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Congress of the United States
House of Representatives
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MEMORANDUM

June 23, 2015

To: **Subcommittee on Health Democratic Members and Staff**

Fr: **Committee on Energy and Commerce Democratic Staff**

Re: **Hearing on "Examining Public Health Legislation: H.R. 2820, H.R. 1344, and H.R. 1462"**

On Thursday, June 25, 2015, at 10:15 am in 2322 Rayburn House Office Building, the Subcommittee on Health of the Committee on Energy and Commerce will hold a hearing entitled "Examining Public Health Legislation: H.R. 2820, H.R. 1344, and H.R. 1462." The subcommittee will review H.R. 2820, the Stem Cell Therapeutic and Research Reauthorization Act of 2015, H.R. 1344, the Early Hearing Detection and Intervention Act of 2015, and H.R. 1462, the Protecting Our Infants Act.

I. BONE MARROW AND STEM CELL RESEARCH AND RELATED DONOR AND TRANSPLANT REGISTRY

A. Background

Each year, nearly 20,000 patients in the United States need a bone marrow, peripheral blood stem cell, or cord blood stem cell transplant (bone marrow transplant).[1] A bone marrow transplant is a life-saving treatment for individuals with blood cancers, such as leukemia, or metabolic or immune system disorder, such as sickle cell anemia.[2] A bone marrow transplant

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[1] Health Resources and Services Administration, *Blood Cell Transplant General Frequently Asked Questions* (online at http://bloodcell.transplant.hrsa.gov/about/general_faqs/index.html).

[2] U.S. National Library of Medicine Medline Plus, *Bone marrow transplant* (online at www.nlm.nih.gov/medlineplus/ency/article/003009.htm).

replaces damaged or destroyed bone marrow with healthy hematopoietic or blood-forming stem cells.[3]

Bone marrow transplants require patients and donors to have closely matching human leukocyte antigen (or HLA), an inherited protein found on most human cells.[4] Generally, a patient is more likely to find a match from a donor from the same racial or ethnic background.[5] Studies show, however, that a cord blood unit does not have to be as closely matched as bone marrow to a patient.[6] While some patients can find a match among their family, approximately 70 percent of patients will need a transplant from an unrelated donor.[7]

B. National Bone Marrow Donor Registry – *Be the Match Registry*

Congress established the National Bone Marrow Donor Registry in 1987, to match unrelated donors to patients in need of a bone marrow transplant.[8] In 2005, the National Bone Marrow Donor Registry was replaced by the C.W. Bill Young Transplantation Program which includes a National Registry for adult donors of bone marrow, peripheral blood adult stem cells, and umbilical cord blood units. This National Registry, also called the *Be the Match Registry*, is administered by the National Marrow Donor Program (NMDP). As of 2014, the *Be the Match Registry* had nearly 12.5 million donors registered and more than 140,000 cord blood units.

A patient's likelihood of matching a donor on the National Registry ranges from 76 percent to 97 percent depending on race and ethnicity.[9] Racial and ethnic minorities are least likely to find a match on the Registry. While Caucasian Americans have a 97 percent chance of finding a match on the Registry, Asian Americans have an 84 percent chance of finding a match, Hispanic Americans an 83 percent chance, and African Americans a 76 percent chance.[10]

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[3] *Id.*

[4] Be the Match, *HLA Matching* (online at https://bethematch.org/for-patients-and-families/finding-a-donor/hla-matching/).

[5] Be the Match, *Why race and ethnicity matter* (online at https://bethematch.org/Transplant-Basics/Matching-patients-with-donors/Why-race-and-ethnicity-matter/).

[6] Be the Match, *Cord blood and transplants* (online at https://bethematch.org/transplant-basics/cord-blood-and-transplants/).

[7] Health Resources and Services Administration, *Blood Cell Transplant: The Need for More Cord Blood Donations* (online at http://bloodcell.transplant.hrsa.gov/cord/the_need/index.html).

[8] Health Resources and Services Administration, *Blood Cell Transplant: Legislation & Contracts* (online at http://bloodcell.transplant.hrsa.gov/about/legislation_and_contracts/).

[9] *Supra* note 5.

