ABSTRACT BOOK

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CHILDREN'S TUMOR FOUNDATION
ENDING NF THROUGH RESEARCH

Platform: High Intensity Focused Ultrasound (HIFU) Treatment of Cutaneous Neurofibromas (cNF): Preliminary Results from a Prospective Dual-Center Clinical Investigation

Monday, June 26, 6:00pm – 6:15pm

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Purpose: Investigation of the safety, local tolerability, and efficacy of high intensity focused ultrasound (HIFU) for treatment of NF1 associated cutaneous neurofibromas (cNFs).

Methods: Adult patients having at least 8 treatable cNFs were recruited in two centers. Focused ultrasound treatment utilizing a 20 MHz HIFU-device with integrated dermoscopic guidance was performed using a handpiece with a focus depth of 2.3 mm below the skin surface. Single dose acoustic energy of 0.7 J/dose of pulse duration 250 ms/dose was manually positioned with distance of 1-2 mm between each applied dose, at repetition frequency of 1-2 seconds until the full cNF including a 1 mm perilesional margin was covered. No anesthetic was applied. Primary endpoint was evaluation of safety and tolerance of the HIFU-treatment. Post-treatment effects were assessed immediately after treatment and at follow-up visits including on-site clinical evaluation, patients' evaluation and clinical photography for 6 months. Further evaluation of the included cNFs was performed by ultrasound scanning (US) in one center and histopathology in the other center.

Results: 20 patients with a total of 147 cNFs (mean 7.35/patient; diameter 2-9 mm) received one treatment of each selected lesion. Mild wheal-and-flare reaction was observed immediately after treatment. Occasionally, erosions/crusts were observed and rarely dyspigmentation after 1 week and 3-6 months post-treatment respectively. No serious adverse events occurred, and no significant scarring was observed. The median reduction in cNF thickness measured by US was 0.62 mm (range -100% to +14%). Visual rating of treated cNFs by the clinical investigator at 6 months showed that 30 out of 92 cNFs (32.6%) had full or substantial reduction; biopsied lesions excluded. Patient reported pain-score during treatment was median 3.5 (range 1-7) on a 0-10 point scale. No pain was reported post-treatment.

Conclusions: HIFU treatment is a new non-invasive treatment modality that with high precision targets intradermal lesions; it is a rapid and tolerable treatment. This study demonstrates the safety, local tolerability, efficacy, and feasibility of HIFU for the treatment of cNFs in adults with NF1. The variation in cNF reduction after HIFU-treatment and the occasional erosions and crusts in the treatment area indicate that dosing needs to be further adjusted. Follow-up pre-clinical and clinical studies to optimize the dose response in adults with NF1 are underway with a goal of applying this therapy to both established and developing cNFs in the future.

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