Palynziq™ (pegvaliase-pqpz) – New drug approval

- On May 24, 2018, the FDA announced the approval of BioMarin's Palynziq™ (pegvaliase-pqpz), to reduce blood phenylalanine (Phe) concentrations in adult patients with phenylketonuria (PKU) who have uncontrolled blood Phe concentrations greater than 600 micromol/L on existing management.

- Patients with PKU are born with an inability to break down Phe, an amino acid present in protein-containing foods and high-intensity sweeteners used in a variety of foods and beverages. If untreated, PKU can cause chronic intellectual, neurodevelopmental and psychiatric disabilities.

    — PKU affects about 1 in 10,000 to 15,000 people in the U.S.

- Palynziq is a PEGylated recombinant Phe ammonia lyase enzyme that targets the underlying cause of PKU by helping the body to break down Phe.

- The safety and efficacy of Palynziq were studied in two clinical studies in adult patients with PKU with blood Phe concentrations > 600 micromol/L on existing management. The first study was a randomized, open-label study where patients were treated with a target dose of 20 mg or 40 mg Palynziq once daily. The second study was an 8-week, placebo-controlled, randomized withdrawal trial in patients who were previously treated with Palynziq.

    — Patients treated with Palynziq achieved statistically significant reductions in blood Phe concentrations from their pre-treatment baseline blood Phe concentrations.

- Palynziq carries a boxed warning for risk of anaphylaxis.

- Warnings and precautions of Palynziq include Palynziq REMS program and other hypersensitivity reactions.

- The most common adverse reactions (at least 20% in either treatment phase) with Palynziq use were injection site reactions, arthralgia, hypersensitivity reactions, headache, generalized skin reactions lasting at least 14 days, pruritus, nausea, abdominal pain, oropharyngeal pain, vomiting, cough, diarrhea, and fatigue.

- The recommended initial dosage of Palynziq is 2.5 mg subcutaneously (SC) once weekly for 4 weeks. Administer the initial dose under the supervision of a healthcare provider.

    — Assess patient tolerability, blood Phe concentrations, and dietary protein and Phe intake throughout treatment.
    — Titrate the Palynziq dosage in a step-wise manner, based on tolerability, over at least 5 weeks, to achieve a dosage of 20 mg SC once daily.
    — Maintain the Palynziq dosage at 20 mg SC once daily for at least 24 weeks.
    — Consider increasing the Palynziq dosage to a maximum of 40 mg SC once daily in patients who have been maintained continuously on 20 mg once daily for at least 24 weeks and who have not achieved either a 20% reduction in blood Phe concentration from pre-treatment baseline or a blood Phe concentration ≤600 micromol/L.

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- The estimated average annual cost of Palynziq is $267,000.
- BioMarin plans to launch Palynziq in June of 2018. Palynziq will be available as 2.5 mg/0.5 mL, 10 mg/0.5 mL and 20 mg/mL single-dose prefilled syringes.