

Microbiome-targeting medicines: Commercial launch is moving centre stage Value, access and reimbursement

Health Technology Assessment
(HTA) case study

March 2023

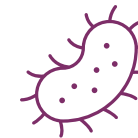
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MICROBIOME

Value,
Access
and
Reimbursement

«Will microbiome product pricing be in line with small molecules or biologics?»

This discussion is fundamentally flawed; key drivers of pricing will be unmet need, indication and standard of care.



In terms of market access and reimbursement, the most established access path exists for small molecules targeting the microbiome (modification).

Compliance with regulatory requirements \neq reimbursement success. Payers will need additional evidence.



High-value but potentially challenging for pricing & value attribution: Add-on with other treatments to enhance or complement their effect - such as in for PD1 inhibitors in oncology.

Cosmetic and consumer health positioning may not be a good enough alternative to a medicinal product – currently industry & research interest seems strongly focused on therapeutic/diagnostic*.

First HTA review of
microbiome therapies:
NICE recommends FMT
treatment approaches



NICE has recommended faecal microbiota transplant (FMT) for recurrent *Clostridium difficile* infection

Evidence levels:

- 5 RCTs
- Economic modelling

Evidence presented to the NICE Medical Technology Advisory Committee shows potential:

- Equivalence or superiority to antibiotic treatment
- Cost-savings
- Fewer antibiotics being used
- Better quality of life

NICE, England: Medical technology guidance 71 [MTG71] - Faecal microbiota transplant (FMT)

NICE National Institute for
Health and Care Excellence

In August 2022 NICE recommended FMT for the treatment of recurrent *Clostridium difficile* infections that has been treated twice or more without success

NOTE: No recommendation for “refractory” *C. difficile* infections.

NICE did not identify any in-scope RCTs comparing FMT with antibiotics for refractory *C. difficile* infections. According to clinical experts, there is no consensus on the definition of refractory *C. difficile* infection

Recommendation wording

1.1 Faecal microbiota transplant (FMT) is recommended as an option to treat recurrent *Clostridioides difficile* infection in adults who have had 2 or more previous confirmed episodes.

1.2 FMT treatment is cheaper than almost all treatment options with antibiotics. It is not cost saving compared with vancomycin taper pulse if it's given using an enema. However, FMT via enema would only be an option for the minority of people who cannot have FMT by another route.

NICE decision drivers were better efficacy and cost savings, shown in several RCTs and economic analyses



EVIDENCE TYPE

- Five randomised controlled trials (RCTs included 274 adults in total)
- Economic literature review: 8 economic studies, 1 used an NHS perspective
- Economic modelling (cohort Markov model)



CLINICAL & PUBLIC HEALTH

- Innovative treatment and new treatment approach
- Reduced need for antibiotics, fighting antimicrobial resistance
- More *C. difficile* infections were resolved with FMT than antibiotic treatment in 4 RCTs; there was no difference in 1 RCT
- Recurrence rate is comparable to or lower than with antibiotic treatment



ECONOMIC

- Economic modelling showed that FMT was cheaper than treatment with almost all antibiotics
- Considerable cost savings of £8,297 when FMT was given as oral capsules compared to vancomycin
- Cost savings of £769 for FMT through colonoscopy compared to vancomycin taper pulse
- FMT by all administration routes evaluated was cost saving in the base case
- FMT remained cost saving in the sensitivity and scenario analyses

But the Institute also identified uncertainties caused by small study sizes, heterogeneous study designs and limited long-term data



Small sample sizes and the relevance of the population to the NHS limit the evidence



