2022 ACNM SB 242 Senate Side (1).pdf
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AFFILIATE OF
AMERICAN COLLEGE of NURSE-MIDWIVES
With women, for a lifetime®

Committee:	**Senate Finance Committee**
Bill Number:	**Senate Bill 242**
Title:	**Maryland Department of Health – System for Newborn Screening – Requirements**
Hearing Date:	**February 3, 2022**
Position:	**Support**

The Maryland Affiliate of the American College of Nurse Midwives (ACNM) supports *Senate Bill 242 – Maryland Department of Health – System for Newborn Screening – Requirements.* This legislation will ensure that Maryland's Newborn Screening Program is kept up-to-date by requiring that disorders recommended by the U.S. Department of Health and Human Services (HHS) are added to the State screening panel within two years.

When considering adding a new disorder to HHS's Recommended Uniform Screening Panel (RSUP), an expert panel reviews the available research to determine the presence of "evidence that supports the potential net benefit of screening, the ability of states to screen for the disorder, and the availability of effective treatments." Among a variety of factors that are considered are the requirement that the disorder under consideration can be identified before a child becomes symptomatic and the presence of available treatment to prevent the child from ever becoming symptomatic. These are important criteria because disorders on the RUSP have significant impacts on the health and lives of children who have them and are often very expensive to treat after a child becomes symptomatic.

Currently, Maryland tests for 34 of the 35 federally recommended disorders. Maryland is not testing for X-linked adrenoleukodystrophy (X-linked ALD), a genetic disorder that mainly affects the nervous system and the adrenal glands.[i] Left untreated, this disorder reduces the ability of nerves to relay information to the brain. Additionally, damage to the adrenal glands may result in weakness, weight loss, skin changes, vomiting, or coma.[ii] In its most severe form, X-linked ALD can result in death during childhood or adolescence. [iii] Although HHS recommended screening for X-linked ALD in 2015, Maryland is still not testing for it, making it more difficult to connect children to life saving treatment.

ACNM supports this bill because it ensures that Maryland children receive newborn screening in accordance with federal recommendations. Thus, families will be able to access life-saving treatment for their children as soon as possible. Thank you for your consideration of our testimony. If you need any additional information, please contact Scott Tiffin at stiffin@policypartners.net or (443) 350-1325.

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[i] https://medlineplus.gov/genetics/condition/x-linked-adrenoleukodystrophy/
[ii] Ibid
[iii] Ibid

MD SB242 Testimony - C Fennell.pdf
Uploaded by: Claudia Fennell
Position: FAV

TO: Chair Shane Pendergrass
AT: Maryland State Senate
RE: Support of MD S.B. 242
SPONSOR: Senator Steve Hershey

FROM: Claudia Fennell, Patient Parent (Batten Disease, CLN2)
DATE: January 19, 2022

Dear Members of the Maryland Senate,

My name is Claudia Fennell, parent to an 8-year-old girl with a rare, neurodegenerative, genetic disorder called Batten Disease type CLN2. Our family is located in Bethesda, Maryland, and I'm here to express my urgent support for **Senate Bill 242, the "System for Newborn Screening - Requirements**," aimed to align Maryland's Department of Health newborn screening program with the federal Recommended Uniform Screening Panel (RUSP) recommendations - to ensure that, when a condition is adopted to the U.S. RUSP, Maryland families will then experience the life-saving benefits of timely diagnosis. I support this bill because diagnosis at birth has the ability to completely transform the lives of Batten Disease families.

Penelope was a healthy child, completely asymptomatic, until she was 3 years old. She played and joked with her two siblings and ten cousins, loved scoring soccer goals and playing in the mud. Then, suddenly, she developed intractable seizures, lost her ability to walk, most of her ability to speak, and could barely eat. It was extremely difficult to find a diagnosis, delaying treatment when every week counted. But, eventually we had answers, and she started an enzyme replacement therapy which dramatically slows the progression of her disorder. Untreated, she would have almost certainly passed away by now, and *earlier* treatment could have completely changed the course of the disease.

The effects of Penelope's delayed diagnosis permeate every aspect of our lives. The complexity of her care, and irregularity of her health status means that I am unable to return to the workforce and limits, among other things, our ability to contribute volunteer hours in our community. Our story is not unique among rare pediatric disorders - consider, if you will, the aggregate impact of delayed diagnosis on Maryland families; it is sure to be enormous.

I urge you to support **Senate Bill 242, the System for Newborn Screening - Requirements** in order to allow families timely access to critical advances in treatment, and reduce the financial impact of illness on families. Thank you for your time today.

Regards,
Claudia Fennell

Maryland RUSP Alignment Sign-on Letter.pdf
Uploaded by: Dylan Simon
Position: FAV

February 1, 2022

The Honorable Emily Shetty and Stephen Hershey

100 State Circle

Annapolis, MD 21401

RE: HB 109 and SB 242- System for Newborn Screening- Requirements

Dear Delegate Shetty and Senator Hershey,

As patient advocacy organizations representing individuals diagnosed with rare diseases and family caregivers in Maryland and across the United States, we write today to thank you for your leadership on newborn screening and express our support for HB 109 and SB 242.

