Eyes on New Product Development

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IN THIS REPORTING period, notable are the number of firms involved in developing treatments for retinal degenerative diseases such as retinitis pigmentosa. The approaches cover a wide range from gene therapy to cell therapy to growth factors, “repurposing” of legacy drugs, and drug delivery systems. Also of note are regulatory and legal efforts surrounding the challenging area of off-label promotion of pharmaceuticals. Several companies have announced results of key Phase 2 and Phase 3 trials in indications from anterior to posterior. In the medical device arena, companies continue to develop micro-invasive glaucoma surgical (MIGS) devices, as well as artificial retinas.

Ophthalmic Pharmaceuticals and Biologics

- Actavis plans to start phase III development of abicipar, a long-acting mono-DARPin that inhibits vascular endothelial growth factor A (VEGF-A), for the treatment of neovascular age-related macular degeneration (wet AMD, May 2015). <http://www.molecularpartners.com/2015-mp-ad-hoc-release/>
- Aerie Pharmaceuticals announced results of the first of its Phase 3 trials of AR-13324 in the treatment of glaucoma (April 2015). <http://investors.aeriepharma.com/releasedetail.cfm?releaseid=908343>
- AGTC is in preclinical research for the treatment of achromatopsia (ACHM) and X-linked retinoschisis (May 2015). <http://globenewswire.com/news-release/2015/03/10/713837/10123971/en/AGTC-Files-Investigational-New-Drug-Application-for-the-Treatment-of-X-Linked-Retinoschisis.html>
- Allegro Ophthalmics has started a Phase 2 clinical study of ALG-1001, an integrin peptide, for the treatment of non-proliferative diabetic retinopathy (May 2015). <http://www.allegroeye.com/press-release/allegro-ophthalmics-begins-phase-2-clinical-trial-of-luminate-alg-1001-for-the-treatment-of-diabetic-macular-edema/>
- Amarantus was granted orphan drug designation from the European Medicine Agency (EMA) for mesencephalic-astrocyte-derived neurotrophic factor for the treatment of retinitis pigmentosa (April 2015). <http://globenewswire.com/news-release/2015/04/29/729850/10131354/en/Amarantus-Granted-European-Union-Orphan-Drug-Designation-for-MANF-for-the-Treatment-of-Retinitis-Pigmentosa.html>
- Aura Biosciences, was granted orphan disease designation by the FDA for its drug AU011 (viral-like nanoparticles) for the treatment of uveal melanoma (May 2015). <http://www.businesswire.com/news/home/20150521005822/en/FDA-Grants-Orphan-Drug-Designation-Aura-Biosciences%E2%80%99VXY3Cc9VhBc>
- BioTime is evaluating OpRegen, a cell-based product composed of retinal pigment epithelial (RPE) cells, derived from human embryonic stem cells (hESC), in a Phase 1/2 study for the treatment of dry macular degeneration (May 2015). <http://finance.yahoo.com/news/biotimeincreportsfirstquarter120000769>
- Eleven Biotherapeutics announced top-line results from pivotal phase 3 study of EBI-005, an IL-1 receptor blocker, in patients with moderate to severe dry eye disease (May 2015). <http://ir.elevenbio.com/releasedetail.cfm?releaseid=913561>
- EyeGate is planning a second Phase 3 trial of its EGP-437 (dexamethasone administered by iontophoresis) for the treatment of anterior uveitis (May 2015). http://www.eyegatepharma.com/pdf/news2012/EyeGate_Uveitis_Release_FINAL_2.pdf
- Icon Bioscience announced results from a pivotal phase 3 study of its IB10090 (intracameral dexamethasone) in inflammation following cataract surgery (April 2014). <http://www.businesswire.com/news/home/20150406005151/en/Icon-Bioscience-Reports-Successful-Outcomes-Phase-3#.VXY3P89VhBc>
- InMed Pharmaceuticals Inc. has filed a patent relating to the treatment of eye diseases. The company “uses bioinformatics tools to identify individual chemical compounds from cannabis and non-cannabis plants which can be targeted to develop therapies for specific diseases and conditions (May 2015). <http://www.prnewswire.com/news-releases/inmed-pharmaceuticals-files-provisional-patent-covering-novel-compounds-for-treating-epidermolysis-bullosa-simplex-ebs-300088450.html>
- Isarna is evaluating ISTH0036, a locked nucleic acid-modified antisense oligonucleotide, in a Phase 1 study for use during glaucoma filtration surgery. The firm has also received orphan designation in Europe for this agent for this indication (May 2015). http://www.isarna-therapeutics.com/fileadmin/user_upload/documents/press-releases/2015/20150416_EN_Isarna_Glaucoma_Phase_1.pdf?

