

December 19, 2019

The Honorable Alex M. Azar, II  
Secretary  
US Department of Health and Human Services  
200 Independence Avenue, SW  
Washington, DC 20201

Dear Secretary Azar:

The 61 undersigned organizations committed to the nation's newborn screening system urge you in the strongest possible terms to ensure that the Advisory Committee on Heritable Disorders in Newborns and Children (ACHDNC or the Committee) resumes its scheduled meetings while Congress continues efforts to reauthorize the Newborn Screening Saves Lives Act. Failure to renew the Committee's activities will significantly disrupt the progress made in newborn screening, hindering a well-established and scientific process for evaluating newborn screening and providing guidance to States.

The ongoing lapse in the Committee's activities, along with its related workgroups, is preventing it from advancing current work and commencing new business. The Committee is currently in the midst of an important update to the nomination process for the Recommended Uniform Screening Panel (RUSP) to ensure that its requirements and considerations are aligned with current science in therapeutics, laboratory technologies and public health. Further, patient advocates preparing to submit RUSP nominations for a number of promising conditions in 2020 are left unsupported.

The ACHDNC was chartered in February 2003 to advise the Secretary regarding the most appropriate application of technologies, policies, guidelines, and standards for effectively reducing morbidity and mortality in newborns. In 2008 and again in 2014, Congress renewed the ACHDNC under the *Newborn Screening Saves Lives Act of 2007* (P.L. 110-204) and the *Newborn Screening Save Lives Reauthorization Act of 2014* (P. L. 113-240), respectively. Today, the Committee provides systematic evidence-based and peer-reviewed recommendations for adding conditions to the RUSP, which now serves as the model for state newborn screening programs. Newborn screening is a dynamic field, constantly evolving and improving. As such, the Committee is continuously reviewing the RUSP and providing the Secretary with recommendations to improve newborn screening programs and to ensure newborns and children with heritable disorders are receiving the highest quality of care.

Prior to the creation of the ACHDNC, the number and quality of newborn screens varied from state to state. In 2002, some states were screening for only four conditions, while others were screening for up to 36. Today, all 50 states, the District of Columbia, and Puerto Rico require screening for at least 30 of the 35 treatable core conditions on the RUSP. This widespread expansion and standardization of newborn screening has led to life-saving treatments and

interventions for the more than 12,000 newborns diagnosed with genetic and endocrine conditions each year.

Given the essential role that the ACHDNC plays in our nation's newborn screening system, we urge you to take immediate action to extend the term of the ACHDNC until reauthorization occurs. The Department of Health and Human Services has ample authority under the Public Health Service Act (42 USC 217a) to extend the Committee's charter prior to reauthorization, as was done when the statutory authority lapsed in 2013. This authority is reinforced by the *Newborn Screening Saves Lives Reauthorization Act of 2014*, which explicitly cites your authority to deem the ACHDNC a Secretarial advisory committee. Furthermore, Congress and numerous advocacy organizations are actively pursuing reauthorization of this important advisory committee. Given your authority and Congress' commitment to passing a reauthorization bill, we urge you to immediately extend the committee charter.

Thank you for your attention to this important matter. If we can provide further information or answer any questions, please contact Rebecca Abbott, Deputy Director of Federal Affairs for Public Health at the March of Dimes, at [rabbott@marchofdimes.org](mailto:rabbott@marchofdimes.org) or 202.292.2750.

Sincerely,

1,000 Days  
AACC  
Aidan Jack Seeger Foundation  
American Academy of Allergy, Asthma & Immunology  
American Academy of Pediatrics  
American Association on Health and Disability  
American Behcet's Disease Association (ABDA)  
American College of Medical Genetics and Genomics  
American Organization for Nursing Leadership  
American Public Health Association  
American Society of Gene and Cell Therapy  
American Society of Hematology  
Amicus Therapeutics  
Association of Maternal & Child Health Programs  
Association of Public Health Laboratories  
Association of State and Territorial Health Officials  
Association of University Centers on Disabilities (AUCD)  
BioMarin Pharmaceutical  
Champions Action Network LLC  
Children's National Hospital  
Children's National Rare Disease Institute  
Coalition for Disability Health Equity  
Colorado School Medicaid Consortium  
Cure SMA

CureDuchenne  
Cystic Fibrosis Foundation  
EveryLife Foundation for Rare Diseases  
Fabry Support & Information Group  
Family Voices  
Family Voices Indiana  
Genetic Alliance  
HCU Network America  
Hydrocephalus Association  
Immune Deficiency Foundation  
Infant Nutrition Council of America  
Jeffrey Modell Foundation  
KIF1A  
March of Dimes  
MLD Foundation  
MSUD Family Support Group  
Muscular Dystrophy Association  
National Birth Defects Prevention Network  
National CMV Foundation, Inc.  
National Organization for Rare Disorders  
Newborn Foundation  
Orchard Therapeutics  
Parent Project Muscular Dystrophy  
Pathways for Rare and Orphan Studies  
PerkinElmer  
Phelan McDermid Syndrome  
PTC Therapeutics  
Regnxbio  
Retrophin, Inc  
Sanofi Genzyme  
Sarepta Therapeutics  
Sister Friend Up  
Society for Maternal-Fetal Medicine  
Somerset County Department of Health/Regional Chronic Disease & Cancer Coalition -  
Morris & Somerset Counties, New Jersey  
T.E.A.M. 4 Travis (Together Ending Asplenia Mortality)  
Ultragenyx Pharmaceutical  
West Valley Neighborhoods Coalition

cc:

Administrator Thomas J. Engels, US Health Resources and Services Administration  
Associate Administrator Michael D. Warren, Maternal and Child Health Bureau, US Health  
Resources and Services Administration