

September 9, 2015

The Honorable Lamar Alexander
Chairman
Committee on Health, Education,
Labor, and Pensions
U.S. Senate
Washington, DC 20510

The Honorable Patty Murray
Ranking Member
Committee on Health, Education,
Labor, and Pensions
U.S. Senate
Washington, DC 20510

Dear Chairman Alexander and Ranking Member Murray:

The undersigned organizations representing patient groups and physician organizations commend you for your leadership in undertaking an examination of the process for getting safe treatments, devices, and cures to patients. As the Committee continues its process of drafting legislation, we ask you to consider including provisions that will address the importance of access to safe and efficacious off-label medications for children with auto immune diseases such as inflammatory bowel disease (IBD).

An estimated 1.6 million Americans are living with IBD (Crohn's disease and ulcerative colitis), with nearly one in four patients diagnosed under 20 years of age. IBD, an Orphan disease, is a chronic condition without a medical cure and commonly requires a lifetime of care.

The vast majority (an estimated 80 percent) of medications prescribed by physicians to treat children with IBD are not approved by the Food and Drug Administration (FDA) for the indication at the time they are given, meaning they are not approved by the FDA for use in children. Medications used to treat IBD are first approved in adults, and approval for children may come many years later, if at all. During this time, these highly effective medications are prescribed "off-label" to children without any mechanism to monitor safety. In addition to medical risk, parents oftentimes incur significant out-of-pocket costs when off-label drugs are prescribed because insurers are not required to cover medications that are not FDA-approved, even though they are prescribed by physicians and are essential to properly and effectively treat these children, for whom there are few FDA-approved options.

Given the frequency with which medications are prescribed as off-label to children with IBD, funding is needed to develop an independent and transparent public, pediatric registry that captures data on the use of these medications. In addition to monitoring the safety of these medications, such a registry could be accessible to physicians and patients to aid in treatment decision-making as well as to industry, investigators and federal regulators. Such a public registry would greatly enhance pediatric drug development and is likely to help expedite drug approvals and encourage drug companies to pursue pediatric indications for FDA-approved drugs by allowing them to access a central data repository rather than establishing cost-prohibitive, proprietary, drug-specific registries for safety monitoring.

Treatment of children with IBD benefits from FDA-mandated registries, but they are often proprietary, single-product registries that do not capture the majority of children who are not enrolled in the registry. Furthermore, significant safety data captured on a competitor's medication may not be made public, and these registries lack uniformity of data collection. Recently, a medication approved for treatment of Crohn's disease in adults has been found to cause a rare but fatal lymphoma in boys who received the medication in combination with another Crohn's treatment. A national safety registry might have identified this problem much earlier.

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We envision that existing IBD registries would share data points with the public IBD registry which would connect to an existing registry for pediatric rheumatology (CARRA — Childhood Arthritis and Rheumatoid Research Alliance). Connection to the CARRA registry would benefit both pediatric IBD and rheumatology patients because these auto-immune diseases are often treated with the same medications. CARRA was started with a \$7.5 million grant to the NIH in 2009 as a result of funding through the American Recovery and Reinvestment Act. We believe building on this federally-funded registry would encourage data sharing, extend the government's return on investment, and allow federal regulators and researchers to access data without having to rely on proprietary registries.

We respectfully ask that you consider our request to provide funding for a public IBD registry that would connect to the existing CARRA registry. The funding would be used to establish the registry, after which we believe the registry would be self-sustaining, financed by industry and private-public collaborations.

On behalf of the thousands of children who live with IBD and take medications for which long-term safety is not known, we thank you in advance for your consideration of our request. For further information, please contact Eric Zuckerman, DO, with the Pediatric IBD Foundation at (248)-227-3999 or ezericzuckerman@gmail.com; or Camille Bonta with the North American Society for Pediatric Gastroenterology, Hepatology and Nutrition at (202) 320-3658 or cbonta@summithealthconsulting.com.

Sincerely,

American Academy of Pediatrics
American College of Gastroenterology
American Gastroenterological Association
American Medical Association
The Association of Pediatric Gastroenterology and Nutrition Nurses
Digestive Disease National Coalition
North American Society for Pediatric Gastroenterology, Hepatology and Nutrition
Pediatric IBD Foundation
Society of Gastroenterology Nurses and Associates