- Lpath announced results for its multicenter, Phase 2 “Nexus clinical trial evaluating iSONEP™ in patients with wet AMD (May 2015). <http://phx.corporate-ir.net/phoenix.zhtml?c=197881&p=irol-newsArticle&ID=2051634>
- Makindus’ MI100, a novel formulation of a legacy compound, received orphan designation for the treatment of Stargardt’s disease from the EMA (May 2015). <http://www.prnewswire.com/news-releases/european-medicines-agency-ema-grants-orphan-designation-for-makindus-mi-100-for-stargardts-disease-300076498.html>
- Mitotech announced results in its U.S. Phase II clinical trial of SkQ1 compound in patients with moderate to severe dry eye syndrome (April 2015). <http://www.mitotechpharma.com/news/Mitotech-successfully-completes-dry-eye-study>
- NightstarX received orphan drug designation from the FDA and EMA for a gene therapy to treat choroideremia (March 2015). <http://www.nightstarx.com/news-events/press-releases/nightstar-receives-us-and-european-orphan-drug-designation-gene-therapy-treat-choroideremia/>
- Ocata Therapeutics announced results from a study of its hESC-derived cells in patients with dry AMD and Stargardt’s disease (April 2015). <http://www.businesswire.com/news/home/20150430005256/en/Ocata-Therapeutics-Announces-Positive-Results-Treating-Macular#.VXY4S89VhBc>
- Ocular Therapeutix plans to submit a new drug application (NDA) for its dexamethasone punctal plug (OTXDP, Dextenza™ for post-operative inflammation (May 2015). <http://www.biospace.com/news/ocular-therapeutix-announces-plans-to-proceed-with/377392>
- Ohr Pharmaceutical announced results from its Phase 2 IMPACT study evaluating OHR-102 (0.2% squalamine lactate ophthalmic solution) combination therapy for the treatment of the wet form of AMD (March 2015). <http://www.biospace.com/News/ohr-pharmas-ohr-102-fails-in-phase-ii-shares/370377>
- Omidria® received a positive opinion from EMA for Omidria®, its fixed dose combination of ketorolac and phenylephrine for use during cataract surgery (May 2015). http://www.morningstar.com/news/Benzinga/BenzBZW_5534392/Omidria-Receives-Positive-Opinion-from-European-Medicines-Agency-Committee-for-Medicinal-Products-for-Human-Use.html
- Portola Pharmaceuticals is developing its Syk inhibitor, PRT2761, in ophthalmic diseases such as allergy (May 2015). <http://globenewswire.com/news-release/2015/05/05/732123/10132590/en/Portola-Pharmaceuticals-Announces-Collaboration-With-Ora-Inc-for-Syk-selective-Inhibitor-PRT2761-in-Ophthalmic-Diseases.html>
