HOUSE OF REPRESENTATIVES STAFF ANALYSIS

BILL #: CS/HM 1165 Newborn Adrenoleukodystrophy Screening
SPONSOR(S): Local & Federal Affairs Committee; La Rosa
TIED BILLS: **IDEN./SIM. BILLS:** SM 1288

REFERENCE	ACTION	ANALYST	STAFF DIRECTOR or BUDGET/POLICY CHIEF
1) Local & Federal Affairs Committee	18 Y, 0 N, As CS	Dougherty	Rojas
2) Health & Human Services Committee			

SUMMARY ANALYSIS

X-linked adrenoleukodystrophy (ALD) is a genetic disorder that occurs primarily in males and mainly affects the nervous system and the adrenal glands, reducing the nerves' ability to relay information to the brain and causing certain hormonal insufficiencies.

Each state administers a newborn screening panel to test for certain genetic disorders at birth. Although each state determines which conditions to include on its panel, the U.S. Department of Health and Human Services provides a list. This list currently has 57 disorders and more can be added by successfully passing a nomination and review process. Despite its nomination in 2012, ALD is not on the list of recommended conditions to include in newborn screening.

CS/HM 1165 urges Congress to recommend that ALD be included in the recommended panel for newborn screening by the Discretionary Advisory Committee on Heritable Disorders in Newborns and Children.

Copies of the memorial will be provided to the President of the United States, the President of the United States Senate, the Speaker of the United States House of Representatives, and each member of the Florida delegation to the United States Congress.

This memorial has no fiscal impact.

FULL ANALYSIS

I. SUBSTANTIVE ANALYSIS

A. EFFECT OF PROPOSED CHANGES:

Present Situation

X-linked Adrenoleukodystrophy (ALD)[1]

X-linked adrenoleukodystrophy is a genetic disorder that occurs primarily in males and mainly affects the nervous system and the adrenal glands. In this disorder, the fatty covering that insulates nerves in the brain and spinal cord is prone to deterioration, which reduces the ability of the nerves to relay information to the brain. In addition, damage to the outer layer of the adrenal glands causes a shortage of certain hormones (adrenocortical insufficiency).

According to the National Institute of Health, the prevalence of ALD is 1 in 20,000 to 50,000 individuals worldwide. This condition occurs with a similar frequency in all populations.

ALD is commonly referred to as Addison disease and cerebral sclerosis, melanodermic leukodystrophy, Schilder-Addison Complex, Schilder disease, Siemerling-Creutzfeldt disease, and X-ALD.

Symptoms

Adrenocortical insufficiency may cause weakness, weight loss, skin changes, vomiting, and coma. Rarely, individuals with ALD develop multiple features of the disorder in adolescence or early adulthood. In addition to adrenocortical insufficiency, these individuals usually have psychiatric disorders and a loss of intellectual function (dementia).

Male Inheritance

X-linked adrenoleukodystrophy is inherited in an X-linked pattern. A condition is considered X-linked if the mutated gene that causes the disorder is located on the X chromosome, one of the two sex chromosomes in each cell. In males (who have only one X chromosome), one altered copy of the gene in each cell is sufficient to cause the disorder. Because females have two copies of the X chromosome, one altered copy of the gene in each cell is usually not problematic; however, some females with one altered copy of the gene have health problems associated with this disorder. The signs and symptoms of X-linked adrenoleukodystrophy tend to appear at a later age in females than in males. Affected women usually develop features of the adrenomyeloneuropathy type.

Types of X-linked Adrenoleukodystrophy

There are three distinct types of ALD: a childhood cerebral form, an adrenomyeloneuropathy type, and a form called Addison disease only.

Childhood Cerebral Form

Children with the cerebral form of X-linked adrenoleukodystrophy experience learning and behavioral problems that usually begin between the ages of 4 and 10. Over time the symptoms worsen, and these children may have difficulty reading, writing, understanding speech, and comprehending written material. Additional signs and symptoms of the cerebral form include aggressive behavior, vision problems, difficulty swallowing, poor coordination, and impaired adrenal gland function. The rate at which this disorder progresses is variable but can be extremely rapid, often leading to total disability within a few years. The life expectancy of individuals with this type depends on the severity of the signs and symptoms and how quickly the disorder progresses. Individuals with the cerebral form of X-linked

[1] See http://ghr.nlm.nih.gov/condition/x-linked-adrenoleukodystrophy.

adrenoleukodystrophy usually survive only a few years after symptoms begin but may survive longer with intensive medical support.

