THRASSA, A Multicenter, Open-label Study Evaluating The Safety, Tolerability And Efficacy Of MaaT013 In Ruxolitinib-refractory Or Intolerant Paediatric/ Adolescent Participants With Gastrointestinal Acute Graft-versus-Host Disease

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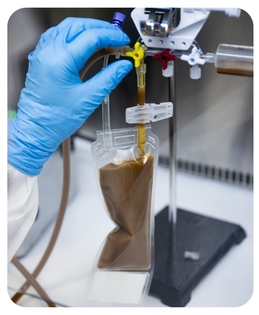
Introduction

Acute graft-versus-host disease (aGvHD) is a serious complication following allogeneic haematopoietic stem cell transplantation (allo-HSCT) and affects many children[1]. The incidence of Grade II–IV aGvHD in children receiving allo-HSCT from unrelated donors varies from 40 to 85%[2]. Corticosteroids (CS) remain first-line therapy, yet fewer than half achieve durable complete responses, requiring second-line treatment[3]. In Europe, ruxolitinib is approved for steroid-refractory (SR) aGvHD in patients ≥28 days old (REACH-2/4). However, ORR ranges from 45–100%[4], and in REACH-4, 49% discontinued due to lack of efficacy or adverse events[5], highlighting persistent unmet need and poor outcomes.

Methods

- Allogeneic, full-ecosystem pooled biotherapeutic intestinal microbiota, drug candidate tested in a completed phase III in adult patients with Gastrointestinal (GI)-aGVHD (NCT04769895) and available on a compassionate use program for patients with aGvHD.
- MaaT013's promising efficacy and acceptable safety profile suggest a favorable benefit–risk profile as a third-line therapy, warranting evaluation in children.



THRASSA is a phase II study designed to evaluate the safety, tolerability and efficacy of MaaT013 as a 3rd line therapy after systemic corticosteroids and ruxolitinib failure in paediatric and adolescent participants with GI-aGvHD (EU-CT#: 2025-524302-15-00).

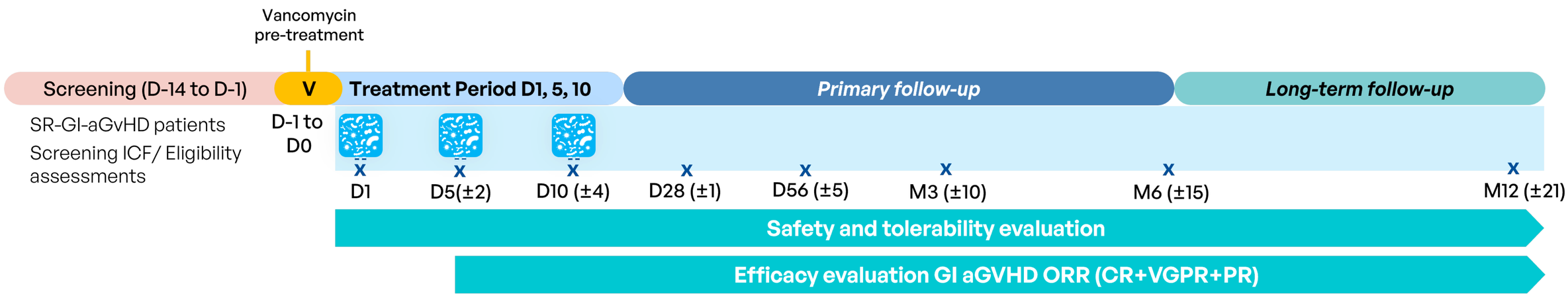
[1] (Vincent et al. 2025), [2] (Andolina et al. 2022; MacMillan et al. 2020), [3] (Gottardi et al. 2023; MacMillan et al. 2020), [4] (Baccelli et al. 2024), [5] (Locatelli et al. 2024).

Study Design

- Protocol Currently Under Regulatory Review -

18 participants followed-up for 12 months (two-cohort).

Open-label, single arm study.



V Vancomycin pre-treatment

MaaT013 administration (3 doses , after D28)

x Visit

Main inclusion criteria

- Subjects age:
  - Cohort 1: ≥ 6 - < 12 years
  - Cohort 2: ≥ 12 - < 18 years
- Allo-HSCT recipients diagnosed with Grade II to IV (MAGIC criteria) GI-aGvHD
- Refractory to steroids and either refractory or intolerant to ruxolitinib
- Karnofsky/ Lansky performance status ≥ 40
- Minimum weight of 15kg

Main exclusion criteria

- Previous lines of systemic aGvHD treatment other than CS and ruxolitinib
- Presence of uncontrolled infection or requiring antibiotics
- Any other diseases that in the opinion of the investigator would contraindicate study treatment
- cGvHD, active cytomegalovirus colitis
- Relapse/ persistent malignancy requiring rapid immune suppression withdrawal
- Severe organ dysfunction or other uncontrolled complication

Objectives

Primary endpoint:

- Safety and tolerability of MaaT013
- From inclusion to M6: Incidence of all AEs treatment-emergent AEs, serious AEs, and assessment of all safety parameters
- From M6 to M12, incidence of SAEs and AESIs only.

Secondary endpoints

- Feasibility of MaaT013 administration in paediatric population
- GI and all-organ overall response rate
- Duration of response, Overall survival
- Progression-free survival of the underlying disease and time to progression

Conclusion

The study is expected to start in May 2026 in France, Italy, Spain, and the Netherlands.

Contacts

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