[10] *Id.*

The National Registry has undertaken several efforts to increase the match rate for racial and ethnic minorities. For example, the National Registry has undertaken an effort to increase the diversity of donors of cord blood units. Because cord blood transplants require less of a match between donor and patient, increasing the diversity of cord blood units on the National Registry makes it possible for more patients to receive a transplant. In 2014, 29 percent of cord blood transplants were for minority patients.[11]

C. How the Registry Is Organized and Operates

The Stem Cell Therapeutic and Research Act of 2005 created the C.W. Bill Young Transplantation Program, which replaced the National Bone Marrow Donor Registry and expanded upon the Federal government's commitment to patients needing a bone marrow or umbilical cord blood transplant. The Transplantation Program has four components: (1) the Office of Patient Advocacy (OPA) and Single Point of Access (SPA); (2) the Bone Marrow Coordinating Center; (3) the Cord Blood Coordinating Center; and (4) the Stem Cell Therapeutic Outcomes Database. The Transplantation Program is operated through contracts between the Health Resources and Services Administration (HRSA) and private entities. The NMDP holds three contracts: the OPA/SPA, Bone Marrow Coordinating Center, and Cord Blood Coordinating Center.[12] The Center for International Blood & Marrow Transplant Research (CIBMTR), a collaboration of the NMDP and Medical College of Wisconsin, holds the contract for the Stem Cell Therapeutic Outcomes Database.[13]

The OPA provides support and information to patients and their families and the SPA is the sole electronic system that is used to search the registry. The Bone Marrow Coordinating Center matches donors with patients, recruits potential marrow donors, and helps facilitate the donation process. The Cord Blood Coordinating Center helps educate expectant parents about donating umbilical cord blood, recruit donors, and facilitate the donation process. The Stem Cell Therapeutic Outcomes Database collects and uses data about transplantation for research purposes.

D. The National Cord Blood Inventory (NCBI)

The Stem Cell Therapeutic and Research Act of 2005 also created the National Cord Blood Inventory. The NCBI provides grants to public cord blood banks to assist them in collecting donated units that are then registered on the National Registry. The goal of the NCBI is to collect at least 150,000 new cord blood units to treat patients. The NCBI also provides cord blood units for research.

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[11] *Supra* note 6.

[12] *Supra* note 8.

[13] *Id.*

E. H.R. 2820, The Stem Cell Therapeutic and Research Reauthorization Act

Representative Matsui (D-CA) and Representative C. Smith (R-NJ) introduced H.R. 2820 on June 18, 2015. This bill would reauthorize the C.W. Bill Young Transplantation Program at $30 million for each of fiscal years 2016 through 2020. The bill would reauthorize the NCBI at $23 million for each of fiscal years 2016 through 2020.

Current authorization for the C.W. Bill Young Transplantation Program and the NCBI will expire in September 2015.

II. EARLY HEARING LOSS DETECTON AND INTERVENTION

A. Background

Hearing loss is a common birth defect in the United States. An estimated 2 to 3 out of every 1000 infants in the United States are born with hearing loss each year.[14] The cause of hearing loss for many babies is not known.

Hearing loss in children can harm a child's normal development. It can affect the ability of children to develop speech, language, and social skills.[15] When a child's hearing loss is identified early, the child's family and doctors can seek services he or she needs at an early age, increasing the likelihood of mitigating or preventing those delays. Early intervention and treatment services can prevent or limit the developmental delays caused by hearing loss.[16] Babies should have a hearing screening before they are 1 month of age.[17] Babies or young children diagnosed as deaf or hard of hearing require treatment plans designed to meet their unique needs. Some treatment options include learning sign language, use of hearing aids and cochlear implants, and medicine and surgery to correct some types of hearing loss.[18]

The Early Hearing Detection and Intervention (EHDI) Program provides grants and technical assistance to states to develop newborn and infant hearing screening and early intervention programs. Statewide EHDI programs include screening (the initial test of infants for hearing loss), diagnostic evaluations (to confirm hearing loss), and early intervention (including medical services, early intervention programs, and family support) to enhance language,

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[14] National Institute on Deafness and Other Communication Disorders, *Quick Statistics* (online at http://www.nidcd.nih.gov/health/statistics/pages/quick.aspx).

[15] Centers for Disease Control and Prevention, *Hearing Loss in Children* (online at http://www.cdc.gov/ncbddd/hearingloss/facts.html).

[16] *Id.*

[17] *Id.*

[18] *Id.*

communication and cognitive and social skill development. When the EHDI program was first implemented, 44 percent of newborns were screened for hearing loss.

Today, approximately 97 percent of newborns in the United States are screened for hearing loss.[19] As a result, each year there are thousands of infants with hearing loss who benefit from early identification. Despite the success of the EHDI Program, much work remains to be done. Many infants and young children do not receive early intervention services that can prevent or eliminate the developmental delays that result from hearing loss. Many infants and young children are lost to follow-up and have limited access to early intervention programs.