- RTech Ueno acquired rights to DE105, an ophthalmic solution of neurotrophic factor peptide, from Santen Pharmaceutical. DE105 is a treatment targeting at persistent corneal epithelial defects (April 2015). <http://www.businesswire.com/news/home/20150427005273/en/R-Tech-Ueno-Announcement-Acquisition-Develop-Commercialize-DE-105#.VXY5F89VhBc> RTech Ueno is also conducting collaborative research with Hokkaido University on a VAP1 inhibitor (RTU1096) for treatment of diabetic retinopathy (DR) and diabetic macular edema (May 2015). <http://www.businesswire.com/news/home/20150518005573/en/R-Tech-Ueno-Announcement-Collaborative-Development-Therapeutic-Agent#.VXY5Ps9VhBc>
- Santen Pharmaceutical is assigning its antirheumatic pharmaceuticals business to Hyperion (May 2015). http://www.santen.com/en/news/20150512_4.pdf
- Shire and the Foundation Fighting Blindness are collaborating on research for a novel treatment for autosomal dominant retinitis pigmentosa (May 2015). <http://www.blindness.org/foundation-news/shire-and-foundation-fighting-blindness-announce-new-research-agreement>
- Spark Therapeutics and Clearside Biomedical entered into an option agreement under which Spark acquired exclusive rights to license Clearside’s microinjector technology to deliver gene therapies to the back of the eye (April 2015). <http://www.bizjournals.com/philadelphia/blog/health-care/2015/04/gene-therapy-company-acquires-rights-to-eye.html>
- Stealth BioTherapeutics, currently evaluating Ocuvia for diabetic macular edema and dry AMD, is expanding to now evaluate it for inherited optic mitochondrial neuropathies (April 2015). <http://www.businesswire.com/news/home/20150427005072/en/Stealth-BioTherapeutics-Expands-Ocuvia%E2%80%99s-Clinical-Development-Rare#.VXY5uc9VhBc>
- ThromboGenics is evaluating Jetrea® (ocriplasmin) as a potential treatment for retinal vein occlusion (April 2015).
- Trefoil Therapeutics signed an agreement with Florida State University for the development of engineered FGF-1 derivatives (eFGF-1s) for therapeutic and cosmetic uses (April 2015). <http://eyewiretoday.com/2015/03/24/trefoil-therapeutics-development-program-to-focus-on-the-treatment-of-corneal-dystrophies>
- Tyrogenex initiated a phase 2 study (APEX) for evaluation of its X82, an orally administered, dual inhibitor of vascular endothelial growth factor (VEGF) and platelet-derived growth factor (PDGF) for wet AMD in previously treated Eylea® (aflibercept, April 2015). <http://www.businesswire.com/news/home/20150429005170/en/Tyrogenex-Initiates-APEX-study-Phase-2-Trial#.VXY55s9VhBc>
- XOMA Corporation announced that the gevokizumab Phase 3 EYEGUARD-B study in uveitic patients with Behcet’s disease reached its target exacerbation event as specified in the study design (May 2015). <http://investors.xoma.com/releasedetail.cfm?ReleaseID=915264>

