Eyes on New Product Development

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IN THIS PERIOD, 2 new products are in late stage evaluation for the treatment of allergic conjunctivitis, and data were reported on a controlled study of a new device for use in glaucoma surgery. Legislative and regulatory actions continue including reorganization within the Food and Drug Administration (FDA) and efforts to manage the affordability of medications. Several gene and cell therapies are in clinical development for the treatment of retinal diseases. A formulation of bevacizumab designed for use in ophthalmology is in late stage clinical trials.

Ophthalmic Pharmaceuticals and Biologics

- Aerie announced that the European Medicines Agency (EMA) recommended marketing authorization approval for its Rhokiinsa™ (netarsudil, sold as Rhopressa® in the United States) for the reduction of intraocular pressure in primary open-angle glaucoma or ocular hypertension (September 2019).
- Aldeyra presented results of its ALLEVIATE phase 3 clinical trial of reproxalap in patients with allergic conjunctivitis (September 2019).
- Allergan and Molecular Partners announced that the EMA has accepted for review a biologics license application for abicipar pegol, an investigational DARPIn therapy for wet age-related macular degeneration (AMD) (September 2019).
- Clearside Biomedical announced that FDA requested additional chemical stability data on the new drug application (NDA) for its Xipere™ (triamcinolone acetate suprachoroidal injectable suspension) currently under review (August 2019).
- Ocular Therapeutix commenced enrollment in a phase 3 clinical trial of its Dextenza™ (dexamethasone intraocular implant) for the treatment of allergic conjunctivitis (September 2019).
- Outlook Therapeutics commenced enrollment in a phase 3 clinical trial of its ONS-5010, a proprietary ophthalmic bevacizumab product candidate for the treatment of wet AMD (July 2019).
- Oxurion presented results from a phase 1 study of its THR-149, a plasma kallikrein inhibitor, in patients with diabetic macular edema (September 2019).
- ProQR received orphan designation from the FDA for its QR-1123, an investigational antisense oligonucleotide for the treatment of autosomal dominant retinitis

pigmentosa due to the P23H mutation in the rhodopsin gene (September 2019).

- Regenxbio and Clearside Biomedical announced an option and license agreement that will give Regenxbio exclusive worldwide rights to Clearside's SCS Microinjector for the delivery of RGX-314 to the suprachoroidal space (September 2019).
- Tarsius Pharma submitted an investigational new drug (IND) application with the FDA for its TRS01, an immune-based treatment, for the treatment of ocular diseases (September 2019).
- Vertical Pharmaceuticals, a subsidiary of Osmotica Pharmaceuticals, submitted a NDA to the FDA for RVL-1201, oxymetazoline, for acquired blepharoptosis treatment candidate (September 2019).

Gene and Cell Therapy

- Adverum reported results on the OPTIC phase 1 trial of its ADV-022 gene therapy designed for long-term vasoactive endothelial growth factor (VEGF) suppression through the expression of aflibercept (September 2019).
- Lineage Cell Therapeutics presented results of the safety and tolerability of OpRegen (Retinal Pigment Epithelial cells) in a dose-escalating phase 1/2a clinical trial in patients with advanced dry AMD accompanied by geographic atrophy (September 2019).
- Ocugen's OCU400, a gene therapy candidate, received a second orphan drug designation from the FDA for the treatment of CEP290 mutation-associated retinal disease (September 2019).
- Wize Pharma entered into an exclusive license agreement to develop and commercialize products based on nonviral gene therapy technology from Copernicus Therapeutics. WP-REP1, Wize's gene therapy product based on Copernicus' technology, will comprise DNA-compromised nanoparticles and designed to treat choroideremia (September 2019).

Medical Devices

- Santen presented results on its DE-128 microshunt in its INN-005 controlled trial in patients with primary open-angle glaucoma not controlled with maximum tolerated glaucoma medications (August 2019),

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Regulatory, Government, and Pharmaceutical Industry

- Sandoz voluntarily recalled all generic forms of Zantac[®] (ranitidine) in the United States after confirmed contamination with N-nitrosodimethylamine, a possible human carcinogen (September 2019).
- The *Economist* presented an analysis that the pressure to reduce the prices of generic medicines has led to an increasingly fragmented and globalized production process, which reduces the number of manufacturers, increasing the risk for drug shortages.¹
- According to a reorganizational plan for the FDA, new ophthalmic drugs, currently reviewed by the Division of Transplant and Ophthalmic Products would be in a new Division of Ophthalmology in the Office of Specialty Medicines (September 2019).
- U.K.'s National Health Service Department of Health Research Authority provided guidance on the impact of a "no-deal" Brexit on clinical trials of medicinal products medical devices (August 2019).
- U.S. House Speaker Pelosi proposed legislation that would allow the federal government to negotiate the prices of prescription drugs for Medicare health care beneficiaries (September 2019).

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Reference

1. Anonymous. A dire scarcity of drugs is worsening, in part, because they are so cheap. *Economist*. 2019.

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