Every year, millions of babies born in the US are screened for a variety of devastating and often fatal diseases and conditions that might otherwise go undetected. These simple screens help provide lifesaving early identification, allowing for the earliest possible diagnosis and immediate access to potentially life-saving treatments for babies. In many cases, early detection can avert costly and risky medical procedures later in life.

HB 109 and SB 242 provide a thoughtful approach to expanding newborn screening in Maryland that ensures that all conditions on the federal Recommended Uniform Screening Panel (RUSP) are added to the screening panel in a reasonable amount of time. The RUSP is periodically updated using a thorough, science and evidence-based deliberative review process involving a national committee of experts in newborn screening. This legislation allows Maryland to efficiently add new conditions by taking advantage of the work done by these medical experts at the federal level to remove obstacles to needed testing and minimizing the irreversible disease progression and loss of life that comes from untreated diseases. States like Georgia, North Carolina, Arizona, and California have passed similar legislation and each time it has had overwhelming bipartisan support.

Maryland is a leader in the field of newborn screening, screening for 34 of 35 conditions currently on the RUSP. However, one of the conditions not currently on the Maryland newborn screening panel, ALD, was recommended for addition more than six years ago. This legislation would require Maryland's Department of Health (MDH) to implement new screening recommendations within **two** years of the RUSP approval, ensuring that babies born in Maryland have the same opportunity for diagnosis and treatments as babies born across state lines.

For these reasons, we are proud to support the newborn screening language. We are grateful for your leadership on this issue and look forward to working with you and your office to ensure this language becomes law.

Sincerely,

EveryLife Foundation for Rare Diseases

MarylandRARE

Duplication Cares

TSC Alliance

Batten Disease Support and Research Association (BDSRA)

Gene Giraffe Project

MTS Sickle Cell Foundation, Inc.

The Akari Foundation

MitoAction

T.E.A.M. 4 Travis

Leukodystrophy Newborn Screening Action Network

United MSD Foundation

International Foundation for CDKL5 Research

Organic Acidemia Association

Project Alive

Acid Maltase Deficiency Association (AMDA)

SCID Angels for Life Foundation

SSADH Association

Undiagnosed Diseases Network Foundation (UDNF)

The Jansen's Foundation

Association for Creatine Deficiencies

HCU Network America

Parent Project Muscular Dystrophy (PPMD)

USA- Prader-Willi Syndrome Association (PWSA)

The Oxalosis and Hyperoxaluria Foundation

Children's PKU Network

Myositis Support and Understanding Association (MSU)

Brian's Hope

International Pemphigus Pemphigoid Foundation

Lymphatic Malformation Institute

National Ataxia Foundation

VHL Alliance

Stronger Than Sarcoidosis

MLD Foundation

STXBP1 Foundation

Histiocytosis Association

Cure GM1 Foundation

Jack McGovern Coats' Disease Foundation

Cure SMA

Cystic Fibrosis Research Institute (CFRI)

ALD Alliance

SYNGAP1 Foundation

Friedreich's Ataxia Research Alliance (FARA)

Pompe Alliance

National PKU Alliance

The E.WE Foundation

Ryker's Foundation for Pompe Disease

Rare Disease Innovations Institute, Inc.

Little Hercules Foundation

Cure Sanfilippo Foundation

Rare and Undiagnosed Network (RUN)

SLC6A1 Connect

The Global Foundation for Peroxisomal Disorders

Fibromuscular Dysplasia Society of America

ADNP Kids Research Foundation

Syngap Research Fund (SRF)

Alport Syndrome Foundation

NTM Info & Research

Remember the Girls

Cure MLD

Hunter Syndrome Foundation

SB242_NewbornScreening_KennedyKrieger_Support.pdf
Uploaded by: Emily Arneson
Position: FAV

Kennedy Krieger Institute

DATE: **February 3, 2022** **COMMITTEE: Senate Finance**
BILL NO: **Senate Bill 242**
BILL TITLE: **Maryland Department of Health - System for Newborn Screening - Requirements**
POSITION: **Support**

Kennedy Krieger Institute supports Senate Bill 242 - Maryland Department of Health - System for Newborn Screening - Requirements

Bill Summary:
Senate Bill 242 requires the Maryland Department of Health's Newborn Screening Program to screen for each condition listed in the U.S. Department of Health and Human Services' Recommended Uniform Screening Panel (RUSP).

Background:
Kennedy Krieger Institute is dedicated to improving the lives of children and young adults with developmental, behavioral, cognitive and physical challenges. Kennedy Krieger's services include inpatient, outpatient, school-based and community-based programs, which serve about 25,000 individuals every year. As part of these services, the Institute is involved in research and clinical care of individuals affected by rare genetic brain disorders. Over the last 5 decades many important research discoveries have led to establishing of new diagnostic tests and new therapies for many of these rare diseases.

Newborn Screening allows the early detection of treatable rare genetic disorders, resulting in a dramatic improvement in the lives of young babies and children, as well as their families (1). X-linked adrenoleukodystrophy (ALD) is a prototype example of rare genetic disorder that is currently identifiable through newborn screening (2). ALD is an inherited disorder of metabolism with recent data indicating an annual incidence of approximately 1 in 14,000 newborn males (3). It is a fatal disorder affecting the brain, the peripheral nervous system and the adrenal glands. Kennedy Krieger Institute, one of the first centers in the world to focus on ALD, currently provides care to about 500 patients affected by ALD. Internationally, Kennedy Krieger serves as both a major clinical and laboratory referral center. In fact, the newborn screening test that is being debated in SB242 was first developed at Kennedy Krieger (4) and has now been replicated by many laboratories across the United States and internationally.

