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Clinical Expert Summary
Afamelanotide (Scenesse®) 16 mg implant

Afamelanotide (Scenesse®) for the prevention of phototoxicity in adult patients with erythropoietic protoporphyria (EPP)

1. Existing guidelines
No guidelines currently exist for the management of EPP.

2. Disease prevalence/incidence
Disease prevalence is difficult to assess as there is no patient registry for people with EPP. During a monthly clinic, experts expect to see approximately two EPP patients per month who are from Wales. As there is no specific treatment at present, many EPP patients seen in the clinic subsequently opt to be monitored in primary care and are therefore not seen regularly by the specialist centre. Experts therefore estimate there to be between 25–50 patients with EPP in Wales who would be eligible for treatment.

3. Current treatment options
There are currently no licensed medicines for the above indication. Various historical medications have been tried but then discontinued due to lack of efficacy. Current management is based around providing information about the condition (including potential complications such as liver failure and vitamin D replacement) and advising on sun protection (behaviour, clothing, special sun creams).

Experts suggested that there may be a benefit from using phototherapy (non-ionising radiation) to toughen the skin without photoactivating the porphyrins. However, phototherapy is not widely used and the evidence is lacking. Furthermore, providing phototherapy has geographical limitations, as patients would need to attend a phototherapy unit up to three times per week over an extended period.

4. Unmet needs
Experts highlighted a ‘huge’ unmet need due to the lack of effective or licensed treatments that can help patients with EPP be less disabled by their condition. Patients’ life choices are limited due to restricted outdoor activities, including everyday activities such as playing outdoors, travelling to and from school, and going shopping.

5. Knowledge of product in given indication
Afamelanotide would be offered as a first-line therapy (specialist only prescribing) in the management of EPP to help patients be less disabled by their condition. There would need to be an agreed process and service in place to support the delivery of the treatment to patients.

Experts noted that not all EPP patients would want an implant-based treatment, but from discussions with patients in clinic they would anticipate most patients would request a trial of this therapy.

It should be noted that one expert involved in compiling this response declared a non-personal specific interest in relation to afamelanotide for the indication under consideration.