Targeting Ocular Disorders

October 6–7, 2014
Westin Boston Waterfront Hotel • Boston, MA

Topics Include:
• New disease pathways
• Alternative drugs
• Anti-inflammatories
• Biologics
• Bi-specific therapies
• Novel therapeutics
• Anti-angiogenics
• Drugs in clinical trials

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humanized form of this antibody may be a novel therapy for diabetic retinopathy. A novel murine monoclonal antibody that antagonizes LRP6 activity which is also observed in human retinas of patients with diabetic retinopathy. We have identified a novel murine monoclonal antibody (mAb), 6F6, to demonstrate significant activity in numerous animal models of retinal disease. SerumAb levels after systemic administration of 6F6 show accumulation of Ab in the periphery suggestive of a peripheral sink mechanism. In summary, anti-Ab mAb treatment can partially prevent or reverse ocular pathologies of the cflr-/- mouse. The data support this therapeutic approach in humans potentially modulating two key elements in the pathogenesis of AMD – Ab and activated, complement C3.

EMERGING THERAPIES FOR RETINAL AND MACULAR DISORDERS

2:40 Oral Therapies for Retinal Degeneration: Case Study of ALK-001
Leonide Saad, Ph.D., CEO, Alkeyus Pharmaceuticals, Inc.
Safely attaining biologically active sustained amounts of a compound in the retina is notoriously difficult. Here we present how compounds delivered orally can find their way to the retina. We further discuss the science behind ALK-001, an oral investigational new drug designed to inhibit the formation of toxic by-products thought to be responsible for major retinal degenerations such as AMD, Stargardt, Best disease or autosomal recessive Retinitis Pigmentosa.

3:10 Wnt Pathway Inhibition for Diabetic Retinopathy
James Larrick, Ph.D., Founder, Managing Scientific Director, CMO, Panorama Research Institute, Wntgen LLC & Velocity Pharmaceutical Development
Activation of the Wnt pathway via LRPS cell surface signaling contributes to the severe retinal/choroidal neovascularization observed in Vldlr-/- mice. Elevated Wnt signaling is also observed in human retinas of patients with diabetic retinopathy. We identified a novel murine monoclonal antibody that antagonizes LRPS activity which demonstrates significant activity in numerous animal models of retinal disease. A humanized form of this antibody may be a novel therapy for diabetic retinopathy.

3:40 MANF - A Novel Neurotrophic Factor for the Treatment of Retinal Disorders
Roman Urfer, Ph.D., Chief Development Officer, NeuroAssets Sarl
Mesencephalic astrocyte-derived neurotrophic factor is the prototype of an emerging family of neurotrophic factors with evolutionary highly conserved structure and function. MANF is expressed in response to cellular stress and during retinal development. MANF’s activity in genetic and pharmacological models of retinal disorders will be presented.

4:55 Combination Therapy in Diseases of Retinal Origin: A New Paradigm
David Sherris, Ph.D., CSO, RestorGenex, Corp.
Neovascular retinal and subretinal diseases as in age-related macular degeneration and proliferative diabetic retinopathy occur through a cascade of events over time. Numerous cytokines have shown involvement throughout the disease process. Anti-VEGF technologies inhibit edema and hemorrhage due to their ability to reduce vascular permeability and to some extent inhibit neovascularization. However, VEGF is not the only cytokine in these pathologies. Here we will discuss the influence of a cytokine storm in back-of-the-eye diseases with the example of PS29, a first-in-class, allosteric, dual dissociative inhibitor of the TORC1 and TORC2 complexes within the PI3K/Akt/mTOR pathway in combination with anti-VEGF.
9:20 Nanobodies® as Next-Generation Therapeutics for Ocular Applications
Tony De Fougerolles, Ph.D., CSO, Ablynx NV

Next-generation ophthalmic therapeutics must aim at being superior to the currently available agents with regards to efficacy and improved drug delivery. We show here that Nanobodies deliver these properties and present a novel class of highly potent and specific drug candidates for the treatment of eye disorders.