Rationale:
Newborn boys with ALD appear perfectly normal and healthy at birth. They are discharged from the newborn nursery with their mothers and develop normally the first few years of life. About half of affected males will first develop behavioral problems around 5-7 years of life and then rapidly lose their vision, their hearing, and then their ability to talk, swallow, and to protect their airways. Next, boys with ALD become bedridden and either die or end up in a vegetative state, all within 2 years after symptom onset. Unfortunately, despite many attempts by various international groups, no effective therapy has been discovered for affected individuals once their first neurological symptoms have manifested (5).

Importantly, while diagnosis after symptom onset is too late for disease modifying therapies, the brain disease in ALD can be effectively halted by undergoing bone marrow transplantation, **but only if affected individuals are diagnosed prior to symptom onset** (6). Therefore, it is vital that affected individuals are diagnosed as early in life as possible to avoid severe suffering and death.

In 2014, following the death of their son, Aidan, owing to complications of ALD, the Seeger family drafted Aidan's Law which led eventually to establishment of ALD newborn screening in New York. In 2016, the then

US Secretary of Health and Human Services signed the recommendation to add ALD to the uniform panel of disorders screened in the newborn period in every state in the United States.

Since that recommendation from HHS in 2016, Kennedy Krieger has, on multiple occasions, petitioned the Maryland Department of Health and the State Newborn Screening Laboratory to establish ALD newborn screening in Maryland. In addition, we have offered to provide technical support for the laboratory testing, if necessary. In the interim, over 20 states in the United States have incorporated newborn screening for ALD into their panels. Unfortunately, Maryland has yet to adopt newborn screening for ALD.

In our opinion, it is extremely important that newborn screening for all condition listed under RUSP, including ALD, is implemented in the State of Maryland to save children affected by these rare disease from suffering and death.

Kennedy Krieger Institute requests a favorable report on Senate Bill 242.

References:

1) Fabie NAV, Pappas KB, Feldman GL. The Current State of Newborn Screening in the United States. Pediatr Clin North Am. 2019 Apr;66(2):369-386
2) Moser AB, Fatemi A. Newborn Screening and Emerging Therapies for X-Linked Adrenoleukodystrophy. JAMA Neurol. 2018 Oct 1;75(10):1175-1176.
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6) Mallack EJ, Turk BR, Yan H, Price C, Demetres M, Moser AB, Becker C, Hollandsworth K, Adang L, Vanderver A, Van Haren K, Ruzhnikov M, Kurtzberg J, Maegawa G, Orchard PJ, Lund TC, Raymond GV, Regelmann M, Orsini JJ, Seeger E, Kemp S, Eichler F, Fatemi A. MRI surveillance of boys with X-linked adrenoleukodystrophy identified by newborn screening: Meta-analysis and consensus guidelines. J Inherit Metab Dis. 2021 May;44(3):728-739..

SB0242_JPayne_Fav_Appendix2.03.2022.pdf
Uploaded by: Jennifer Payne
Position: FAV

February 3, 2022

The Honorable Delores G. Kelley
Chair, Finance Committee
3 East Miller Senate Office Building
11 Bladen Street
Annapolis, MD 21401

The Honorable Brian J. Feldman
Vice Chair, Finance Committee
104 James Senate Office Building
11 Bladen Street
Annapolis, MD 21401

Re: Favorable_SB242 Testimony of Jennifer Payne 2/3/2022 at 1:00 p.m. Finance Committee

Appendix to Testimony of Jennifer Payne:

A dialogue with Carole Weiland, United States Senator Paul Sarbanes, and the State of Maryland illustrating the impact of withdrawing federal funding from Maryland's NBS program circa 1982.

Respectfully submitted,

/s/

Jennifer Weiland Payne, PharmD, MAPP
Independent Advocate, Adult with PKU
7115 John Calvert Ct
Elkridge, MD 21075
443-535-5322
pkupioneer@gmail.com

United States Senate

WASHINGTON, D.C. 20510

October 18, 1982

Mrs. Carole Weiland
15064 Joshua Tree Road
Gaithersburg, Maryland 20878

Dear Mrs. Weiland:

Thank you very much for getting in touch with me to express your concerns regarding Maryland's PKU program. As one who has been a consistent and vigorous supporter in Congress of measures designed to ensure that adequate and affordable health care is available to all Americans, I greatly appreciate your taking the time to share your thoughtful and well-considered comments and personal experience with me. In order to be of assistance to you, I have contacted the appropriate officials of the Maryland Department of Health urging a careful review of this situation. You can be certain that I will be in touch with you as soon as I receive a response.

As you may know, I have strongly opposed the Reagan Administration's budget cuts which have such a detrimental effect on the health and well-being of our citizens. I share your strong commitment to the continuation of effective health care programs for the citizens of our nation, and want to assure you that I will continue to do all I can in the Senate in this most important area.

