

INITIAL STATEMENT OF REASONS

Summary of the Proposal

This regulatory emergency amendment is to increase the Newborn Screening (NBS) Program's all-inclusive program participation fee from \$129.25 to \$141.25 in Title 17, California Code of Regulations (17 CCR), Division 1, Chapter 4, Subchapter 9, Group 3, Article 4, Newborn Screening Fee Collection, section 6508, subdivision (b). The increase is necessary for the expansion of statewide screening of newborns to include screening for Mucopolysaccharidosis Type I (MPS I) and Pompe disease.

Policy Statement Overview

Problem Statement:

The California Department of Public Health's (Department) Genetic Disease Screening Program (GDSP) administers the NBS Program, as mandated by Health and Safety Code sections 125000, 125001, and 125025 to provide organized, quality assured screenings for all births in California for several genetic disorders. Disorders mandated for testing are established in Health and Safety Code sections 124977, 125000, 125001 and 125025, and in Title 17 CCR section 6508, subdivision (b).

The NBS Program panel screens for over 80 disorders that include amino acid disorders such as phenylketonuria, hemoglobinopathies, organic acid disorders, fatty acid oxidation disorders, galactosemia, congenital hypothyroidism, congenital adrenal hyperplasia, sickle cell anemia, cystic fibrosis, biotinidase deficiency, severe combined immune deficiency, and adrenoleukodystrophy.

Senate Bill (SB) 1095 (Chapter 393, Statutes of 2016) established Health and Safety Code section 125001, subdivision (d) and required the GDSP to expand statewide screenings of newborns by adding new tests within two years of the disease screen being adopted by the federal Recommended Uniform Screening Panel (RUSP). At the time the bill was chaptered, there were two disorders on the RUSP that were not on the NBS Program panel: MPS I and Pompe disease, which were added to the RUSP in 2016 and 2015, respectively. Therefore, as specified in state statutes, the Department is required to add these disorders to the NBS Program panel by August 30, 2018.

Health and Safety Code sections 124977 and 124996 require the NBS Program to be "fully supported from fees collected." The Department's Director may adjust this fee as needed to meet costs. The NBS Program previously collected \$129.25 for each newborn tested. The fees collected from the institution of birth were deposited into a special fund called the Genetic Disease Testing Fund (GDTF). The GDTF is used to pay expenses of program operations including costs of supplies, forms, educational materials, and contracts with private vendors for laboratory analysis, tracking, and follow-up of positive test results, data processing, and fee collection.

To begin screening for Pompe disease and MPS I, a test method for each disorder must be developed by the Department. In Fiscal Year (FY) 2017-2018, the 2017 Budget Act included \$2.25 million in one-time Local Assistance funding and \$139,000 in State Operations funding to plan, prepare for, and incorporate MPS I and Pompe disease into

the Screening Information System and support one Research Scientist II to start up test method development activities.

The FY 2018-2019 Budget Change Proposal for routine screening for Pompe and MPS I to support SB 1095 to expand the NBS Program was approved to add fifteen positions to address the routine testing and ongoing workload associated with the addition of Pompe disease and MPS I. An increase in Local Assistance expenditure authority was approved to purchase the consumables, supplies, and reagents related to the ongoing screening and testing activities. The FY 2018-2019 Budget Change Proposal authorizing the expansion of the NBS Program and the November and May Revision Estimates authorizing Local Assistance expenditures were approved by the Department of Finance and the Legislature during the FY 2018-2019 budget building process. All of the expenditure authority was included in the budget that was signed by the Governor on June 27, 2018.

The Department's GDSP works to improve the quality of testing by preventing false negative tests and keeping false positive test rates as low as possible. Incorporating new testing strategies to reduce false positive test rates to prevent unnecessary stress and anxiety for parents is vital. By coupling the primary screening method with a second linked test that is more specific than the original method, the Department can improve diagnostic specificity (fewer false positives) without reducing sensitivity (the rate of false negatives). A second-tier test uses the same blood specimen from the original test, eliminating that additional burden to families or hospital personnel, and measures additional metabolites that either strongly supports the presumption of a true positive case or shows that the patient does not have the disorder.

