National Association of Neonatal Nurses

S. 2041 Promoting Life-Saving New Therapies for Neonates Act

The Issue

Each year, approximately 200,000 newborns in the United States require admission to a neonatal intensive care unit for treatment of prematurity, costing more than $26 billion per year. Prematurity is the leading cause of newborn mortality and the second leading cause of infant mortality. Of the survivors, one in five faces health problems that persist for life such as intellectual disabilities, cerebral palsy, chronic lung disease, and deafness.

The Research

Federal legislation implementing a combination of pediatric 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. Despite these policy efforts, few drug labeling changes have included neonates (premature and full term infants up to 28 days of age); and the last new drug for neonates was approved in 1999.

Current incentives have not been sufficient to stimulate innovation for the neonatal population due to numerous challenges. For example, very few patients are available for study at any given time so it is difficult and costly to get sufficient numbers. The patients are also very sick so it is difficult to get informed consent from caregivers. Furthermore, many conditions for which drug therapy is needed in the neonatal population do not occur in older children and adults. And even if a drug has been shown to be safe and effective in older populations, the data cannot be extrapolated to neonates because of their distinctive developmental status and immature physiology.

Current Legislation

Senator Casey and Senator Cassidy introduced S. 2041, “Promoting Life-Saving New Therapies for Neonates Act of 2015,” which will act as a stimulus for neonatal drug development. The legislation will:

- Close the treatment gap by stimulating the development of drugs for neonates, a challenging and neglected pediatric population.
- Ensure that new neonatal drugs address the neonatal population’s unique needs and priorities by working with stakeholders like the NIH, the Critical Path Institute, and patient advocacy groups.
- Create a new model, a voucher program, to incentivize neonatal product development by drug sponsors.

Recommendations

Overall, this legislation will spur innovation for new neonatal drug therapies, improving outcomes for devastating neonatal conditions and giving our most vulnerable children the chance to become healthy, productive citizens.

References

1. http://www.newbornhealth.org/nann.html
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3. Food and Drug Administration Modernization Act of 1997, 21 U.S.C. §§ 101-501 (1997).
4. McCune SK, Mulugeta YA. Regulatory science needs for neonates: a call for neonatal community collaboration and innovation. Front Pediatr. 2014;2.
5. Offringa M, Davis JM, et.al. Applying regulatory science to develop safe and effective medicines for neonates: Report of the US Food and Drug Administration first annual neonatal scientific workshop. Therapeutic Innovation & Regulatory Science, DOI: 10.1177/2168479015597730 Sept, 2015
6. First Annual Neonatal Scientific Workshop – Roadmap for Applying Regulatory Science to Neonates**.** October 28-29, 2014 Workshop at the FDA (http://www.fda.gov/Drugs/NewsEvents/ucm410863.htm)