Adrenomyeloneuropathy Type

Signs and symptoms of the adrenomyeloneuropathy type appear between early adulthood and middle age. Affected individuals develop progressive stiffness and weakness in their legs (paraparesis), experience urinary and genital tract disorders, and often show changes in behavior and thinking ability. Most people with the adrenomyeloneuropathy type also have adrenocortical insufficiency. In some severely affected individuals, damage to the brain and nervous system can lead to early death.

Addison Disease Only Form

People with X-linked adrenoleukodystrophy whose only symptom is adrenocortical insufficiency are said to have the Addison disease only form. In these individuals, adrenocortical insufficiency can begin anytime between childhood and adulthood. However, most affected individuals develop the additional features of the adrenomyeloneuropathy type by the time they reach middle age. The life expectancy of individuals with this form depends on the severity of the signs and symptoms, but typically this is the mildest of the three types.

Advisory Committee

In 2003, the U.S. Department of Health and Human Services created the Secretary's Advisory Committee on Heritable Disorders in Newborns and Children (SACHDNC).[2] The purpose of this committee was to advise the Secretary[3] on the best ways to reduce morbidity and mortality among babies who have, or are at risk for, certain heritable disorders. SACHDNC determined national recommendations for newborn screening panels, the Recommended Uniform Screening Panel (RUSP). On April 24, 2013, the Discretionary Advisory Committee on Heritable Disorders in Newborns and Children (DACHDNC) was chartered[4] to fulfill the functions previously undertaken by SACHDNC.

The committee advises the Secretary on the most appropriate application of universal newborn screening tests, technologies, policies, guidelines, and standards. Specifically, the committee provides the Secretary the following:[5]

- advice and recommendations concerning grants and projects authorized awarded or funded related to screening heritable disorders in newborns and children;
- technical information to develop Heritable Disorders Program policies and priorities to enhance the ability of the state and local health agencies to provide screening, counseling, and health care services for newborns and children who have or are at risk for heritable disorders; and
- recommendations, advice, and information to enhance, expand, or improve the ability of the Secretary to reduce mortality and morbidity from heritable disorders in newborns and children.

The Committee's next meeting is scheduled for May 29-30, 2014.

State Newborn Screening Panels

Each state administers a newborn screening panel, which tests for a variety of conditions at birth, and each state public health department decides both the number and types of conditions on its panel. Each state's panel is largely determined by the following factors:

- the laws of the state;
- the financial costs of screening;
- the frequency of the disorder in the state;

[2] Established under the Section 1111 of the Public Health Service (PHS) Act, 42 U.S.C. 300b-10, as amended in the Newborn Screening Saves Lives Act of 2008 (Act).
[3] Secretary, U.S. Department of Health and Human Services.
[4] Established under the Public Health Service Act (PHS), 42 U.S.C. 217a: Advisory councils or committees.
[5] See http://www.hrsa.gov/advisorycommittees/mchbadvisory/heritabledisorders/index.html.

- the availability of treatments for each condition; and
- the funding sources for the newborn screening program.

Recommended Uniform Screening Panel (RUSP)

Although each state ultimately determines which conditions to test for, the Committee compiles a list of nationally recommended disorders. The Committee follows a rigorous selection process to determine the included disorders. This selection is based on the *Newborn Screening: Towards a Uniform Screening Panel and System*.[6] The Committee recommends that every newborn screening program include a Uniform Screening Panel[7] that screens for 31 core disorders and 26 secondary disorders.[8]

Newborn screening is specifically designed to identify core conditions, which share the following classifying characteristics:

- there is a specific and sensitive test available to detect it;
- the health outcomes of the condition are well understood;
- there is an available and effective treatment; and
- identification of the condition could affect the future reproductive decisions of the family.

When looking for a core condition, other genetic conditions may be identified unintentionally. These are secondary conditions.

