



BOTOX (onabotulinumtoxinA)

RATIONALE FOR INCLUSION IN PA PROGRAM

Background

Botulinum toxin (abbreviated either as BTX or BoNT) is a protein neurotoxin produced by the bacterium *Clostridium botulinum*. The botulinum toxins are characterized as 7 separate neurotoxins (labeled as types A, B, C [C1, C2], D, E, F, and G), which are antigenically and serologically distinct but structurally similar. The neuromuscular blockade is achieved through prevention of docking/fusion of neurosecretory with the nerve synapse plasma membrane and release of neurotransmitters (1).

The various botulinum toxins have approved cosmetic and non-aesthetic uses. They possess individual potencies, and care is required to assure proper use and avoid medication errors. Recent changes to the established drug names by the FDA were intended to reinforce these differences and prevent medication errors (1-2).

Regulatory Status

FDA-approved indications: Botox is an acetylcholine release inhibitor and a neuromuscular blocking agent indicated for: (3)

1. Treatment of overactive bladder (OAB) with symptoms of urge urinary incontinence, urgency, and frequency, in adults who have an inadequate response to or are intolerant of an anticholinergic medication.
2. Treatment of urinary incontinence due to detrusor over-activity associated with a neurologic condition [e.g., spinal cord injury (SCI), multiple sclerosis (MS)] in adults who have an inadequate response to or are intolerant of an anticholinergic medication.
3. Treatment of neurogenic detrusor overactivity (NDO) in pediatric patients 5 years of age and older who have an inadequate response to or are intolerant of anticholinergic medication.
4. Prophylaxis of headaches in adult patients with chronic migraine (≥ 15 days per month with headache lasting 4 hours a day or longer).
5. Treatment of spasticity in patients 2 years of age and older.
6. Treatment of cervical dystonia in adult patients, to reduce the severity of abnormal head position and neck pain.
7. Treatment of severe axillary hyperhidrosis that is inadequately managed by topical agents in adult patients.



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8. Treatment of blepharospasm associated with dystonia in patients ≥ 12 years of age.
9. Treatment of strabismus in patients ≥ 12 years of age.

Limitations of Use:

Safety and effectiveness of Botox have not been established for the prophylaxis of episodic migraine (14 headache days or fewer per month) or for the treatment of hyperhidrosis in body areas other than axillary (3).

Off-Label Uses: (4-11)

1. Achalasia
2. Chronic anal fissures
3. Essential tremor
4. Excessive salivation secondary to advanced Parkinson's disease
5. Hemifacial spasm
6. Spasmodic dysphonia (laryngeal dystonia)

Safety and effectiveness of Botox have not been established for the treatment of hyperhidrosis in body areas other than axillary (4).

Botulinum toxins are not interchangeable. Total accumulated dose should not exceed 400 IU over a 3-month interval (3).

Some products have cosmetic indications which are excluded from coverage.

Summary

Botulinum toxin (abbreviated either as BTX or BoNT) is a protein neurotoxin produced by the bacterium *Clostridium botulinum*. The botulinum toxins are characterized as 7 separate neurotoxins (labeled as types A, B, C [C1, C2], D, E, F, and G), which are antigenically and serologically distinct but structurally similar (1).

The various botulinum toxins have approved cosmetic and non-aesthetic uses. They possess individual potencies, and care is required to assure proper use and avoid medication errors. Recent changes to the established drug names by the FDA were intended to reinforce these differences and prevent medication errors (1-2).



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Prior authorization is required to ensure the safe, clinically appropriate, and cost-effective use of Botox while maintaining optimal therapeutic outcomes.

References

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