

Off-label, on target: new hope for children with inflammatory bowel disease

FAYETTEVILLE, GA, UNITED STATES, August 19, 2025 /EINPresswire.com/ -- [Off-label treatments](#) are offering new hope to children battling inflammatory bowel disease (IBD), especially when conventional therapies fall short. A comprehensive new review explores the real-world application of biologics and small molecule drugs originally approved for adults—such as ustekinumab, vedolizumab, upadacitinib, and ozanimod—in pediatric IBD cases. These alternative approaches are showing encouraging results, including higher rates of steroid-free clinical remission and improved disease management in treatment-resistant patients. While these therapies remain technically unapproved for pediatric use, growing evidence suggests they may provide critical relief when other options have been exhausted. The review underscores both the promise and the caution needed when venturing into off-label territory.

Pediatric inflammatory bowel disease (IBD)—encompassing Crohn's disease and ulcerative colitis—is a chronic and often debilitating condition that disrupts digestion, growth, and development in children. Unlike adult patients, children often present with more aggressive symptoms and face greater risks of developmental complications. Yet only two biologics are formally approved for use in pediatric IBD, leaving many children with limited therapeutic options. As a result, clinicians are increasingly turning to off-label medications designed for adults, hoping to replicate positive outcomes in younger patients. Due to these limitations and complexities, there is a growing need to evaluate the safety, effectiveness, and dosing strategies of such treatments specifically in the pediatric context.

In a review (DOI: [10.1002/pdi3.70011](#)) published in [Pediatric Discovery](#) in June 2025, researchers from the University of Texas Southwestern Medical Center and the Children's Hospital of Chongqing Medical University examined the landscape of off-label therapies for pediatric IBD. The team analyzed how adult-approved drugs—like ustekinumab, vedolizumab, and Janus kinase (JAK) inhibitors—are being used to treat children with severe or treatment-resistant IBD. Drawing from clinical trials, real-world studies, and case reports, the review provides a detailed picture of how off-label treatments are shaping the next frontier in pediatric IBD management.

The review highlights several key off-label therapies and their real-world performance in children with IBD. Ustekinumab, which targets IL-12 and IL-23 pathways, demonstrated strong steroid-free remission outcomes, especially in biologic-naïve pediatric patients. Studies showed up to 90% remission in some cohorts, with a favorable safety profile. Similarly, vedolizumab, a gut-targeted antibody, achieved clinical remission in nearly half of pediatric patients in multiple trials.

and real-world settings. For more severe or refractory cases, JAK inhibitors like tofacitinib and upadacitinib offered rapid symptom relief—even among patients who had failed multiple prior treatments. One case reported full remission in a 12-year-old Crohn's patient just weeks after starting upadacitinib.

The review also explored combination therapies and dual-drug regimens, noting high remission rates but also highlighting safety concerns such as infection and thrombosis. Emerging therapies like S1P receptor modulators—though still experimental in children—have been cautiously introduced based on adult data, signaling new possibilities. Across all treatments, the authors emphasize the importance of therapeutic drug monitoring, individualized dosing strategies, and long-term safety assessments to maximize benefits while minimizing risks.

“Pediatric IBD care is at a turning point,” said Dr. Xiaoqin Zhou, senior author of the study. “We’re increasingly seeing success with therapies that were never designed with children in mind. But that doesn’t mean we can treat them like small adults. Every decision must balance innovation with safety.” Dr. Zhou noted that off-label therapies can offer lifelines to patients who have exhausted conventional options, but emphasized the need for rigorous oversight, collaborative decision-making, and continued investment in pediatric-focused clinical trials.

The findings from this review could help reshape treatment strategies for pediatric IBD, giving clinicians more tools to manage complex or refractory cases. As real-world evidence accumulates, off-label therapies may eventually transition into mainstream pediatric care—particularly if future trials confirm their efficacy and safety. For now, these treatments offer a valuable option when standard drugs fail, helping children achieve clinical remission and avoid long-term complications. The review also highlights the need for better regulatory pathways, personalized treatment frameworks, and pediatric-specific research to close the gap between adult and child IBD care.

References

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