With best regards,

Sincerely,

Paul Sarbanes

Paul S. Sarbanes
United States Senator

PSS/jhl

United States Senate

WASHINGTON, D.C. 20510

November 18, 1982

Mrs. Carole Weiland
15064 Joshua Tree Road
Gaithersburg, Maryland 20878

Dear Mrs. Weiland:

Following up on our previous correspondence regarding budget cuts in Maryland's PKU program I am enclosing a copy of a letter which I recently received from the Maryland Department of Health and Mental Hygiene in response to your comments. I hope that the information provided will be helpful in answering your questions about this matter.

Please do not hesitate to let me know if there is any way in which I might be of further assistance.

With best regards,

Sincerely,

Paul Sarbanes

Paul S. Sarbanes
United States Senator

PSS/jhl
Enclosure

4 October 1982

Senator Paul Sarbanes
2327 Dirkson Bldg.
Washington, D.C. 20510

Dear Senator Sarbanes:

I wrote to you sometime ago and I am very happy to say that you were most helpful and I hope that will apply in this case also.

I am writing on behalf of two Maryland State Dept. of Health employees (in the Baltimore offices), Mrs. Lib Walker and Mrs. Sue Crosby. They work with children who have a hereditary genetic disease called PHENYLKETONURIA (PKU). PKU children lack an enzyme in their bodies which is responsible for breaking down an amino acid called phenylalanine. Fortunately, this particular disease isn't fatal; it is controlled by a special low protein diet and a regular blood test. If a child isn't diagnosed properly in the very first few weeks of life and put on the diet immediately, the phenylalanine builds up and causes retardation. Unfortunately, there have been cases where the child wasn't diagnosed early and the result is that the child is retarded. Therefore, it is very important that the diet be controlled and monitored and a regular blood test taken (which determines the phenylalanine content in the blood). If it is too high or too low, the diet is adjusted accordingly.

I have two children with this disease (the oldest a girl 9 years and a boy 7 years). Fortunately, due to the immediate response and quick action on the part of the pediatrician and the PKU Program of Maryland, my children were caught early in life and put on the diet immediately. I am very happy to say they have grown normally and do above average work in school. No one would ever know that they have this disease by looking at them; the difference shows when we are at the table for a meal and their plates do not have the same food as the others in the family. Their progress has a great deal to do with the work that Mrs. Crosby and Mrs. Walker do.

Mrs. Walker is a nutritionist and it is to her we go for advice when we have questions regarding the diet. She has been coming to our home for nine years and adjusting the children's diet to keep it under proper control (in accordance with the children's growth). She is always available and many times we have had to call her at her home for advice. She has been very helpful and gone above and beyond the call of duty. When our third child was born she and Mrs. Crosby came to our home the same day we returned from the hospital and took a sample of our child's blood. I told her how anxious I was and that everytime the phone rang I thought it was going to be the doctor telling me that the test was positive. After leaving our home she and Mrs. Crosby took the blood sample to the laboratory in Baltimore and asked the technician if he would do the blood test right away - he complied - and she called immediately to tell us the good news - that our third child didn't have the disease. This saved us days of worry and anxiety. Mrs. Walker has been with the PKU program for 18 years.

Mrs. Crosby is a nurse. She administers the blood tests each month. She' makes the blood test out to be more of a game than a medical test, which is imperative to the children's well being. Because of her kindly manner the children no longer fear the blood tests and she has become such a good friend to them (and to us). She is also one we can turn to for advice and help. She has never in 9 years failed to come to our home to administer the blood test, even at times when she wasn't in the best of health.

We have known both of these ladies for over nine years and they have become more than medical advisors; they have become good friends and more than that, a part of our family. I know this feeling is shared by the numerous other PKU families in this State.

This past week they came for the usual monthly visit but this time they had very distressing news. They informed us that as of December of this year they won't be making home visits anymore. Why? Because of budget cuts. Mrs. Walker (after 18 years of service) will still remain in the program but only available by letter or phone (which is not sufficient in most cases). Mrs. Crosby (after 15 years of service) will no longer be making home visits - she has been cut completely from the PKU program. After all of these years of dedication to PKU children and parents she will have to start in a new field.

Any parent who has a child with any type of disorder, especially one that the child must carry with him throughout life finds it very heartbreaking. The first year of life for a child of this type is very difficult for the parent and is a learning experience and a time for adjustment to the fact that your child isn't like other children. At times like this the parents need a kind face and kind words and compassion; they need someone they can turn to. Mrs. Walker and Mrs. Crosby fulfill this need. I know from personal experience! They have always been there when I needed them; especially in the beginning when it was so important to me. I looked forward to and counted the days until their next visit. I always had so many questions and fears about my child's growth and mental well being. They were always very understanding and compassionate. If their jobs are cut from the program this one on one relationship won't be there and I just can't stress the importance of it, especially for parents who have just been told their child has a hereditary disease. That news is so devastating to a parent and without Mrs. Crosby and Mrs. Walker, they won't have anyone to communicate with on a personal basis; only telephone availability.

By these home visits Mrs. Walker and Mrs. Crosby can see the progress the children are making and know that they have been a significant part of that progress. This is very rewarding. They have personal relationships with all of their "children".

Since I am a PKU parent and know about the PKU program in other states, I can honestly say that Maryland probably has the best program in the country and I am very greatful that our children were born here. The following are some of the reasons why Maryland has such a marvelous PKU program.

