FLORIDA HOUSE OF REPRESENTATIVES
BILL ANALYSIS

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BILL #: HB 1089	**COMPANION BILL:** SB 524 (Harrell)
TITLE: Newborn Screenings	**LINKED BILLS:** None
SPONSOR(S): Booth	**RELATED BILLS:** SB 524 (Harrell)

Committee References

Health Professions & Programs 17 Y, 0 N ➤ Health Care Budget ➤ Health & Human Services

SUMMARY

Effect of the Bill:

HB 1089 requires the Florida Newborn Screening Program to add screening for Duchenne muscular dystrophy to the state's newborn screening program by January 1, 2027.

Fiscal or Economic Impact:

The bill will have a significant, negative fiscal impact on the Department of Health (DOH). DOH estimates a total cost of $2,678,989 ($2,580,241/recurring and $98,748/nonrecurring) to implement the provisions of the bill.

JUMP TO SUMMARY ANALYSIS RELEVANT INFORMATION BILL HISTORY

ANALYSIS

EFFECT OF THE BILL:

The Florida Newborn Screening (NBS) program, within the Department of Health (DOH), screens all infants born in the state for metabolic, hereditary, and congenital disorders known to result in significant impairment of health or intellect. The NBS Program currently uses blood spot testing to screen for 37 core conditions and 23 secondary conditions (a total of 60 conditions). Conditions are included in NBS Program screening based primarily on federal recommendations and the needs of the state. Duchenne Muscular Dystrophy (DMD) is the most common pediatric-onset muscular dystrophy affecting approximately one in 5,000 live male births. The NBS Program does not currently screen for DMD.

HB 1089 requires DOH to adopt rules requiring the NBS Program to screen newborns for DMD beginning January 1, 2027. (Section 1).

DOH anticipates that screening will identify approximately 900 newborns whose first-tier test results will require outreach and further genetic testing. DOH estimates that of those 900, approximately 20-30 male newborns may be diagnosed with DMD.[1]

The bill provides an effective date of July 1, 2025. (Section 2).

[1] Department of Health, *Agency Analysis of HB 1089* (2025). On file with the Health Professions & Programs Subcommittee.

RULEMAKING:

Current law authorizes the Department of Health (DOH) to adopt rules administering the NBS program. The bill modifies this authority to require DOH to adopt rules to implement the provisions of the bill.

Lawmaking is a legislative power; however, the Legislature may delegate a portion of such power to executive branch agencies to create rules that have the force of law. To exercise this delegated power, an agency must have a grant of rulemaking authority and a law to implement.

FISCAL OR ECONOMIC IMPACT:

STATE GOVERNMENT:

DOH estimates a total cost of $2,678,989 ($2,580,241/recurring and $98,748/nonrecurring) to implement the provisions of the bill as follows:[2]

- Total Non-recurring expenses: $98,748:
  - $75,000 for changes to the Laboratory Information Management System (LIMS);
  - $23,748 for professional non-recurring expense standards.
- Total recurring expenses: $2,580,241:
  - $984,000 for first- and second- tier test kits;
  - $388,581 for salary and benefits for three laboratory personnel and one registered nurse consultant;
  - $1,148,469 for three contracted specialty care centers to hire additional staff;
  - $30,336 for travel expenses;
  - $27,448 for other expenses;
  - $1,407 for human resources outsourcing.

RELEVANT INFORMATION

SUBJECT OVERVIEW:

Newborn Screening

Federal Recommendations for Newborn Screening

Newborn screening is a preventive public health program provided in every state 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 federal government produces a standardized list of conditions that it recommends every newborn be screened for, but each state determines which conditions are screened for under their respective state's newborn screening program.[3]

The U.S. Department of Health and Human Services (HHS) Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC) was established to reduce morbidity and mortality in newborns and children who have, or are at risk for, heritable disorders. The ACHDNC advises the Secretary of HHS on the most appropriate application of universal newborn screening tests, technologies, policies, guidelines, and standards.[4]

The Recommended Uniform Screening Panel (RUSP) is a list of disorders recommended by the Secretary of HHS, based on advice from the ACHDNC, for states to screen as part of their newborn screening program. Inclusion of a disorder on the RUSP is determined on evidence that supports the potential net benefit of screening, the ability of

