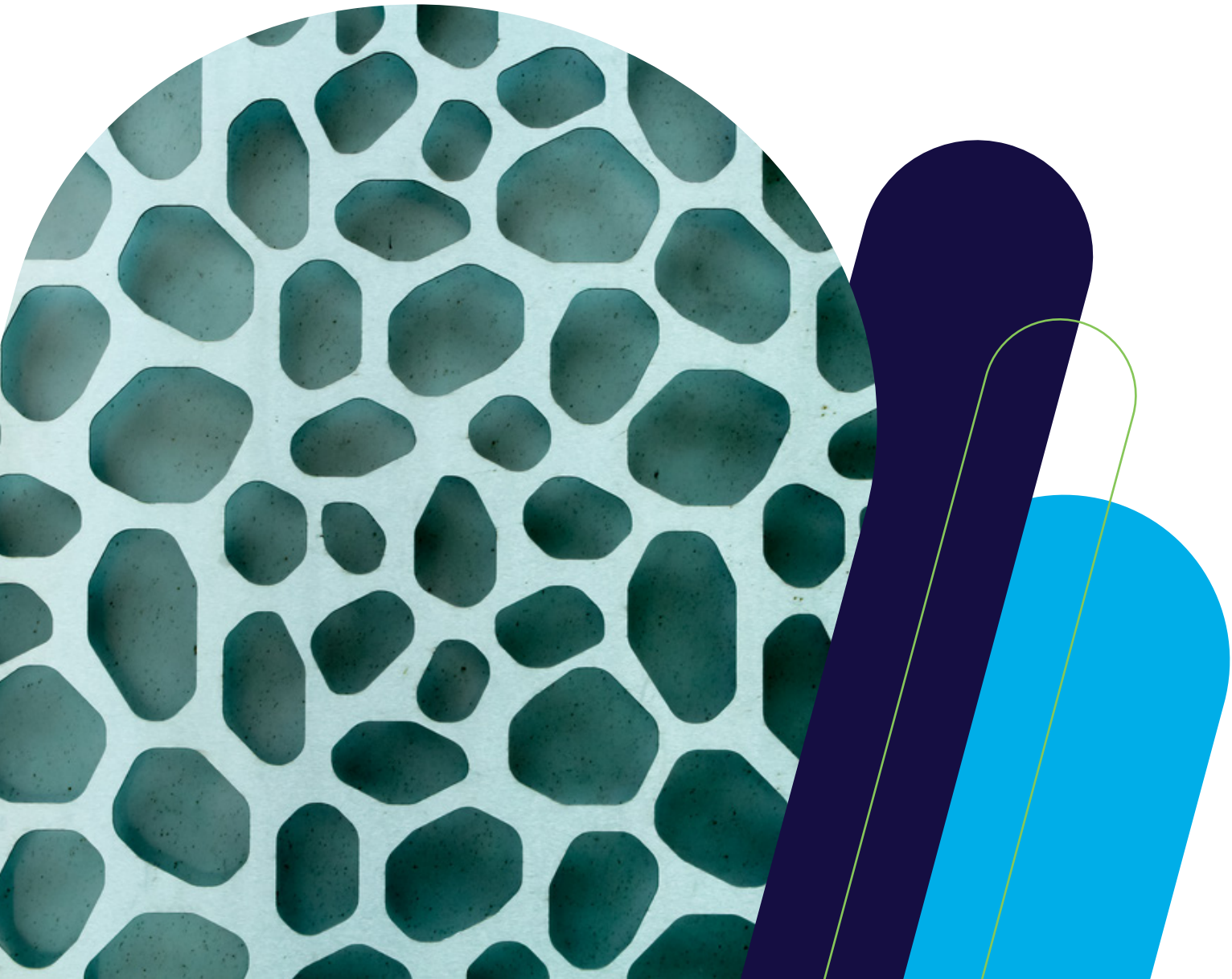
White Paper

Harnessing the Multitudes: Charting the Future of Microbiome Therapeutics

Voices from the microbiome frontier

STEFAN LUTZMAYER, Senior Consultant, EMEA Thought Leadership, IQVIA
ISMA HACHI, Senior Principal, Consulting Services, IQVIA
ISABELLE DE CREMOUX, CEO and Managing Partner, Seventure Partners
TOMAS DE WOUTERS, CEO & Co-founder, PharmaBiome



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Abstract

The FDA approved the first FMT-based microbiome drugs developed by Ferring Pharmaceuticals and Seres Therapeutics in November 2022 and April 2023 respectively. This milestone sent waves of enthusiasm through the entire field who aims to bring the human microbiome and its modulation as an attractive modality into the spotlight. Two years later, it is time to reflect on the impact these approvals have had on patients and how, going forward, companies want to harness the power of the microbiome for better health outcomes. This paper consolidates industry insights and company perspectives on the microbiome, providing a comprehensive overview of trends in the microbiome field. It also provides an analysis of the challenges raised by microbiome stakeholders, as well as the key success factors shaping the field. By addressing these challenges through a focus on broad therapeutic potential and innovation, fostering ecosystem collaboration, building stakeholder trust, embracing patient-centric approaches, and pursuing regulatory clarity, the microbiome field is poised to overcome its obstacles and unlock its immense potential.

Introduction — quo vadis microbiome

As the microbiome therapeutics field matures, companies, regulators, and investors face a shared challenge: how to turn scientific potential into scalable products, with credible health impact. Across the industry, there is growing alignment around the therapeutic potential of the microbiome. A wave of scientific advances, clinical progress, and regulatory recognition is helping the field transition from early exploration to focused, evidence-based product development and validation. Companies are advancing from live bacterial consortia to targeted microbiome modulation, supported by increasingly sophisticated data analytics and translational models. At the same time, efforts to harmonise clinical trial design, manufacturing standards, and regulatory pathways are gaining traction — laying the foundation for broader adoption and impact.

This paper examines how the field is navigating that complexity. Drawing on clinical and pipeline data, as well as interviews with leaders across biotech, investment, and regulation, we explore how companies are redefining success, focusing pipelines, and building stakeholder trust. Their perspectives are integrated throughout the analysis to ground our findings in the realities of development and commercialisation.

Our focus is on microbiome-modulating therapies currently in development, including live biotherapeutic products, rather than the broader microbiome space of diagnostics, nutrition, or wellness products (Box 1). We aim to equip biopharma strategists, innovation leaders, and investors with a clear-eyed view of what's working, what's stalling, and where the greatest opportunities now lie.

Box 1: Mapping microbiome therapeutics

Microbiome-modulating therapies span a continuum — from minimally processed microbial communities to highly defined biological products.[1] Each therapy must be assessed based on its degree of characterisation, control, and intended use.

