

Safety and Efficacy of AQX-1125 in Interstitial Cystitis/Bladder Pain Syndrome - Results of the Phase 2 LEADERSHIP Trial

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Introduction and Objectives: AQX-1125, a novel SH2-containing inositol-5'-phosphatase 1 (SHIP1) activator represents a potential therapy for bladder pain syndrome (BPS). This 6-week randomized, double-blind, placebo-controlled Phase 2 trial assessed the safety and efficacy of AQX-1125 on average daily bladder pain and in standardized BPS questionnaires.

Methods: Women with BPS, having cystoscopic evidence of inflammation within 3 years and a baseline mean pain score of ≥ 5 were randomized to daily AQX-1125 or placebo for 6 weeks. Daily average and maximal pain scores and urinary frequency were recorded by e-diary and in the clinic. O'Leary-Sant Symptom and Problem Indices (ICSI/PI), Bladder Pain IC Symptom Scale (BPIC-SS) and Short Form 12 Health Survey (SF-12v2) questionnaires were administered. Safety data was collected through 6 weeks treatment and 4 weeks follow up.

Results: 69 women were randomized to AQX-1125 (n=37) and placebo (n=32). Mean age (52.6 years), weight (71.5 kg) and duration of BPS at diagnosis (69.4 months) were balanced between arms. At 6 weeks, the daily average pain score was reduced by 2.4 points (AQX-1125) versus 1.4 points (placebo) ($p=0.061$ ANCOVA). AQX-1125 significantly improved ICSI with a reduction of 3.7 points versus 1.3 on placebo ($p=0.005$) and ICPI with a reduction of 3.5 versus 1.5 points ($p=0.015$). Maximum pain and BPIC-SS results will also be presented. AQX-1125 was well tolerated with no SAEs reported. Adverse event rates were similar between AQX-1125 (51.4%) and placebo (78.1%).

Conclusions: Women with moderate to severe BPS treated with AQX-1125 reported greater reduction in bladder pain and symptoms at 6 weeks, compared to placebo. AQX-1125 was well tolerated. This compelling data supports continued development of AQX-1125 for BPS.