Let the Sun Shine for Everyone:
Patient Organization Successfully Appeals NICE!

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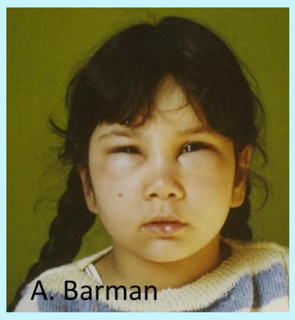
Erythropoietic protoporphyria (EPP)

Patients with the ultra-rare (1:150.000) genetic condition erythropoietic protoporphyria (EPP) suffer from severly painfull phototoxic reactions after being exposed to a few minutes of sunlight and strong artificial light sources.



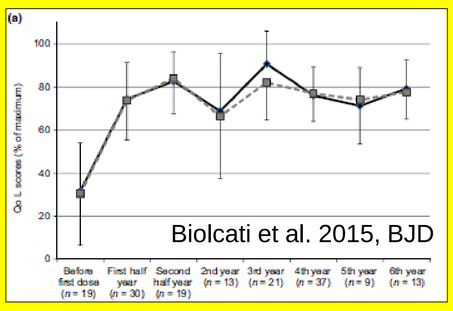
Phototoxic reactions associated with severe neuropathic pain in patients with EPP. As the visible light causes the burns, UV protection measures do not help in EPP.

Biopsy sample: Chronic damage of the blood vessels.

The first treatment for EPP:

In 2014, „afamelanotide“ was approved in the EU for the treatment of EPP: Patients under treatment consistently report life-changing effects and that they are able to spend hours in direct sunlight without developing any painful phototoxic reactions.

Unter treatment with afamelanotide, EPP patients lead an almost normal life and can expose for several hours per day to sunlight.



Quality of life increased from 31% (± 24%) at baseline to 75% (± 17%) **under** treatment.

Negative evaluation by NICE

Despite the transformative effects, the Highly Specialized Technologies Committee of NICE in England recently assessed the benefit of the afamelanotide treatment in EPP as being „small“. As a consequence, **sufferers** in England might not gain access to the only treatment for their condition.

Benefit is not „small“! Successful Appeal by the International Porphyria Patient Network

The International Porphyria Patient Network (IPPN) together with other stakeholders filed **an** Appeal against that decison and took part in the oral Appeal Hearing held **on** 30 July 2018 in London. Three of the Appeal points from IPPN **and other stakeholders** were upheld by the Appeal panel, including that the benefit of the treatment is not to be assessed as „small“.

We now hope that NICE will change its mind and grant access to the only treatment for this severely affected small group of patients

“As EPP is connected with uniquely painful, disfigurating, debilitating, socially disabling and in the long run potentially life-threatening phenotypic manifestations and no authorised medicinal products exists for EPP there is currently a clear unmet medical need for treatment of patients with EPP.”

European Medicines Agency:
European Public Assessment Report (EPAR) of afamelanotide, p.103-104

Resources:
Evaluation by NICE: “Afamelanotide for treating erythropoietic protoporphyria [ID927]”: https://www.nice.org.uk/guidance/indevelopment/gid-hst10009
European Public Assessment Report (EPAR) of afamelanotide: https://www.ema.europa.eu/medicines/human/EPAR/Scenesse
Biolcati, G., Marchesini, E., Sorge, F., Barbieri, L., Schneider-Yin, X., & Minder, E. I. (2015). Long-term observational study of afamelanotide in 115 patients with erythropoietic protoporphyria. British Journal of Dermatology, 172(6), 1601-1612.