The top 10 research priorities for inflammatory bowel disease in children and young adults

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The top 10 research priorities for inflammatory bowel disease in children and young adults: results of a James Lind Alliance Priority Setting Partnership

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Inflammatory bowel disease (IBD), including Crohn's disease and ulcerative colitis, is a chronic idiopathic disorder characterised by relapsing and remitting inflammation of the gastrointestinal tract.[1] Recent large population-based registry studies in Europe showed that around 8% of patients with IBD are diagnosed before age 18 years.[2,3] Over the past three decades, IBD has become a global disease with rising incidence rates, especially in low-income and middle-income countries in Asia, South America, and Africa.[4] In the paediatric population, the prevalence of IBD continues to rise worldwide, with the highest prevalence rates reported in Europe, ranging from 31 cases per 100 000 people to 75 cases per 100 000 people.[5]

Panel: **Top 10 research priorities for inflammatory bowel disease (IBD) in children and young adults**

1. What are the causes of fatigue in children and young adults with IBD, and what steps can they take to reduce their fatigue levels?
2. How can health-care professionals more accurately predict the disease course of IBD in children and young adults, including flares and disease complications, and can this prediction aid in preventing disease progression?
3. What external factors, such as diet, infections, medication, and living environment, contribute to the development of IBD in children and young adults?
4. What are the potential long-term effects of medication use in children and young adults with IBD?
5. When and how should medication tapering be done in children and young adults with IBD?
6. What role does the gut microbiome play in the development of IBD in children and young adults?
7. Which foods, nutrients, and eating habits are beneficial for managing IBD in children and young adults?
8. Is it possible to predict which medications will work best on an individual basis?
9. How can children and young adults with IBD receive support in coping with their disease?
10. Are there diagnostic procedures possible for children and young adults with IBD that are less invasive than endoscopy?

Childhood-onset IBD differs from adulthood-onset IBD in several aspects, including response to specific therapies, early disease progression, substantial effects on growth, pubertal and skeletal development, the psychosocial burden on patients and their families, and school absenteeism.[6,7] Research in paediatric IBD is relatively scarce compared with that in adults with IBD, illustrating the need for future work covering a variety of research areas. Developing a paediatric IBD research agenda could guide researchers in prioritising research questions. Current research agendas, including the future priorities in paediatric IBD, have been stipulated by scientists, academia, industry, and funding agencies. The patients' perspective is rarely considered.[8] To address this mismatch, the James Lind Alliance (JLA) developed a method for bringing research consumers together in a priority setting partnership (PSP), allowing for the identification and prioritisation of research questions that matter for patients, caregivers, and health-care professionals.[9]

A Dutch PSP for children and young adults with IBD was established in cooperation with the patient association Crohn & Colitis Netherlands (CCNL), the Dutch research working group Kids with Crohn's and Colitis (KiCC), and the JLA. In April, 2021, a steering group was established, including four children and young adults with IBD, four parents, and eight health-care professionals, including a dietitian, adult and paediatric gastroenterologists, nurse practitioners, and psychologists. The steering group agreed on a broad scope of research uncertainties, including the cause, diagnosis, symptoms, treatment, prognosis, self-management, and influence on the daily lives of children and young adults with IBD (age up to 21 years). The PSP followed the JLA step-wise approach.[10] The steps involved: (1) gathering research uncertainties from patients, caregivers, and health-care professionals with online and in-person surveys; (2) checking these research uncertainties against the existing literature; (3) interim prioritisation via a second online survey; and (4) a final workshop, agreeing

on the top 10 research priorities. In both surveys, respondents provided demographic and disease-specific details, including age, education level, region, type of health-care professional, and phenotype and duration of IBD, to ensure that a broad clinical, caregiver, and patient population was represented.

With the first survey (completed between November, 2021, and February, 2022), 763 uncertainties were submitted by 222 respondents, including 74 patients, 89 caregivers, and 59 health-care professionals. Among these 763 uncertainties, 103 were out of scope and removed. The remaining 660 uncertainties were categorised into 13 themes (appendix p 1) and refined into 64 indicative questions. A literature check was done with the support of a medical information specialist, which resulted in five answered questions and 59 unanswered questions. In the second online survey, completed between October, 2022, and January, 2023, 263 respondents (70 patients, 127 caregivers, and 66 health-care professionals) voted for their personal top 10 from the 59 unanswered questions. The 25 questions most voted for from the prioritisation survey were included in the final consensus workshop (on March 18, 2023), which was attended by ten patients, 12 caregivers, and eight health-care professionals. A consensus was reached on the final top 10 research priorities by use of the nominal group technique.[10]

We here outline the top 10 research priorities from the viewpoint of children and young adults with IBD, their caregivers, and health-care professionals (panel). The 25 questions that remained until the final workshop are summarised in the appendix (pp 2–3). This patient-centred IBD research agenda covers a broad range of research areas, including the cause and management of fatigue, the aetiology of IBD, the need for non-invasive and personalised diagnostic and management strategies, and support in coping with their disease. The results of this unique PSP were presented to the Dutch Paediatric Association and to ZonMw, the major national health-care funding agency. These top 10 priorities will be made available to the global IBD research community for consideration when planning clinical research. The translation of these research priorities into studies will provide the greatest benefit to children and young adults with IBD, their families, and health-care professionals.

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See **Online** for appendix

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