Faecal microbial transplantation (FMT)
Minimally processed stool-derived material, typically from screened donors. Donor origin plays a major role in benefit-risk assessment. Often, made at the hospital, variable composition; limited characterisation and control.

Microbiome ecosystem therapies
Medical products prepared at industrial scale following reproducible industrial manufacturing process and according to consistent quality, safety and efficacy standards. These products are of standardised consistency and are intended to be distributed to patients on a large scale and, therefore, need marketing authorisation to be commercialised.

Live biotherapeutic products (LBPs)
Engineered or defined consortia of live microorganisms developed as drugs, typically for specific clinical indications. Regulated as biologics or drugs.

Probiotics, prebiotics and postbiotics
Found in food, supplements, or consumer health products. Generally, not intended to treat disease; regulated as food or dietary supplements.

Non-living biotherapeutics and phage therapies
Include inactivated microbes or bacteriophages. No live organisms; offer targeted action with full product control. See our recent blog post "The good virus — phage therapeutics" for more details.

Microbiome innovation

Building on the first pivotal regulatory approvals, the microbiome field has entered a new phase of innovation that prioritises mechanistic rigour, validated endpoints, and translational potential.

After years of exploratory hype, developers are sharpening their focus on programmes that demonstrate reproducibility, scalability, and regulatory readiness.

A central part of this shift involves moving beyond observational studies toward interventions grounded in defined biological mechanisms. One such area of promise is the identification of disease-related microbial signatures — particularly within the gut microbiome — which is increasingly viewed by researchers and early-stage developers as a tool for patient stratification and therapeutic targeting. This is especially under investigation in fields like immuno-oncology, inflammatory bowel disease, and metabolic disorders, where microbial composition may influence disease progression or treatment response. Companies like GMT Science and BioCorteX are taking innovative approaches. GMT Science applies AI-driven metagenomic profiling to predict response to immunotherapy in oncology, while BioCorteX's Carbon Mirror™ platform models host–microbiome–drug interactions to optimise therapeutic strategies.[2,3] Both are working toward clinically validated applications with defined mechanisms and measurable outcomes — an essential step for credibility in microbiome therapeutics.

Meanwhile, consumer health companies such as ZOE have found faster traction by offering microbiome-based wellness insights. ZOE's PREDICT studies leverage large-scale multi-omics data to personalise nutrition, while Viome uses meta-transcriptomics to inform dietary

choices.[4] These platforms benefit from rapid development cycles and looser regulatory constraints, enabling them to reach market and users quickly. While they don't meet the evidence standards of prescription drugs, they illustrate public appetite and commercial viability — albeit in a lower-stakes context.

On the research and platform side, companies such as Sequentia Biotech and Cmbio are helping drive the field forward with scalable, data-rich infrastructure for microbiome discovery and clinical translation. While Sequentia has supported numerous omics-based studies through its bioinformatics platforms, Cmbio, a newly formed European consortium, brings together leading microbiome CROs with a strong publication record and experience supporting clinical and therapeutic development.

Progress towards clinical-grade diagnostics will rely on linking microbial composition, function, and patient outcomes through high-quality, contextualised data. Ultimately, such predictions must then be validated clinically and adhere to strict regulatory compliance. Metabolomics and proteomics can further enhance understanding by analysing the metabolite and protein expressions of microbes, providing a deeper insight into their functional roles in health and disease. Though beyond the scope of this paper, phage therapeutics — a modality leveraging bacteriophages to selectively modulate microbial communities — are gaining renewed attention for their precision and scalability. Interested readers can explore this topic further in our dedicated blog post.[5]

Clinical and translational progress

The landscape for microbiome therapeutics is marked by steady clinical advancement. Developers are building on the first approvals in treating recurrent *Clostridioides Difficile* Infections (CDI) through additional evidence-based programs.

Microbiome research is also expanding beyond the gut to include other microbial environments such as the skin and vaginal microbiomes, as well as emerging applications in animal health and agriculture.

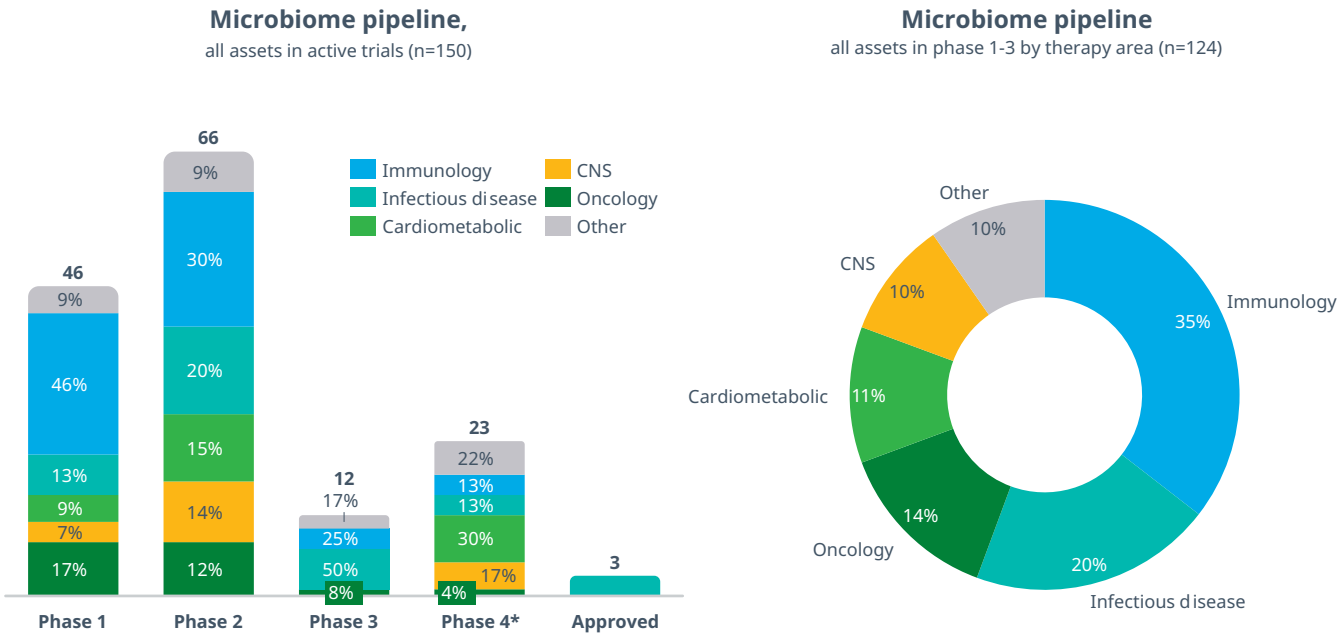
Several late-stage trials now show promising data across (haem-) oncology, infectious, inflammatory, and metabolic indications; thus, bringing the field closer to broader credibility and regulatory confidence.

