Rystiggo® (rozanolixizumab-noli) – New orphan drug approval

- On June 27, 2023, UCB announced the FDA approval of Rystiggo (rozanolixizumab-noli), for the treatment of generalized myasthenia gravis (gMG) in adult patients who are anti-acetylcholine receptor (AChR) or anti-muscle-specific tyrosine kinase (MuSK) antibody positive.

- Rystiggo is a humanized IgG4 monoclonal antibody that binds to the neonatal Fc receptor (FcRN), resulting in the reduction of circulating IgG.

    — Rystiggo is the first FDA approved treatment for both anti-AChR and anti-MuSK antibody-positive gMG.

- The efficacy of Rystiggo was established in a randomized, double-blind, placebo-controlled study in 200 adults with gMG who were anti-AChR antibody or anti-MuSK antibody positive. During the treatment period of the study, Rystiggo (two different doses) or placebo was administered once a week for 6 weeks. The primary endpoint was the comparison of the change from baseline between treatment groups in the Myasthenia Gravis-Activities of Daily Living (MG-ADL) total score at day 43. The MG-ADL total score ranges from 0 to 24, with the higher scores indicating more impairment.

    — A statistically significant difference favoring Rystiggo was observed in the MG-ADL total score change from baseline (-3.4 points in Rystiggo-treated group at either dose vs. -0.8 points in the placebo-treated group; $p < 0.001$ for both doses).

- Warnings and precautions for Rystiggo include infections, aseptic meningitis, and hypersensitivity reactions.

- The most common adverse reactions (≥ 10%) with Rystiggo use were headache, infections, diarrhea, pyrexia, hypersensitivity reactions, and nausea.

- The recommended dose of Rystiggo is based on body weight (see table below). Rystiggo should be administered as a subcutaneous (SC) infusion using an infusion pump at a rate of up to 20 mL/hour once weekly for 6 weeks. Subsequent treatment cycles should be administered based on clinical evaluation. The safety of initiating subsequent cycles sooner than 63 days from the start of the previous treatment cycle has not been established.

Body weight of patient	Dose	Volume to be infused
Less than 50 kg	420 mg	3 mL
50 kg to less than 100 kg	560 mg	4 mL
100 kg and above	840 mg	6 mL

- Rystiggo should only be prepared and infused by a healthcare provider.

- UCB plans to launch Rystiggo in third quarter 2023. Rystiggo will be available as a 280 mg/2 mL (140 mg/mL) solution in a single-dose vial.