Objective: To assess the safety and efficacy of incobotulinumtoxinA for benign essential blepharospasm (BEB) in toxin-naïve subjects.

Background:
IncobotulinumtoxinA is efficacious for BEB. This was the first, randomized, Phase III study (NCT01896895) in toxin-naïve subjects. Here we present data from the complete study.

Design/Methods: Subjects (18–80 years) with bilateral BEB, Jankovic Rating Scale (JRS) severity subscore ≥2, and no BEB treatment with any botulinum neurotoxin (BoNT) serotype within past ≥12 months, were enrolled. In the main period (MP), subjects were randomized (1:1:1) in a double-blind manner to single intramuscular injections of incobotulinumtoxinA 25U (12.5U/eye), 50U (25U/eye) or placebo, with an observation period (OP) of 6–20 weeks. Subjects with a need for re-injection (JRS severity subscore ≥2 at final MP visit) were eligible for the open-label extension period (EP): a single dose of incobotulinumtoxinA ≤70U (≤35U/eye) with a 6–20-week OP. Mean change from baseline in JRS severity subscore and safety were assessed.

Results: Overall, 61 subjects were randomized (mean 55.0 years; 59.0% female); 55 completed the MP and 39 entered and completed the EP. At MP Week 6, JRS severity subscore significantly improved from baseline with incobotulinumtoxinA 50U versus placebo (p=0.0004), and numerically improved with incobotulinumtoxinA 25U versus placebo. Sustained improvements were seen with incobotulinumtoxinA ≤70U from EP baseline to EP Week 6 (−1.2) and to EP final visit (−0.7), and from MP baseline to EP final visit (−1.0) (all p<0.0001). In the MP, more adverse events (AEs) were reported with incobotulinumtoxinA ≤70U (42.1%) versus 25U (31.8%) or placebo (30.0%). AEs were less frequent in the EP (all incobotulinumtoxinA-treated: 28.2%). Most AEs were of mild-to-moderate severity.

Conclusions: IncobotulinumtoxinA showed sustained efficacy in toxin-naïve subjects with BEB. Long-term safety results were in line with the known safety profile.