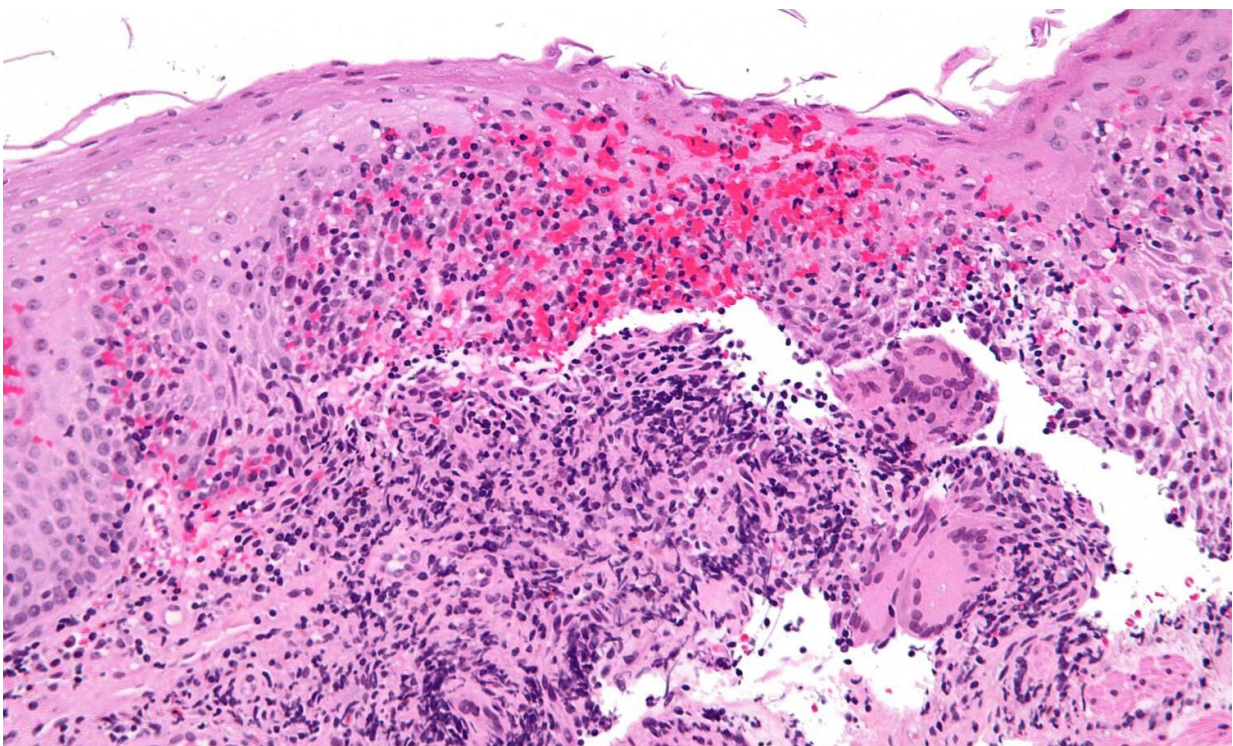


Researchers announce findings from landmark clinical trial for pediatric Crohn's disease

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High magnification micrograph of Crohn's disease. Biopsy of esophagus. H&E stain. Credit: Nephron/Wikipedia

Crohn's disease is a lifelong inflammatory bowel disease (IBD) that is characterized by swelling in the lining of the digestive tract, causing

severe diarrhea, abdominal pain, and rectal bleeding. For pediatric patients, the disease is harder to treat. If untreated, it can impair growth, psychosocial development, and puberty.

A significant clinical trial under the direction of Michael Kappelman, MD, MPH, professor of pediatrics at UNC School of Medicine, found that patients receiving the tumor necrosis factor inhibitor adalimumab combined with a low dose of methotrexate, a second immunosuppressant, did better than those treated with adalimumab alone. However, patients initiating infliximab, another tumor necrosis factor inhibitor, had similar outcomes with or without methotrexate.

Their findings [were published](#) in *Gastroenterology*, the leading journal in the field of gastrointestinal [disease](#).

Adalimumab and infliximab, the key drugs of interest, belong to a class of medications called anti-inflammatory tumor necrosis factor (TNF) inhibitors. These medications suppress the body's natural response to TNF, a protein which is produced by white blood cells during inflammatory events. Adalimumab was approved by the Food and Drug Administration in 2014 for treatment in [pediatric patients](#) with Crohn's disease, whereas infliximab was approved in 2006.

"We know that both adalimumab and infliximab have proven to be effective and safe," said Kappelman. "We also know that they that they don't work for all patients and don't work forever. This pivotal trial comparing anti-TNF alone or in combination with methotrexate has provided clear results that can immediately impact [patient care](#)."

The clinical trial had been in the works for a decade. Starting with the important decision of which research question was the most urgent and pressing need, Kappelman then engaged key stakeholder groups through a collaboration with [ImproveCareNow](#), a national pediatric community

that brings together [medical professionals](#), patients, and families to advance care through research and [quality improvement](#), support for psychosocial functioning and mental health, and escalating initiatives to address disparities, equity, and inclusivity.

David Wohl, MD, professor of medicine in the Division of Infectious Diseases in UNC's Department of Medicine, joined ImproveCareNow soon after his son was diagnosed with pediatric Crohn's disease at nine years old. He was surprised at how little evidence there was behind treatment decisions.

"I became vocal about the 'data desert' parents like me have to wander through to find answers," said Wohl. "I immediately joined as a parent representative and became active in the organization and at our UNC site. My superpower was not only being a parent of a child with IBD, but also a clinician-researcher."

After Wohl's son started seeing Kappelman for clinical care, they spoke about a need for better clinical trial data. Kappelman then approached Wohl with an idea, which would later become the landmark clinical trial. Subsequently, Wohl assumed a lead role in patient and parent engagement and enrollment, along with Lisa Pitch from Nevada and other parents through ImproveCareNow.

"To see the study now provide us with a clear answer about what medicines work best in kids with Crohn's is incredibly gratifying," said Wohl. "It is truly a game-changer."

Kappelman and his team, with UNC-Chapel Hill acting as the lead site, were successful in obtaining an \$8-million funding award from the Patient-Centered Outcomes Research Institute (PCORI) and substantial co-funding from the Helmsley Charitable Trust. They recruited and followed patients at over thirty centers across the country, rigorously

collecting, quality checking, and analyzing data, and synthesizing results. Two of the sites were located in North Carolina—one at UNC Children's Hospital and another at Levine Children's Hospital in Charlotte.

"We are grateful to our funders and to the hundreds of physicians and staff at sites across the country who worked hard to make this project a success," said Kappelman. "Most importantly, we would like to thank all of the patients and families who bravely and generously volunteered to be part of this study."

The results will also be presented at Digestive Disease Week, a prestigious meeting the hosted by the American Gastroenterological Association.

More information: Michael D. Kappelman et al, Comparative Effectiveness of Anti-TNF in Combination with Low Dose Methotrexate vs Anti-TNF Monotherapy in Pediatric Crohn's Disease: a Pragmatic Randomized Trial, *Gastroenterology* (2023). [DOI: 10.1053/j.gastro.2023.03.224](https://doi.org/10.1053/j.gastro.2023.03.224)

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