Safety and efficacy of repeated NT 201 (Botulinum neurotoxin type A free from complexing proteins) injections of patients with blepharospasm

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Background
Blepharospasm is a form of focal dystonia that is characterized by excessive muscle tone in the ocular muscles of the eyelid. This leads to spasms of the eyelids, which can be painful and interfere with the patients' daily lives.

The visual impairment caused by blepharospasm is associated with significant disability that can impact on many aspects of daily life. For example, for people with blepharospasm may find it difficult, or impossible, to read, watch television, or use a computer. Furthermore, frequent travel may be challenging.

NT 201 (Merz, Merz Pharmaceuticals, Germany) is a preparation of Botulinum toxin type A (BoNT/A) that is free from complexing proteins, and is indicated for the symptomatic treatment of blepharospasm.

In a previous double-blind, randomized, clinical study of 170 patients with blepharospasm, NT 201 demonstrated comparable efficacy and safety to oculimuscle of the eyelid. This leads to a sporadic or continuous contraction of the eyelid muscles, thus producing involuntary spasms.

Methods
Study design
The study was an open-label extension (OLEX) of a 2-week placebo-controlled, double-blind study of NT 201 in patients with blepharospasm.

In the double-blind, placebo-controlled portion of the study, patients were assigned to single treatment with NT 201 or placebo and followed up for 22 days. All 170 patients enrolled in the study were dose-escalated to 6 injection sessions as per the Investigator's discretion, and dosing was tailored to the patient's global response to NT 201 treatment as ‘very good’ or ‘good’ in the large majority of patients.

Patient global response
Across the OLEX period, the range of patients who rated their global response to NT 201 treatment as ‘improved’ was between 63.9% and 96.6% of cases. This included ratings of ‘very improved’ (34.5%–81%) and ‘complete abolition of all signs’ (5%–16%).

Safety outcomes

Patient global response

Safety outcomes

Conclusions

Repetitive injections with NT 201 were effective in improving symptoms (JRS score) and disability (BSDI scores) in patients with blepharospasm.

The efficacy of NT 201 was maintained across up to 20 weeks. An open-label extension study (OLEX) with repeated injections of NT 201 was also well tolerated in the long-term, with no safety concerns associated with repeated dosing.

Overall, investigators rated the global tolerability of NT 201 injections as ‘very good’ or ‘good’ in the large majority of cases (93.3%–100%).