[2] *Id.*
[3] Health Resources & Services Administration, *History of the ACHDNC*. Available at https://www.hrsa.gov/sites/default/files/hrsa/advisory-committees/heritable-disorders/hrsa-timeline-interactive.pdf (last visited March 14, 2025).
[4] U.S. Department of Health and Human Services, *Advisory Committee on Heritable Disorders in Newborns and Children*. Available at http://www.hrsa.gov/advisorycommittees/mchbadvisory/heritabledisorders/index.html (last visited March 14, 2025).

states to screen for the disorder, and the availability of effective treatments.[5] Adding a condition to the RUSP is a multistep process beginning with a nominator submitting a nomination package for review by the ACHDNC and may or may not result in the Committee recommending the condition for inclusion on the RUSP. The length of time from when a nomination is first presented to the ACHDNC to when the Secretary of HHS adds the condition to the RUSP varies widely, but most often it has taken three to four years.[6] Anyone can nominate a condition for inclusion by completing a nomination package.[7] The RUSP currently recommends screening for 36 core conditions and 26 secondary conditions. Duchenne Muscular Dystrophy has been nominated for inclusion in the RUSP, but has not been recommended by ACHDNC.[8]

Florida Newborn Screening Program

The Florida Newborn Screening (NBS) Program was initially established in 1965 to screen newborns for a single condition, phenylketonuria.[9] The NBS Program has since evolved to screen for a wide range of congenital conditions. The NBS program is housed within the Department of Health (DOH) and serves to promote the screening of all newborns for metabolic, hereditary, and congenital disorders known to result in significant impairment of health or intellect.[10] The NBS Program attempts to screen all newborns to identify, diagnose, and manage newborns at risk for select disorders that, without detection and treatment, can lead to permanent developmental and physical damage or death.[11] Parents and guardians may decline the screening if they choose to do so.[12]

The Florida Genetics and Newborn Screening Advisory Council (GNASC) advises DOH on disorders to be included in the panel of screened disorders and the procedures for collecting and transmitting specimens.[13] The NBS Program currently screens for 37 core conditions and 23 secondary conditions, nearly all of which are screened through the collection and testing of blood spots. Hearing screening, critical congenital heart disease, and targeted testing for congenital cytomegalovirus are completed at the birthing facility through point of care testing.[14]

Under current law, when a new condition is added to the federal RUSP, GNASC is required to consider the condition and make a recommendation to DOH as to whether the condition should be included in the state NBS panel within one year.[15] GNASC reviews the recommendation to ensure:[16]

- The state's readiness to screen, diagnose, and treat the condition;
- The condition is known to result in significant impairment in health, intellect, or functional ability if not treated before clinical signs appear;
- The condition can be detected using screening methods which are accepted by current medical practice;

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[5] Health Resources & Services Administration, *Recommended Uniform Screening Panel* (2024). Available at https://www.hrsa.gov/advisory-committees/heritable-disorders/rusp (last visited March 14, 2025).
[6] Health Resources & Services Administration, *Frequently Asked Questions* (2022). Available at https://www.hrsa.gov/advisory-committees/heritable-disorders/frequently-asked-questions (last visited March 17, 2025).
[7] Health Resources & Services Administration, *Condition Nomination and Review* (2022). Available at https://www.hrsa.gov/advisory-committees/heritable-disorders/condition-nomination (last visited March 14, 2025).
[8] Health Resources & Services Administration, *Previously Nominated Conditions* (2025). Available at https://www.hrsa.gov/advisory-committees/heritable-disorders/rusp/previous-nominations (last visited March 14, 2025).
[9] *See*, Tatiana Wing, R.C. Philips Research and Education Unit, *Newborn Screening Update* (2020). Available at https://genetics.pediatrics.med.ufl.edu/wordpress/files/2019/11/RCPU-Newborn-screening-update.pdf (last visited March 14, 2025); Watson, S., Lloyd-Puryear, M., & Howell, R. (2022). The Progress and Future of US Newborn Screening. International Journal of Neonatal Screening, 8:41, https://doi.org/10.3390/ijns8030041. Phenylketonuria (PKU) is a rare inherited disorder that causes an amino acid called phenylalanine to build up in the body resulting in dangerous symptoms unless a specific diet is adhered to. PKU was the first inheritable condition for which a relatively simple and repeatable blood test was able to be conducted at a high enough throughput to enable population-level screening.
[10] S. 383.14(1), F.S.
[11] Florida Department of Health, *Florida Newborn Screening 2022 Guidelines*. Available at https://floridanewbornscreening.com/wp-content/uploads/NBS-Protocols-2022-FINAL.pdf (last visited March 14, 2025).
[12] S. 383.14(4), F.S.; Rule 64C-7.008, F.A.C.; The health care provider must attempt to get a written statement of objection to be placed in the medical record.
[13] S. 383.14(5), F.S.
[14] *Supra*, note 1.
[15] S. 383.14(6), F.S.
[16] *Supra*, note 14.