Microbiome pipeline

As shown in Figure 1, over 150 microbiome therapeutics are currently in clinical development, with the majority focused on immunology, infectious disease, oncology, and cardiometabolic conditions. Emerging biopharma (EBP)* continues to drive most of the pipeline activity, supported by a strategic narrowing of focus to financially viable assets and validated endpoints. In parallel, 23 clinical trials in phase 4 are ongoing, highlighting the field's growing maturity and the need for real-world validation of safety and efficacy. These late-stage studies span a diverse range of conditions, from functional constipation and metabolic disorders to (haem-) oncology, neurodevelopmental and mental health indications. Importantly, many of these trials include both prescription microbiome therapeutics and evidence-generating studies on probiotic formulations.

*Emerging Biopharma Companies (EBPs) — defined as those with less than an estimated $200Mn in R&D spend per year, including those 'pre-commercial' companies with no revenue as well as smaller emerging companies with up to $500Mn per year in annual revenue. Large and-mid-sized companies — those with more than $10Bn and more than $5Bn, respectively, in global revenue.

Figure 1: Microbiome Pipeline



*Includes probiotics
Source: Citeline Trial Trove Q4 2024, company websites

Recent readouts reinforce this trajectory. MaaT Pharma's MaaT013, a donor-derived microbiome therapeutic for acute Graft vs Host Disease (GvHD), achieved positive phase 3 results — exceeding its primary endpoint — and the company has now submitted a marketing authorisation application to the EMA.[6] The other leading product from Vedanta is VE202, a cocktail of bacterial strains. Unfortunately, the company announced that it failed to meet the primary endpoints in a phase II ulcerative colitis (UC) trial.[7] The company is now analysing the full clinical dataset to identify potential patient responder subgroups and to explore selection or stratification approaches for future UC trials. These insights are relevant not only to Vedanta but also to peers such as MRM Health (MH002), which are pursuing biotherapeutic approaches for UC. Together, these programmes are helping to validate the broader therapeutic promise of the microbiome across different scientific approaches.

ASCO 2025 strongly emphasised that the gut microbiome is both modifiable factor and a potential therapeutic target. During a panel discussion, physicians underscored the importance of microbiome-focused strategies as antibiotics usage during Immune Checkpoint Inhibitor (ICI) therapy was associated with poorer outcomes and preserving the gut microbiome should be a strategic priority for oncologists.[7]

Microbiotica, for example, is developing microbiome-based therapeutics and biomarkers designed to enhance ICI response in oncology, with clinical programmes grounded in datasets that link microbial profiles to patient outcomes, which underscores the field's therapeutic potential in immuno-oncology.

There is also growing interest in understanding the role of the microbiome in cardiometabolic health and obesity. Characteristics of the gut microbiome have been linked to obesity. A recent meta-analysis of 10 trials involving 334 patients revealed significant negative correlations between faecal microbiota transplantation (FMT) and abdominal adiposity, caloric intake, fasting glucose, insulin resistance, blood pressure, and total cholesterol.[8] This suggests potential benefits, especially for obese individuals with

metabolic complications. The Akkermansia Company (formerly A-Mansia Biotech, acquired by Danone in July 2025) has advanced this field significantly. *Akkermansia muciniphila,* a keystone gut bacterium, has been shown in a randomised controlled trial to improve insulin sensitivity, reduce cholesterol, and lower inflammation in overweight and obese individuals when administered in pasteurised form.[9] This pasteurised version received novel food approval from the European Food Safety Authority (EFSA) in 2021, and the recent acquisition by Danone, signals increased commercialisation traction. Moreover, gut bacteria composition can affect weight loss success, possibly through increased GLP-1 secretion.[10]

A microbiome-based approach may present an interesting potential alternative to GLP-1 receptor agonist drugs. In a small study sponsored by Pendulum Therapeutics in people with type-2-diabetes, administration of five bacterial strains including *Akkermansia muciniphila* improved glucose control.[11] This probiotic method is suggested to naturally boost GLP-1 production. While it is an attractive concept, more human clinical trials are necessary to understand how bacterial strains influence GLP-1 levels and metabolic health. In contrast to this probiotic approach, Zehna Therapeutics is pursuing a prescription drug pathway developing non-bactericidal inhibitors of specific gut microbial enzymes to treat cardiometabolic diseases, with chronic kidney disease as a lead indication.[12] It is worth mentioning that there are some controversies about IP ownership in this field and that in February 2025, there was a judgment on the IP dispute between Pendulum and The Akkermansia company in favour of the latter.

These examples illustrate the growing translational potential of microbiome-based interventions in metabolic disease and underscore the scientific and commercial interest. While it is crucial to differentiate between consumer products and those undergoing rigorous clinical trials for regulatory approval as drugs, it is also important to acknowledge that many consumer health products are actively generating clinical evidence to support their claims. Though regulatory thresholds for efficacy differ significantly, the ongoing research and development in both sectors contribute valuable insights and pathways for future microbiome-based solutions.

The gut and oral microbiomes are increasingly recognised as potential contributors to neurodegenerative diseases and may serve as early indicators of disease risk and progression.[13]

In Alzheimer's disease, substantial evidence has implicated oral bacteria such as *Porphyromonas gingivalis*, whose neurotoxic metabolites (gingipains) have been directly linked to Alzheimer's pathology, inflammation, and cognitive decline.[14,15] Preclinical and clinical evidence suggests a role for the microbiome also in Amyotrophic Lateral Sclerosis (ALS). Excitingly, a recent phase 1b trial of MaaT033 showed a slower rate of disease progression-although the sample size was limited.[16] Similarly, in Parkinson's disease, dysbiosis of gut microbiota and altered microbial metabolite profiles have been associated with inflammatory pathways, disease onset, and progression.[17] Clinical studies continue to highlight specific microbial metabolites and bile acid profiles as potential biomarkers or targets for therapeutic modulation. Emerging therapeutics targeting these microbial mechanisms (e.g., Axial Therapeutics, Ultimate Medicine, Piton Therapeutics) underscore the potential of microbiome modulation as a therapeutic approach for neurodegeneration.

