Prospective Registry Study and Clinical Observation of Children With Crohn's Disease

July 24, 2024

Study Protocol

Brief title	Prospective cohort establishment and clinical observation of children with Crohn's disease
Research institution	Tongji Hospital, Tongji Medical College, Huazhong University of Science and Technology
Major investigators	Sainan SHU professor, Biao Zou attending doctor
Date	July 24, 2024
Study population	Children with Crohn's Disease
Purpose of research	**Primary Outcome Measure:** To evaluate the clinical manifestations, treatment options, and improved clinical outcome of children with Crohn's disease in real-world Settings: (1) analysis of clinical manifestations; (2) probability of using the same treatment options; (3) Clinical outcome; **Secondary Outcome Measures:** (1) Evaluate the clinical application of enteral nutrition (EEN/ PEN, reason, route of administration, dose, course of treatment); (2) Evaluate the clinical use of glucocorticoids (reason, route of administration, dose, course of treatment); (3) Evaluate the use of immunosuppressants (reason, route of administration, dose, course of treatment); (4) Evaluate the clinical application of biologics (including domestic infliximab (Taizhou Mabtech Pharmaceutical Co.,Ltd)) (dose, duration, effectiveness, safety, and economy); (5) Evaluate the application of fecal microbiota transplantation (cause, transplantation routes, dose, course of treatment); (6) To evaluate the medical costs and resource consumption of the treatment of Crohn's disease in children (state insurance costs, commercial insurance costs, self-financing costs, others); **Other Pre-specified Outcomes:** To conduct registration studies to obtain information on the efficacy and safety of biologics (e.g. infliximab (Taizhou Mabtech Pharmaceutical Co.,Ltd)) used in children with Crohn's disease, and to evaluate the dose, duration, and economics of clinical use of biologics (e.g. infliximab (Taizhou Mabtech Pharmaceutical Co.,Ltd)).

Study endpoint	**Primary efficacy endpoint:** 14 weeks clinical response rate. **Secondary efficacy endpoints:** (1) Clinical remission rates at 14 and 54 weeks (PCDAI≤10)； (2) Clinical response rate at 54 weeks (PCDAI reduction ≥15 points and total PCDAI≤30 points) (3) Endoscopic response rate at 14 and 54 weeks (SES-CD≤2); (4) Mucosal healing rate at 14 and 54 weeks (SES-CD=0); (5) Changes of serum C-reactive protein, erythrocyte sedimentation rate and fecal calcarein from baseline at 14 and 54 weeks; (6) Changes in intestinal flora from baseline at 14 and 54weeks; (7) Changes in serum new inflammatory markers from baseline at 14 and 54 weeks; **Safety end point:** The incidence of adverse events/adverse reactions after the first dose was recorded (observed up to 102 weeks).
Study design	Prospective, single center, registry study. This study is an observational study, without intervention in the treatment of patients, patients will accept the treatment plan formulated by the attending physician, researchers and patients respectively truthfully register the corresponding data generated by the clinical practice, collect the laboratory examination and the evaluation data of doctors during the visit, and combine the hospital visit and the electronic information system. Statistical analysis was performed after follow-up.
Eligibility Criteria	Inclusion Criteria: (1) Age 0~17 years old; (2) Children with a definite diagnosis of Crohn's disease; (3) Patients and their guardians must sign informed consent Exclusion Criteria: Other conditions deemed inappropriate by the doctor to participate in the study.

Study Population Description	100
Research visit	Total observation 102 weeks; A total of 4 interview points were set up: baseline, 14 weeks, 54 weeks, 102 weeks; According to the patient's wishes, the course of the disease, and the actual diagnosis and treatment, the visit can be ended early.
Planned study time	Estimated total project time: 3-5 years

Statistic analysis	For continuous variables, the number of cases, mean, standard deviation, median, 25th and 75th percentiles, minimum, and maximum are described. For discrete variables, the numbers and percentages of each type are described. Missing values are not included in the calculation of percentages unless otherwise noted. Unless otherwise noted, all statistical tests will use a two-sided test with $\alpha = 0.05$, $P < 0.05$ is considered statistically significant, and a two-sided 95% confidence interval (CI) is calculated if necessary.

Study flow chart

Interview point	**Baseline**	**treatment period**		
Visit time	Visit time 1	Visit time 2	Visit time 3	Visit time 4
	-14d~0d	14w±7d	54w±7d	102w±7d
informed consent	√			
Demographic data	√			
physical examination	√	√	√	√
vital signs	√	√	√	√
medical history	√			
Medication history	√			
Operation History	√			
family history	√			
allergic history	√			
Laboratory index				
Blood routine examination	√	√	√	√
blood biochemistry	√	√	√	√
routine urine test	√	√	√	√
stool routine	√	√	√	√
fecal calprotectin	√	√	√	√
Serum 25-(OH)-D3	√	√	√	√
coagulation function	√	√	√	√
inflammatory factor	√	√	√	√
C-reactive protein	√	√	√	√
erythrocyte sedimentation rate	√	√	√	√
PCDAI score	√	√	√	√
endoscopy	√	√	√	√
histopathological examination	√			
imageological examination	√	√	√	√

medication administration record	√	√	√	√
adverse event	√	√	√	√
Combination medication/concomitant therapy	√	√	√	√
medical cost	√	√	√	√