

## Media Release

# Rebiotix and Ferring announce world's first with positive preliminary pivotal Phase 3 data for investigational microbiome-based therapy RBX2660

- *Rebiotix and Ferring are the first to announce positive preliminary results on primary efficacy endpoint from ongoing pivotal Phase 3 clinical trial for RBX2660*
- *RBX2660 is an investigational, non-antibiotic, microbiome-based therapy, developed to reduce *Clostridioides difficile* (*C. diff*) infection recurrences*
- *CDC defines *C. diff* as a major burden to patients and doctors and an urgent healthcare threat causing an estimated half a million illnesses and thousands of deaths annually in the US alone<sup>1,2</sup>*

**Roseville, Minnesota and Saint-Prex, Switzerland – 6 May, 2020, 07:00 EST –** Today, Rebiotix and Ferring Pharmaceuticals announced positive preliminary findings from their ongoing pivotal Phase 3 trial of the investigational microbiome-based treatment, RBX2660. These preliminary positive efficacy findings mark an important milestone, advancing RBX2660 in its clinical development program with a goal of bringing a US FDA approved therapy to patients. The clinical development program for RBX2660 is the most advanced in the world in evaluating the safety and efficacy of a standardized, non-antibiotic microbiome-based therapy.

RBX2660 is being developed to reduce *C. diff* infection recurrences, an urgent unmet need for patients and healthcare providers worldwide. Antibiotics, the current standard of care, have been shown to disrupt the microbiome and increase the risk of *C. diff* recurrence.<sup>3</sup> *C. diff* causes nearly 30,000 deaths each year in the US; in Europe, the incidence of *C. diff* is increasing, with recurrent bouts of infection representing 10-15% of all healthcare-related infections in hospitals annually.<sup>4,5</sup> As a live biotherapeutic, aiming to help restore the gut microbiome community, RBX2660 may bring an innovative therapeutic option to patients suffering from this potentially deadly infection.

“*C. diff* infection is a significant public health threat that has limited treatment options. These positive preliminary findings represent a major step forward towards bringing an innovative, non-antibiotic option to patients that may help restore their gut microbiome, said Per Falk, Ferring’s President and Chief Science Officer. With health systems under increasing pressure due to viruses like COVID-19 and the rising threat of antimicrobial resistance, the need for new therapies is greater than ever. We believe the power of the microbiome has great potential and we look forward to bringing RBX2660 to patients soon.”

“Since founding Rebiotix in 2011, our mission has been to harness the power of the microbiome to treat complex diseases. Our first goal was to address *C. diff*, which poses a significant health threat to thousands worldwide every year,” said Lee Jones, CEO and founder of Rebiotix, a Ferring company. The positive preliminary data on the primary efficacy endpoint are a major stepping stone for the RBX2660 development program, bringing us closer to an approved microbiome therapy

available for healthcare providers to help patients. As a first-in-class, potentially paradigm-changing technology, we look forward to discussing our final data with the FDA in the latter part of this year.”

The ongoing Phase 3 trial is a randomized, multicenter, double-blinded, placebo-controlled study. The trial also incorporates a safety assessment intended to follow patients for several months after receiving the investigational drug. The safety data will provide insight into the potential of using microbes as a therapeutic intervention. The full data package is anticipated in the second half of 2020.

This trial builds on nearly a decade of research and evaluation of the formulation, with robust clinical and microbiome data collected over multiple controlled trials under the proprietary MRT™ drug platform.

## **Notes to editors**

### **About *Clostridioides difficile* infection (*C. diff*)**

*C. diff* is a bacterium that causes diarrhea and colitis (an inflammation of the colon).<sup>6</sup> It is estimated to cause up to half a million illnesses in the US alone every year and is considered an urgent threat to public health by the CDC, and can lead to severe complications, including hospitalization, surgery, and death.<sup>2</sup> While antibiotics are the standard of care to address the infection, they are also the primary risk factor for disease recurrence.<sup>3</sup> Recurrence of *C. diff* occurs in approximately 15-50% of patients.<sup>7</sup>

### **About the microbiome**

The human microbiome is a complex community of microorganisms which live on every surface of the body. The microbiome aids in the maintenance and development of the immune system, metabolism, and other functions essential to human life.<sup>8</sup> The gastrointestinal tract houses the most dense and complex population of microbiota, which has an incredible influence over daily health – from aiding in food digestion to fighting disease. Clinical and scientific studies indicate antibiotics, viruses, stress and other factors can disturb the gut microbiota. This disruption, often referred to as “dysbiosis,” may have negative health impacts, and promote conditions for infections like *C. diff* infection to take hold.<sup>9</sup> Rebiotix and Ferring believe there is tremendous potential in microbiota-based therapies to address such illnesses, and are evaluating this therapeutic option through their pioneering microbiota-based MRT™ drug platform, beginning with recurrent *C. diff* infection.

### **About RBX2660**

The investigational RBX2660 formulation is the first-in-class microbiota-based therapy to achieve positive preliminary Phase 3 study results. RBX2660 is being developed to help break the cycle of recurrent *C. diff* infection. The therapy has been granted Fast Track, Orphan, and Breakthrough Therapy designations from the US FDA. The RBX2660 ongoing pivotal Phase 3 trial, PUNCH CD3, is a randomized, multicenter, double-blinded, placebo-controlled study. For more information about the RBX2660 Phase 3 study, visit [www.clinicaltrials.gov](http://www.clinicaltrials.gov) (NCT03244644).

### **About Ferring Pharmaceuticals**

Ferring Pharmaceuticals is a research-driven, specialty biopharmaceutical group committed to helping people around the world build families and live better lives. Headquartered in Saint-Prex, Switzerland, Ferring is a leader in reproductive medicine and maternal health, and in specialty areas within gastroenterology and urology. Founded in 1950, privately-owned Ferring now employs

approximately 6,500 people worldwide, has its own operating subsidiaries in nearly 60 countries and markets its products in 110 countries.

Learn more at [www.ferring.com](http://www.ferring.com), or connect with us on [Twitter](#), [Facebook](#), [Instagram](#), [LinkedIn](#) and [YouTube](#).

### **About Rebiotix**

Rebiotix Inc, part of the Ferring Pharmaceuticals Group, is a late-stage clinical microbiome company focused on harnessing the power of the human microbiome to revolutionize the treatment of challenging diseases. Rebiotix has a diverse pipeline of investigational drug products built on its pioneering microbiota-based MRT™ [drug platform](#). The platform consists of investigational drug technologies designed to potentially rehabilitate the human microbiome by delivering a broad consortium of live microbes into a patient's intestinal tract. For more information on Rebiotix and its pipeline of human microbiome-directed therapies for diverse disease states, visit [www.rebiotix.com](http://www.rebiotix.com), or connect with us on [Twitter](#), [Facebook](#), [LinkedIn](#) and [YouTube](#).

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### **References**

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