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 severely painful phototoxic reactions after being exposed to a few minutes of sunlight and strong artificial light sources.

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.

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 decision 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, disfiguring, 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: