Procysbi® (cysteamine bitartrate) – New formulation approval

- On February 18, 2020, Horizon Therapeutics announced the FDA approval of Procysbi (cysteamine bitartrate) delayed-release oral granules, for the treatment of nephropathic cystinosis in adults and pediatric patients 1 year of age and older.

- Procysbi was previously approved as a delayed-release oral capsule formulation. The granules and capsules share the same indication.

- Warnings and precautions for Procysbi include Ehlers-Danlos-like syndrome, skin rash, gastrointestinal ulcers and bleeding, central nervous system symptoms, leukopenia and/or elevated alkaline phosphatase levels, and benign intracranial hypertension.

- The recommended dose of Procysbi in cysteamine-naïve patients is weight-based. In patients switching from immediate-release cysteamine (Cystagon®) to Procysbi, start with a total daily dose of Procysbi equal to the previous total daily dose of immediate-release cysteamine bitartrate.

    - The Procysbi dose should be given every 12 hours.
    - The maximum dosage of Procysbi is 1.95 grams/m^2 of body surface area per day.
    - The oral granules should not be crushed or chewed. The granules should be sprinkled and mixed in applesauce, berry jelly or fruit juice (except grapefruit juice).
    - Refer to the Procysbi drug label for complete dosing and administration recommendations.

- Horizon Therapeutics plans to launch the oral granule formulation of Procysbi in the first half of this year. The new formulation will be available as 75 mg and 300 mg single-use packets.