# New Drug Fact Blast

## Clinical Services

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| **Drug/Manufacturer:** | **Scenesse® (afamelanotide) [Clinuvel Pharmaceuticals LTD.]** |
| **Dosage Formulations:** | Rod, Implant 16mg |
| **FDA Approval Date:**<br>**FDB File Date:** | FDA: October 8, 2019<br>FDB: March 15, 2020; became rebateable October 27, 2021 |
| **Indication:** | Increase pain free light exposure in adult patients with a history of phototoxic reactions from erythropoietic protoporphyria (EPP). |
| **Mechanism of Action:** | Scenesse is a synthetic tridecapeptide and a structural analog of α-melanocyte stimulating hormone (α-MSH). Scenesse is a melanocortin receptor agonist and binds predominantly to MC1-R, resulting in increased production of eumelanin (type of melanin pigment) in the skin independently of exposure to sunlight or artificial ultraviolet (UV) light sources. |
| **Dose/ Administration:** | Insert a single Scenesse implant (containing 16mg of afamelanotide) subcutaneously above the anterior supra-iliac crest every 2 months.<br>• Implant is to be inserted by a healthcare provider (outpatient setting) proficient in subcutaneous implant procedure.<br>• SFM implantation canula, sterile gloves, local anesthetic, needle, syringe, blunt forceps, sterile gauze, adhesive bandages and pressure bandages are required for proper implantation (not included).<br>• Monitoring 30 minutes post-implant should be required to ensure no adverse reactions |
| **Disease State Clinical Highlights** | • Erythropoietic protoporphyria (EPP) is a rare autosomal recessive disorder caused by mutations leading to impairment in ferrochelatase (FECH) activity, an enzyme involved in heme production. The decrease in ferrochelatase activity leads to an accumulation of protoporphyrin IX (PPIX) in the body, which can react with light reaching the skin, causing intense pain and skin changes, including redness and thickening.<br>• Few EPP patients develop hepatic complications which can include cholelithiasis or chronic liver disease progressing to rapid acute liver failure.<br>• Erythropoietic protoporphyria comprises about 90% phenotypic presentations, but prevalence is not well characterized in the US. Prevalence estimates range from 1 in 75,000 and 1 in 200,000 in the Netherlands and Wales respectively and prevalence in 17 European countries is estimated at 1 in 140,000.<br>• Gold standard test for the diagnosis of EPP is biochemical analysis (PEE / Porphyrins Evaluation, Whole Blood). |
| **Drug Clinical Highlights** | • Granted priority review and orphan drug designation by FDA<br>• Scenesse is the first implant therapy available to treat erythropoietic protoporphyria patients by increasing light exposure time.<br>• Safety of Scenesse was assessed based on two randomized, prospective, vehicle controlled clinical trials involving 167 participants that assessed time with exposure to direct sunlight without phototoxic pain. A total of 86 subjects received Scenesse and 81 subjects received vehicle implants. The first trial with Scenesse patients had 64.1 exposure hours without pain compared to 40.5 hours with the vehicle group in 180 days. The second trial followed patients over 270 days and determined median pain score of 6 in the Scenesse group versus 17.5 in the vehicle group and total lower maximum pain score per phototoxic episode in comparison to placebo patients (4.0 vs. 6.0). Patients also reported improvement in quality of life and an increase in direct sunlight contact hours.<br>• Contraindications: none indicated |

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| | • Warnings: Skin monitoring due to risk of increased skin pigmentation and darkening of pre-existing nevi and ephelides because of its pharmacologic effect.<br>• ADE/ADRs: implant site reaction, nausea, oropharyngeal pain, cough, fatigue, dizziness, skin hyperpigmentation, somnolence, melanocytic nevus, respiratory tract infection, non-acute porphyria and skin irritation<br>• Bioresorbable sterile rod<br>• In order to receive Scenesse, patients must enroll through the manufacturer's website. Scenesse is only available through accredited EPP specialty centers. There is one EPP specialty center in Missouri, located in Springfield. |
| **Price Per Unit (AWP):** | $56,330.00 per implant |
| **Therapeutic Alternatives:** | This agent is the first FDA approved therapy available for erythropoietic protoporphyria to treat patients with the most common EPP mutation. Symptom and preventative management were first line treatment for erythropoietic protoporphyria. Therapies included:<br>• Sun and UV light exposure avoidance/prevention using zinc oxide or titanium oxide paste<br>• Vitamin D supplementation<br>• Pain control/anti-inflammatory agents<br>• Routine monitoring and interventions |
| **Prior Authorization Approval Criteria:** | <u>Initial Therapy:</u><br>• Documented diagnosis of erythropoietic protoporphyria (ICD-10: E80.0) **AND**<br>• Dermatologic evidence of EPP (i.e. edema, sun-induced erythema, acute painful photodermatitis, urticaria) **AND**<br>• Genetic testing to confirm diagnosis: metal-free protoporphyrin in hemolyzed anticoagulated whole blood (PEE / Porphyrins Evaluation, Whole Blood) followed by Ferrochelatase gene analysis if elevated free protoporphyrin detected<br>  ○ Reference Values:<br>  ▪ Porphyrins, Total, RBC: <80 mcg/dL packed cells<br>  ▪ Free Protoporphyrin: <20 mcg/dL packed cells<br>  ▪ Zinc-Complexed protoporphyrin: <60 mcg/dL packed cells<br>  ○ The proportion of zinc-protoporphyrin compared to metal-free protoporphyrin should be examined to determine EPP from XLP. Protoporphyrin that is metal free should be >90% of total.<br>  ○ See Mayo Clinic Labs Algorithm for diagnostic algorithm on last page **AND**<br>• Prescribed by or in consultation with an appropriate specialist for the disease state **AND**<br>• Patient is ≥18 years **AND**<br>• Documented recent baseline whole body skin exam **AND**<br>• Max 3 implants per 12-month period, with no more than 1 implant every 2 months and length of therapy is at physician's discretion per Clinuvel summary of product characteristics<br>  ○ Indicated for months when sun exposure risk is at its highest (spring/summer months) **AND**<br>• Lack of Bowen's disease, basal cell carcinoma, squamous cell carcinoma, other malignant or premalignant skin lesions, personal history of melanoma or dysplastic nevus syndrome and any other photodermatosis such as polymorphic light eruption, discoid lupus erythematosus or solar urticaria **AND**<br>• Initial approval for one treatment<br><br><u>Continuation of Therapy:</u><br>Subsequent treatments may be approved with documentation of the following:<br>• Full body skin examination twice yearly to monitor pre-existing and new skin pigmentary lesions. |

