Clinical Trial Summary Attachment (based on RRF)	**SA**

1	**Sponsor:** CHU de Bordeaux
2	**Naming of the experimental drug(s):** polyethylene glycol
3	**Naming of the experimental active(s) substance(s):** polyethylene glycol
4 4.1 4.2 4.3	**Study identification** **Official Title:** Effect of Polyethylene Glycol Treatment on Intestinal Inflammation Associated With Cystic Fibrosis In children. **Acronym:** MUCOLAX **Unique Protocol ID:** CHUBX 2019/25
5.1 5.2	**ID EudraCT:** 2019-004958-29 **ID NCT :** NCT04458129
6	**Investigator(s):** Dr Raphaël ENAUD Service de Gastroentérologie pédiatrique – CRCM pédiatrique Hôpital des Enfants – Groupe hospitalier PELLEGRIN, Place Amélie Raba-Léon, CHU de Bordeaux
7	**Location(s):** -CHU de Bordeaux - CRCM pédiatrique Hôpital des Enfants - Dr Raphaël ENAUD -CHU Toulouse - CRCM pédiatrique – Dr Marie MITTAINE
8	**Paediatric regulatory details if necessary: ☐yes / ☐no**
9 9.1	**Trial status / Results Analysis stage:** ☒prematurely ended (Terminated) ☐withdrawn ☐no subjects recruited ☒only partial results are available ☐trial transitioned to CTIS and its results will be posted there **Detailed reason:** Target number of subject not reached Changes in marketing authorizations and protein therapies, which have increased the patient exclusion rate, making inclusion too difficult as a result.
10 10.1 10.2	**Notification of the end of the trial to the NCA : ☒yes / ☐no** **ANSM: May 15 2026** **RRF (Résumé du Rapport Final): May 15 2026**
11 11.1 11.2 11.3	**Study duration** - First inclusion: 09/29/2020 - Primary completion date: 01/05/2021 - Study completion date: May 18 2022
12. **Clinical Trial Phase / Type:** II	
13 13.1 13.2 13.3	**Population of trial subjects / Enrollment** - **Number of subjects expected:** 23 - **Actual Number of subjects included in the trial: 1** - **Actual Number of subjects analysed in the trial: 1**
14	**Main objective:** Study the impact of a 3-month polyethylene glycol treatment on intestinal inflammation associated with cystic fibrosis in children aged 4 to 18 years, as measured by the fecal calprotectin assay.

14.1	**Secondary objectives:** • Study the impact of a 3-month treatment with polyethylene glycol: - On digestive symptoms and quality of life, - On the intestinal microbiota (bacterial and fungal) • Study the co-evolution of inflammation between the gut and lungs ("the gut-lung axis") during the 3 months of polyethylene glycol treatment
15	**Research strategy:** Bi-centric, non-comparative, prospective, Phase II trial according to a Fleming scheme One arm, treated for 3 months with polyethylene glycol
16	**Medical condition or disease studied:** Cystic Fibrosis
16.1	The main objective of the study is to evaluate the effectiveness of polyethylene glycol treatment on intestinal inflammation in children with cystic fibrosis. In this test, a method adapted from the Fleming onestep scheme will be used. The success rate is measured by the proportion of patients with fecal calprotectin levels < 250 µg/g at 3 months after treatment initiation.
16.2	**Inclusion Criteria:** • Age ≥ 4 years old and <18 years old ; • Patient with cystic fibrosis (sweat test > 60 mmol/l and/or molecular biology identifying mutations in the CFTR gene) with associated pancreatic insufficiency (fecal elastase <100 µg/g); • With a rapid calprotectin assay result via the IBDoc test (Bühlmann®) superior or equal to 250 µg/g; • Person affiliated or benefiting from a social security scheme; • Free, informed and written consent signed by the holders of parental authority and the investigator before any examination required by the research and oral and/or written consent by the participant (depending on his/her age). **Exclusion Criteria:** • Ongoing processing that can modulate the functionality of the CFTR (such as lumacaftor-ivacaftor protein therapy); • Patient already on polyethylene glycol or other laxative within 3 months before the inclusion visit; • Patient with diarrhea at inclusion (diarrhea will be defined as the presence of 3 or more stools / day in the 7 days prior to the inclusion visit); • Acute viral or bacterial diarrhea in the month prior to the inclusion visit (associated with fever); • Cure of oral or intravenous antibiotics or antifungals in the month preceding the collection of samples; • Change in background treatment in the month prior to the inclusion visit (oral or inhaled corticosteroid therapy, azithromycin, inhaled antibiotic therapy, inhaled antifungal agent, proton pump inhibitors); • Taking probiotics in the month before the inclusion visit; • Transplanted patient (on immunosuppressants); • Patient with IBD or celiac disease; • Patient with digestive perforation or risk of digestive perforation; • Patient with ileus or suspicion of intestinal obstruction, symptomatic stenosis; • History of hypersensitivity to macrogol or any of the excipients • Holders of parental authority enjoying judicial protection.
17	**Experimental drug(s) studied (name, dose, route of administration, lot number):** polyethylene glycol (Macrogol 4000), powder for oral solution, in 4g and 10g sachets. Dosage of 0.7 g/kg/day, with a maximum dose of 20 g/day
18	**Treatment duration:** 3-month
19	**Reference Experimental drug(s) (name, dose, route of administration, lot number):** NA
20	**Evaluation criteria** *Efficacy / Security / other*
20.1	**Primary endpoint:** Impact of a 3-month polyethylene glycol treatment on intestinal inflammation assessed by measurement of fecal calprotectin (µg / g) by an ELISA biological test proportion of patients with fecal calprotectin <250 µg / g measured by an ELISA test 3 months after initiation of treatment with polyethylene glycol, testifying to the absence of intestinal inflammation or slight inflammation.

