Procysbi® (cysteamine bitartrate) – Expanded indication

- Horizon Pharma announced the FDA approval of Procysbi (cysteamine bitartrate), for the treatment of nephropathic cystinosis in adults and pediatric patients 1 year of age and older.
    - Previously, Procysbi was indicated in adult and pediatric patients 2 years of age and older.
- Nephropathic cystinosis is a rare, life-threatening metabolic lysosomal storage disorder that causes toxic accumulation of cystine in all cells, tissues, and organs in the body. If untreated, elevated cystine accumulation leads to progressive, irreversible tissue damage and multi-organ failure, including kidney failure, blindness, muscle wasting and premature death.
    - It is estimated that about 2,000 people worldwide have this disorder.
- The approval of the expanded indication was based on an open-label study that enrolled 17 people with nephropathic cystinosis, including 15 children between the ages of 1 and 5 years old, who had not previously been treated with cysteamine therapy.
    - Children enrolled in the study experienced lowering of white blood cell (WBC) cystine levels from poor controlled to well controlled at 12 and 18 month measurements.
    - The children also experienced improvements in growth milestones including weight and height.
- The most common adverse reactions (> 10%) with Procysbi use in patients 1 year to less than 6 years naïve to cysteamine treatment were vomiting, gastroenteritis/viral gastroenteritis, diarrhea, breath odor, nausea, electrolyte imbalance and headache.
- In cysteamine-naïve patients, the recommended maintenance dosage of Procysbi after initial dose escalation is 1.3 g/m^2/day divided into two doses given every 12 hours. Start treatment with a dosage equal to 1/6 to 1/4 of the maintenance dosage.
    - The maximum dosage of Procysbi is 1.95 grams/m^2/day. Dosage should be rounded to the nearest incremental dosage that can be administered using the available capsule strengths. Only use whole capsules.
    - WBC cystine concentration should be monitored and Procysbi dosage titrated as needed to achieve the therapeutic target WBC cysteine concentration.
    - Consult the Procysbi drug label for further recommendations about dosage initiation, titration, and WBC cysteine concentration monitoring. A dosing table based on body weight is also provided in the drug label.