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| | • Annual documentation of improvement or stability in disease state based on assessment of decrease in phototoxic reactions and increase in sun exposure time without phototoxic reaction based on lack of new lesion development with skin exams. |
| **Implication to State Medicaid Program:** | • The FDA granted the application for Scenesse priority review, fast track status and orphan drug designation.<br>• Data is lacking in certain populations, including pregnancy/lactation and pediatric populations. A small geriatric population size was included in previous studies, but do not have enough evidence to imply safety in patients above 65.<br>• Clinuvel has included post-marketing proposals to clinically follow-up EPP patients over the long-term, which can further provide insight for state level management.<br>• LOE: 4.8.2027 |

**References:**


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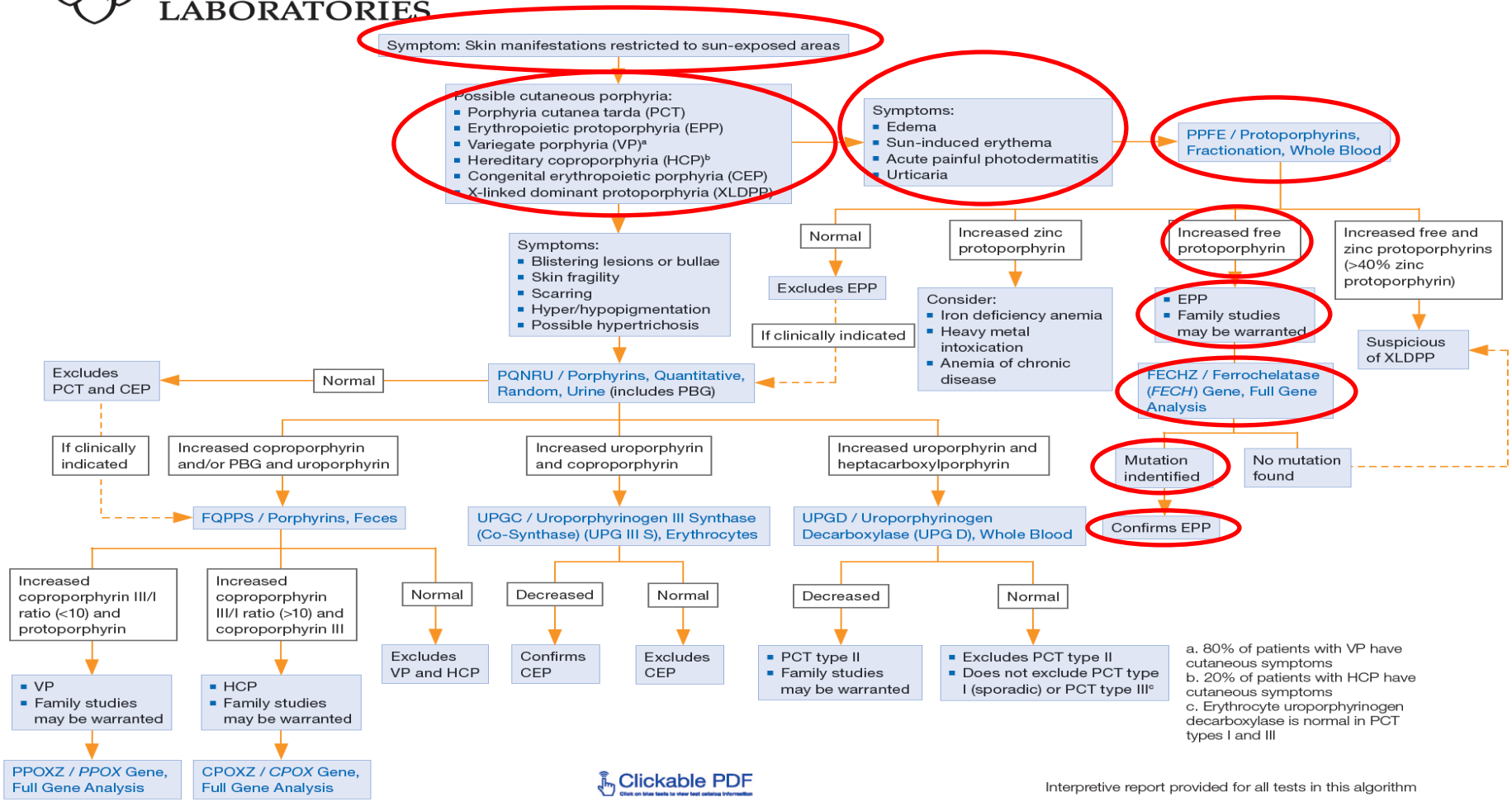
# Porphyria (Cutaneous) Testing Algorithm*

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Interpretive report provided for all tests in this algorithm



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