(1) Mandatory diagnostic newborn screening (which prevents retardation when diagnosed in the first weeks of life). Some states do not have this.

(2) The home visits (which include advice on nutrition and regular blood tests so that the parents do not have to take the children to a hospital or clinic).

(3) Yearly psychologicals and yearly physical exams for the PKU child.

(4) Yearly meetings of parents and medical personnel to keep us abreast of current PKU developments.

(5) Availability of services to children and parents.

Up to this point all of the above services have been free of charge. I understand that as of 1983 we will still have some of these services but the costs will be to the parents which will run into quite a bit of money.

There are societies and organizations for every kind of disease except PKU; all you have to do is look in the phone book (March of Dimes, Heart and Lung Disease, Cancer Institute, Muscular Dystrophy, and the list goes on.) These organizations are there to help and in most cases treatment and advice are given freely. There is no such organization for PKU. That is why we rely so heavily on Mrs. Walker and Mrs. Crosby. If they are cut from the program who can we turn to? They have even been so gracious as to give out their home phone numbers when there is a medical problem and a doctor can't be reached. I also understand the above services are all endangered; in fact, the entire PKU program is in jeopardy. Please help us keep it alive and active by sufficient funding.

I can't believe that the budget is so tight that Mrs. Walker's and Mrs. Crosby's services must be cut from the program. The purpose of this letter is to keep them in their present capacity. It is because of them that the program is successful. They are a vital part of it and if we lose them our children will be losing so much more.

What is the State doing with the money that they supposedly are saving by eliminating these services? Is it put into another program? What is the dollar difference in keeping them on in their present positions versus the new positions? I am sure it is such a minute amount that the budget could be adjusted accordingly.

I am sorry to have gone on so but I think it is imperative that you know exactly how important these ladies are and the importance of the entire PKU program. I would like a response at your earliest convenience as December isn't far away and that is when their present positions will cease to exist. If at all possible I (and possibly other PKU parents and children) would like an audience with you to discuss this further. This will be affecting quite a number of families in the State and this certainly is worthwhile to do a little research and investigating.

Thanking you in advance,

Mrs. Carole Weiland

Mrs. Carole Weiland
15064 Joshua Tree Rd.
Gaithersburg, Md. 20878

(301) 279-7196

State of Maryland

OFFICE OF THE SECRETARY
DEPARTMENT OF HEALTH AND MENTAL HYGIENE
201 WEST PRESTON STREET • BALTIMORE, MARYLAND 21201 • Area Code 301 • 383-2843

Harry Hughes, Governor

Charles R. Buck, Jr., Sc.D. Secretary

November 9, 1982

The Honorable Paul S. Sarbanes
United States Senator
2327 Dirksen Senate Office Building
Washington, D.C. 20510

Dear Senator Sarbanes:

In response to your letter of October 18, 1982, I have asked the Preventive Medicine Administration for information about the PKU Program.

As I understand from Mrs. Weiland's letter, her primary concern is for the quality of services which will be available to families of children with PKU and other hereditary metabolic disorders discovered by Maryland's Newborn Screening Program when Federal funds are withdrawn. Specifically, she is requesting that Mrs. Lib Walker and Mrs. Sue Crosby continue performing the same jobs which they have carried out with dedication for many years. The Department shares Mrs. Weiland's concerns about quality of services and certainly understands her uncertainties about the upcoming changes in the program.

The intensive home visiting, blood-drawing, and dietary monitoring services described by Mrs. Weiland were part of a detailed study protocol beginning in the early years of Maryland's PKU program. At that time, Maryland was part of a multi-state collaborative study which was necessary to clarify the effects and benefits of dietary treatment. Careful monitoring of blood levels and developmental milestones was available only through the two specially trained professionals under the federally supported PKU demonstration program.

Through Maryland's participation with other states, the collaborative study has now followed sufficient children to carefully verify the safety, efficacy, and success of PKU treatment. As a result, the intensive, home-based services which were necessary for the study will now be restructured. This must be done to accomodate termination of federal PKU funding and to concentrate Mrs. Walker's nutritional expertise on the more vulnerable new infant cases. These infants will receive home services until their condition is stable and their families understand the therapeutic regime.

It is true that Mrs. Walker and Mrs. Crosby will not make home visits to the Weilands anymore. Such parents of older children will now have the option of either learning to draw a small blood specimen themselves (the state will provide an "autolet" to make this procedure as simple as possible) or using their local health department to have the specimen taken at regular intervals.

The results will be forwarded to Mrs. Walker who will be stationed centrally in the State Health Department in Baltimore. She will monitor the blood levels of phenylalanine on all children and make diet changes as appropriate. She will be readily available for consultation by telephone, which is not the case at present. Because of this change, results should be communicated to parents more quickly than in the past. It is the Department's feeling that using Mrs. Walker's experience and expertise in this way is far more efficient than having her spend alot of unproductive time in transit, which also results in her being unavailable to other families needing consultation.