Medical Devices

- InnFocus completed enrollment in a Phase 1 study of its MicroShunt glaucoma drainage implant (April 2015). <http://innfocusinc.com/index.php/news/innfocus-expands-board-of-directors-and-responsibilities/>
- Pixium Vision is conducting preclinical studies on PRIMA, its photovoltaic wireless subretinal implant (April 2015). http://www.pixium-vision.com/file_bdd/dynamic_content/file_pdf_pdf_en/1430755709_Pixium_Vision-PRIMA_ARVO_POSTERS-ENG_FINAL.pdf

Regulatory, Government, and Pharmaceutical Industry

- Alexion is buying Synavega BioPharma. Both firms specialize in treatments for rare diseases (May 2015).

<http://www.bloomberg.com/news/articles/2015-05-06/alexion-to-pay-8-4-billion-to-buy-synageva-in-rare-disease-push>

- Express Scripts is seeking an arrangement with pharmaceutical firms selling oncology treatments that would set prices relative to efficacy (May 2015). <http://www.bloomberg.com/news/articles/2015-01-22/express-scripts-targets-150-000-cancer-drug-prices-in-2016-plan>
- Indiana passed a “right to try” law (March 2015). <http://chicago.cbslocal.com/2015/03/24/indiana-right-to-try-law-gives-terminally-ill-access-to-experimental-drugs/>
- Johnson & Johnson appointed a bioethicist to create a panel that will make decisions about patients’ requests for potentially lifesaving medicine. It comes as a small but growing number of patients with terminal illnesses have sought the right to obtain drugs still in the testing phase that show promise for treating their diseases (May 2015). <http://www.cnbc.com/id/102657370>
- Mallinckrodt is suing the FDA over its decision to downgrade from “AB” to “BX” the Therapeutic Equivalence (“TE”) rating its generic version of Concerta® (methylphenidate HCl). FDA also downgraded the TE rating for another generic version of Concerta® marketed by Kudco Ireland Ltd. FDA has asked both ANDA sponsors to either confirm, within 6 months, the bioequivalence of their products using the Agency’s revised bioequivalence standards, or voluntarily withdraw their products from the market. (May 2015). http://www.fdalawblog.net/fda_law_blog_hyman_phelps/2014/11/mallinckrodt-sues-fda-after-agency-downgrades-therapeutic-equivalence-rating-for-generic-concerta.html
- Novartis’ biosimilar drug for Amgen’s Neupogen® was blocked by a U.S. appeals court (May 2015). <http://www.pharmalive.com/novartis-ags-biosimilar-drug-sale-blocked-by-u-s-appeals-court/>
- San Mateo county (Northern California) adopted an ordinance that mandates the pharmaceutical industry underwrite the costs of a takeback program (May 2015). <http://www.sfexaminer.com/sanfrancisco/proposed-san-mateo-county-ordinance-would-require-drug-companies-to-pay-for-disposal-program/Content?oid=2916717>
- The Netherlands Medicines Evaluation Board stated that there are “... no relevant differences between biosimilars and reference products” (April 2015). <http://www.biopharma-reporter.com/Markets-Regulations/Netherlands-Medicines-Evaluation-Board-says-no-relevant-differences-between-biosimilars-reference-products>
- The U.S. FDA further defined draft guidance outlining how the agency intends to regulate compounding and repackaging of biological products, including bevacizumab

(Avastin®), at compounding facilities. This guidance suggests that a “beyond use data” of 4 hours (February 2015). <http://www.fda.gov/Drugs/GuidanceComplianceRegulatoryInformation/PharmacyCompounding/ucm375804.htm>

- The U.S. FDA and Congress are developing the 21st Century Cures law, supporting medical product development, the development of biomarkers, and strengthening FDA’s human resources. As part of this law, there is a public meeting this summer to address drug company concern that restrictions on what they can say about off-label use of drugs violate their First Amendment right to free speech. This is part of 21st Century Cures (May 2015). <http://www.fda.gov/NewsEvents/Testimony/ucm404647.htm> In a related story, Amarin has filed suit against the U.S. FDA, claiming that the company’s First Amendment right of free speech has been squashed by the government in its promotion of it drug, icosapent ethyl (May 2015). http://www.nytimes.com/2015/05/08/business/drugmaker-sues-fda-over-right-to-discuss-off-label-uses.html?_r=0 (Subscription required to access Web site.)
- The U.S. NIH has warned over a global preponderance of fake medicines to treat a wide range of diseases including malaria, AIDS and tuberculosis (April 2015). <http://www.nih.gov/news/health/apr2015/fic-20.htm>
- U.S. Senators Dianne Feinstein (D-California) and Susan Collins (R-Maine) have introduced an amendment to the federal Food, Drug and Cosmetic Act that would give the Food and Drug Administration more power and oversight to regulate personal care products in a bill called the Personal Care Products Safety Act (April 2015). <http://www.feinstein.senate.gov/public/index.cfm/2015/4/feinstein-collins-introduce-bill-to-strengthen-oversight-of-personal-care-products>

Author Disclosure Statement

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