Oxervate™ (cenegermin-bkbj) – New orphan drug approval

- On August 22, 2018, the FDA announced the approval of Dompé's Oxervate (cenegermin-bkbj) for the treatment of neurotrophic keratitis.
    - Oxervate was granted priority review designation.
- Oxervate is the first ever topical biologic medication approved in ophthalmology. It is the first ever application of a human nerve growth factor (NGF) and the only FDA approved treatment available for this indication in the U.S.
- Neurotrophic keratitis is a rare orphan condition that affects fewer than 65,000 persons in the U.S. It is a degenerative disease resulting from impaired function of corneal nerves, which can be caused by infections, ocular surface injuries, ocular or neurologic surgeries, and some systemic conditions that can impair corneal sensation. The loss of corneal sensation causes progressive damage to the top layer of the cornea, including corneal thinning, ulceration, and perforation in severe cases.
- Cenegermin-bkbj is a novel recombinant human NGF that is structurally identical to the NGF protein that is made in the human body, including in the ocular tissues
- The safety and efficacy of Oxervate as compared to vehicle was based on two, eight-week clinical studies of 151 patients with neurotrophic keratitis. All eye drops in both studies were given six times daily in the affected eye(s) for eight weeks and underwent a follow up period.
    - In study NGF0212, 72.0% of patients with disease in one eye experienced complete corneal healing with Oxervate vs. 33.3% with vehicle (difference: 38.7% [95% CI: 20.7%, 56.6%]; $p < 0.01$).
    - In study NGF0214, 65.2% of patients with disease in both eyes experienced complete corneal healing with Oxervate vs 16.7% with vehicle (difference: 48.6% [95% CI: 24%, 73.1%]; $p < 0.01$).
- Warnings and precautions of Oxervate include eye discomfort and use with contact lenses.
- The most frequently reported adverse reactions (> 5%) with Oxervate use were eye pain, ocular hyperemia, eye inflammation, and increased lacrimation.
- The recommended dose of Oxervate is one drop instilled in the affected eye(s), 6 times a day at 2-hour intervals for eight weeks.
- Dompé plans to launch Oxervate by early 2019.Oxervate will be available as a 20 mcg/mL eye drop solution supplied in a weekly carton containing 7 multiple dose vials.