Mrs. Crosby will not be out of the PKU Program entirely. However, through her work in the PKU Program she too has gained a great deal of experience and expertise in dealing with parents of children with newly diagnosed hereditary conditions. The Division of Hereditary Disorders has been given the responsibility of implementing HB 351 - Sentinel Birth Defects Registry. An important part of the program is to provide parents of infants with one of twelve birth defects with information about available services and resources, such as the parents groups mentioned by Mrs. Weiland. Mrs. Crosby is needed to help train the hospital personnel who will then be able to serve distressed parents of a newly diagnosed infant with a birth defect. Again, it is the Department's feeling that particularly in these times of reduced funds, it must use its resources to its best advantage. But for families accustomed to having Mrs. Crosby and Mrs. Walker visit them at home, these changes will be understandably difficult.

Speaking to Mrs. Weiland's "reasons why Maryland has such a marvelous PKU program," we wish to emphasize that we agree with her that the program is among the best, nation-wide. The Department is committed to maintaining the Newborn Screening Program and concomitant follow-up services. These follow-up services will include monitoring of diet, nutrition advice, yearly meetings of parents and medical personnel, and the potential for local health department, home or clinic services.

Local health departments have agreed to expand their role, and the Division of Hereditary Disorders will help train staff so they can counsel families and collect bloods. Whenever possible, families will be seen at the genetics outreach clinic closest to their home. This will save them some inconvenience of travel and some expense. It is expected that, within the next year in compliance with state law, fees will have to be instituted for psychological services. Ways of assuring third party payments for these services are being explored in an effort to reduce the families' out of pocket expenses to the minimum.

Mrs. Weiland has beautifully described the benefits of comprehensive child health care including home health services. It is Preventive Medicine Administration's plan to strengthen such services through local public and private health resources for which additional state or federal funds may be required. These have been identified in the Department's FY 84 plan priorities, but funding is presently uncertain.

I hope that the above information is helpful to you. We will be happy to provide you with further information if necessary.

Sincerely,

Fran Tracy

Fran Tracy, Director
Office of Government Relations

FT:psp

SB0242_JPayne_Fav_Testimony2.03.2022 (1).pdf
Uploaded by: Jennifer Payne
Position: FAV

IN SUPPORT OF SB0242 REQUIRING THE NEWBORN SCREENING PROGRAM IN THE MARYLAND DEPT. OF HEALTH TO REQUIRE TESTING FOR EACH CONDITION LISTED BY THE RECOMMENDED UNIFORM SCREENING PANEL (RUSP)

The Honorable Delores G. Kelley
Chair, Finance Committee
3 East Miller Senate Office Building
11 Bladen Street
Annapolis, MD 21401

The Honorable Brian J. Feldman
Vice Chair, Finance Committee
104 James Senate Office Building
11 Bladen Street
Annapolis, MD 21401

Re: Favorable_SB242 Testimony of Jennifer Payne 2/3/2022 at 1:00 p.m.

Madame Chair, my name is Jennifer Payne. I thank you the opportunity to share my testimony with you today in support of SB242 as an Ambassador for the greater Maryland Rare disease community.

My Connection: Maryland, Dept. of Health and Mental Hygiene, Preventative Medicine Administration It is with deep and heartfelt gratitude for the State of Maryland's Newborn Screening (NBS) Program that I come to you today as an adult beneficiary and *among the first diagnosed in the State with the rare, genetic disorder, phenylketonuria (PKU) in 1973*. Thanks to NBS, timely diagnosis and early treatment spared me a lifetime of institutional care. Because of my age, only I can offer you "living" testimony to the power of prevention - with my historical perspectives on growing up in Maryland's PKU Program[1] (early days) and speak directly to the legislative impact of this powerful public health policy tool that saved my life - and my children's lives.

I ask you to Reaffirm this Commitment: with *passage of SB242* because ***preventing*** devastating illnesses and diseases before they become too serious and too costly to treat benefits all Americans to live well, to stay healthy, and to freely live their lives.

Historical Framing of Testimony PKU: Early detection and early treatment are critical for the clinical management of PKU, the effects (for which the brain and central nervous system are the target organs of damage) stem from a deficiency or inability of the liver to metabolize phenylalanine (phe), a building block of protein found in virtually every food. Given my

[1] See enclosed: NCATS NIH Video Profile. Payne, Jennifer; July 2016, https://youtu.be/btSQQYcxnjk quoted in NIH National Center for Advancing Translational Sciences, Genetic and Rare Diseases Information Center (GARD) website, https://rarediseases.info.nih.gov/diseases/7383/phenylketonuria In this NCATS Video Profile, Jennifer Payne discusses living with phenylketonuria (PKU). Left untreated, PKU results in psychological disorders, neurological deterioration, mental illness and brain damage. Dietary management and access to medical foods are a critical part of PKU care.

medical history and risk with PKU, knowing the accumulation of phe is also teratogenic to offspring of untreated mothers, I can proudly say my children are all healthy and alive thanks to NBS.

Benefits and Impact of the Recommended Uniform Screening Panel (RUSP) Alignment: As I have demonstrated, Maryland's participation in RUSP alignment is critical to saving lives. And, having the necessary resources available to fund the conditions added to RUSP is as equally critical.[2] For not only was our family impacted with dire and direct consequences of this action, screening and ensuring a continuation in these effective health programs will only result in a net gain to taxpayers and cost-savings to the government. Again, I offer you living proof.

Conclusion: Speaking professionally and personally, when it comes to public health - the mission we share is one in the same. It comes down to saving lives. Thank you for the opportunity to speak in support of SB242.