9:50 Local Inhibition of Cytokine Signaling to Treat Anterior and Posterior Ocular Disorders
Eric Furline, Ph.D., CSO, Eleven Therapeutics

Cytokines, chemokines, and growth factors mediate anterior and posterior eye diseases. Our lead product, the IL-1 receptor inhibitor EBI-005, was designed and engineered for the topical treatment of dry eye disease and was biologically active in subjects with dry eye disease. In addition, we engineered an IL6 inhibitor with potential for local treatment diabetic macular edema. Finally, a novel soluble receptor inhibitor of cytokines IL-17A and IL-17F was engineered for the local treatment of uveitis. Both IL-6- and IL-17-targeted drugs were designed and engineered for intravitreal administration.

10:20 Coffee Break in the Exhibit Hall with Poster Viewing

10:50 Topical Aganirsen in Eye Diseases: From Phase III Results in the Cornea to Retinopathies - A New Revolution
Eric Thorin, Chief Development Officer, Gene Signal International SA

The antisense aganirsen inhibits the expression of Insulin Receptor Substrate-1 (IRS-1). The phase III showed that its topical application induces regression of pathological corneal neovascularization and reduces the need for corneal transplantation. Topical aganirsen reaches the retina, which is at the basis of its clinical development for the treatment of AMD and DME. Aganirsen should become the first therapy active by topical application for the treatment of angiogenesis-based retinopathies.

11:20 NLRP3 Inflammasome Activation in Retinal Pigment Epithelial Cells by Lysosomal Destabilization: Implications for Age-Related Macular Degeneration
Bruce R. Ksander, Ph.D., Associate Professor, Ophthalmology, Scheepens Eye Research Institute/Massachusetts Eye and Ear

NLRP3 upregulation occurs in the RPE during the pathogenesis of advanced AMD, in both geographic atrophy and neovascular AMD. Destabilization of RPE lysosomes induces NLRP3 inflammasome activation, which may contribute to AMD pathology through the release of the proinflammatory cytokine IL-1β and through caspase-1-mediated cell death, known as “pyroptosis.”

11:50 Maximizing Chances of Success in the Clinic: The Role of Animal Models in the Development of Ocular Therapeutics
Claire M. Geffman, Ph.D., Director, Pre-Clinical Services, Ora

Achieving proof-of-concept in animal models of ophthalmic disease is a commonly desired prerequisite for advancing drugs into the clinic. The choice of endpoints for evaluation should mirror, when possible, those that will be evaluated in the clinic. In this presentation, we will review several models that recapitulate specific features of human ophthalmic disease, and discuss the integration of that information into the larger clinical development plan in order to maximize chances of success.
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For sponsorship and exhibit information, please contact:
Carolyn Benton – Business Development Manager
781-972-5412 | cbenton@healthtech.com

*Inquire about exhibit space, branding and more!*

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# Pricing and Registration Information

## CONFERENCE PRICING

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<tr>
<td>PREMIUM PACKAGE</td>
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<td>(Includes access to Targeting Ocular Disorders, and a copy of Insight Pharma Reports Ocular Disorders: Rising Therapeutics, Technologies, and Devices)</td>
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<td>Registrations after August 22 and on-site</td>
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<td>STANDARD PACKAGE</td>
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<td>(Includes access to Targeting Ocular Disorders, excludes report)</td>
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<tr>
<td>Registrations after August 22 and on-site</td>
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- **Poster Submission - Discount ($50 Off):** Poster abstracts are due by September 5, 2014. Once your registration has been fully processed, we will send an email containing a unique link allowing you to submit your poster abstract. If you do not receive your link within 5 business days, please contact jring@healthtech.com. *CHI reserves the right to publish your poster title and abstract in various marketing materials and products.*

- **REGISTER 3 - 4th IS FREE:** Individuals must register for the same conference or conference combination and submit completed registration form together for discount to apply.

- **Alumni Discount:** Cambridge Healthtech Institute (CHI) appreciates your past participation in Targeting Ocular Disorders. As a result of the great loyalty you have shown us, we are pleased to extend to you the exclusive opportunity to save an additional 20% off the registration rate. Alumni and Register 3 - 4th is free discounts cannot be combined

- **Group Discounts:** Discounts are available for multiple attendees from the same organization. For more information on group rates contact David Cunningham at +1-781-972-5472.

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How to Register: **Healthtech.com/Targeting-Ocular-Disorders**

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