The dynamic interplay between the microbiome and women's health is a rapidly expanding area of research with significant therapeutic promise.[18]

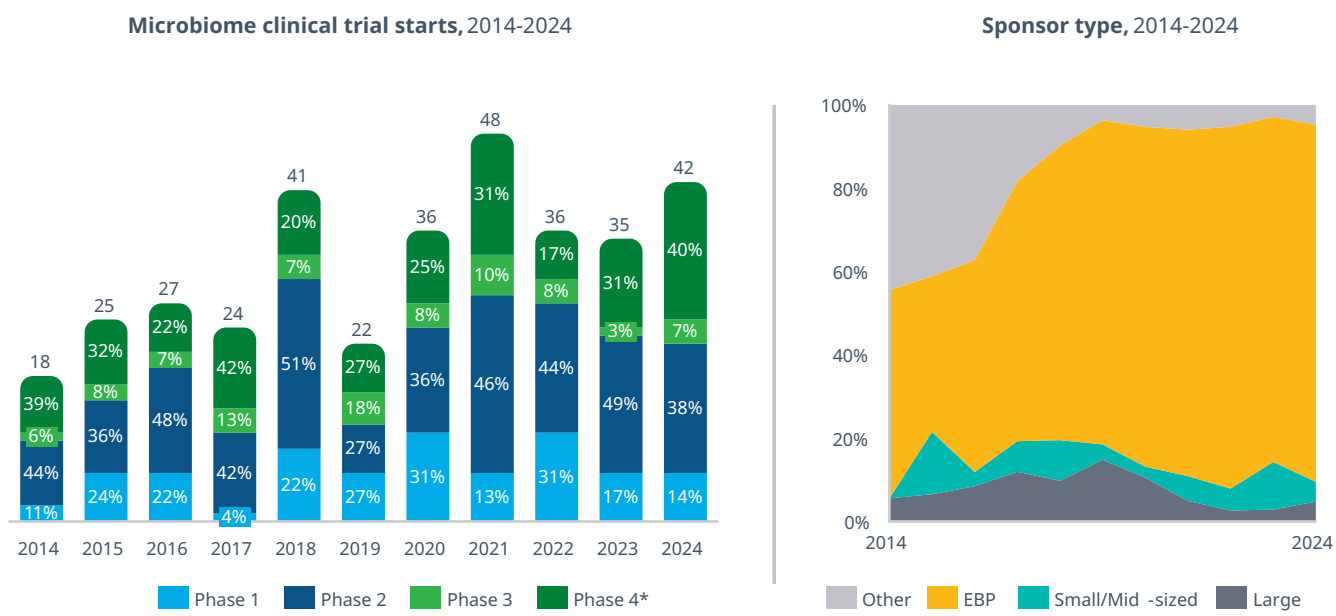
Significant progress is being made in the vaginal microbiome space, with Osel Inc. leading the field with its product Lactin-V, which is currently in phase 3 clinical trials for the prevention of recurrent bacterial vaginosis, demonstrating the strong translational potential in this area.[19] Nevertheless, microbial influences on women's

health extend far beyond the reproductive tract. The gut microbiome plays a critical role in hormonal regulation via the estrobolome — a collection of gut microbial genes capable of metabolising oestrogens — as well as in bone metabolism, immune modulation, and mental well-being. Dysbiosis in gut communities has been implicated in female-specific conditions such as Polycystic Ovary Syndrome (PCOS) and endometriosis.[20] In addition, the gut-brain and gut-hormone axes are increasingly recognised as key mediators of gender-specific health outcomes.

Clinical trial data

The total number of clinical trial starts has stabilised and returned to pre-pandemic levels.[21] Microbiome-related trial activity shows a similar trend. In 2021, there was a peak in the total number of trials starts followed by a decline in the subsequent two years and has levelled off at 42 last year (Figure 2 left panel). Across all therapeutic areas, EBP are the leading source of biopharma innovation and accounted for 63% of global industry-sponsored clinical trial starts in 2024.[15] In microbiome, this is even more pronounced and EBPs had a share of over 85% last year (Figure 2 right panel). Small, mid-sized and large pharma accounted only for less than 10% of clinical trial starts.

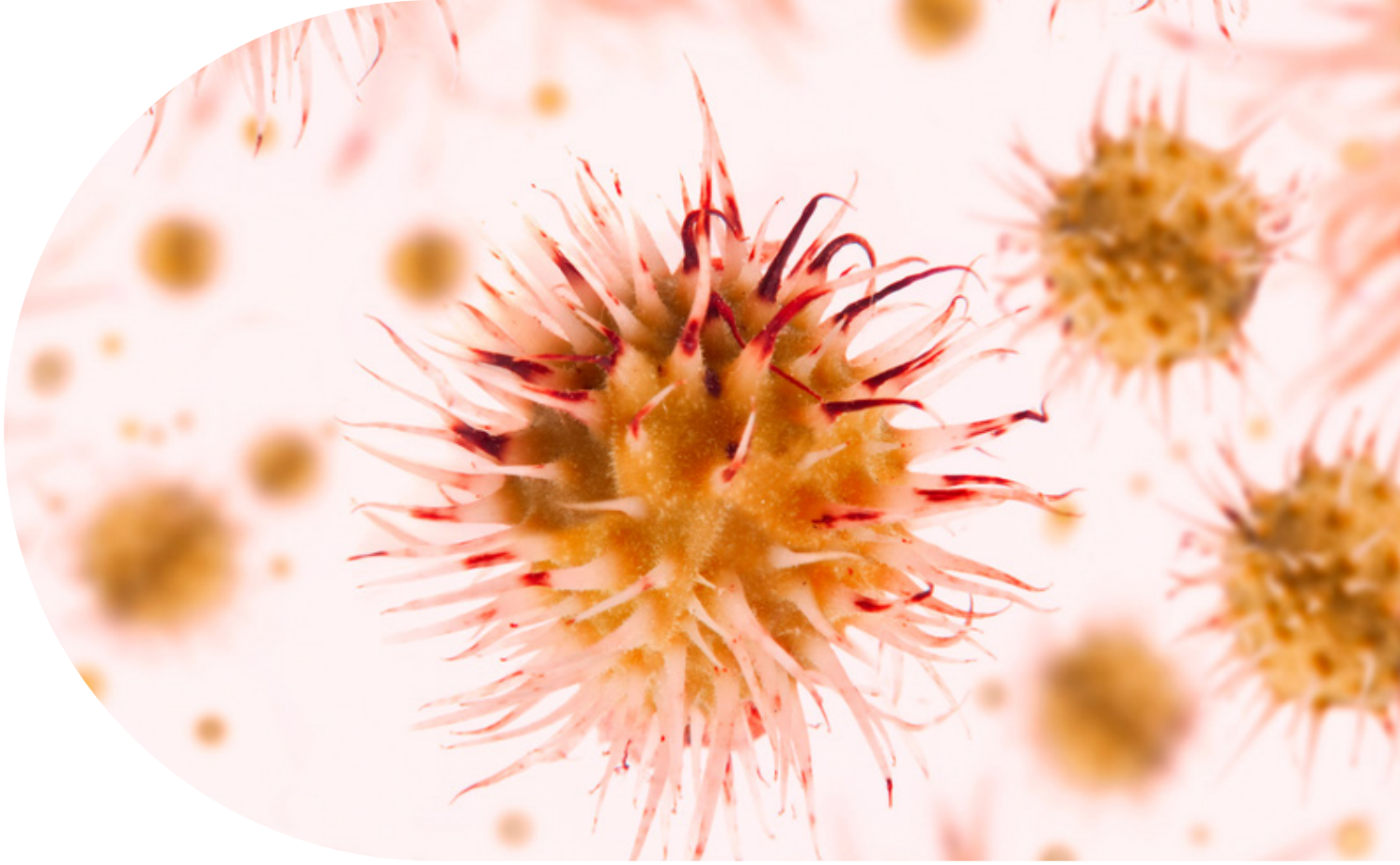
Figure 2: Microbiome clinical trial activity



