John Bel Edwards
GOVERNOR

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SECRETARY

State of Louisiana

Department of Health and Hospitals
Office of Public Health

April 29, 2016

The Honorable Fred H. Mills, Jr., Chairman
Senate Health and Welfare Committee
P.O. Box 94183, Capitol Station
Baton Rouge, LA 70804

The Honorable Frank A. Hoffmann, Chairman
House Health and Welfare Committee
P.O. Box 94062, Capitol Station
Baton Rouge, LA 70804

Subject: DHH response to SCR 3 of the 2016 Regular Legislative Session

Dear Honorable Chairs:

Senate Concurrent Resolution 3 of the 2016 Regular Session requires the Department of Health and Hospitals (DHH) to submit a report on the health benefits and healthcare costs associated with adding Adrenoleukodystrophy (ALD) to the newborn screening panel.

Please accept this letter as a response to this request.

Overview of the Condition

ALD is lysosomal storage disorder (LSD) caused by mutations in the ABCD1 gene located on the X chromosome. There are three phenotype classifications X-linked ALD (X-ALD): adrenocortical insufficiency ("Addison's-only"), cerebral demyelination (child, adolescent, and adult cerebral ALD) and progressive paraparesis (adrenomyeloneuropathy or AMN). The most serious form of X-ALD is the childhood cerebral ALD. This condition affects more males than females, with symptoms appearing between 2.5 and 10 years of age and is associated with rapid neurologic decline and death within 3 years after onset.

The overall prevalence of X-ALD is estimated at 6 per 100,000 births regardless of sex. For males with cerebral ALD, the prevalence is 1 in 100,000. Based on these rates, at least one case of ALD will be expected in Louisiana, once every 2 years.
(Source: External Review Report of the Advisory Committee on Heritable Disorders in Newborns and Children, August 2015)

Health Benefits of Adding X-ALD to the Newborn Screening Panel

In order for a new condition to be added to the panel for screening, an interested group must submit a nomination form to the U.S. Department of Health and Human Service's Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC). This form undergoes federal administrative review and then an evidenced based review by an external body to essentially determine if the proposed test has a a) clear definition, b) if a clear test result can be established and c) if there is an effective treatment for the condition. Based on information obtained by the review, the ACHDNC has decided to recommend universal screening for X-ALD.

Testing for ALD is done by a process called tandem mass spectrometry (TMS) that the DHH Office of Public Health's (OPH) Laboratory currently uses to test for over 20 other conditions on the newborn screening panel. TMS is normally used in conjunction with a U.S. Food and Drug Administration (FDA) approved test kit. Although SCR 3 proposes testing by January 1, 2017, a delay in testing is requested since it is not possible to start ALD testing in the OPH without an FDA approved test kit being available. The OPH Laboratory will have the capacity to test for ALD in mid-2017 when an FDA-approved kit is expected to be released.

Successful treatment of ALD requires early diagnosis. One of the most prevalent early symptoms of children with ALD is adrenal insufficiency, which has been identified in up to 86% of males prior to any other signs of neurologic involvement. Results from newborn screening may lead to earlier diagnoses than clinical findings and other clinical tests. Another benefit of early screening is improved treatment which includes adrenal cortisol replacement therapy. Hematopoietic stem cell transplantation (HSCT) from a related or matched donor can reduce the risk of progression of neurologic symptoms if the condition is detected early enough.

Healthcare Costs of Adding ALD to the Newborn Screening Panel

It is estimated that start up testing for ALD would cost the state at least $1,051,291 for year 1, which includes the cost of equipment, supplies, staff, and professional services; and at least $438,091 for years 2 through 5, which includes supplies, staff, maintenance of equipment, and professional services. The cost per test per infant is expected to be approximately $20 for the first year of testing and will drop to approximately $8 per infant for the subsequent years.

- Lab Costs
    - Equipment:

        - 2 Tandem Mass Spectrometers, year 1 only, at $350,000 each ($700,000)
        - Rainin Liquidator, year 1 only ($20,000)
        - Plates, tips, and solvents, approximately $0.20 per infant, 0.2*52,000 ($10,400)
        - Reagent from Perkin Elmer approximately $1.00 per sample, 1*52,000 ($52,000)
    - Staff:
        - 1 Lab Scientist, $61,709 salary plus fringe ($84,850 annually)
    - Maintenance:
        - 2 Tandem Mass Spectrometers annual service contract costs beginning in Year 2 ($105,000)
        - Rainin Liquidator Maintenance cost beginning in Year 2 ($3,000)
    - Cost per year:
        - Year 1 – $867,250
        - Years 2 through 5 – $255,250 annually
- Genetics Program Costs
    - Equipment:
        - Computer and printer, year 1 only ($1200)
    - Staff:
        - 1 Program Monitor to contact physician and family to ensure reporting and treatment, $60,248 salary plus fringe ($82,841)
    - Professional Services:
        - Contracts with specialists (neurologist, endocrinologist, and hematology oncologist) from Tulane and LSU to hold clinics in regional health units ($100,000)
    - Cost per year
        - Year 1: $184,041
        - Years 2 through 5: $182,841
- Total cost per test
    - Year 1, approximately $20 per test
    - Years 2 through 5, approximately $8 per test

Note: Treatment (bone marrow transplant) is approximately $200,000 to $300,000 per case.

Once ALD is added to the newborn screening panel, OPH will seek reimbursement from Medicaid (to be determined) and private insurers to offset the cost of testing and follow-up as newborn screening is a covered service under health insurance plans.

Based on the evidence presented in this letter, and with the proper resources in place, DHH supports adding ALD to the newborns screening panel.

If you have further questions or concerns regarding this report or any public health matter, please contact Beth Scalco at (225) 342-8093 or beth.scalco@la.gov.

Sincerely,

Andrew Tuozzolo
Chief of Staff

cc: David R. Poynter, Legislative Research Library