- The condition can be detected prior to the infant becoming 2 weeks of age, or at the appropriate age as indicated by accepted medical practice;
- After screening for the disorder, reasonable cost benefits can be anticipated through a comparison of tangible program costs with those medical, institutional, and special educational costs likely to be incurred by an undetected population; and
- When screening for a condition, sufficient pediatric medical infrastructure is available.

The NBS Program involves coordination across several entities, including the Bureau of Public Health Laboratories Newborn Screening Laboratory (state laboratory), DOH Children's Medical Services (CMS) Newborn Screening Follow-up Program, and referral centers, birthing centers, and physicians throughout the state.[17] Health care providers in hospitals, birthing centers, perinatal centers, county health departments, and school health programs provide screening as part of the multilevel NBS Program screening process.[18]

Health care providers in hospitals and birthing centers collect drops of blood from the newborn's heel on a standardized specimen collection card which is then sent to the state laboratory for testing.[19] Point-of-care testing is used at the birthing facility to screen for the conditions which cannot be screened for with blood spot testing: pulse oximetry tests for critical congenital heart defect and hearing screening to detect hearing loss.[20]

Screening results are released to the newborn's health care provider; in the event of an abnormal result, the baby's health care provider, or a nurse or specialist from the Follow-up Program provides follow-up services and referrals for the child and his or her family.[21]

DOH is authorized to charge and collect a fee not to exceed $15 per live birth occurring in a hospital or birth center to administer the NBS Program.[22] DOH must calculate the annual assessment for each hospital and birth center, and then quarterly generate and mail each hospital and birth center a statement of the amount due.[23] DOH bills hospitals and birth centers quarterly using vital statistics data to determine the amount to be billed.[24] DOH is authorized to bill third-party payers for the screening tests and bills insurers directly for the cost of the screening.[25] DOH does not bill families that do not have insurance coverage.[26]

Duchenne Muscular Dystrophy

Muscular dystrophies are a group of genetic diseases that cause a person's muscles to become weak. Each kind of muscular dystrophy affects specific muscle groups, appears at different ages, and varies in severity.[27] Duchenne Muscular Dystrophy (DMD) is a rare genetic condition, but it is also the most common childhood-onset form of muscular dystrophy. Estimates of the affected population vary significantly, but DMD affects approximately one in every 3,300-5,000 live male births.[28] DMD is an X-linked inherited neuromuscular disorder; because it is X-linked, DMD can be carried by girls, but typically only presents with symptoms in boys.[29]

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[17] S. 383.14, F.S.
[18] *Id.*
[19] Florida Newborn Screening Program, *What is Newborn Screening?* Available at https://floridanewbornscreening.com/parents/what-is-newborn-screening/ (last visited March 14, 2024). *See also,* Florida Newborn Screening, *Specimen Collection Card*, http://floridanewbornscreening.com/wp-content/uploads/Order-Form.png (last visited March 14, 2025).
[20] Department of Health, *Agency Analysis of HB 499* (2024). On file with the Health & Human Services Committee.
[21] Department of Health, *Agency Analysis of HB 499* (2024). On file with the Health & Human Services Committee.
[22] S. 383.145(3)(g)1., F.S.
[23] *Id.*
[24] S. 383.145(3)(g), F.S.
[25] S. 383.145(3)(h), F.S.
[26] S. 383.14, F.S.
[27] Centers for Disease Control and Prevention. *About Muscular Dystrophy* (2025). Available at https://www.cdc.gov/muscular-dystrophy/about/index.html (last visited March 13, 2025).
[28] *See*, American College of Medical Genetics and Genomics, *Newborn Screening ACT Sheet: Duchenne and Becker Muscular Dystrophy* (2022). Available at https://www.acmg.net/PDFLibrary/DMD_Pathogenic_Variants.pdf (last visited March 15, 2025); U.S. Food & Drug Administration, *FDA Approves Targeted Treatment for Rare Duchenne Muscular Dystrophy Mutation* (2021). Available at https://www.fda.gov/news-events/press-announcements/fda-approves-targeted-treatment-rare-duchenne-muscular-dystrophy-mutation-0 (last visited March 15, 2025); U.S. Food & Drug Administration, *FDA Approves First Gene Therapy for Treatment of Certain Patients with Duchenne Muscular Dystrophy* (2023). Available at https://www.fda.gov/news-events/press-announcements/fda-approves-first-gene-