*Includes probiotics
Source: Citeline Trial Trove Q4 2024; IQVIA EMEA Thought Leadership

Alongside early-phase development, a notable share of microbiome trials — between 17% and 40% annually since 2014 — are phase 4 studies also including probiotics. They often include interventions for e.g., gastrointestinal disorders, metabolic health, or immune support and are commonly sponsored by academic groups, hospitals, or public-private consortia, rather than industry. This trend underscores a dual dynamic in the field: while innovation is largely led by emerging biotech, clinical validation efforts are maturing in parallel through robust real-world evidence generation.

Despite the promising clinical progress, microbiome experts consistently highlighted ongoing challenges, particularly the need for harmonised trial methodologies. Interviewees emphasised that inconsistent approaches to sample handling, patient selection, and endpoint validation can weaken evidence comparability and delay regulatory acceptance. They see addressing these inconsistencies as critical for moving microbiome therapeutics from clinical promise to widespread medical practice.



Collaboration and regulatory alignment

The microbiome therapeutics field is rapidly maturing scientifically, but collaboration remains essential to accelerate translation into clinical and commercial success. Current microbiota diagnostics and clinical protocols lack consistent standards, posing challenges for regulators, clinicians, and innovators alike.

Experts interviewed broadly agreed on the urgent need for harmonisation across microbiome trial methodologies, sample handling, and data analysis practices.

Initiatives like the Microbiome Therapeutics Innovation Group (MTIG) in the U.S. and the European Microbiome Innovation for Health (EMIH) aim to directly address these gaps.[22,23] By fostering dialogue between academia, industry, and regulatory bodies, these groups are creating frameworks to speed up innovation, incentivise investment, and accelerate patient access.

Similarly, the International Human Microbiome Standards (IHMS) project has made strides in developing standard operating procedures from sample collection to data analysis, an essential foundation for robust and reproducible evidence generation.[24] Europe, currently lagging in microbiome-specific regulation, is actively catching up. The Pharmabiotic Research Institute (PRI), a non-profit alliance of microbiome-focused companies and researchers, plays a central role in facilitating dialogue with regulators and advancing frameworks for clinical-grade microbiome therapeutics. Its work is particularly relevant to harmonising standards around live biotherapeutic products (LBPs) and aligning with the EU's upcoming Substances of Human Origin (SoHO) regulatory framework.[25]

The SoHO now formally includes human-derived microbiome samples. When it enters into force in 2027, SoHO will require that any material sourced from the human microbiome and used in therapeutic applications comply with stringent donor screening, quality control, and traceability requirements. Developers using such materials will need to register as authorised SoHO entities and undergo product-specific authorisation processes.[26]

During our sessions with microbiome experts, regulatory clarity emerged as one of the field's most urgent needs. Interviewees pointed to persistent regional discrepancies and the lack of microbiome-specific guidelines as key barriers, complicating trial design, delaying approvals, and stalling investment. That said, many praised the FDA's constructive engagement with developers, particularly in advancing frameworks for LBPs. The EMA is also making strides, with stakeholders acknowledging growing receptiveness and alignment efforts in Europe.

Encouragingly, regulatory momentum is also building in Europe. The submission of Xervyted (MaaT013) to the EMA is a case in point. Likewise, the EU's forthcoming SoHO regulation, set for 2027, explicitly addresses microbiome-based therapies, promising a more harmonised regulatory landscape. Recent comments from the FDA leadership explicitly recognising microbiome's therapeutic significance signal confidence for the entire field.[27]

This progress, coupled with a clear stakeholder call for harmonisation, positions the microbiome therapeutics ecosystem at an inflection point. Collaborative efforts across academia, industry, and regulators will be crucial to translating the field's scientific potential into sustainable, scalable healthcare solutions. One example illustrating the potential of such cross-sector collaboration is the Seerave Foundation. This international non-profit family foundation focuses on improving cancer outcomes by investigating the role of the gut microbiome in modulating immune responses, particularly in relation to immunotherapy. Supported by emerging scientific evidence, including studies published in Nature Medicine, Seerave-backed research has identified associations between specific gut bacteria and improved responses to immune checkpoint inhibitors in melanoma patients. The Foundation funds research into microbiome-related biomarkers, modulation of the microbiome by diet and medications, and interventional strategies such as live bacterial products and faecal microbiota transplantation. Seerave regularly convenes globally leading stakeholders from these disparate fields of science and medicine to foster advancements that come from their shared insights.

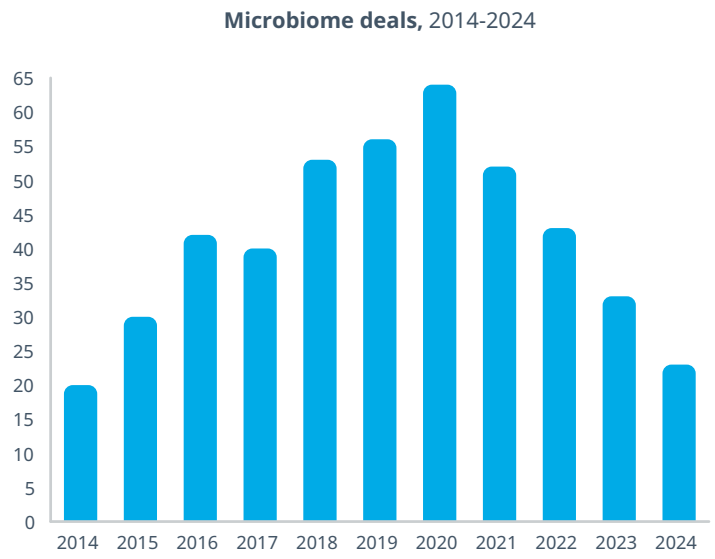
Navigating investor caution to unlock microbiome opportunities

Investment in microbiome therapeutics has experienced a significant evolution over recent years. As with many emerging therapeutic fields, investors approach the microbiome sector cautiously — attracted by profound scientific promise, yet wary of complexity, uncertain timelines, and perceived immaturity of the market.

