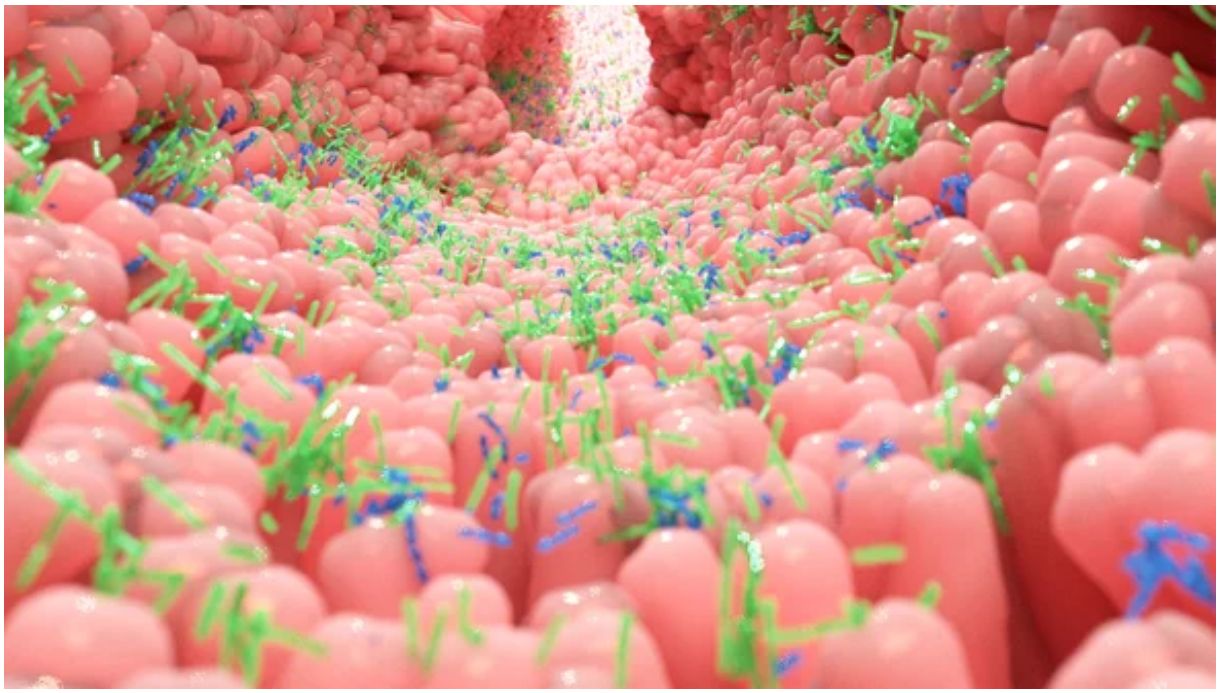
Observations

We're Starting to Harness the Microbiome to Treat Disease

But strong regulation is a must to protect patient safety

By Lee Jones on August 1, 2019



Credit: Marcin Klapcyznski *Getty Images*

Our bodies are home to an incredible array of microorganisms, invisible to the naked eye, collectively referred to as the "human microbiome." We depend on these organisms and their communities to survive and are just beginning to understand the tremendous impact that they have on our health.

One of the first therapies using the gut microbiome, fecal microbiota transplantation (called "FMT" and known in popular media as a "poop transplant") has shown success in treating people who have a recurrent intestinal infection called *Clostridioides difficile (C. diff),* characterized by severe diarrhea. Now FMT is being investigated for other conditions as well.

Unfortunately, in some cases, the promise of this treatment has outpaced concern for following stringent safety precautions before patient use; most recently, the use of this investigational therapy resulted in a patient death through the use of stool from an inappropriately screened donor, trigging the FDA to issue a safety alert. This unfortunate death serves as a reminder that all investigational therapies have associated risks and that clear and rigorous oversight from a regulatory body is needed to help minimize those risks to patients.

HARNESSING THE MICROBIOME TO TREAT DISEASE

The microbiome is a complex community of microorganisms that live on every surface of the human body. The most dense and diverse community is found in the human digestive tract; it aids in the digestion of food, the development and maintenance of the immune system and the regulation of our metabolism. The gut microbiome is often compared to the Amazon rainforest, as both have interwoven ecosystems functioning together to maintain life. When that diversity is destroyed, invasive species can take root. This is similar to what happens in the gut; the complex diversity of microbes work together to function, but if the community is damaged, the protective health benefits are lost.

Research has shown that the microbial communities can be damaged by many factors, including antibiotic use, viruses or stress, and that this disruption can leave the host susceptible to diseases like *C. diff*.

In 2013, *C. diff* was named a national health threat by the Centers for Disease Control (CDC) and a subsequent 2015 CDC study found that it caused almost half a million infections in the United States in a single year. An estimated 15,000 deaths are directly attributable to *C. diff* infections. The condition can be deadly, and the treatments can be serious, such as lifelong antibiotic therapy or removal of the colon.

Unlike antibiotics that kill microbes and continue to leave the patient susceptible to disease, new therapies are being developed that utilize a healthy donor's gut microbiota to help reestablish the community of microorganisms to a more normal state. By using a donor's microbiome, patients can regain an ability to fight disease themselves. This has shown great promise in *C. diff* patients, and there may be opportunity for "bugs as drugs" beyond *C. diff* as well. To date, the microbiome is being investigated in the treatment of a number of other conditions and therapeutic areas spanning from ulcerative colitis to reproductive health and oncology.

A CLEAR NEED FOR REGULATION

As the hype around FMT and its potential to treat *C. diff* has continued to grow, the demand for physicians to perform FMT has also increased. In 2013, the FDA issued an enforcement discretion policy (PDF) in an attempt to provide physicians the ability to practice FMT for their own patients by not requiring them to create an application for an investigational new drug (IND) as is normally the practice with an unapproved therapy. This unintentionally opened the door for stool bank companies to provide thousands of unqualified and unregulated doses of FMT to patients with no safety protection and regulatory oversight. This overuse and lack of controls likely led to the unfortunate patient death.

At Rebiotix, we feel strongly that standardized manufacturing processes, donor screening and validated quality testing under FDA oversight is imperative in this new microbiome industry to avoid further safety issues. Clinical and scientific data need to be collected and reviewed to assess product quality, safety and efficacy. It is our duty to take no shortcuts in bringing innovative treatments to vulnerable patients.

As such, we were the first company to engage with the FDA regarding a regulatory pathway for our investigational microbiota-based drug product platform. Our path towards regulatory approval has not been a linear one, but our learnings and

collaborative relationship with the FDA will hopefully ensure we can eventually bring safe, efficacious products to patients in need.

Microbiota-based therapies have tremendous potential and our aim is to deliver much-needed therapies that help address significant unmet needs. We remain deeply committed to helping to research and educate the field about the true power of the human microbiome, how it may be utilized to change the way we approach treating debilitating illnesses, and the need for regulatory oversight to protect the patients. Through these avenues we can ensure that the microbiome's true potential is put into practice.

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Lee Jones is CEO of Rebiotix, a Ferring company.

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