Eyes on New Product Development

Gary D. Novack^{1,2}

IN THIS PERIOD, there was a first-time approval for an intravenous treatment for thyroid eye disease, and an approval for an intraocular dye to assist in retinal surgery. In dry eye, trials for new pharmaceutical agents are in various clinical phases. A novel treatment for allergic conjunctivitis is in late stage development, a gene therapy firm released interim results on their new technology, and a novel therapy for the treatment of glaucoma is in early stage clinical development. In the regulatory area, a Food and Drug Administration (FDA) approval gives more precedent regarding policy on orphan drugs, and there is continued discussion on issues such as the use of real-world evidence and women in clinical trials. Importantly, a recent report suggests that “predatory” journals have little impact on science, and the real value of masking of clinical trials is discussed.

Ophthalmic Pharmaceuticals and Biologics

- Aerie announced that the European Medicines Agency has accepted for review the marketing authorization application for Roclanda (netarsudil and latanoprost ophthalmic solution, currently marketed as Rocklatan in the United States (January 2020).
- Aerpio announced results from the fifth cohort of subjects from a phase 1b trial of a topical ocular formulation of AKB-9778 in patients with ocular hypertension or primary open angle glaucoma (January 2020).
- Aldeyra started enrollment in its Phase 3 INVIGORATE Trial of topical ocular reproxalap in patients with allergic conjunctivitis. The firm also started enrollment in a phase trial of ADX-2191 (intravitreal methotrexate) for the prevention of recurrent retinal detachment due to proliferative vitreoretinopathy (January 2020).
- Allysta Pharmaceuticals started enrollment in a phase 1/2a trial of ALY688, a novel peptide agonist that binds to and activates adiponectin receptors, in patients with dry eye disease (January 2020).
- Dutch Ophthalmic Research Centre (DORC) received FDA approval for its brilliant blue for the visualization of internal limiting membranes (December 2019).
- Genentech and Roche started enrollment in a phase 3 trial of its Port Delivery System with ranibizumab (PDS) in people with diabetic macular edema (DME, January 2020).

- Horizon received FDA approval for its Tepezza™ (teprotumumab) as a treatment for thyroid eye disease (January 2020).
- Kala Pharmaceuticals completed enrollment in its Short Term Relief In Dry Eye (STRIDE-3) phase 3 clinical trial for KPI-121 0.25% (loteprednol etabonate) for the short-term treatment of dry eye disease (January 2020).
- Ocuphire entered into an agreement with Apexian Pharmaceuticals, Inc., granting Ocuphire an exclusive worldwide sublicense to the Apexian’s Ref-1 Inhibitor program, including its lead drug candidate APX3330, for all ophthalmic and diabetic indications (January 2020).
- Oyster Point Pharma announced the top-line results from its phase 2 MYSTIC study of OC-01 (in dry eye disease (January 2020)).¹

Gene Therapy

- Applied Genetic Technologies Corporation reported interim 6-month data from its ongoing phase 1/2 clinical program in X-linked retinitis pigmentosa (January 2020).

Regulatory, Government, and Pharmaceutical Industry

- Bartlett et al. conducted a cross-sectional analysis on the potential to use Real Word Data² to replicate clinical trial evidence. They found that only 15% of the U.S.-based clinical trials published in high-impact journals in 2017 could be feasibly replicated through analysis of administrative claims or EHR data. These findings suggest the potential for observational data to complement but not completely replace randomized clinical trials (January 2020).³
- Björk et al. found that there are few citations to “predatory” journals, suggesting that they have little impact.^{4,5}
- The FDA Center for Drug Evaluation and Research provided a list of 48 approved novel drugs in the calendar year 2019. Of these, 2 are ophthalmic products (Tissue Blue™, brilliant blue G ophthalmic solution to DORC, and Beovu®, broclicizumab-dblb to Novartis). Also approved in the same time period, but not including on this “novel drug” list were Lotemax SM®,

¹PharmaLogic Development, Inc., San Rafael, California.

²Department of Ophthalmology, School of Medicine, University of California, Davis, Sacramento, California.

- loteprednol etabonate to Bausch and Lomb, Rocklatan® netarsudil/latanoprost to Aerie, Tetracaine to Bausch and Lomb, and Avaclyr™, acyclovir to Fera.
- FDA policy on orphan designation and approval for orphan products [21 CFR 316.3(b)(3)] was evidenced by its approval of a nasal spray of diazepam to treat frequent seizure activity (Valtoco™, Neurelis), and denial of a citizen's petition by Aquestive investigating a buccal film formulation of diazepam, in which it requested staying of the approval pending additional clinical studies. It also appears that this new product will receive 7 years of marketing exclusivity (January 2020).
 - Glaxo Smith Kline voluntarily halted production of a long-time produced over-the-counter product, Excedrin® (acetaminophen and aspirin) due to inconsistencies in manufacturing methods (January 2020).
 - Godlee, editor-in-chief of the British Medical Journal (BMJ), referencing several articles in a recent issue, concluded that "new research published in the BMJ casts doubt on the benefits of [masking]" of clinical studies that can add cost and compromise patient safety, in favor of large studies with appropriate randomization.⁶⁻⁹
 - Goldstein and Walensky highlight the issue of lack of parity in enrolling women in clinical trials, especially those of anti-HIV treatments.¹⁰
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Author Disclosure Statement

The author consults for numerous ophthalmic, pharmaceutical, and medical device firms. A complete listing of all of Dr. Novack's consulting affiliations can be found online.

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Address correspondence to:

Dr. Gary D. Novack
PharmaLogic Development, Inc.
17 Bridgegate Drive
San Rafael, CA 94903

E-mail: gary_novack@pharmalogic.com