

The Coalition for Pediatric Medical Research



Akron Children's Hospital
Ann & Robert H. Lurie Children's Hospital of Chicago
Baystate Children's Hospital
Boston Children's Hospital
Children's of Alabama
Children's Healthcare of Atlanta
Children's Hospital Colorado
Children's Hospital Los Angeles
Children's Hospital of Pittsburgh of UPMC
Children's Hospital of Wisconsin
Children's Mercy Hospital
Children's National Medical Center

Cincinnati Children's Hospital Medical Center
LeBonheur Children's Hospital
Nationwide Children's Hospital
Nemours Children's Health System
Penn State Hershey Children's Hospital
Primary Children's Hospital
Rainbow Babies & Children's Hospital
Seattle Children's
St. Jude Children's Research Hospital
St. Louis Children's Hospital
The Children's Hospital of Philadelphia
University of Iowa Children's Hospital

April 18, 2016

The Honorable Lamar Alexander
Chairman
Committee on Health, Education
Labor and Pensions
428 Dirksen Senate Office Building
Washington, DC 20510

The Honorable Patty Murray
Ranking Member
Committee on Health, Education,
Labor and Pensions
428 Dirksen Senate Office Building
Washington, DC 20510

Dear Chairman Alexander and Ranking Member Murray:

On behalf of the Coalition for Pediatric Medical Research, which consists of about two dozen of our nation's leading children's hospitals and research organizations including LeBonheur Children's Hospital, Seattle Children's and St. Jude Children's Research Hospital, we offer our strong support for including the **Kids' Innovative Drugs Act** within the Senate Innovation bill.

The KIDS Act will help advance development and delivery of novel cancer therapies to our children by ensuring more potential therapies for children are studied in this special population. Congress has long recognized that children are not simply small adults. The Pediatric Research Equity Act (PREA) and the Best Pharmaceuticals for Children Act (BPCA) recognize this reality and have sought to drive additional drug study in pediatric populations.

While PREA and BPCA have both had a positive impact, under current law a sizeable gap exists within PREA. Specifically, PREA only requires pediatric studies to be done on drugs that will be indicated for use in children. But because indications for cancer drugs focus on the type of cancer by pathology or organ system, such as breast or prostate cancer, most novel cancer drugs are not studied in kids even though their underlying molecular target does manifest in children. For example, while lung cancers are rarely seen in children, an underlying genetic mutation responsible for certain forms of lung cancer is also a driver of neuroblastomas. But if a drug sponsor simply pursues an indication for lung cancer, under PREA today they are not required to do a pediatric study. The KIDS Act seeks to close this gap by amending PREA to require pediatric studies for any oncology drug or biologic where the molecular target exists in a pediatric population. It will also limit similar exemptions for rare disease oncology targets when the molecular target presents in pediatric population. As a result of these reforms, many more drugs that are currently only

indicated for adult populations would be subject to PREA requirements if molecular targets present in pediatric population.

The KIDS Act would produce a significant expansion in the number of novel cancer therapies approved each year that have been studied in children. This would provide clinicians in our institutions and beyond with greater assurances as to the safety and efficacy of potential therapies that are now largely used off-label. By expanding the number of cancer therapies approved for pediatric patient use, we will expand our therapeutic arsenal and, hopefully, lead to more kids who are treated successfully.

The Coalition for Pediatric Medical Research strongly supports including the KIDS Act within the Senate innovation package. We thank you for considering this request and would be pleased to discuss this more fully at your convenience. If you have any questions, please contact us through Nick Manetto at 202.312.7499 or nicholas.manetto@faegrebd.com.

Sincerely,

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