in Florida. The majority of the test results are reported within 24 to 48 hours. The CMS program, within DOH, provides follow-up services for all abnormal screening results.

### Adrenoleukodystrophy (ALD)

Adrenoleukodystrophy (ALD) is a genetically determined neurological disorder that affects 1 in every 17,900 boys worldwide. The presentation of symptoms occurs somewhere between the ages of 4 and 10, and affects the brain with demyelination. Demyelination is the stripping away of the fatty coating that keeps nerve pulses confined and maintains the integrity of nerve signals. This process inhibits the nerves ability to conduct properly, thereby causing neurological deficits, including visual disturbances, auditory discrimination, impaired coordination, dementia, and seizures. Demyelination is an inflammatory response and nerve cells throughout the brain are destroyed.<sup>9</sup>

### Screening for ALD

ALD was first nominated to the SACHDNC in 2012. At that time, the nomination did not progress and SACHDNC did not recommend a full evidence review because sufficient prospective data was not yet available from a large pilot study at the Mayo Biochemical Genetics Laboratory.<sup>10</sup> In September 2013, ALD was again nominated for consideration. Following that nomination, at a January 2014 meeting of the SACHDNC, the SACHDNC recommended a full evidence review of ALD and requested the External Evidence Review Group to present a full report to the DACHDNC. There was a preliminary report presented on February 12, 2015, however a recommendation was not made at that time.<sup>11</sup>

ALD is currently screened for in Connecticut, New Jersey, and New York, and in 2014 legislation was proposed in California and Maryland.<sup>12</sup> The Florida Genetics and Newborn Screening Advisory Council has not yet considered whether ALD should be added to Florida's NSP panel of disorders. If the DACHDNC recommends ALD to be included on the RUSP, their recommendation will be submitted in writing to the HHS Secretary who will have final approval before the condition is added to the RUSP.<sup>13</sup>

### **Effect of Proposed Changes**

The bill amends s. 383.14, F.S., to direct DOH to expand statewide newborn screening to include screening for ALD when ALD is added to the RUSP. This will make ALD only the second disease statutorily required to be screened under the NSP. Typically, DOH has the discretion to determine which disorders must be screened after consultation with the Genetics and Newborn Screening Advisory Council.<sup>14</sup>

The effective date of the bill is July 1, 2015.

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<sup>9</sup> Adrenoleukodystrophy Foundation, *ALD Information*, [http://aldfoundation.org/ald\\_info.html](http://aldfoundation.org/ald_info.html) (last visited March 13, 2015).

<sup>10</sup> U.S. Department of Health and Human Services, *Letter of Secretary's Advisory Committee on Heritable Disorders in Newborns and Children*, October 1, 2012, available at

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

<sup>11</sup> Alex R. Kemper, *Newborn Screening for X-linked Adrenoleukodystrophy (X-ALD): Preliminary Report from the Condition Review Workgroup (CRW)*, February 12, 2015, available at

<http://www.hrsa.gov/advisorycommittees/mchbadvisory/heritabledisorders/meetings/2015/sixth/crupupdatealdkemper2.pdf> (PowerPoint on file with Health Quality Subcommittee)

<sup>12</sup> Alex R. Kemper, *Newborn Screening for X-linked Adrenoleukodystrophy (X-ALD): Preliminary Report from the Condition Review Workgroup (CRW)*, February 12, 2015, available at

<http://www.hrsa.gov/advisorycommittees/mchbadvisory/heritabledisorders/meetings/2015/sixth/crupupdatealdkemper2.pdf> (last visited March 14, 2015).

<sup>13</sup> Florida Department of Health, *2015 Agency Legislative Bill Analysis HB 403*, January 28, 2015 (on file with Health Quality Subcommittee).

<sup>14</sup> See, s. 383.14(2), F.S. (stating that DOH "shall adopt and enforce rules requiring that every newborn in this state shall, prior to becoming 1 week of age, be subjected to a test for phenylketonuria" and specifying that DOH "may deem" it necessary to screen for other disorders).

**B. SECTION DIRECTORY:**

**Section 1:** Amends s. 383.14, F.S., relating to screening for metabolic disorders, other hereditary and congenital disorders, and environmental risk factors.

**Section 2:** Provides an effective date.

**II. FISCAL ANALYSIS & ECONOMIC IMPACT STATEMENT**

**A. FISCAL IMPACT ON STATE GOVERNMENT:**

1. Revenues:

None.

2. Expenditures:

The bill will have a significant negative fiscal impact on DOH if ALD screening begins. This will include an annually recurring cost of \$2,550,000 for the DOH State Laboratory to conduct the screening at a cost \$8.50 per specimen for approximately 300,000 newborns screened.<sup>15</sup> DOH will also incur an annual recurring cost for the presumptive positive results that are sent to the newborn screening genetic centers of \$83,000.<sup>16</sup> In addition, DOH will incur a one-time cost of \$50,000 to modify its current data system to incorporate ALD screening, follow-up, and tracking.<sup>17</sup>

The bill will also have a significant negative fiscal impact on AHCA for newborns covered under Florida Medicaid:

Fiscal Year	Medicaid Newborns	Increased Cost
2015-2016	130,081	\$2,146,344
2016-2017	131,669	\$2,182,531
2017-2018	133,275	\$2,199,029

Annual screening costs were determined by multiplying the unit cost per the CPT Code of \$16.50 and the estimated number of Medicaid newborns each year. In 2013, according the Florida Vital Statistics Annual Report, there were 215,194 births, of which 59% were Medicaid eligible.<sup>18</sup> The calculations estimate a 1.22% increase in total number of births each year.

**B. FISCAL IMPACT ON LOCAL GOVERNMENTS:**

1. Revenues:

None.

2. Expenditures:

None.

**C. DIRECT ECONOMIC IMPACT ON PRIVATE SECTOR:**

None.

**D. FISCAL COMMENTS:**

None.

<sup>15</sup> Florida Department of Health, 2015 Agency Legislative Bill Analysis HB 403, January 28, 2015 (on file with Health Quality Subcommittee); email from Department of Health, *Question about HB 403/SB 632*, March 18, 2015 (on file with Health Quality Subcommittee).

<sup>16</sup> Id.

<sup>17</sup> Id.

<sup>18</sup> Florida Agency for Health Care Administration, 2015 Agency Legislative Bill Analysis HB 403, January 22, 2015 (on file with Health Quality Subcommittee).

### **III. COMMENTS**

#### **A. CONSTITUTIONAL ISSUES:**

##### **1. Applicability of Municipality/County Mandates Provision:**

Not Applicable. This bill does not appear to affect county or municipal governments.

##### **2. Other:**

None.

#### **B. RULE-MAKING AUTHORITY:**

DOH will be required to adopt rules related to newborn screening requirements for ALD when it is added to the RUSP. Specifically, Rule 64C-7.002, F.A.C., which includes the list of congenital conditions/diseases for which newborns are screened and specifies when the blood specimen is to be collected, would need to be amended to include screening for ALD once it is added to the RUSP.

#### **C. DRAFTING ISSUES OR OTHER COMMENTS:**

None.

### **IV. AMENDMENTS/ COMMITTEE SUBSTITUTE CHANGES**

On March 16, 2015, the Health Quality Subcommittee adopted a strike-all amendment to HB 403 and reported the bill favorably as a committee substitute. The amendment requires DOH to expand the statewide screening of newborns to include screening for ALD once HHS adds ALD to the federal RUSP. The analysis is drafted to the Health Quality Subcommittee substitute.