Recommendations for pharmacological clinical trials in children with irritable bowel syndrome: the Rome foundation pediatric subcommittee on clinical trials

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Abstract

Background: There is little published evidence of efficacy for the most commonly used treatments. Thus, there is an urgent need to conduct clinical trials on existing and novel therapies. Purpose In order to address these issues the Rome Foundation and members of the Pediatric Committee of the European Medicines Agency formed a subcommittee on clinical trials to develop guidelines for the design of clinical trials in children with irritable bowel syndrome (IBS). The following recommendations are based on evidence from published data when available and expert opinion.

Key recommendations: The subcommittee recommends randomized, double-blind, placebo-con- trolled, parallel-group, clinical trials to assess the efficacy of new drugs. The combined endpoints for abdominal pain are a decrease in intensity of at least 30% compared with baseline and to meet or exceed the Reliable Change Index (RCI) for the sample. Stool consistency is measured with the Bristol Stool Scale Form (BSFS). The subcommittee recommends as entry criteria for abdominal pain a weekly average of worst abdominal pain in past 24 h of at least 3.0 on a 0–10 point scale or at least 30 mm in 100 mm Visual Analog Scale. For stool endpoints the committee recommends an average stool consistency lower than 3 in the BSFS during the run-in period for clinical trials on IBS-C and an average stool consistency greater than 5 in the BSFS during the run-in period for clinical trials on IBS-D. Changes in stool consistency are the primary endpoints for both IBS with diarrhea (IBS-D) and IBS with constipation (IBS-C).

Keywords abdominal pain, children, clinical trials, endpoints, irritable bowel syndrome, stool consistency.

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