Tarpeyo™ (budesonide) – New orphan drug approval

- On December 15, 2021, the FDA announced the approval of Calliditas Therapeutics' Tarpeyo (budesonide), to reduce proteinuria in adults with primary immunoglobulin A nephropathy (IgAN) at risk of rapid disease progression, generally a urine protein-to-creatinine ratio (UPCR) ≥ 1.5 g/g.

    — This indication is approved under accelerated approval based on a reduction in proteinuria. It has not been established whether Tarpeyo slows kidney function decline in patients with IgAN. Continued approval for this indication may be contingent upon verification and description of clinical benefit in a confirmatory clinical trial.

- IgA nephropathy, also known as Berger's disease, is a rare kidney disease that occurs when IgA deposits build up in the kidneys, causing inflammation that damages kidney tissues. The deposits can cause the kidneys to leak blood and protein into the urine. IgA nephropathy complications can include high blood pressure and chronic kidney disease, which can sometimes progress to kidney failure.

- The efficacy of Tarpeyo was established in a randomized, double-blind study in 199 patients with biopsy-proven IgAN who were on a stable dose of a maximally-tolerated renin-angiotensin system (RAS) inhibitor. Patients were randomized to either Tarpeyo or placebo. The primary endpoint was the percentage reduction in UPCR at 9 months compared to baseline

    — The percentage reduction in UPCR from baseline was 34% and 5% with Tarpeyo and placebo, respectively (Tarpeyo vs. placebo percentage reduction: 31, 95% CI: 16, 42; p = 0.0001).

- Warnings and precautions for Tarpeyo include hypercorticism and adrenal axis suppression, risks of immunosuppression, and other corticosteroid effects.

- The most common adverse reactions (≥ 5%) with Tarpeyo use were hypertension, peripheral edema, muscle spasms, acne, dermatitis, weight increase, dyspnea, face edema, dyspepsia, fatigue, and hirsutism.

- The recommended dose of Tarpeyo is 16 mg orally once daily for a recommended duration of therapy of 9 months. When discontinuing therapy, the dosage should be reduced to 8 mg once daily for the last 2 weeks of therapy.

- Calliditas Therapeutics plans to launch Tarpeyo in early first quarter 2022. Tarpeyo will be available as 4 mg delayed release capsules.