Rebyota™ (fecal microbiota, live - jslm) – New orphan drug approval

- On November 30, 2022, the FDA announced the approval of Ferring Pharmaceuticals' Rebyota (fecal microbiota, live - jslm), for the prevention of recurrence of *Clostridioides difficile* (*C. difficile*) infection (CDI) in individuals 18 years of age and older following antibiotic treatment for recurrent CDI.
    - Rebyota is not indicated for treatment of CDI.
- The intestinal tract contains millions of microorganisms, often referred to as the "gut microbiome." Certain situations, such as taking antibiotics to treat an infection, may change the balance of microorganisms in the gut, allowing *C. difficile* to multiply and release toxins causing diarrhea, abdominal pain, and fever. In severe cases, CDI can lead to organ failure and death. After recovering from CDI, individuals may get the infection again, a condition known as recurrent CDI.
    - In the U.S., CDI is associated with 15,000 to 30,000 deaths annually.
    - The administration of fecal microbiota is thought to facilitate restoration of the gut flora to prevent further episodes of CDI.
- Rebyota is the first FDA approved fecal microbiota product. It is prepared from stool donated by qualified individuals.
- The efficacy of Rebyota was established in a randomized, double-blind, placebo-controlled, phase 3 study (Study 1), which formally integrated treatment success rates from a placebo-controlled phase 2 study (Study 2). Adults in both studies were 18 years of age or older and had a confirmed diagnosis of recurrent CDI. The integrated efficacy analysis set included 262 patients in Study 1 and 82 patients in Study 2. Treatment success was defined as the absence of CDI diarrhea within 8 weeks of blinded treatment.
    - The estimated rate of treatment success was significantly higher in the Rebyota group (70.6%) than in the placebo group (57.5%) through 8 weeks after completing blinded treatment, resulting in a difference of 13.1% (95% CI: 2.3, 24.0) which corresponds to a 99.1% posterior probability that Rebyota is superior to placebo.
- Warnings and precautions for Rebyota include transmissible infectious agents, management of acute allergic reactions, and potential presence of food allergens.
- The most common adverse reactions (≥ 3%) with Rebyota use were abdominal pain, diarrhea, abdominal distention, flatulence, and nausea.
- The recommended dose of Rebyota is a single dose of 150 mL administered rectally.
    - Rebyota should be administered 24 to 72 hours after the last dose of antibiotics for CDI.
- Ferring Pharmaceuticals' launch plans for Rebyota are pending. Rebyota will be available as a 150 mL suspension.

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