Effect of Aminaftone on Raynaud's Phenomenon (RP) Secondary to Systemic Sclerosis: A Double-Blind Prospective, Randomized, Placebo-Controlled Pilot Study

SUMMARY

BACKGROUND: In this study aminaftone (AMNA), a derivate of 4-aminobenzoic acid has been tested to treat R.P. secondary to Systemic Sclerosis (SS) because of its previous described anti-endothelin 1 activity.

METHODS: it was a single centre, double-blind , placebo-controlled clinical trial. Patients received either 75 mg of AMNA 3 times daily for 12 weeks in wintertime or matching doses of placebo. The Investigators compared the number and the severity of RP attack at baseline and after 12 weeks. Furthermore the effect of AMNA vs PLACEBO has been detected in controlling serum ET-1 levels (baseline vs 12 weeks).

RESULTS: Twenty-five patients were randomized to received AMNA or PLACEBO, 23 patients (12 AMNA; 11 PLACEBO) complete the study. No drug-related adverse events has been detected. The two distint groups were statistically overlapping for baseline clinical and demographical characteristics. An encoraging and strong trend on the reduction of the number of RP attacks in the AMNA vs PLACEBO group was observed (median – 67.9% (-40.7% - - 83.3%) vs -44.2% (-15.5% - 54.3%) p=0.06 (Mann Whitney Test); no differences in RP severity scores or RP duration scores were observed. ET-1 serological concentrations were markedly and significantly reduced in the treatment arm compared to the placebo arm (median -43.5% (-25.5% - - 46.2%) VS 4.9% (-0.6% - +15.9%) p=0.02 (Mann-Whitney Test).

CONCLUSIONS: Although the protocol primary clinical end-point was not meet, most likely due to the low sample size, AMNA was found to have a meaningful and statistical significative effects on ET-1 serum levels. In light of the results of a previous report showing the non-inferiority of AMNA compared to bosentan in the long-term control of the peripheral SS-related vascular clinical manifestations, when bosentan was not indicated, it is reasonable to plan more focused studies on a big sample size of patients in order to confirm the efficacy of AMNA in the treatment of RP secondary to SS.