Process to Add Disorders to the RUSP

Nomination[9]

In order for the Committee to consider adding a particular disorder to the RUSP, the condition must be nominated. The Committee encourages individuals and organizations to form multi-disciplinary teams to submit nominations for conditions to be considered for inclusion on the RUSP. Teams should include researchers and/or clinicians with expertise on the condition being nominated, advocacy and/or professional organizations with knowledge of issues relevant to newborn screening, and interested consumers/individuals. A Nomination Package must be assembled and submitted to the Committee.

Nomination and Prioritization Workgroup

The Committee's Nomination and Prioritization Workgroup reviews the completed Nomination Package and compiles a summary for the Committee's consideration. The Committee decides if sufficient evidence is available, and votes to assign, or not assign, the nominated condition to the external Condition Review Workgroup. Nominators whose conditions are not assigned to the Condition Review Workgroup are provided with feedback.

Condition Review Workgroup

The external Condition Review Workgroup completes a systematic review, provides updates, and presents a final report to the Committee on assigned conditions. The Committee votes to recommend, or not recommend, adding the nominated condition to the RUSP for consideration by the Secretary. Nominators whose conditions are not recommended for addition to the RUSP are provided with feedback. The Secretary makes the final decision on whether to add, or not add, a recommended condition to the RUSP.[10]

[6] See http://www.hrsa.gov/advisorycommittees/mchbadvisory/heritabledisorders/uniformscreening.pdf.
[7] See http://www.hrsa.gov/advisorycommittees/mchbadvisory/heritabledisorders/recommendedpanel/uniformscreeningpanel.pdf.
[8] For the lists od core and secondary conditions as of April 2013, see http://www.hrsa.gov/advisorycommittees/mchbadvisory/heritabledisorders/recommendedpanel/uniformscreeningpanel.pdf.
[9] See http://www.hrsa.gov/advisorycommittees/mchbadvisory/heritabledisorders/nominatecondition/index.html.
[10] For the latest Nominated Conditions, see http://www.hrsa.gov/advisorycommittees//mchbadvisory/heritabledisorders/nominatecondition/workgroup.html#conditions.

X-linked Adrenoleukodystrophy's Former Nomination

The Stop ALD Foundation nominated ALD for consideration in 2012, but it did not pass the SACHDNC's internal review and therefore was not sent to the Condition Review Workgroup.[11]

Effect of Proposed Changes

CS/HM 1165 urges Congress to recommend that X-linked Adrenoleukodystrophy be included in the Recommended Uniform Screening Panel for state newborn screening programs by the Discretionary Advisory Committee on Heritable Disorders in Newborns and Children.

Legislative memorials are not subject to the Governor's veto power and are not presented to the Governor for review. Memorials have no force of law—they are mechanisms for formally petitioning the U.S. Congress to act on a particular subject. This memorial does not have a fiscal impact.

Copies of the memorial will be provided to the President of the United States, the President of the United States Senate, the Speaker of the United States House of Representatives, and each member of the Florida delegation to the United States Congress.

B. SECTION DIRECTORY:

Not applicable.

II. FISCAL ANALYSIS & ECONOMIC IMPACT STATEMENT

A. FISCAL IMPACT ON STATE GOVERNMENT:

1. Revenues:

   None.

2. Expenditures:

   None.

B. FISCAL IMPACT ON LOCAL GOVERNMENTS:

1. Revenues:

   None.

2. Expenditures:

   None.

C. DIRECT ECONOMIC IMPACT ON PRIVATE SECTOR:

None.

D. FISCAL COMMENTS:

None.

III. COMMENTS

[11] The decision letter is available at http://www.hrsa.gov/advisorycommittees/mchbadvisory/heritabledisorders/nominatecondition/reviews/alddecisionletter.pdf.

A. CONSTITUTIONAL ISSUES:

1. Applicability of Municipality/County Mandates Provision:

None.

2. Other:

None

B. RULE-MAKING AUTHORITY:

None.

C. DRAFTING ISSUES OR OTHER COMMENTS:

None.

IV. AMENDMENTS/ COMMITTEE SUBSTITUTE CHANGES

On April 3, 2014, the Local & Federal Affairs Committee considered and adopted a technical amendment correcting the name of the federal oversight committee.