B. Authorization of Early Hearing Detection and Intervention Program

Congress initially provided grant funding to states to develop newborn and infant screening and intervention programs through the Consolidated Appropriations Act of 2000. Subsequently, Congress authorized the EHDI Program through the Children's Health Act of 2000. The law requires HRSA to award funding to states to develop statewide newborn and infant hearing screening, evaluation, and intervention programs and systems. In addition to promoting screening and intervention, those programs and systems facilitate data collection and public health surveillance. The law also required the Centers for Disease Control and Prevention (CDC) to award funding to provide technical assistance and data management to state EHDI programs and to conduct applied research. The law also required the National Institutes of Health (NIH) to continue research related to early hearing detection and intervention.

C. H.R. 1344, The Early Hearing Detection and Intervention Act of 2015

Representatives Lois Capps (D-CA) and Brett Guthrie (R-KY) introduced H.R. 1344, the Early Hearing Detection and Intervention Act, on March 10, 2015. This legislation would reauthorize the EHDI Program from 2017 through 2022. Current authorization of the EHDI Program will expire in September 2015.

Under the legislation, the grant program for state EHDI programs would be reauthorized at $17.8 million for each of fiscal years 2017 through 2022. It would also reauthorize the program for technical assistance, data management, and applied research at $10.8 million for each of fiscal years 2017 through 2022. In addition, this legislation would expand the focus of state EHDI programs to include young children and strengthen their focus on preventing infants and young children from being loss to follow-up.

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[19] *Id.*

III. NEONATAL ABSTINENCE SYNDROME (NAS) -- OPIOID AND HEROIN USE

A. Background

The CDC has declared prescription drug abuse in the United States an epidemic and has found drug overdose to be the leading cause of injury death in the United States.[20] Between 2000 and 2010, there was a fourfold increase in the use of prescribed opioids for the treatment of pain.[21] This increase in opioid prescriptions has been mirrored by a fourfold increase in opioid-related overdose deaths: between 1999 and 2010, the death rate from prescription opioids more than quadrupled.[22]

Opioid painkillers appear to be widely prescribed among reproductive age women. According to the CDC, more than a third of reproductive age women enrolled in Medicaid, and more than a quarter of those with private insurance, filled a prescription for an opioid pain medication each year during 2008-2012.[23] One study in the journal *Pediatrics* found that of 112,029 pregnant women enrolled in the Tennessee Medicaid Program, 28 percent were prescribed an opioid pain reliever during pregnancy.

The rate of heroin overdoses has also increased dramatically in recent years. In 2010, approximately 3,000 drug-poisoning deaths were connected to heroin. In 2013, the number jumped to a total of 8,000 overdose deaths.[24] Some evidence suggests that individuals switch to heroin when prescription drugs are harder to obtain, due to cost or limited supply. For example, a 2012 *New England Journal of Medicine* study found that heroin use nearly doubled after the introduction of an abuse-deterrent formulation of OxyContin.[25]

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[20] Office of National Drug Control Policy, *Prescription Drug Abuse* (online at www.whitehouse.gov/ondcp/prescription-drug-abuse).

[21] Substance Abuse and Mental Health Services Administration, *SAMHSA Opioid Overdose Prevention Toolkit* (2014).

[22] Nora D. Volkow et al., *Medication-Assisted Therapies – Tackling the Opioid Overdose Epidemic,* New England Journal of Medicine (May 29, 2014).

[23] Centers for Disease Control and Prevention, *Opioid Prescription Claims Among Women of Reproductive Age—United States, 2008-2012*, Morbidity and Mortality Weekly Report (January 23, 2015).

[24] National Institute on Drug Abuse, *Overdose Death Rates* (online at www.drugabuse.gov/related-topics/trends-statistics/overdose-death-rates).

[25] Thomas J. Cicero, Matthew S. Ellis, and Hilary L. Surrat, *Effect of Abuse-Deterrent Formulation of OxyContin,* New England Journal of Medicine, (July 2012).

The incidence of neonatal abstinence syndrome (NAS) has also skyrocketed. NAS is a group of problems that occur in newborns who were exposed to opiates while in their mother's womb.[26] Symptoms often begin within 1 to 3 days of birth but can take up to a week to appear.[27] Some symptoms of NAS include diarrhea, excessive crying or high-pitched crying, fever, irritability, tremors, or seizures.[28]

B. Recent Research and Findings

According to a recent study in the *New England Journal of Medicine*, from 2004 through 2013, the incidence of NAS quadrupled.[29] NAS is also associated with preterm births, low birthweight, and complications such as respiratory distress and seizures.[30] Newborns with NAS require specialized care—often in a neonatal intensive care unit—which results in longer hospital stays and increased costs. From 2004-2013, the percentage of neonatal intensive care unit (NICU) days nationwide associated with NAS increased from 0.6 percent to 4.0 percent.[31] In 2012, aggregate hospital charges for NAS were an estimated $1.5 billion, with 81 percent attributed to state Medicaid programs.[32]