/s/

Jennifer Weiland Payne, PharmD, MAPP
Independent Advocate, Adult with PKU
7115 John Calvert Ct
Elkridge, MD 21075
443-535-5322
pkupioneer@gmail.com

Enclosed:

NCATS NIH Video Profile. Payne, Jennifer; July 2016, https://youtu.be/btSQQYcxnjk quoted in NIH National Center for Advancing Translational Sciences, Genetic and Rare Diseases Information Center (GARD) website, https://rarediseases.info.nih.gov/diseases/7383/phenylketonuria

Under Separate Attachment:

Appendix: A dialogue with Carole Weiland, United States Senator Paul Sarbanes, and the State of Maryland illustrating the impact of withdrawing federal funding from Maryland's NBS program circa 1982.

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[2] See attached Appendix.

SB 242_mgoldstein_fav 2022.pdf
Uploaded by: Mathew Goldstein
Position: FAV

Secular Maryland secularmaryland@tutanota.com

February 03, 2022

SB242 - SUPPORT

Maryland Department of Health – System for Newborn Screening – Requirements

Dear Chair Kelley, Vice-Chair Feldman, and Members of the Finance Committee:

The Recommended Uniform Screening Panel (RUSP) is a list of disorders that the Secretary of the Department of Health and Human Services (HHS) recommends for states to screen as part of their state universal newborn screening (NBS) programs. According to HHS, disorders on the RUSP are chosen based on evidence that supports the potential net benefit of screening, the ability of states to screen for the disorder, and the availability of effective treatments. It is recommended that every newborn be screened for all disorders on the RUSP. The RUSP currently consists of 35 core conditions and 26 secondary conditions. Secondary conditions screening is recommended when the screening of the core conditions indicated a risk for the secondary condition.

This bill requires our state to adopt RUSP. Maryland currently screens newborns for 50 conditions. There are 34 conditions in the RUSP that Maryland currently fails to screen [Carbamoyl Phosphate Synthetase I Deficiency, Congenital Toxoplasmosis, , Gaucher, Glucose-6-Phosphate Dehydrogenase Deficiency, Guanidinoacetate Methyltransferase Deficiency, Human Immunodeficiency Virus, Hyperornithine with Gyrate Deficiency, Hyperornithinemia-Hyperammonemia-Homocitrullinuria Syndrome , Krabbe , Mucopolysaccharidosis Type-II, Niemann-Pick Disease, Nonketotic Hyperglycinemia , Ornithine Transcarbamylase Deficiency, Prolinemia, Pyroglutamic Acidemia, T-cell Related Lymphocyte Deficiencies]. Most states screen for the majority of disorders on the RUSP. Some states also screen for additional disorders.

Secular Maryland advocates for state laws and policies to follow the consensus recommendations of the experts as closely as possibly. Accordingly we favor passage of this bill.

Mathew Goldstein
3838 Early Glow Ln
Bowie, MD

SB0242_FAV_MedChi, MDAAP_MDH - System for Newborn

Uploaded by: Pam Kasemeyer

Position: FAV

MedChi

The Maryland State Medical Society

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Maryland Chapter
American Academy of Pediatrics
Speaking for Maryland Kids!

TO: The Honorable Delores G. Kelley, Chair
Members, Senate Finance Committee
The Honorable Stephen S. Hershey, Jr.

FROM: Pamela Metz Kasemeyer
J. Steven Wise
Danna L. Kauffman
Christine K. Krone

DATE: February 3, 2022

RE: **SUPPORT** – Senate Bill 242 – *Maryland Department of Health – System for Newborn Screening – Requirements*

On behalf of the Maryland State Medical Society and the Maryland Chapter of the American Academy of Pediatrics, we submit this letter of **support** for Senate Bill 242.

Senate Bill 242 proposes to strengthen the effectiveness of the State's system for newborn screening by requiring that the Maryland Department of Health (MDH) include in the system screening for each condition listed in the U.S. Department of Health and Human Services' (DHHS) Recommended Uniform Screening Panel. In addition, beginning on January 1, 2023, MDH will be required to include in the system any core or secondary condition added to the recommended uniform screening panel within 2 years after the addition of the condition to the panel.

MDH administers the system for newborn screening in consultation with the State Advisory Council on Hereditary and Congenital Disorders. One of the charges of administering the system is to determine the screening tests that the Department's public health laboratory is required to perform. Requiring the inclusion of all conditions listed in the recommended uniform screening panel issued by DHHS will ensure that Maryland's system for newborn screening is comprehensive and reflects all recommended conditions. It will also allow the State Advisory Council to focus its efforts on enhancing the effectiveness of the program's objectives – which are to facilitate the rapid identification and treatment of affected children – as opposed to spending unnecessary time reviewing and evaluating conditions for inclusion in the system. We strongly urge a favorable report.

