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MaaT Prepares For EMA Filing, US Expansion After Positive GVHD Readout

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The French biotech plans to file for EMA approval in graft-versus-host disease in mid-2025 while starting a US Phase III trial, which the CEO said in an interview is contingent on financing.

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MaaT Pharma is planning to first target the European market with MaaT013 for acute graft-versus-host disease with gastrointestinal involvement (GI-aGVHD) as it seeks to raise money to bring its microbiome-based drug to the US market.

The Lyon, France-based biotech company announced 8 January data from the single-arm, open-label Phase III ARES trial, testing the drug as a third-line treatment among patients refractory to steroids and refractory or intolerant to Incyte's Jakafi (ruxolitinib). The trial met its primary endpoint of gastrointestinal overall response rate (GI-ORR).

Shares of the company closed up 13.2% at €9.08 ($9.35).

MaaT plans to file a marketing authorization application with the European Medicines Agency in mid-2025 and launch a Phase III trial in the US in 2025. MaaT013 is part of the company's suite of microbiome ecosystem therapies (MET), which are designed to use a full microbiome ecosystem to restore balance and maximize clinical benefits for patients with severe, treatment-induced dysbiosis in acute diseases.

Key Takeaways

- MaaT announced positive results from its Phase III ARES trial of MaaT013 in acute graft-versus-host disease with gastrointestinal involvement, including a 64% overall response rate.
- The company plans to file for EMA approval in mid-2025, foreseeing a 65% market penetration there.
- For the US, the plan is to start a Phase III study this year, but that depends on financing, which could come from investment or a partnership.

Positive Response Rate Data

Among 66 patients, there was a 62% GI-ORR at day 28, with 38% of patients experiencing a complete response (CR) and 20% experiencing a very good partial response (VGPR). Across all evaluable organs, ORR occurred in 64% of patients, with 36% CR and 18% VGPR rates. The 12-month probability of survival was 54%, with median survival not reached. There was a higher probability of survival in patients who responded at day 28 than in non-responders, with respective rates of 67% and 28% ($p<0.0001$).

The ARES data safety monitoring board confirmed MaaT013's safety in October 2023 for the first 30 patients, showing the drug was well-tolerated and did not produce increased risk of infection or treatment-related fatal adverse events. Pharmacovigilance and DSMB surveillance are ongoing.

"You can look at any product that has been providing data in GVHD – you will never see an overall response rate of 64%, so that's joyful for us, but that's super, super promising for the patients," MaaT CEO Hervé Affagard told *Scrip* in an interview. "And that's a key takeaway because here, we're talking about a totally new modality."

Seeking EMA Approval With US Expansion Plans

MaaT has an open investigational new drug application with the US Food and Drug Administration and plans to start Phase III development in the US this year. The FDA had placed a hold on the Phase III program for MaaT013 in 2022 amid concerns about the company's "pooling" approach of mixing fecal donations from multiple donors, but lifted the hold in April 2023.

In Europe, the company is forecasting potential yearly peak sales of €750m ($772.6m) in hematology-oncology overall, including €250m ($258m) for MaaT013 and €500m ($515.1m) for MaaT033, its asset for allogeneic stem cell transplant. The timeline for the MaaT013 MAA is six months ahead of what the company had originally forecast.

"Because once we had accessed the data, we formed the conviction that those data are sufficient to suit the marketing authorization," Affagard told a 9 January analyst call, adding that MaaT forecasts an initial market of about 3,000 GVHD patients and a 65% market penetration.

Expanding into the US market will depend on finding sufficient financing to do so. In the meantime, MaaT has an early-access program that it expanded into the US in December.

As of 30 September, MaaT had €27m ($27.8m) in cash with runway extending into the second quarter of 2025.

"Everything will be dictated by the fundraising," Affagard said in the interview. "Everything is more expensive in the US, and we need to protect the cash we have today."

To that end, the company is seeking financing from both investors and potential partners.

"It's going great, not only on the front of financing, but also partnerships, which would also be an accelerator," he said, noting that MaaT is in discussions with a set of partners. But investment is a potential path as well.

"The ideal investor would be a kind of crossover investor, taking us from where we are today with the ability to deploy significant financial resources so then they can help take us into the US with the appropriate amount of financing," he said.