



April 6, 2016

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

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

Dear Senators Cassidy and Casey:

We are writing to express our support for your legislation, the Promoting Life Saving New Therapies for Neonates Act of 2015 (S. 2041), to create important incentives for the development of critical new therapies for neonates. Despite the serious challenges that can confront newborns, innovation in neonatal health has 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 costs endemic to our regulatory approval process for new drugs. Your innovative proposal will help to reverse this untenable *status quo* and spur the necessary investment and restructuring required to bring groundbreaking cures for newborns into the 21st Century.

Prematurity is among the most significant and under-recognized problems 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 200,000 newborns are admitted into NICUs every year. Despite our best efforts, prematurity continues to be the leading cause of newborn mortality and the second leading cause of infant mortality. And among those who survive, one in five face health problems that persist for life such as cerebral palsy, intellectual disabilities, chronic lung disease, and deafness.¹

In the critical days and weeks after birth, we need innovative treatments that are specifically tailored for patients' fragile state of development. While most treatments developed for adults are designed to cure mature humans of illness, infants' organs have not yet had the time to strengthen, 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, with more than 90 percent not approved by the Food and Drug Administration (FDA) for the prescribed indication.

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.

While previous legislative efforts to encourage pediatric drug development have been broadly successful, their efficacy in driving new treatments for neonates has been underwhelming. Federal legislation implementing a combination of incentives and obligations has led to an increase in pediatric studies submitted to the FDA, resulting in over 500 product labeling changes since the enactment of the Food and Drug Administration Modernization Act (FDAMA) in 1997.² But despite this promising data, there has not been a single FDA-approved medication released for newborns since 1999.³

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 measure will create an opportunity for vital studies and research that would otherwise be too costly to perform. Further, the legislation is thoughtfully drafted to target only 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 the nation’s leading children’s hospitals, whose doctors and nurses make the protection of newborn lives their personal and professional calling, we thank you for introducing this important legislation and encourage your colleagues to join this worthy effort.

Sincerely,

Cheyenne Children’s Clinic (WY)
Children’s Healthcare of Atlanta
Children’s Hospital of Philadelphia
Children’s Hospital of Pittsburgh
Children’s Mercy Hospital and Clinics (MO)
Nationwide Children’s Hospital (OH)

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.