Stakeholders interviewed consistently highlighted common challenges faced when attracting investment, particularly misconceptions arising from decades of unproven consumer probiotic products, complexity of the science, and unclear regulatory pathways. This confusion often makes it challenging for investors to differentiate clinically validated microbiome therapeutics from general wellness probiotics, creating additional barriers to investment decision-making.

Despite these acknowledged barriers, there are meaningful signs of investor engagement and growing financial confidence in carefully selected microbiome opportunities. While microbiome deal activity has declined since its peak in 2021 (Figure 3), recent examples like MaaT pharma raising €37.5m, Enterome raising $19m, EnteroBiotix raising £27 million, as well as successful funding rounds for innovators such as Siolta, Holobiome, Ancilia Biosciences, Microbiotica, Neobe Therapeutics, and many others indicate that credible therapeutic propositions can secure substantial investor backing.[28,29]

Figure 3: Microbiome deal activity



Microbiome investments & deals, selection

Companies	Operation	Scope / Objective
Nestlé Health Sciences / Seres Therapeutics	Asset acquisition	Nestlé Health Sciences acquires global rights to VOWST™
Clinical Microbiomics / Cosmos ID / MS-Omics / DNASense / Microba R&D	Merger	Formation of the Cmbio brand
EnteroBiotix	Investment	Funding to advance clinical programmes
Programmes Biosciences	Investment	Investment to develop microbial therapies for bacterial vaginosis

Source: IQVIA Pharma Deals; Desk research

Notable strategic partnerships also highlight growing interest. For example, Nestlé Health Science acquired full global rights to VOWST from Seres Therapeutics at the end of 2024, marking a major endorsement of the commercial potential of microbiome-based therapeutics and signalling mainstream confidence in the field's long-term viability.[30] Furthermore, the creation of Cmbio — a consortium of specialist microbiome and metabolomics/metagenomics service providers — illustrates increasing sector maturity and collaboration readiness.[31]

Encouragingly, investor confidence in the microbiome field continues to build.

High-quality companies across the ecosystem are securing capital through a combination of venture investment and non-dilutive grant funding — reflecting both scientific progress and diversified funding strategies. Several family offices and specialised investors have entered the space, drawn by the convergence of microbiome science with precision medicine and personalised health. Stakeholders point to venture firms such as Seventure Partners, whose proactive approach has helped boost sector credibility and support the growth of clinically focused microbiome companies. Investments in firms such as MaaT Pharma, now advancing toward commercialisation following positive phase 3 results, highlight the ability of well-validated microbiome assets to attract long-term capital and strategic backing.

Going forward, interviewees expressed cautious optimism, emphasising the importance of showcasing successful clinical outcomes, clear regulatory paths, and cross-sector collaborations to increase investor confidence. Transparent communication of microbiome therapeutic successes and clearer differentiation from general probiotic products will be crucial to sustain investor momentum. Overall, the investment landscape, while understandably cautious, is gradually shifting towards greater confidence, informed by robust scientific evidence, validated clinical progress, and improving regulatory clarity.

Given the increasing number of clinical trials currently underway for live biotherapeutic products (LBPs), manufacturing is a strategic priority for microbiome therapeutics. Producing therapies that contain living microorganisms presents unique challenges, requiring specialised facilities, cold chain infrastructure, complex fermentation and purification processes, and rigorous quality assurance. Despite these complexities,

significant strides have been made in scaling up production and ensuring product stability, bringing more LBPs closer to patients.

For instance, the achievement of regulatory approvals for certain oral LBPs underscores the industry's growing capability to produce complex microbiome therapies at commercial scale and meet stringent regulatory requirements. Contract Development and Manufacturing Organizations (CDMOs) like Biose Industrie, Sacco, LIST and Wacker Biotech (with its LIBATEC® platform) have also played a crucial role. They have developed specialised expertise in handling anaerobic strains, optimising fermentation regimes, and ensuring GMP-compliant production for a wide range of LBPs, including single strains and multi-strain consortia.

Interviewees stressed that ensuring safety, purity, potency, and contamination control demands advanced technologies and significant investment in infrastructure and technical expertise. As one industry leader noted, manufacturing capabilities are no longer simply operational — they are now key differentiators and critical enablers of regulatory approval and commercial viability.

Despite these challenges, stakeholders remain optimistic. Companies that proactively invest in specialised production capacity, resilient supply chains, and stringent quality systems will not only accelerate approval timelines but also position themselves more competitively in a rapidly evolving market.

Harnessing the potential of the microbiome: Key success factors for advancing innovation

Having mapped the state of the microbiome field— its clinical momentum, regulatory shifts, investment dynamics, and scientific progress — it is clear that sustained success depends on coordinated execution. The following eight success factors represent enablers of individual product success, as well as levers for system-wide maturity. They are drawn from both stakeholder input and strategic analysis and represent the capabilities and coordination the field must build now to transition from potential to impact (Figure 4). The microbiome pipeline is particularly robust, with multiple microbiome products demonstrating highly encouraging emerging results in phase 3 clinical trials. While not every product reaches approval, the accumulating data from pioneers such as MaaT Pharma (with positive Phase III outcomes for MaaT013 in graft-versus-host disease and EMA submission in June 2025), Enterome (advancing OncoMimics™ in cancer), Seres (whose VOWST received FDA approval in April 2023), and Ferring (with an FDA approval in November 2022) collectively underscore the immense promise of the field. Parallel to this, the consumer health sector is increasingly prioritising clinical validation to support its product claims, reflecting a broader industry shift towards a more evidence-based approach.

Figure 4: Key success factors

Attracting sustainable investment: To advance microbiome-based therapies, companies must attract investors by clearly communicating their clinical and economic benefits, leveraging early successes, and forming strategic partnerships.

Establishing value-based pricing models: Developers of microbiome therapies must align pricing with clinical and economic value, using value-based models to ensure patient access and payer support, to clearly articulate long-term benefits for broad adoption.

Fostering stakeholder trust and education: To ensure the adoption of microbiome therapies, developers must gain trust through transparent communication, distinguish rigorously tested therapies from general probiotics, and increase awareness.

