Scenesse® (afamelanotide) – New orphan drug approval

- On October 8, 2019, the FDA announced the approval of Clinuvel's Scenesse (afamelanotide), to increase pain free light exposure in adult patients with a history of phototoxic reactions from erythropoietic protoporphyria (EPP).

- EPP is a rare disorder caused by mutations leading to impaired activity of ferrochelatase. The decrease in ferrochelatase activity leads to an accumulation of protoporphyrin IX (PPIX) in the body. Light reaching the skin can react with PPIX causing intense skin pain and skin changes, such as redness and thickening.

- Scenesse is a melanocortin-1 receptor agonist that increases the production of eumelanin in the skin independent of exposure to sunlight or artificial light sources.

- The approval of Scenesse was based on two vehicle-controlled studies designed to assess exposure to direct sunlight on days with no phototoxic pain. The first study enrolled 93 patients, and the second study enrolled 74 patients.

    - For the first study, the median total number of hours over 180 days spent in direct sunlight between 10 am and 6 pm on days with no pain was 64.1 hours for patients receiving Scenesse and 40.5 hours for patients receiving vehicle.
    - For the second study, the median total number of hours over 270 days spent outdoors between 10 am and 3 pm on days with no pain for which "most of the day" was spent in direct sunlight was 6.0 hours for patients in the Scenesse group and 0.75 hours for patients in the vehicle group.

- A warning and precaution for Scenesse is skin monitoring.

- The most common adverse reactions (> 2%) with Scenesse use were implant site reaction, nausea, oropharyngeal pain, cough, fatigue, dizziness, skin hyperpigmentation, somnolence, melanocytic nevus, respiratory tract infection, non-acute porphyria, and skin irritation.

- The recommended dose of Scenesse is a single 16 mg implant inserted subcutaneously above the anterior supra-iliac crest every 2 months.

    - Scenesse should be administered by a health care professional.
    - Sun and light protection measures should be maintained during treatment with Scenesse to prevent phototoxic reactions related to EPP.

- Clinuvel's launch plans for Scenesse are pending. Scenesse will be available as a 16 mg sterile rod implant.

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