Date of Hearing: May 8, 2024

ASSEMBLY COMMITTEE ON APPROPRIATIONS
Buffy Wicks, Chair
AB 2563 (Essayli) – As Introduced February 14, 2024

Policy Committee: Health Vote: 16 - 0

Urgency: No State Mandated Local Program: No Reimbursable: No

SUMMARY:

This bill requires the California Department of Public Health (CDPH) to expand the Newborn Screening Program to include screening for Duchenne Muscular Dystrophy (DMD), and expands the purposes for which moneys from the Genetic Disease Testing Fund are expended.

FISCAL EFFECT:

CDPH estimates first-year costs of adding DMD screening to be $8 to $11 million and ongoing costs in the range of $6 million to $8 million dollars thereafter (Genetic Disease Testing Fund). The first-year cost includes equipment and modification to the Screening Information System. The ongoing costs include supplies, consumables, design of result mailers, development of patient and provider educational materials, staffing for screening specimens for DMD, and follow-up activities at special care centers throughout California. CDPH states it does not have the capacity to include DMD in the screening panel and will need to create a new screening process. CDPH also notes DMD is still under review by the federal Recommended Uniform Screening Panel (RUSP), and there is currently no laboratory-developed DMD testing and no U.S. Food and Drug Administration (FDA)-approved kits for this disorder.

State law requires newborn screening in the Genetic Disease Screening Program (GDSP) be fee-supported, so the addition of DMD will require a fee increase to cover these costs.

COMMENTS:

1) **Purpose.** This bill is sponsored by Destroy Duchenne. According to the author:

> I am honored to be authoring AB 2563, a simple yet important bill which will add Duchenne Muscular Dystrophy (DMD) to the statewide newborn screening panel. DMD is a debilitating and often fatal disorder that causes progressive muscle deterioration and weakness, affecting approximately 1 in 3,600 male infants worldwide. Early intervention and treatment can make a critical difference in the life of a DMD patient…
>
> AB 2563 will bring California in line with the policies already enacted in states like Ohio and New York, along with many others currently considering similar proposals. California can and should

> be a leader in this space, as newborn screenings for DMD will save and improve thousands of lives each year.

2) **Background.**

DMD. According to the Muscular Dystrophy Association, DMD is a genetic disorder characterized by progressive muscle degeneration and weakness due to the alterations of a protein called dystrophin that helps keep muscle cells intact. DMD symptom onset usually occurs between ages two and three years and primarily affects boys. Muscle weakness is the principal symptom, first affecting the muscles close to the core of the body, and later affecting the distal limb muscles. Usually, the lower external muscles are affected before the upper external muscles. Progressive weakness and scoliosis result in impaired pulmonary function, which can eventually cause acute respiratory failure. Life expectancy for those with DMD is about 25 years, but has increased, with some patients surviving beyond 30 years. In Europe and North America, the prevalence of DMD is approximately one in 3,500 to 5,000 newborns.

In March 2024, the U.S. Food and Drug Administration (FDA) approved Duvyzat (givinostat) oral medication for the treatment of DMD in patients six years of age and older. Duvyzat is the first nonsteroidal drug approved to treat patients with all genetic variants of DMD.

Newborn Screening for DMD. NBS for DMD has been performed, primarily as pilot studies, in various parts of the world since the 1970s as early detection was believed to be beneficial. The benefits include early treatments such as physical therapy, allowing families to prepare for supporting a child with DMD by accessing appropriate resources and considering family planning options for future children. In 2023, Ohio became the first state to mandate universal NBS for DMD, with New York following shortly thereafter.

Adding DMD to the California Newborn Screening Program. DMD screening is currently undergoing evidentiary review by the federal Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC). This bill would add DMD to California's Newborn Screening Panel. However, SB 1095 (Pan), Chapter 393, Statutes of 2016, requires CDPH to expand statewide screening of newborns to include screening for any disease that is detectable in blood samples as soon as practicable, but no later than two years after the disease is adopted by the RUSP.

3) **Prior Legislation.**

   a) SB 1095 (Pan) requires CDPH to expand statewide screening of newborns to include screening for any disease that is detectable in blood samples as soon as is practicable, but no later than two years after the disease is adopted by RUSP or enrollment of SB 1095, whichever is later.

   b) AB 1559 (Pan), Chapter 565, Statutes of 2014, requires the Newborn Screening Program to expand statewide screening of newborns to include screening for adrenoleukodystrophy (ALD) as soon as ALD is adopted by the RUSP.

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