Recommendations for pharmacological clinical trials in children with functional constipation: The Rome foundation pediatric subcommittee on clinical trials

M. Saps, J. V. Lavigne, S. Nurko, I. J. N. Koppen, J. A. J. M. Taminiau, C. di Lorenzo, M. A. Benninga

Abstract

Background: Evidence for the efficacy of commonly used drugs in the treatment of childhood functional constipation (FC) is scarce, studies are often of low quality and study designs are heterogeneous. Thus, recommendations for the design of clinical trials in childhood FC are needed.
Purpose: Members of the Rome Foundation and a member of the Pediatric Committee of the European Medicines Agency formed a committee to create recommendations for the design of clinical trials in children with FC.
Key Recommendations: This committee recommends conducting randomized, double- blind, placebo-controlled, parallel-group clinical trials to assess the efficacy of new drugs for the treatment of childhood FC. Pediatric study participants should be included based on fulfilling the Rome IV criteria for FC. A treatment free run-in period for baseline assessment is recommended. The trial duration should be at least 8 weeks. Treatment success is defined as no longer meeting the Rome IV criteria for FC. Stool consistency should be reported based on the Bristol Stool Scale. Endpoints of drug efficacy need to be tailored to the developmental age of the patient population.

KEYWORDS
children, constipation, pediatrics, RCT, trial

(2018). Neurogastroenterology & Motility, 30(4), e13294.