Eyes on New Product Development

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IN THIS PERIOD, several new drug delivery technologies have been used to deliver therapeutic doses of existing agents to the posterior segment, and several gene therapy companies provided clinical updates. The U.S. Food and Drug Administration (FDA) noted safety issues with compounded and off-label ophthalmic therapy. Two new chemical entities are being evaluated for topical use in corneal disease, and late stage activities/approvals were reported in treatment two orphan ophthalmic diseases. Finally, 2 groups donated to the Henrietta Lacks Foundation in recognition of their use of HeLa cells in research.

Ophthalmic Pharmaceuticals and Biologics

- Aerie reported phase 2 clinical results for its AR-1105 (Dexamethasone Intravitreal Implant, July 2020).
- Clearside submitted an Investigational New Drug Application (IND) to the U.S. FDA for its CLS-AX (axitinib injectable suspension) to conduct a phase 1/2a clinical trial in wet age-related macular degeneration (AMD) patients by the end of 2020 (August 2020).
- Genentech received U.S. FDA approval for its Enspryng™ (satralizumab-mwge), a subcutaneous injection for treatment of adult with anti-aquaporin-4 (AQP4) antibody positive neuromyelitis optica spectrum disorder (August 2020).
- Iveric started dosing in a second phase 3 trial of its Zimura® (avacincaptad pegol) for the treatment of geographic atrophy secondary to AMD (July 2020).
- Kubota Vision received an orphan product clinical trial grant from the U.S. FDA to support the ongoing phase 3 study of emixustat in Stargardt disease (August 2020).
- Neuroptika completed enrollment in a phase 2 controlled double-masked clinical trial of NRO-1 for the treatment of patients with dry eye disease (August 2020).
- Ocugen received orphan designation status for its gene therapy (OCU400) to treat rhodopsin mutation-associated retinal degenerative disease (July 2020).
- Oculis announced that based upon a meeting with the U.S. FDA, they are pursuing phase 3 clinical trials for OCS-01 (topical dexamethasone) for the treatment of inflammation and pain after cataract surgery as well as diabetic macular edema (DME, July 2020).
- Roche announced results of its phase 3 study of the Port Delivery System with ranibizumab for the treatment of neovascular AMD (July 2020).

- **Trefoil Therapeutics today initiated a clinical trial of its Fibroblast Growth Factor-1, TTHX1114, for the regenerative treatment of people with corneal endothelial dystrophies, including Fuchs Endothelial Corneal Dystrophy (August 2020).

Cell and Gene Therapy

- 4D Molecular Therapeutics commenced enrollment for a phase 1/2 clinical trial of 4D-125 (AAV gene) for X-linked retinitis pigmentosa (XLRP, August 2020).
- Applied Genetic Technologies Corporation (AGTC) announced expansion of its ongoing phase 1/2 clinical study testing its gene therapy treatment for XLRP caused by mutations in the Retinitis Pigmentosa GTPase Regulator gene (July 2020).
- jCyte announced results from its phase 2b clinical trial of its cell therapy for patients with retinitis pigmentosa (July 2020).
- Regenxbio reported data from patients in cohorts 4 and 5 of the phase 1/2a trial of RGX-314 for the treatment of wet AMD. They have also activated a phase 2 trial of RGX-314 for the treatment of wet AMD delivered to the suprachoroidal space (AAVIATE, August 2020).

Ophthalmic Devices

- Olympic Ophthalmics received FDA *de novo* request clearance for its iTEAR100, an external prescription device designed to temporarily increase acute tear production in adults by stimulating a cutaneous nerve (June 2020).
- Gyroscope Therapeutics received 510(k) clearance from the FDA for the Orbit subretinal delivery system (August 2020).

Regulatory, Government, and Pharmaceutical Industry

- Abcam, a life sciences firm, and the laboratory of Smaara Reck-Peterson, PhD, at the University of California, San Diego, are donating funds to the Henrietta Lacks Foundation in recognition of their use of HeLa cells in research (August 2020).
- Moderna and the National Institute of Allergy and Infectious Diseases (NIAID) started a phase 3 clinical

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trial of an investigational vaccine, mRNA-1273, for the prevention of COVID-19 disease (July 2020).

- The U.S. FDA has received 29 cases of TASS (Toxic Anterior Segment Syndrome) after intraocular administration of compounded drugs using moxifloxacin as a bulk drug substance, as well as reports associated with the intraocular administration of repackaged and/or diluted FDA-approved moxifloxacin drugs (August 2020).

** Indicates news items with scientific articles in this issue.

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