Heterogeneous study characteristics may limit the evidence

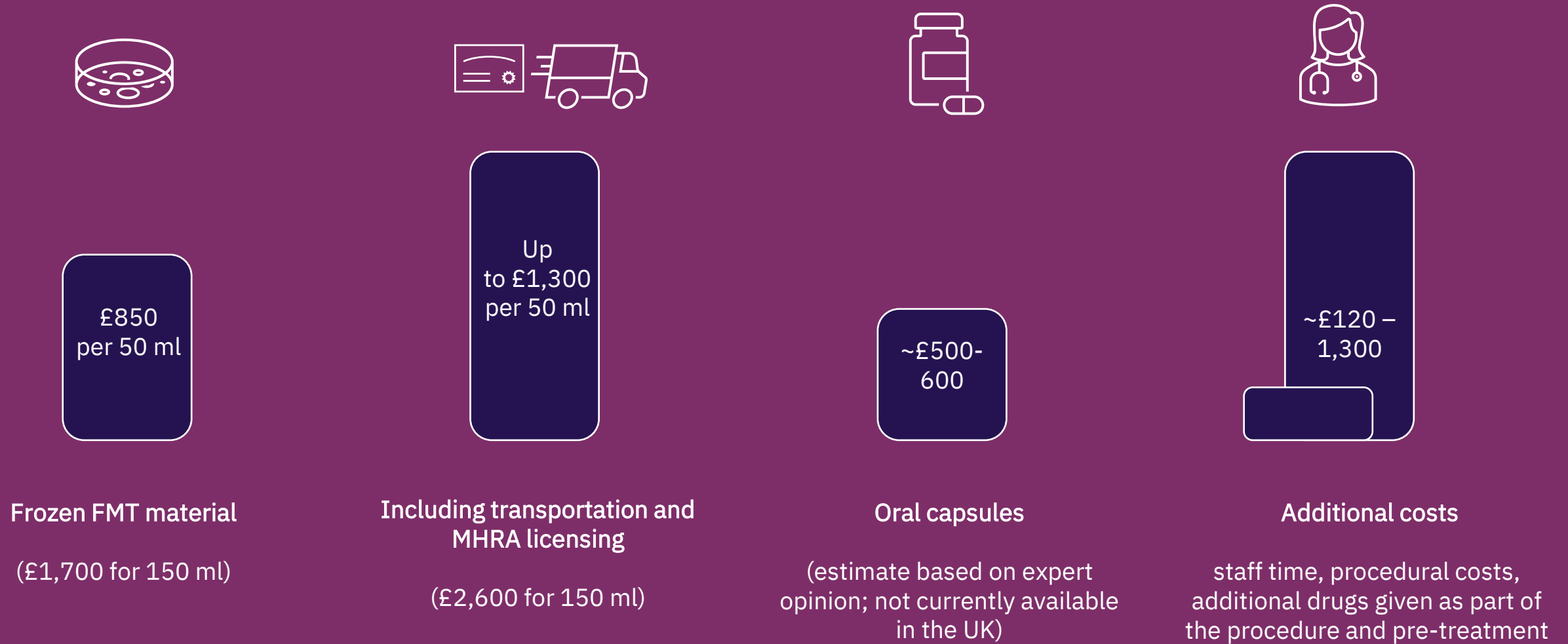
- Studies used different FMT administration routes
- None of the included trials evaluated FMT delivered via capsule, nasogastric tube or flexible sigmoidoscopy.



The quality of the clinical evidence limits the economic model

- Limited data on the long-term outcomes of FMT treatment
 - NICE encourages establishing a registry
- Need to identify potential long-term adverse events
- Reduced uncertainty in the economic model
- More RCT evidence comparing capsulised FMT with antibiotic treatment needed for a better evidence base

Economic modelling showed that FMT was cheaper than treatment with almost all antibiotics: FMT costs in the UK NHS



Thinking access & reimbursement: Payers and Health Technology Assessment bodies have requirements beyond those of Regulators



REGULATORS

PAYERS



Evidence Requirements



Get in touch for more insights on Microbiome value, access and reimbursement, including our full White Paper



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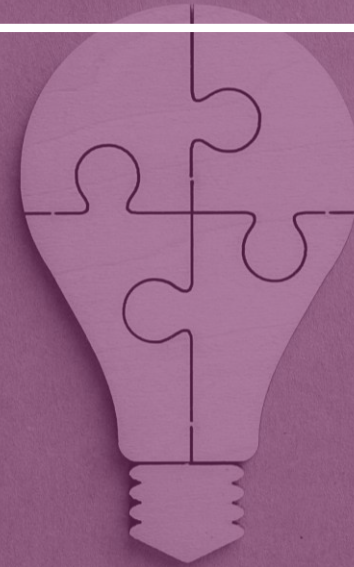
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