On average, boys begin showing early observable signs of DMD around two and a half years of age, but won't receive a confirmatory diagnosis of DMD until approximately five years old.[30] DMD is characterized by progressive skeletal and cardiac muscle weakness with children losing independent mobility by 9.5 years of age, developing cardiomyopathy by 14.5 years of age, with death occurring in their early twenties, on average.[31]

Diagnosis

The diagnostic process usually begins after a toddler has presented with early motor delays such as weakness, clumsiness, or difficulty with stair climbing. Ideally, the child is then promptly referred to a neuromuscular specialist, with input from a geneticist or genetic counsellor, and a diagnosis can be made soon after the onset of early symptoms.

Testing for DMD is typically conducted in two phases; first a blood test to check creatine kinase (CK) levels, high levels of which are indicative of DMD, followed by genetic testing to confirm DMD.[32] On average, two and a half years pass between the observation of early symptoms and a confirmatory diagnosis; this length time until diagnosis has not improved in the last several decades of monitoring DMD nationally.[33]

Treatment

DMD is considered a lethal condition for which there is no curative treatment. However, care for individuals with DMD has evolved significantly in the last 15 years resulting in prolonged survival and a focus on improving quality of life. Appropriate care for individuals with DMD involves a multidisciplinary approach. Careful management of a person's neuromuscular, rehabilitation, endocrine, gastrointestinal and nutritional needs are vital to prolonging life, slowing the progression of disease, and maintaining quality of life. [34]

Physiotherapy and treatment of glucocorticoids are well-established mainstays of DMD treatment. These treatment methods should begin as early as possible and continue after loss of independent mobility. Direct physical, occupational, and speech and language therapy should be used throughout life to delay muscle degeneration and loss of function. Beginning glucocorticoid therapy before significant physical decline and continued long-term glucocorticoid therapy have been shown to delay the loss of independent mobility, preserve upper limb and respiratory function, and mitigate severe scoliosis.[35]
Beyond the conventional approach, there are emerging disease-modifying treatments that are in various stages of development and approval. Four injectable exon-skipping drugs for treatment of specific subtypes of DMD and one gene therapy treatment for certain patients with DMD have been approved by the U.S. Food & Drug Administration

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therapy-treatment-certain-patients-duchenne-muscular-dystrophy (last visited March 15, 2025); Romitti, P. A., et al. (2015). *Prevalence of Duchenne and Becker muscular dystrophies in the United States*. Pediatrics, 135(3), 513–521. https://doi.org/10.1542/peds.2014-2044

[29] Venugopal, V. & Pavlakis, S., *Duchenne Muscular Dystrophy*. (2023). StatPearls Publishing. Available at https://www.ncbi.nlm.nih.gov/books/NBK482346/ (last visited March 15, 2025).

[30] Thomas, S., Conway, K. M., Fapo, O., Street, N., Mathews, K. D., Mann, J. R., Romitti, P. A., Soim, A., Westfield, C., Fox, D. J., Ciafaloni, E., & Muscular Dystrophy Surveillance, Tracking, and Research Network (MD STARnet) (2022). *Time to diagnosis of Duchenne muscular dystrophy remains unchanged: Findings from the Muscular Dystrophy Surveillance, Tracking, and Research Network, 2000-2015*. Muscle & nerve, 66(2), 193–197. https://doi.org/10.1002/mus.27532

[31] Paramsothy, P., Wang, Y., Cai, B., Conway, K. M., Johnson, N. E., Pandya, S., Ciafaloni, E., Mathews, K. D., Romitti, P. A., Howard, J. F., Jr, & Riley, C. (2022). *Selected clinical and demographic factors and all-cause mortality among individuals with Duchenne muscular dystrophy in the Muscular Dystrophy Surveillance, Tracking, and Research Network*. Neuromuscular disorders: NMD, 32(6), 468–476. https://doi.org/10.1016/j.nmd.2022.04.008

[32] *Supra*, note 34.

[33] *Supra*, note 30.

