Neue Studie belegt Wirksamkeit von Scenesse!

In 8 europäischen Porphyrie-Zentren in 6 Ländern wurde zusammen eine Scenesse –Studie mit 74 EPP Patienten durchgeführt und auf dem Kongress in Luzern (15.-18. Mai 2013) vorgestellt.
Sie zeigt, dass die Betroffenen im Behandlungszeitraum **20.4 Stunden in der Sonne** verbringen konnten ohne Schmerzen zu entwickeln **im Gegensatz zu 5.6 Stunden ohne Scenesse**. Ausserdem war die Anzahl der Attacken und ihr Schweregrad signifikant geringer, wenn Scenesse gegeben wurde.
In bisherigen Untersuchungen wurde ein Fragebogen verwendet, der einfach nicht die spezielle Situation der EPP-patienten wiedergespiegelt hat – die Patienten berichteten von sehr positiven Erfahrungen unter Behandlung, aber diese Wirksamkeit hat sich nicht in den Daten wiedergespiegelt. In dieser Studie wurde deshalb ein eigener, neu entwickelter Frageboden verwendet, der besser auf die EPP zugeschnitten ist.

Original Zusammenfassung der Studie:

OC15 Afamelanotide Implants Effectively Reduce Pain and Prolong Sun-Tolerance in Patients with Erythropo ï etic Protoporphyria; Results of a Phase III, Multicenter, Double-Blind, Randomized, Placebo-Controlled Trial

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Sponsor: Clinuvel Pharmaceuticals Ltd.
Introduction: EPP patients have a life-long painful photosensitivity which results in strict light avoiding behavior. The symptoms result from an accumulation of photosensitizing protoporphyrin IX. Afamelanotide is a potent 〈 -MSH analogue that stimulates eumelanin production in the skin, which blocks the penetration of light into the skin. The aim of this study was to determine whether afamelanotide can 1) reduce the severity and number of phototoxic reactions, defined as number of days with a Likert pain scale score (LSS) of ε 4, for ε one consecutive day, 2) improve light tolerance, and 3) improve quality of life (QOL) in EPP patients.
Methods: A randomized placebo-controlled trial was conducted in 8 European centers. Seventy-four patients with EPP were enrolled in the study; 38 received 16 mg afamelanotide implants and 36 received placebo implants. The implants were administered subcutaneously on days 0, 60, 120, 180 and 240, via a 14G needle. The median age of the subjects was 36.0 years (range 19-70) and there were no differences between the groups in age, gender, weight or skin type.
The range of erythrocyte free protoporphyrin IX levels was 5.3-274 ⌠ mol/l.
Results: The severity (sum of LSS during all episodes) and the

number of phototoxic episodes per subject was lower in the group who received afamelanotide 18.3 (mean, SD 27.8) and 2.0 (2.7) episodes compared to 52.9 (98.2) and 4.9 (8.5) episodes in those who received placebo ($p = 0.035$ and 0.044 resp.). Patients who received afamelanotide could spent more time in direct sunlight without pain, than placebo recipients (resp. 20.4 vs 5.6 hours during the study period; $p = 0.005$).). No serious adverse events were reported and afamelanotide was well tolerated. The QOL measured by SF36 was not different between the groups, neither at baseline nor after treatment. A new EPP-QOL questionnaire demonstrated a significant improvement in QOL in the afamelanotide group compared to the placebo group (average score of 10 points and 12 points resp. decreased to -1.1 points and 3.2 in treated vs placebo group; $p = 0.011$).

Conclusion: Afamelanotide administration resulted in a more than 3 fold prolongation of the time EPP patients can spend in direct sunlight without pain. Afamelanotide was safe and well tolerated. It ' s effect in improving light tolerance and reducing the risk of phototoxic reactions could possibly result in a major improvement in the quality of life of EPP patients.