20.2	**Secondary endpoints:** Evolution between the ignition of the treatment at J0 and after 3 months of treatment (M3) with polyethlene glycol of : - Quality of life and Gastrointestinal symptoms - Gastrointestinal inflammatory response - Composition of the bacterial and fungal gut microbiota, - Pulmonary inflammation
21	**Statistical analysis of the data** Analysis of the main objective: At alpha risk 5% (unilateral test), the hypothesis that the proportion of success (patients with fecal calprotectin < 250 µg/g) is greater than 20% will be tested by comparing the lower bound of the bilateral confidence interval at 90% of the proportion of success at 6 months to the theoretical value of 20%.
22 22.1 22.2	**Trial Summary – Results** - **Outcomes / Efficacy evaluation:** No statistical analyses available - **Adverses events / Security evaluation:** *Adverse events information* *Adverse event reporting group* *Serious Adverse Events* *Non-serious adverse event*
23	**Conclusion** The target of 23 patients to be included in the study was not met. 9 patients were screened. 2 patients were included (because their fecal calprotectin levels, as measured by the IBDoc rapid test, were > 250 µg/g), but 1 patient had to be excluded (because confirmation of fecal calprotectin levels via the reference ELISA test ultimately showed values < 250 µg/g). In total, only 1 patient was included in this study and has completed follow-up. Statistical analysis was not possible due to a lack of data. <u>The recruitment issue was analysed:</u> Protein therapies have been developed to restore the function of the CFTR protein. Among these modulators, the lumacaftor-ivacaftor combination has held a Marketing Authorization (MA) in France since November 2015. We therefore conducted a preliminary retrospective study involving 15 children, as no data were available regarding the impact of lumacaftor-ivacaftor on gastrointestinal complications of cystic fibrosis, specifically: - Intestinal inflammation - The gut microbiota (bacterial and fungal). The results showed that calprotectin levels were significantly reduced after 3 months of treatment with lumacaftor-ivacaftor. At the time the MUCOLAX study was submitted in 2019, the marketing authorization for lumacaftor-ivacaftor applied only to patients with cystic fibrosis over the age of 12 who were homozygous for the DeltaF508 mutation—representing approximately 30% of the patients followed at the Bordeaux and Toulouse University Hospitals. By the end of 2022, the marketing authorization for elexacaftor-tezacaftor-ivacaftor was expanded to include patients aged 6–11 years. As a result, approximately 90% of patients with cystic fibrosis are now eligible for a modulator in 2022, which has called into question the feasibility of the MUCOLAX study. The decisions of prematurely termination of MUCALAX has been made by the scientific committee of the STUDY.
24	**<u>More Information</u>**: *Global Substantial Amendments:* *Global Interruptions and re-starts:* *Limitations & Caveats:*
25	**Publication(s):**
26	**Establishment date: 07/21/2026**