Mycapssa® (octreotide) – New orphan drug approval

- On June 26, 2020, Chiasma announced the FDA approval of Mycapssa (octreotide), for long-term maintenance treatment in acromegaly patients who have responded to and tolerated treatment with octreotide or lanreotide.

- Acromegaly is a rare chronic disease often caused by a benign pituitary tumor and characterized by excess production of growth hormone and insulin-like growth factor-1 hormone.

- Mycapssa is the first and only oral somatostatin analog approved by the FDA.

- Octreotide is also available generically as a solution for injection, as branded Sandostatin® LAR Depot powder for suspension for injection, and branded Bynfezia® pen injection.

    — These formulations of octreotide are approved for acromegaly, carcinoid tumors, and vasoactive intestinal peptide tumors.

- The efficacy of Mycapssa was established in a randomized, double-blind, placebo-controlled study in 56 patients with acromegaly. Patients received Mycapssa or placebo. The primary efficacy endpoint was somatostatin dose-adjusted proportion of patients who maintain their biochemical response, defined as an insulin-like growth factor 1 (IGF-1) levels less than or equal to the upper limit of normal (ULN) at the end of 9 months of treatment.

    — Overall, 58% of patients treated with Mycapssa vs. 19% of patients treated with placebo maintained their biochemical response.

- Warnings and precautions for Mycapssa include cholelithiasis and complications of cholelithiasis; hyperglycemia and hypoglycemia; thyroid function abnormalities; cardiac function abnormalities; and decreased vitamin B_{12} levels and abnormal Schilling's tests.

- The most common adverse reactions (> 10%) with Mycapssa use were nausea, diarrhea, headache, arthralgia, asthenia, hyperhidrosis, peripheral swelling, blood glucose increased, vomiting, abdominal discomfort, dyspepsia, sinusitis, osteoarthritis.

- The recommended initial dose of Mycapssa is 40 mg daily, administered as 20 mg orally twice daily. The dosage should be titrated based on IGF-1 levels and patient's signs and symptoms. The dosage should be increased in increments of 20 mg daily.

    — For Mycapssa dosages of 60 mg daily, administer as 40 mg in the morning and 20 mg in the evening.
    — For Mycapssa dosages of 80 mg daily, administer as 40 mg twice daily.
    — The maximum recommended dosage of Mycapssa is 80 mg daily.

- Chiasma plans to launch Mycapssa in the fourth quarter 2020. Mycapssa will be available as 20 mg capsules.