Eyes on New Product Development

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IN THIS REPORTING PERIOD, several drug delivery firms reported results on Phase 2 and Phase 3 trials with glaucoma and anti-inflammatory therapies, as did a firm using RNAi technology. Cell and gene therapies are making progress in the treatment of retinal diseases. One firm has re-submitted a request for approval of a combination product for the treatment of corneal disease. A novel biologic treatment is being evaluated in the giant cell arteritis, a disease with no approved therapy.

Ophthalmic Pharmaceuticals and Biologics

- Ampio Pharmaceuticals is continuing to develop Optina™ (danazol) for the treatment of diabetic macular edema in patients refractory to currently available drugs (October 2015). www.prnewswire.com/news-releases/ampio-pharmaceuticals-inc-announces-updates-on-the-regulatory-path-for-fda-approval-of-optina-an-oral-treatment-for-diabetic-macular-edema-dme-300159361.html
- Avedro, Inc. resubmitted its New Drug Application (NDA) for riboflavin ophthalmic solution/KXL® System to the US Food and Drug Administration (FDA) for the treatment of progressive keratoconus and corneal ectasia following refractive surgery (October 2015). <http://avedro.com/press-releases/avedro-announces-resubmission-of-new-drug-application-to-fda-for-corneal-cross-linking/>
- BioTime, Inc. and its subsidiary Cell Cure Neurosciences Ltd. received fast track designation from the US FDA for OpRegen®, a cell-based therapeutic product consisting of retinal pigment epithelial (RPE) cells designed to block the progression of the severe dry form of age-related macular degeneration (September 2015). http://investor.biotimeinc.com/phoenix.zhtml?c=83805&p=irol-newsArticle_print&I0=2090854
- Envisia Therapeutics reported results from a Phase 2a trial of ENV515 (travoprost XR) in patients with glaucoma and ocular hypertension (October 2015). www.envisiatherapeutics.com/category/announcements/
- Gene therapy based on a mouse model of Leber hereditary optic neuropathy at the National Institutes of Health¹ is now in a clinical trial (October 2015). www.nih.gov/news/health/oct2015/nei-05.htm
- Genentech is conducting a Phase 3 trial of tocilizumab for the treatment of giant cell arteritis (October 2015).

www.healio.com/rheumatology/vasculitis-and-giant-cell-arteritis/news/online/%7Ba4147dee-3341-42c5-aef8-df5ce7a144b2%7D/phase-3-actemra-trial-underway-for-patients-with-giant-cell-arteritis

- Ocular Therapeutix submitted an NDA for Dextenza® (sustained release of dexamethasone in punctal plugs) for the treatment of postsurgical ocular pain. It also started a third Phase 3 study for this product in this indication, as well as reported results for this product in this study for the treatment of allergic conjunctivitis. Finally, the firm reported results for a Phase 2b study of travoprost for the treatment of glaucoma and ocular hypertension (September and October 2015). beta.finance.yahoo.com/news/ocular-therapeutixtm-reports-topline-results-203300345.html; [www.streetinsider.com/Corporate+News/Ocular+Therapeutix+\(OCUL\)+Announces+Statistically+Significant+Topline+Data+from+DEXTENZA+Phase+3/10995694.html](http://www.streetinsider.com/Corporate+News/Ocular+Therapeutix+(OCUL)+Announces+Statistically+Significant+Topline+Data+from+DEXTENZA+Phase+3/10995694.html); <http://investors.ocutx.com/phoenix.zhtml?c=253650&p=irol-newsArticle&ID=2090846>; <http://investors.ocutx.com/phoenix.zhtml?c=253650&p=irol-newsArticle&ID=2097159>
- Shire received a complete response letter from the US FDA for their NDA for lifitegrast for dry eye. Additional clinical data were requested (October 2015). www.benzinga.com/news/15/10/5921835/shire-receives-fda-crl-for-lifitegrast-nda-and-plans-to-respond-with-opus-3-trial
- Spark Therapeutics announced results from a Phase 3 trial of its gene therapy product candidate, SPK-RPE65, for the treatment of RP65-mediated inherited retinal dystrophies (October 2015). <http://ir.sparktx.com/phoenix.zhtml?c=253900&p=irol-newsArticle&ID=2093863>
- Sylentis announced results of a dose-finding Phase 2 study for the novel RNAi therapeutic bamosiran, which blocks the production of β_2 -adrenergic receptors in patients with open-angle glaucoma or ocular hypertension (October 2015). www.eurekalert.org/pub_releases/2015-10/p-sat100215.php

Regulatory, Government, and Pharmaceutical Industry

- The American Society of Cataract and Refractive Surgery (ASCRS) has released an official position statement opposing directives that restrict 2 well-established presurgical ophthalmic practices: the use of diluted povidone-iodine

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solution for topical infection prophylaxis and the use of multidose topical eye drops on multiple patients. ASCRS finds dilution of marketed 10% povidone-iodine solution with saline to prepare a 5% solution to be “safe and effective.” They also support the use of multidose eye drops on multiple patients as part of the preoperative surgical protocol (October 2015). www.prweb.com/releases/2015/08/prweb12907908.htm

- Supporting Initiatives to Redistribute Unused Medicine (SIRUM) is providing unused, unexpired prescription medications to low-income patients (October 2015). www.wsj.com/articles/match-com-for-unused-medicine-1443798827*

Author Disclosure Statement

The author consults for numerous ophthalmic, pharmaceutical, and medical device firms.

Reference

1. Yu, H., Koilkonda, R.D., Chou, T.H., et al. Consequences of zygote injection and germline transfer of mutant human mitochondrial DNA in mice. *Proc. Natl. Acad. Sci. U. S. A.* 112:E5689–E5698, 2015.

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