September 30, 2016

The Honorable Bill Cassidy
United States Senate
703 Hart Senate Office Building
Washington, D.C. 20510

The Honorable Bob Casey
United States Senate
339 Russell Senate Office Building
Washington, D.C. 20510

Dear Senators Cassidy and Casey:

The National Coalition for Infant Health (NCfIH) writes to extend our support for your bill, the Promoting Life Saving New Therapies for Neonates Act of 2015 (S. 2041). We applaud you for introducing this important legislation that creates new incentives for the development of essential therapies for neonates. Persisting threats to newborn health have not been met by innovation in the industry. Advancements in neonatal treatments have stalled due to the difficulty of performing clinical trials, the economic challenge of driving investment in treatments for this narrow category of patients, and the cost endemic to the regulatory process for approving new drugs and medical devices. With your help, we may be able to reverse the *status quo*. An improved environment will inspire critical reform through investment and restructuring to allow the development of groundbreaking therapies for newborns.

As a collaborative of professional, clinical, community health, and family support organizations focused on improving the lives of premature infants and their families, we appreciate your efforts to bring the issues that plague the neonatal health industry to light. The National Coalition for Infant Health (NCfIH) prides ourselves on educating and advocating for improved access to and the development of new therapies for premature infants and their families. Today, we proudly bring together passionate preemie parents, multidisciplinary leaders of national and community-based movements, nurses, social workers, neonatologists, pediatricians, educators and more – all working together to focus on equal access to a continuum of quality care, patient safety, education, and policy to meet the needs of this important and vulnerable population.

Prematurity is among the most significant under-recognized problem facing our healthcare system today. Every year, our hospitals see hundreds of thousands of infants enter our neonatal intensive care units (NICU) with health challenges related to prematurity; nationwide, approximately 500,000 newborns are admitted into NICUs every year. Despite our best efforts, prematurity continues to be the leading cause of neonatal mortality within the first month of life and the second leading cause of infant mortality within the first year of life. And of those who survive, one in five face health problems that persist into adulthood, including cerebral palsy, intellectual disabilities, chronic lung disease, and deafness. [1]

In the critical days and weeks after birth, providers need innovative treatments that are specifically tailored to this fragile state of development. Neonates have their own unique pathology and physiology

[1] Davis JM et al. The Need for Rigorous Evidence on Medication Use in Preterm Infants: Is It Time for a Neonatal Rule? JAMA (2012); 308(14): 1435-1436.

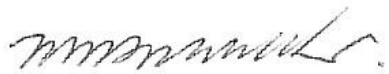
that makes them distinct from older children and adults. While most treatments developed for adults are designed to cure mature humans of illness, infants' organs have not yet had the time to mature, and thus many of the treatments utilized for adults prove toxic. Moreover, unlike treatments used in other fields of medicine, most medications administered to preterm infants lack convincing data to support their safety and efficacy. More than 90 percent of the medications in regular use in this population are not approved by the Food and Drug Administration (FDA) for the prescribed indication. Performing clinical trials to determine pharmaceutical safety and efficacy in neonates is fraught with challenges – such as high population variability, low participation numbers in clinical trials, and increasingly expensive trial costs.

While previous legislative efforts to encourage pediatric drug development have been successful, their efficacy in driving new treatments for neonates has been underwhelming. Since the enactment of the Food and Drug Administration Modernization Act (FDAMA) in 1997, there has been an increase in pediatric studies submitted to the FDA, resulting in over 500 product labeling changes.[2] But despite this promising data, there have been far too few new FDA-approved medications with a specific indication for newborns.[3]

Your legislation, the Promoting Life-Saving New Therapies for Neonates Act, marks a promising step forward in creating appropriate incentives to bring new treatments forward to benefit this vulnerable population. By creating a transferrable "exclusivity voucher" for drug manufacturers who successfully develop products in critical areas of neonatal health, your legislation will create an opportunity for vital studies and research that would otherwise be too costly to perform. Further, the bill is thoughtfully drafted to target the most critical needs in the neonatal population – fostering cooperation among multiple stakeholders, such as the National Institutes of Health (NIH), the Critical Path Institute, and patient advocacy groups to identify priority conditions for research.

On behalf of neonatal support organizations around the nation, we are committed to providing quality healthcare for premature infants. Thank you for spearheading this vital legislative initiative. We hope that your colleagues from both sides of the aisle rally to this cause in the spirit of bipartisanship.

Sincerely,



Mitchell Goldstein, MD
Medical Director, National Coalition for Infant Health

CC: U.S. Senators

[2] Laughon M et al. Drug Labeling and Exposure in Neonates. JAMA Pediatr (2014); 168(2):130-136.
[3] Stiers J & Ward R. Newborns, One of the Last Therapeutic Orphans to Be Adopted. JAMA Pediatrics (2014); 168(2): 106-108.