In FY 2017-2018, the 2017 Budget Act included a one-time increase of \$300,000 in State Operations expenditure authority, and a one-time transfer of \$330,000 from Local Assistance to State Operations expenditure authority, for the purpose of mass spectrometry equipment and testing method development. These one-time increases provided expenditure authority for the initial start-up activities for second-tier testing.

The FY 2018-2019 Budget Change Proposal was approved to support the workload associated with Second-Tier Newborn Testing. A shift of \$460,000 from Local Assistance to State Operations was approved to fund three positions to perform the ongoing second-tier testing that will begin in FY 2018-2019. The FY 2018-2019 Budget Change Proposal was approved by the Department of Finance and the Legislature during the FY 2018-2019 budget building process. All of the expenditure authority was included in the budget that was signed by the Governor on June 27, 2018.

Pompe disease is an inherited disorder caused by the buildup of a complex sugar called glycogen in the body's cells. The accumulation of glycogen in certain organs and tissues, especially in muscles, impairs their ability to function normally.

MPS I is a condition that affects many parts of the body. Children with MPS I often have no signs or symptoms of the condition at birth, but it is a progressive disease if not

treated early. Individuals with MPS I may have a large head (macrocephaly), heart valve abnormalities, and an enlarged liver and spleen.

Benefits and Objectives (Goals):

California newborns and their families will benefit from the expansion of statewide screening of newborns by adding new tests within two years of the disease screen being adopted by the federal RUSP. An early diagnosis through newborn screening coupled with appropriate treatment can slow the progress of the diseases before some irreversible disease processes have begun. An early diagnosis is the first step in an appropriate medical care treatment plan. An early diagnosis provides parents with more time to become educated about how to provide the best possible care for a child with either condition and helps parents make informed life choices, including reproductive and health plan decisions.

The cost of including the new disorders to the NBS Program panel requires complicated and labor-intensive development activities, all of which require the highest standards of medical and scientific procedures and protocols. New equipment is necessary as well as additional staff to run the test. The Department cannot absorb the additional workload associated with adding both disorders without an increase in fees, appropriation authority, and associated staffing.

This rulemaking is in response to changes in technology, testing methodologies, and the expansion of the NBS Program. Because the NBS Program is fully fee supported as required by state statute, a fee increase is required to provide revenue to ensure the expansion to Pompe disease and MPS I is fully implemented and that sufficient resources are available on an ongoing basis. This funding will support expenditures associated with the ongoing workload of processing biospecimens at the Department's Genetic Disease Laboratory; staff needed to perform the screening; testing of chemicals; equipment acquisition; IT upgrades; and supplies used to run the assay. Funding will also be utilized to support follow-up costs for "Screen Positive" cases, such as case management; diagnostic work-up; confirmatory processing; provider and family education; informative result mailers; as well as incorporation and maintenance on an ongoing basis of Pompe and MPS I into the Screening Information System.

Background/Authority

The Legislature has found that timely implementation of changes in genetic screening programs and continuous maintenance of quality statewide services requires expeditious regulatory action and administrative procedures (Health and Saf. Code § 124977, subd. (c)(1).) The Health and Safety Code provides authority for the Department to adopt emergency regulations. Health and Safety Code section 124977, subdivision (d)(1) specifies that the adoption of these regulations shall be deemed an emergency and necessary for the immediate preservation of the public peace, health and safety, or general welfare, and that the regulations shall not be subject to the review and approval of the Office of the Administrative Law (OAL); shall be submitted directly to the Secretary of State for filing; and shall become effective immediately upon filing by the Secretary of State. Health and Safety Code section 124977, subdivision (d)(1) also

requires the Department to conduct a public hearing within 120 days of filing with the Secretary of State, and to submit to the OAL the adopted regulation, a final statement of reasons, and an updated informative digest. Health and Safety Code section 124977, subdivision (d) (2) specifies that this emergency regulation shall not be repealed by the OAL and shall remain in effect until revised or repealed by the Department.

The GDSP will continue to monitor NBS Program revenues and expenses in order to determine whether future increases or decreases in the NBS Program's participation fees are warranted.