Scaling manufacturing and building resilient supply chains: Successfully commercialising and scaling microbiome therapies requires addressing manufacturing and supply chain challenges, developing specialised facilities, partnering with CDMOs and ensuring robust quality management for resilient supply chains.

Generating high-quality evidence: Advancing microbiome-based therapies requires robust evidence, standardized protocols, and advanced technologies to ensure reliable research and innovative, data-driven solutions.

Embracing patient-centric development and access: To ensure the success of microbiome therapies, developers must prioritize patient-centered strategies, involving patients early, educating them, and focusing on clinical and economic value from their perspective.

Building regulatory clarity and alignment: Clear and harmonised regulatory guidelines, along with early engagement with agencies and international cooperation, are essential for accelerating the development and approval of microbiome-based therapies.

Collaboration across ecosystems: Collaboration among academia, industry, and regulatory bodies is crucial for establishing infrastructure, standardising practices, and translating scientific discoveries into scalable microbiome-based healthcare solutions.

Source: IQVIA EMEA Thought Leadership

1 Attracting sustainable investment

The microbiome field needs capital aligned with long innovation cycles and translational complexity. Strategic investors — including family offices and life science VCs — can bridge the funding gap between early science and scalable product platforms. To secure this capital, companies must clearly communicate not only clinical milestones but also the platform economics of microbiome development. Public-private partnerships and blended financing models can further de-risk early-stage ventures.

2 Generating high-quality evidence

Credibility in microbiome therapeutics requires more than data; it demands consistency, comparability and clinical context. Harmonising trial methodologies, sample processing protocols, and microbial analytics will enable cross-trial synthesis and accelerate regulatory trust. The field also needs to move toward endpoints that reflect microbial mechanism, not limited to symptomatic improvement alone. Multi-omics and systems biology approaches will be most effective when embedded in reproducible frameworks.

3 Establishing value-based pricing models

Reimbursement will hinge on proving differentiated clinical and economic value. Value-based pricing models — anchored to outcomes like recurrence prevention or reduced healthcare utilisation — can support payer adoption. Companies should work with payers early to design post-approval evidence plans or pilot reimbursement agreements tied to therapeutic performance. Clear articulation of the long-term benefits of microbiome-based therapies will be essential to gaining reimbursement approvals and driving adoption in healthcare systems worldwide.

4 Embracing patient-centric development and access

Patient relevance must be built into therapeutic design from the outset. This includes incorporating patient-reported outcomes, designing for usability and adherence, and involving patient organisations in education and trial awareness. Additionally, focusing on clinical and economical value from a patients' perspective will ensure that these groundbreaking therapies reach the patients who need them most, fostering broader acceptance and adoption.

5 Fostering stakeholder trust and education

Gaining the trust of regulators, clinicians, patients, investors, payers and the public is essential for the adoption of microbiome-based therapies. Transparent communication of clinical evidence, combined with efforts to distinguish rigorously tested therapies from general wellness probiotics, can help dispel misconceptions. Educational initiatives tailored to patients and healthcare providers will further build confidence and promote informed decision-making. Advocacy groups and healthcare providers can serve as key partners in bridging the gap between scientific innovation and real-world application.

6 Building regulatory clarity and alignment

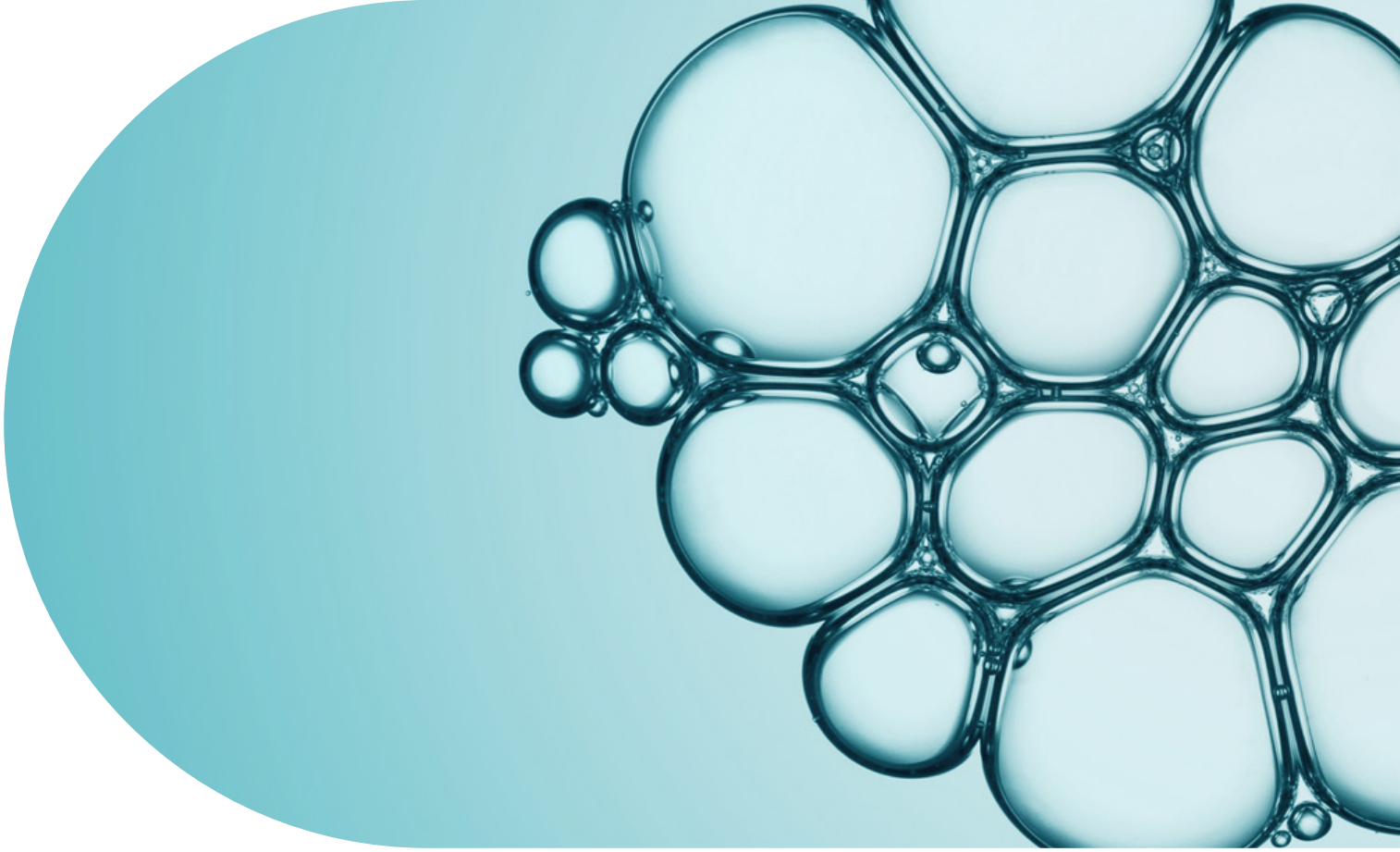
Clear and harmonised regulatory guidelines are critical to accelerating the development and approval of microbiome-based therapies. Engaging early and consistently with regulatory agencies can help define transparent requirements for products such as live biotherapeutic products (LBPs) and microbiota transplantation therapies. At the same time, promoting international harmonisation of regulatory frameworks will streamline global development and market entry, reducing uncertainty for developers.

