Firdapse® (amifampridine) – New orphan drug approval

- On November 28, 2018, the FDA announced the approval of Catalyst Pharmaceutical's Firdapse (amifampridine), for the treatment of Lambert-Eaton myasthenic syndrome (LEMS) in adults.

- LEMS is a rare autoimmune disease that affects approximately 1 in 100,000 people in the U.S. The most common symptoms of LEMS are proximal muscle weakness and fatigue. Symptoms can be life threatening when the weakness involves respiratory muscles. Approximately 50% of LEMS patients have an underlying malignancy, typically small cell lung cancer.

- Firdapse is a broad spectrum potassium channel blocker. The mechanism by which Firdapse exerts its therapeutic effect in LEMS patients has not been fully elucidated.

- The efficacy of Firdapse was demonstrated in two placebo-controlled studies enrolling 64 patients with LEMS. The two co-primary measures of efficacy in both studies were the change from baseline to the end of the discontinuation period in the Quantitative Myasthenia Gravis (QMG) score, which assesses muscle weakness, and in the Subject Global Impression (SGI) score, which is an overall impression of the patient's physical wellbeing.

    - Lower QMG scores represent less muscle weakness while higher scores represent more weakness. Lower SGI scores represent lower perceived benefit with the study treatment.
    - In study 1, the QMG score change from baseline was 0.4 for the Firdapse group vs. 2.2 for the placebo group (difference = -1.7 [95% CI: -3.4, 0.0]; p = 0.045). The SGI score change from baseline was -0.8 for the Firdapse group vs. -2.6 for the placebo group (difference = 1.8 [95% CI: 0.7, 3.0]; p = 0.003).
    - In study 2, the QMG score change from baseline was 0.00 for the Firdapse group vs. 6.54 for the placebo group (difference = -6.54 [95% CI: -9.78, -3.29]; p = 0.0004). The SGI score change from baseline was -0.64 for the Firdapse group vs. -3.59 for the placebo group (difference = 2.95 [95% CI: 1.53, 4.38]; p = 0.0003).

- Firdapse is contraindicated in patients with a history of seizures and hypersensitivity to Firdapse or another aminopyridine.

- The most common adverse reactions (> 10%) with Firdapse use were paresthesia, upper respiratory tract infection, abdominal pain, nausea, diarrhea, headache, elevated liver enzymes, back pain, hypertension, and muscle spasms.

- The recommended starting dose of Firdapse is 15 mg to 30 mg daily, taken orally in divided doses (3 to 4 times daily).

    - The dosage can be increased by 5 mg daily every 3 or 4 days.
    - The maximum recommended total daily dosage is 80 mg.
    - The maximum single dose is 20 mg.

- Catalyst Pathways™ is a free, personalized program that offers patients and their families one-on-one support throughout their treatment journey with a dedicated team of specialists to help them manage their unique challenges.

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- Catalyst Pharmaceuticals plans to launch Firdapse early in the first quarter of 2019. Firdapse will be available as 10 mg tablets.