For more information call:
Pamela Metz Kasemeyer
J. Steven Wise
Danna L. Kauffman
Christine K. Krone
410-244-7000

HB242_Testimony_SSullivan_2_3_2022.pdf
Uploaded by: Susan Sullivan
Position: FAV

IN SUPPORT OF SB0242 REQUIRING THE NEWBORN SCREENING PROGRAM IN THE MARYLAND DEPT. OF HEALTH TO REQUIRE TESTING FOR EACH CONDITION LISTED BY THE RECOMMENDED UNIFORM SCREENING PANEL (RUSP)

Senator Delores G. Kelley
Chair, Finance
3 East
Annapolis, MD 21401

Senator Brian J. Feldman
Vice Chair, Finance
3 East
Annapolis, MD 21401

Re: Testimony of Susan Sullivan 2/3 at 1:00 p.m.

Madame Chair, my name is Susan Sullivan, and I am a constituent of District 3b. As a representative for the greater Maryland Rare disease community, I thank you for the opportunity to share my testimony today in support of SB242.

My Connection: I am a mother whose son Daniel passed away in 2011 from metachromatic leukodystropy (MLD), a neurological disease that was curable if detected at birth. I cannot express the pain of knowing that if we had done the right test at the right time, Daniel would be with us today. As science advances, more diseases are becoming like my son's—treatable at birth before irreversible damage is done. Newborn screening for disease like this is our most effective way to ensure that all babies have an equal chance at life.

I ask you to Affirm this Commitment: with passage of SB0242 because Maryland's newborn screening lags behind its potential. Maryland should begin screening for diseases like MLD as soon as they are added to the Recommended Uniform Newborn Screening Panel (RUSP). Maryland's newborn screening efforts trail other states. Adrenoleukodystropy (ALD) was added to the RUSP in 2016 and, while 25 states and DC all test for the disorder, Maryland does not six years later.

Historical Framing of Testimony: Early detection is critical for many diseases, particularly neurological diseases like my son's. Treatment can only halt the damage to the brain and nervous system. If the disease is diagnosed through the symptoms, it is too late for treatment.

Benefits and Impact of the Recommended Uniform Screening Panel (RUSP) Alignment: Since ALD was added to the RUSP in 2016, an estimated 12 babies have been born in Maryland with the severe version of ALD and have gone undiagnosed. If Maryland had begun screening in 2 years as this bill proposes, eight of those children would have been saved.

Conclusion: Thank you for the opportunity to speak in support of SB0242. Maryland is ranked 5th in the nation in healthcare yet we are trailing in newborn screening. I want us to have a state where other families do not have to experience the pain I did. Maryland has been a leader in rare disease research and I you ask that we become a leader in newborn screening once again. Your statehood should not determine your baby's opportunity for a healthy life.

Susan Sullivan
Cure MLD, parent of a deceased child with a treatable disease.
3126A Basford Rd
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SB 242 Amendment.pdf
Uploaded by: Steve Hershey
Position: FWA

SB0242/403924/1

AMENDMENTS PREPARED BY THE DEPT. OF LEGISLATIVE SERVICES

24 JAN 22
15:03:10

BY: Senator Hershey
(To be offered in the Finance Committee)

AMENDMENTS TO SENATE BILL 242
(First Reading File Bill)

AMENDMENT NO. 1

On page 1, in line 5, after "each" insert "core".

AMENDMENT NO. 2

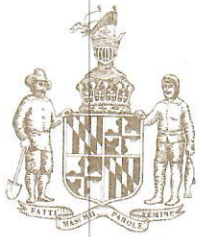
On page 3, in line 2, after "**EACH**" insert "**CORE**"; and in line 6, strike "**OR SECONDARY**".

SB 242 Testimony .pdf
Uploaded by: Steve Hershey
Position: FWA

STEPHEN S. HERSHEY, JR.
Legislative District 36
Caroline, Cecil, Kent, and
Queen Anne's Counties

Finance Committee
Executive Nominations Committee

Legislative Policy Committee



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The Senate of Maryland
ANNAPOLIS, MARYLAND 21401

February 1, 2022

The Honorable Delores Kelley.
Finance Committee
Senate Bill 242 – Maryland Department of Health – System for Newborn Screening - Requirements

Dear Chairwoman and Members of the Committee:

Senate Bill 242 requires the Department of Health Newborn Screening Program to screen for each condition listed in the U.S. Department of Health and Human Services Recommended Uniform Screening Panel.

The Newborn Screening Program is a statewide system for screening all newborn infants in Maryland for certain hereditary and congenital disorders associated with severe problems of health or development. This ensures that all newborns in Maryland receive the most updated, comprehensive medical attention they deserve from the very beginning of their lives.

Currently in Maryland, when babies are born, unless the parents choose to opt-out, they receive the benefit of a newborn screening test as part of the State's universal newborn screening program. It is a special test that is used to test babies for certain serious medical conditions in order to identify babies who have specific disorders before they get sick, helping them to get treatment as soon as possible. It does not test for everything – only 50 conditions including cystic fibrosis, severe immunodeficiency disorders, the presence of abnormal red blood cells or sickle cell disease and more.

Maryland develops its list of disorders from the federal Recommended Uniform Screening Panel that is developed by the US Health and Human Services Department. The recommendations is a list of disorders that HHS recommends for states to screen as part of their newborn screening program; however each state utilizes its own discretion on what to screen for, while others screen for additional disorders.

I have submitted amendments in file to clarify that Senate Bill 242 will only apply to core conditions listed on the panel rather than to both core and secondary conditions. This will align Maryland with six other states that have passed similar legislation.

I request a favorable vote on Senate Bill 242.