Specific Discussion of Regulatory Action

Title 17, Division 1, Chapter 4, Subchapter 9, Group 3, Article 4
Section 6508, subdivision (b), Newborn Screening Fee Collection.

Subsection (b) was revised to raise the Newborn Screening panel fee from \$129.25 to \$141.25. This revision is necessary because an increase of \$12.00 is required to fully support the current NBS Program and the additional service expansions necessary to implement Pompe and MPS I screening components. The additional expenditures are described in the dollar amounts in Attachment 1 to the Economic and Fiscal Impact Statement (STD. 399) Form. The comments are based on experience with implementation of newborn screening expansions; information collected from discussions with vendors; and comparison costs for similar services in other states and the private sector.

The amendment increases the NBS Program participation fee as necessary for the expansion of statewide screening of newborns to include screening for MPS I and Pompe disease.

Statement of the Results of the Economic Impact Assessment

- (b)(1) The Department has determined that the rulemaking will not significantly affect the following pursuant to Government Code Sections 11346.3, subd. (b)(1)(A), (B), (C), and (D):
- (A) **The Creation or Elimination of Jobs within the State of California**
The regulation will not create or eliminate jobs in California. The impact to insurers in processing the participation fee increase, and to insurance/health plan members, will be minimal.
 - (B) **The Creation of New Businesses or the Elimination of Existing Businesses within the State of California.** The regulation will not create new businesses or eliminate existing businesses within the State of California. The cost impact to insurers of \$12.00 for each covered newborn is unlikely to have a significant impact on any affected business, or insurance/health plan members.

- (C) **The Expansion of Businesses Currently Doing Business within the State of California.** The regulation will not expand businesses within the state of California. The impact to insurers in processing the participation fee increase will be minimal.
- (D) **Worker Safety** This regulation does not affect worker safety because it does not affect workers;
California's Environment This regulation does not affect the State's environment.
The Department has determined that the rulemaking impacts the following pursuant to Government Code section 11346.3, subdivision (b)(1)(D):
1. **Health and Welfare of California Residents.** The regulation is expected to increase and strengthen the health and welfare of California residents. An increase in the participation fee for the NBS Program ensures that the NBS Program remains self-sufficient and protects statewide access to newborn screening and follow-up services, thereby reducing the emotional and financial burden of disability and death caused by genetic and congenital disorders.
 2. The amendment increases the NBS Program participation fee as necessary for the NBS Program to expand statewide screening of newborns to include screening for MPS I, and Pompe disease.

Reasonable Alternatives to the Regulation and the Department's Reasons for Rejecting those Alternatives

The Department has made an initial determination that there are no acceptable alternatives to the regulation to fund the operations of the NBS Program and protect the public interest in maintaining a statewide NBS Program. There is no alternative to compliance with SB 1095 and Health and Safety Code section 125001, which requires the Department to add MPS I, and Pompe disease to the NBS Program panel by August 30, 2018.

Technical, Theoretical, and/or Empirical Study, Reports or Documents Relied Upon

Department of Finance Demographic Research Unit (DRU) Birth Projections for calendar year 1990-2040, ([Birth Projections](#).)

Evidence Supporting No Significant Statewide Adverse Economic Impact on Business

The Department has made an initial determination that the emergency regulations will not have a significant statewide adverse economic impact directly affecting businesses, including the ability of California businesses to compete with businesses in other states.

While the Department directly bills hospitals and midwives, health insurance and health plans and their capitated medical groups and health insurers generally cover the screening test. As such, the hospitals and midwives are generally reimbursed for the

cost of the screening test and the health plans and health insurers' account for the increase in the form of increased premiums and such increases are typical for health care costs in any given year.

The total annual costs of this regulation are estimated to be \$5,739,852 (478,321 estimated numbers of newborns to be screened in FY 2018-19 X \$12.00). It is unlikely that a \$12.00 increase in Newborn Screening fees, paid by the hospital of birth to the Department, is sufficient to require any significant increase in premiums for health insurance charged to businesses. Past increases in Newborn Screening fees had no adverse business impacts that were reported to the Department.

Mandated by Federal Law or Regulations

These regulatory changes do not conflict with or duplicate any federal or state statutes, regulations, or policies.