Zilbrysq® (zilucoplan) – New orphan drug approval

- On October 17, 2023, UCB announced the FDA approval of Zilbrysq (zilucoplan), for the treatment of generalized myasthenia gravis (gMG) in adult patients who are anti-acetylcholine receptor (AChR) antibody positive.

- gMG is a rare autoimmune disease in which patients can experience a variety of symptoms, including severe muscular weakness that can result in double vision, drooping eyelids, difficulty with swallowing, chewing and talking, as well as life-threatening weakness of the muscles of respiration.

    - The prevalence of gMG is 100 to 350 cases per every 1 million people.

- Zilbrysq is a C5 inhibitor and the first gMG targeted therapy for self-administration by adult patients with anti-AChR antibody-positive gMG.

- The efficacy of Zilbrysq was established in a randomized, double-blind, placebo-controlled study in 174 adult patients with gMG who are anti-AChR antibody positive. Patients were randomized to receive Zilbrysq or placebo. The primary endpoint was a comparison of the change from baseline between treatment groups in the Myasthenia Gravis-Specific Activities of Daily Living scale (MG-ADL) total score after 12 weeks of treatment. A total score ranges from 0 to 24, with the higher scores indicating more impairment.

    - The least square mean change from baseline in the MG-ADL total score was -4.39 and -2.30 for Zilbrysq and placebo, respectively (mean difference of -2.09, 95% CI: -3.24, -0.95; $p < 0.001$).

- Zilbrysq carries a boxed warning for serious meningococcal infections.

    - Because of the risk of serious meningococcal infections, Zilbrysq is available only through a restricted program under a Risk Evaluation and Mitigation Strategy (REMS) called Zilbrysq REMS.

- Zilbrysq is contraindicated in patients with unresolved *Neisseria meningitidis* infection.

- Additional warnings and precautions for Zilbrysq include other infections and pancreatitis and other pancreatic conditions.

- The most common adverse reactions (≥ 10%) with Zilbrysq use were injection site reactions, upper respiratory tract infection, and diarrhea.

- The recommended dose of Zilbrysq is given once daily as a subcutaneous (SC) injection and is dependent on actual body weight (see table below).

Body weight	Once daily dosage
Less than 56 kg	16.6 mg
56 kg to less than 77 kg	23 mg
77 kg and above	32.4 mg

    - Zilbrysq is intended for use under the guidance and supervision of a healthcare professional. Patients may self-inject Zilbrysq after training in SC injection technique.

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- UCB's launch plans for Zilbrysq are pending. Zilbrysq will be available as 16.6 mg/0.416 mL, 23 mg/0.574 mL, or 32.4 mg/0.81 mL single-dose prefilled syringes.