Egaten™ (triclabendazole) – New orphan drug approval

- On February 13, 2019, Novartis announced the FDA approval of Egaten (triclabendazole), for the treatment of fascioliasis in patients 6 years of age and older.

- Fascioliasis, commonly known as liver fluke infestation, is a tropical disease that infects 2.4 million people worldwide. It is caused by two species of parasitic flatworms (*Fasciola hepatica* or *Fasciola gigantica*) that can infect humans following ingestion of larvae in contaminated water or food.

- Egaten is currently the only medicine for fascioliasis recommended by the World Health Organization (WHO) and is on the WHO Model List of Essential Medicines. It is supplied by WHO during epidemic outbreaks and for periodic use in endemic countries.

    — Novartis has been donating Egaten to the WHO since 2005.

- Egaten is an anthelmintic against *Fasciola* species.

- The efficacy of triclabendazole was based on an open label study conducted in Vietnam in 100 patients with acute symptomatic fascioliasis. Patients received Egaten or oral artesunate (not FDA approved in the U.S.).

    — At three months after treatment, 92% and 76% (difference 16%; 95% CI: 1.7, 30.8; p = 0.035) of patients in the Egaten and artesunate arms respectively, reported no clinical symptoms.

- In addition, triclabendazole was evaluated in six open label studies performed in Cuba, Bolivia, Peru, Chile, and Iran in a total of 245 adult and pediatric patients with stool-confirmed fascioliasis. The studied triclabendazole doses ranged from 5 mg/kg to 20 mg/kg administered on days 1 to 3. Cure was defined as the absence of *Fasciola* eggs in the stool at day 60 in patients who were positive at baseline.

    — The day 60 cure rate was highest (95.5%; 95% CI: 77, 100) for the 20 mg/kg dose, followed by cure rates of 88% (95% CI: 64, 99), 80% (95% CI: 73, 86), and 50% (95% CI: 27, 73) in the 15 mg/kg, 10 mg/kg, and 5 mg/kg dose groups, respectively.

- A warning and precaution of Egaten is QT prolongation.

- The most common adverse reactions (> 2%) with use of the Egaten 20 mg/kg dose are abdominal pain, hyperhidrosis, nausea, decreased appetite, headache, urticaria, diarrhea, vomiting, musculoskeletal chest pain, and pruritus.

- The recommended dose of Egaten is 2 doses of 10 mg/kg given 12 hours apart, with food.

    — The 250 mg tablets are functionally scored and divisible into two equal halves of 125 mg. If the dosage cannot be adjusted exactly, the dose should be rounded upwards.

- Novartis' launch plans for Egaten are pending. Egaten will be available as a 250 mg tablet.