[34] Birnkrant, D. J., Bushby, K., Bann, C. M., Apkon, S. D., Blackwell, A., Brumbaugh, D., Case, L. E., Clemens, P. R., Hadjiyannakis, S., Pandya, S., Street, N., Tomezsko, J., Wagner, K. R., Ward, L. M., Weber, D. R., & DMD Care Considerations Working Group (2018). *Diagnosis and management of Duchenne muscular dystrophy, part 1: diagnosis, and neuromuscular, rehabilitation, endocrine, and gastrointestinal and nutritional management.* The Lancet. Neurology, 17(3), 251–267. https://doi.org/10.1016/S1474-4422(18)30024-3

[35] *Id*.

(FDA).[36] Clinical trials for DMD treatments are ongoing; due to the nature of rare diseases, clinical trials have difficulty in reaching the necessary capacity because of the low number of patients who qualify for participation.[37]

Newborn Screening of DMD

Newborn screening has been proposed as a method for ensuring early diagnosis of DMD. Advocates for newborn screening for DMD point to evidence suggesting that emerging DMD therapies might prove to be most effective if they are initiated before the onset of symptoms and the overall benefits of beginning treatments as early as possible.[38] Furthermore, delayed diagnosis of DMD leads to lost opportunities for genetic counseling, implementation of appropriate standards of care, access to newly approved disease-modifying medications, and participation in clinical trials. However, there are ethical, legal, and social concerns relating to the development and implementation of newborn screening for DMD. These concerns include the limited treatment options available, whether both males and females should be screened, and the high rate of false-positives resulting from the first-tier diagnostic test.[39]

Newborn screening for DMD has been adopted in several states; it has been implemented in Minnesota and Ohio, and is in planning phases in New York and Massachusetts.[40] The method of screening is similar to the conventional diagnostic method for suspected cases of DMD. A blood spot test is conducted to measure CK levels, followed by a confirmatory genetic test. One of the primary concerns with this screening method is the relatively high frequency of elevated CK levels in newborns that are unrelated to DMD, leading to false positives and unnecessary genetic testing.[41]

DMD has been nominated for inclusion in the federal RUSP by Parent Project Muscular Dystrophy and the Muscular Dystrophy Association. The review process began in February 2023, but a pause in the review process was requested by the nominators after ACHDNC determined there was insufficient evidence to move forward and requested additional information regarding the diagnostic process and clinical utility.[42] The RUSP is largely restricted to neonatal-onset disorders for which early treatment shows improved outcome. DMD differs from the majority of conditions included on the RUSP because onset does not occur until later in childhood.[43]

BILL HISTORY

COMMITTEE REFERENCE	ACTION	DATE	STAFF DIRECTOR/ POLICY CHIEF	ANALYSIS PREPARED BY
Health Professions & Programs Subcommittee	17 Y, 0 N	3/20/2025	McElroy	Osborne
Health Care Budget Subcommittee				
Health & Human Services Committee				

[36] *Supra*, note 28. *See also*, Rare Disease Advisor, *Alongside Gene Therapy, Exon Skipping Remains Key Target in Duchenne Research* (2024). Available at https://www.rarediseaseadvisor.com/features/exon-skipping-key-target-duchenne-dmd-research/ (last visited March 15, 2025).
[37] *Supra*, note 34.
[38] *Supra*, note 34; *See also*, Parent Project Muscular Dystrophy, *Newborn Screening Action Center*. Available at https://www.parentprojectmd.org/advocacy/newborn-screening-action-center/ (last visited March 15, 2025).
[39] *Supra*, note 30.
[40] Parent Project Muscular Dystrophy, *Newborn Screening Action Center*. Available at https://www.parentprojectmd.org/advocacy/newborn-screening-action-center/ (last visited March 15, 2025).
[41] *Supra*, note 34.
[42] U.S. Department of Health and Human Services, Advisory Committee on Heritable Disorders in Newborns and Children, Chair Letter to DMD Nominators (2023). Available at https://www.hrsa.gov/sites/default/files/hrsa/advisory-committees/heritable-disorders/resources/chair-letter-dmd-nominators.pdf (last visited March 15, 2025); Health Resources & Services Administration, *Summary of Nominated Conditions to the Recommended Uniform Screening Panel* (2024). Available at https://www.hrsa.gov/sites/default/files/hrsa/advisory-committees/heritable-disorders/rusp/summary-nominated-conditions.pdf (last visited March 15, 2025).
[43] Supra, note 34.