Lampit® (nifurtimox) – New orphan drug approval

- On August 7, 2020, Bayer announced the FDA approval of Lampit (nifurtimox), in pediatric patients (birth to less than 18 years of age and weighing at least 2.5 kg) for the treatment of Chagas disease (American Trypanosomiasis), caused by *Trypanosoma cruzi*.

    - This indication is approved under accelerated approval based on the number of treated patients who became immunoglobulin G (IgG) antibody negative or who showed an at least 20% decrease in optical density on two different IgG antibody tests against antigens of *T. cruzi.*
    - Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory trial(s).

- Chagas disease is caused by the *T. cruzi* parasite and is primarily transmitted to humans via the feces of infected triatomines, insects that are also known as “kissing bugs” where the disease is present. Chagas disease can also be transmitted by infected organ transplantation, infected blood transfusion, during pregnancy or birth from an infected mother to her child, and less frequently through ingestion of contaminated food.

    - According to the World Health Organization, 6 to 7 million people around the world are infected with Chagas disease. The majority of infected individuals are in Latin America.

- Lampit is an antiprotozoal medication. The exact mechanism of action is not fully understood, but the drug is active against all three stages of *T. cruzi.*

- The efficacy of Lampit was established in a double-blind study of 330 pediatric patients with serologic evidence of *T. cruzi* infection and without Chagas disease-related cardiac or gastrointestinal symptoms. Patients were randomized to a 60-day or 30-day treatment regimen with Lampit. Serological response to treatment was defined as ≥ 20% decrease in optical density measured by lysate and recombinant ELISA in subjects > 8 months to < 18 years or seroconversion to negative (defined as negative immunoglobulin G concentration in all patients) at 1-year post-treatment follow-up.

    - The results for the lysate ELISA showed superiority in favor of the Lampit 60-day arm (32% serological response) vs. 30-day arm (19% serological response) (Difference: 13%; 95% CI: 3.5%, 22.6%; p = 0.007).
    - Results from recombinant ELISA assay were similar, showing serological response rates of 35% for the Lampit 60-day arm and 22% for Lampit 30-day arm (Difference: 13%; 95% CI: 3.2%, 23.0%; p = 0.010).

- Lampit is contraindicated in patients who consume alcohol during treatment and with known hypersensitivity to nifurtimox or any of the excipients in Lampit.

- Warnings and precautions for Lampit include potential for genotoxicity and carcinogenicity; embryo-fetal toxicity; worsening of neurological and psychiatric conditions; hypersensitivity; decreased appetite and weight loss; and porphyria.

- The most common adverse reactions (≥ 5%) with Lampit use were vomiting, abdominal pain, headache, decreased appetite, nausea, pyrexia, and rash.

- The recommended dose of Lampit is administered orally three times a day with food based on the patient’s body weight. The recommended duration of treatment with Lampit is 60 days.

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  - Lampit are functionally scored tablets which can be split into one-half at the scored lines by hand. Lampit tablets should not be broken mechanically with a tablet splitting device.
  - Lampit tablets can be made into a slurry as an alternative method of administration for patients who cannot swallow the tablets.
  - Refer to the Lampit drug label for further weight-based dosing recommendations and instructions.

- Bayer's launch plans for Lampit are pending. Lampit will be available as 30 mg and 120 mg tablets.