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Eyes on New Product Development

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In this review period, there was substantial regulatory progress on the use of a small molecule to treat Leber Hereditary Optic Neuropathy (LHON). Approved in Japan was a novel prostaglandin for the treatment of glaucomatous disease. Several firms announced results of Phase 2 stage trials for investigational ophthalmical products. Gene and cell therapy studies in ophthalmology continue, although a large pharmaceutical firm announced stopping its (systemic) cell therapy programs. The U.S. FDA announced a number of new programs to support U.S. drug development, as well as increasing enforcement of current regulations on direct-to-consume advertising.

Ophthalmical Pharmaceuticals and Biologics

- Chiesi submitted a New Drug Application to the U.S. FDA for idebenone (Raxone™) as a treatment for LHON. The firm also received approval from the UK-based National Institute for Health and Care Excellence (NICE) for this drug and indication (September 2025).
- iVeena Delivery Systems is developing topical eye drops to control pediatric myopia and other refractive disorders (September 2025).
- Kala Bio announced results of its CHASE (Corneal Healing After SEcretome therapy) trial of a phase 2b trial of its KPI-012 (mesenchymal stem cell secretome) in persistent corneal epithelial defects (PCED, September 2025).
- Nacuity Pharmaceuticals announced results from the SLO-RP Phase 1/2 clinical trial evaluating NPI-001 tablets to treat patients with retinitis pigmentosa (RP) associated with Usher syndrome (USH, September 2025).
- Ollin Biosciences completed enrollment in a clinical trial of its OLN324, a high-potency VEGF/Ang2 bispecific antibody, in the treatment of wet age-related macular degeneration and is also developing OLN102, a TSHR/IGF-1R bispecific antibody for the treatment of thyroid eye disease and Graves' disease (September 2025).
- Opus Genetics commenced dosing in its LYNX-3 study of phentolamine ophthalmical solution 0.75% for the treatment of chronic night driving impairment in keratorefractive patients with reduced mesopic vision (September 2025).
- Outlook Therapeutics received a complete response letter from the U.S. FDA for its ONS-5010, bevacizumab, for treating patients with wet AMD (September 2025).
- Roche received the European Union (EU) CE mark for its Port Delivery Platform of ranibizumab (Contivue™ in the EU, Susvimo® in the US) for the treatment of wet AMD (September 2025).
- Santen obtained marketing approval in Japan for SETANEO™ ophthalmical solution 0.002% (sepetaprost, STN1012600) for the treatment of glaucoma and ocular hypertension (August 2025).
- VivaVision Biotech reported communication with the US FDA regarding the development of its VVN461 ophthalmical solution, a dual JAK1/TYK2 inhibitor for the treatment of postoperative inflammation following cataract surgery (June 2025).

Gene Therapy

- REGENXBIO completed enrollment in its ATMOSPHERE and ASCENT pivotal studies evaluating its surabgene lomparvovec (sura-vec, ABBV-RGX-314) in wet AMD. This is a gene therapy delivered using subretinal delivery (October 2025).
- Sepul Bio, a unit of Thea, commenced dosing in its Phase 3 HYPERION clinical trial for sepofarsen, an RNA therapy, in individuals with CEP290-associated Leber Congenital Amaurosis Type 10 (LCA10, October 2025).
- Takeda announced discontinuation of its cell therapy efforts (October 2025).

Regulatory, Government, and Pharmaceutical Industry

- Harrow is acquiring Melt Pharmaceuticals, which has an investigational systemic sedation product for use in medical procedures, including cataract surgery (September 2025).
- There are a number of publications on the lack of compliance with timely reporting of results from clinical trials.[1–3]
- The U.S. FDA (September/October 2025):
  - Announced changes to the Centers of Excellence in Regulatory Science and Innovation academic program.

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- Is increasing enforcement on compliance of direct-to-consumer advertising by pharmaceutical firms with current regulations.
- Created a new program to expedite reviews for domestically manufactured generic drugs.
- Announced FDA PreCheck, a new program to strengthen the domestic pharmaceutical supply chain by increasing regulatory predictability and facilitating the construction of manufacturing sites in the United States.
- Announced the impact of the closure of the federal government on 1 October. This includes the inability for sponsors to submit New Drug Applications due to the inability to accept user fees.
- Published a large number of "complete response letters" (non-approvals). It is unclear if these were previously available.
- Published Guidances on: Post approval methods to capture safety and efficacy data for cell and gene therapy products, innovative designs for clinical trials of cellular and gene therapy products in small populations, Biosimilars (Analytical Assessment and Other Quality-Related Considerations), and Expedited Programs for Regenerative Medicine Therapies for Serious Conditions.

- U.S. National Institutes of Health:
  - Established an organoid development center to reduce reliance on animal models (September 2025).
  - There is discussion to close the National Eye Institute of the U.S. National Institutes of Health (September 2025).

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