Access to Pediatric Inflammatory Bowel Disease Therapies

Crohn's disease and ulcerative colitis are chronic, degenerative, immune-mediated diseases collectively known as inflammatory bowel disease (IBD). Nearly one in every 100 Americans has been diagnosed with IBD, and cases among children have risen dramatically over the last decade. The rapidly increasing rate of pediatric IBD is especially concerning given the fact that these diseases are typically more severe and aggressive in children, often causing more extensive bowel damage and displaying higher relapse rates than adult IBD. Children's bodies are also at an increased risk for complications related to malnutrition, developmental delays, and growth impairment. The management of pediatric IBD is made all the more difficult by the fact that the Food and Drug Administration (FDA) continues to delay approvals for effective pediatric IBD treatments.

Pediatric Patients are Left Behind

Since 2000, drug therapies using six different mechanisms of action have been approved by the FDA for the treatment of adult IBD. Yet, in that same period, only a fraction of those medications have received FDA approval for use in children under 18, and all but one are in the same therapeutic class. Currently, there is only one class of medication available for pediatric patients with ulcerative colitis.

Pediatric IBD approvals have been delayed in part because the FDA has imposed a more restrictive evidentiary standard for the same medications when they are used in pediatric IBD than for use in other pediatric disease states, including other autoimmune diseases (e.g., arthritis and psoriasis).

The ongoing lack of pediatric indications prolongs suffering and delays effective disease management by unnecessarily limiting treatment options for children with IBD.

Steps FDA Can Take to Address the Gap and Help Children with IBD

- **No Placebos:** For new IBD therapies, create a pediatric clinical trials framework that does not require the use of placebos. Children with active IBD should not be forced to forgo treatment under any circumstance, and certainly not when there are alternatives (such as data extrapolation) that the FDA could use to evaluate the safety and efficacy of new therapies in children.
- **Enforce Existing Law:** For all (new and existing) IBD therapies used in adult populations, ensure compliance with the Pediatric Research Equity Act (PREA) mandate for pediatric dosing studies. Currently, only one-third of the required PREA studies have been completed, with follow-up that often takes more than seven years.
- **Use Real World Evidence:** For therapies that have been approved for use in adult IBD patients and are widely used "off-label" in children, we encourage the FDA to work with sponsors, clinicians, and the patient community to develop strategies to utilize existing real-world evidence to assess therapeutic safety, efficacy, and dosing.

We encourage the FDA to act quickly to modernize the processes it uses to review medicines that can help children fight Crohn's disease and ulcerative colitis to achieve a goal everyone shares: helping children fight these difficult chronic diseases.

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