7 Embracing patient-centric development and access

Manufacturing is a strategic capability, not merely an operational task. Addressing manufacturing and supply chain challenges early is essential, involving specialised facilities, partnerships with Contract Development and Manufacturing Organisations (CDMOs), and innovative technologies for consistent quality at scale. Robust quality management systems are critical to ensure safety, viability, and efficacy from clinical trials through commercialisation. Because viability is a key quality attribute, many microbiome therapeutics rely on cold chain logistics. For example, Rebyota®, a donor-derived enema therapy, requires storage at –60 °C to –90 °C and must be used within five days once thawed — highlighting the complexity of handling whole-ecosystem products. Advances in formulation — such as lyophilised full-ecosystem, consortia, spore-formers, and micro-encapsulated phages — are now enabling more stable products at 2–8 °C or even ambient temperatures. Early investment in such innovations will be key to reducing costs, improving global access, and building resilient supply chains.

8 Collaboration across ecosystems: building a foundation for microbiome innovations

Collaboration among academia, industry, and regulatory bodies is essential to establishing the infrastructure required for the microbiome field to flourish. Partnerships can drive progress by defining clear regulatory frameworks and standardizing best practices for the development, approval, and commercialization of microbiome-based products. Interdisciplinary efforts also promote harmonised methodologies for research protocols, such as sample collection, processing, and data analysis, ensuring reproducibility and comparability across studies. By aligning stakeholders, these collaborative ecosystems can streamline the translation of scientific discoveries into scalable, real-world solutions. Such cooperation is critical to overcoming the unique challenges of the microbiome field and unlocking its transformative potential in healthcare.



Conclusion

The human microbiome has been implicated in everything from basic immunity to metabolic disease, from neuroinflammation to cancer response. Its therapeutic promise touches nearly every domain of modern medicine. And yet, outside of *Clostridioides Difficile* Infections (CDI), we are still waiting for the data to fully catch up with this vision.

Like any emerging modality, microbiome therapeutics must now pass through the hard middle: the space between early enthusiasm and durable proof. The field is consolidating. Funding is selective. Pipelines are narrowing to where signals are strongest. This is not retreat: it's maturation.

Success will come not from novelty alone, but from the ability to coordinate across data, delivery, and disciplines. This paper has charted the contours of that future. What remains is to harness the multitudes — strategically, rigorously, and with patients in mind.

Acknowledgments

The authors would like to thank the many experts across industry, academia, and investment who generously shared their perspectives through interviews and discussions during the development of this paper. Their insights were invaluable in shaping the themes and recommendations presented here.

We are particularly grateful to the following individuals for their time and thoughtful input: Cameron Rodd (Ferring Pharmaceuticals), Bertrand Rodriguez (Gnosis by Lesaffre), Jonathan Chriqui (MaaT Pharma), Pierre Belichard (Enterome), and Cristina Blanco (IQVIA).

We also thank Julia Levy (IQVIA) and Denise Kelly (Seventure Partners) for their valuable input during the development of this paper. Their thoughtful contributions were instrumental in refining its framing, focus, and overall direction.

About the authors

STEFAN LUTZMAYER
Sr Consultant, EMEA Thought Leadership, IQVIA

Stefan has over 9 years of experience working in academia and life sciences. He joined the Thought Leadership team in 2021 where he creates novel materials on emerging next-generation biotherapeutic platforms like the microbiome or gene-editing, new developments across therapeutic areas, including central nervous system or cardiometabolic diseases as well as vaccines and cold chain pharmaceuticals.

Stefan has prior experience working as an IT consultant advising healthcare and life sciences clients. He holds a PhD degree from the University of Vienna, is trained in molecular biology and data analysis and has published multiple peer-reviewed articles in internationally-renowned journals.

ISMA HACHI
Senior Principal, Consulting Services, IQVIA

Isma Hachi brings over 16 years of international consulting experience in innovation and strategy. With a scientific background and a PhD, she combines deep analytical rigor with strategic insight to drive impactful outcomes. As a Senior Principal in IQVIA's Consulting Practice, she serves as a trusted Senior Client Partner guiding clients from initial opportunity discussions throughout the delivery of high-impact insights and actionable business recommendations. Her career includes experience at leading Big Four consulting firms, where she advised a wide range of biopharma companies across the U.S. and Europe on developing innovative business strategies and operating models. Isma is deeply passionate about emerging technologies and cross-sector trends, particularly how non-traditional investment strategies can accelerate innovation. She has a strong interest in personalised nutrition and medicine, advocating for a holistic approach centered on prevention, early diagnosis, healthy diet, and lifestyle.

ISABELLE DE CREMOUX
CEO and Managing Partner, Seventure Partners

Isabelle is a leading European venture capital firm specializing in life sciences — with a strong focus on the microbiome — as well as digital, nutrition, and blue economy sectors. With over 25 years of international experience in business development and finance, Isabelle has held leadership roles at Arthur Andersen, Pfizer, and Fournier/Abbott, where she negotiated numerous international deals.

An engineer by training (École Centrale Paris), she also holds DECF and ISEB qualifications. Isabelle is a recognised thought leader in the microbiome field, frequently speaking at global conferences. She served on the board of Naturex/Givaudan and currently chairs the private equity branch of France's AFG association. She is also a founding member of the French Conseil de l'innovation, advising the government on national innovation strategy.

TOMAS DE WOUTERS
CEO and Co-Founder, PharmaBiome

Tomas worked on understanding the intestinal microbiota and its role in human health since his studies in food engineering and biotechnology at the ETH Zurich. During his PhD at the INRAE in Jouy-en-Josas near Paris — a hotspot of microbiome research - he developed a solid scientific understanding of the microbiome in human health during the early 2000's. His engineering background and PhD have strongly supported his transition from fundamental to applied and translational science, starting with spinning PharmaBiome out of ETH Zurich.

With his team, he has pioneered interacting bacterial consortia as a therapeutic modality. He is currently acting CEO of PharmaBiome.

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