The *Pediatrics* study found that prescription opioid use in pregnancy is strongly associated with neonatal complications. The study found that 65 percent of infants with NAS were exposed to opioid painkillers that were legally prescribed to their mothers during pregnancy. Of those women who participated in the study and had been prescribed opioids, nearly all of them received immediate release medications (96.2 percent). A small percentage

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[26] U.S. National Library of Medicine Medline Plus, *Neonatal abstinence syndrome* (online at http://www.nlm.nih.gov/medlineplus/ency/article/007313.htm).

[27] *Id.*

[28] *Id.*

[29] Veeral N. Tolia et al., *Increasing Incidence of Neonatal Abstinence Syndrome in U.S. Neonatal ICUs*, The New England Journal of Medicine (Apr. 26, 2015)(online at www.nejm.org/doi/full/10.1056/NEJMsa1500439?query=featured_home).

[30] Stephen W. Patrick, *Neonatal Abstinence Syndrome and Associated Health Care Expenditures United States, 2000-2009*, JAMA Internal Medicine (May 9, 2012).

[31] Veeral N. Tolia et al., *Increasing Incidence of Neonatal Abstinence Syndrome in U.S. Neonatal ICUs*, The New England Journal of Medicine (Apr. 26, 2015)(online at www.nejm.org/doi/full/10.1056/NEJMsa1500439?query=featured_home).

[32] S.W. Patrick et al., *Increasing Incidence and Geographic Distribution of Neonatal Abstinence Syndrome: United States 2009 to 2012*, Journal of Perinatology (Apr. 30, 2015).

(0.6 percent) received long-acting preparations, and 2.7 percent received maintenance treatment for opioid use disorders.[33]

A recent U.S. Government Accountability Office (GAO) report in February entitled "Prenatal Drug Use and Newborn Health: Federal Efforts Need Better Planning and Coordination," identified a number of different research gaps in the treatment of opioid use during pregnancy and the treatment of infants with NAS. Specific research gaps relate to best practices for treating pregnant women with opioid use disorders, the long-term effects of prenatal drug exposure, and best practices in the screening, diagnosis, and treatment of NAS.[34]

Agency officials and other experts surveyed also identified a number of gaps in treatment programs and provider education, such as a lack of coordinated provision of obstetric care and addiction treatment, and a lack of provider training in how to recognize substance abuse in patients. Agency officials cited several reasons for these program gaps: 1) stigma and criminalization of pregnant women who use drugs, resulting in pregnant women not getting substance abuse treatment or prenatal care out of fears of criminal charges; 2) gaps in current research on best practices; 3) lack of awareness of NAS as a public health problem; 4) and a lack of funding for programs to address prenatal opioid use or NAS.

Hospitals and health care professionals have begun to identify best practices for treating NAS, but there is no comprehensive national resource for coordinating or implementing these practices.

Federal agencies obligated $21.6 million for 18 research projects related to NAS from 2008-2014.

C. H.R. 1462, the Protecting Our Infants Act of 2015

Representatives Katherine Clark (D-MA) and Steve Stivers (R-OH) introduced H.R. 1462, the Protecting Our Infants Act, on March 19, 2015. The bill would require the Secretary of the Department of Health and Human Services (HHS), acting through the Agency for Healthcare Research and Quality (AHRQ), to develop recommendations for preventing and treating prenatal opioid abuse and NAS.

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[33] Women on maintenance medications for opioid abuse disorders have different risks and different benefits for using opioids. For women with heroin dependency, for instance, maintenance medications have been shown to improve both maternal and neonatal outcomes, including improved fetal growth and decreased preterm births. It is not recommended that pregnant women on opioid maintenance medications, i.e. methadone and buprenorphine, be tapered off of these medications during pregnancy, because withdrawal is associated with high relapse rates. *See* American College of Obstetricians and Gynecologists, *Committee Opinion: Opioid Abuse, Dependence, and Addiction in Pregnancy* (May 2012).

[34] Government Accountability Office, *Prenatal Drug Use and Newborn Health: Federal Efforts Need Better Planning and Coordination* (Feb. 2015) (GAO-15-203).

The bill would also require HHS to develop a strategy to coordinate its efforts and to close gaps in research and programming, as recommended by GAO. Finally, it would require the CDC to assist states in collecting public health data relative to prenatal opioid abuse and NAS.

IV. WITNESSES

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