Topics Include:

- New disease pathways
- Alternative drugs
- Anti-inflammatory
- Biologics
- Bi-specific therapies
- Novel therapeutics
- Anti-angiogenics
- Drugs in clinical trials

Preserving Vision through Innovative Drug Discovery and Development

Healthtech.com/Targeting-Ocular-Disorders
2:00 A Special Intraocular Contemplation for New Format Antibody Formulations

Dhananjay Jere, Ph.D., Group Leader, PTDE, Hoffmann-La Roche

Development of protein products to target ocular diseases requires special considerations from the molecular designing aspect and also from the formulation, stability, and regulatory aspect. The protein intended to stay in eye has to be compatible and stable in the intraocular environment. This presentation describes key development aspects of new format antibody formulation to make it apt for the intraocular application.

2:30 Intrinsic Toxicity Pathways Affecting Survival of Ganglion Cells in Injured Retina

Val Shestopalov, Ph.D., Associate Professor, Ophthalmology, Bascom Palmer Eye Institute, University of Miami Ophthalmology

Aberrant activation of pannexin-1 membrane channel disrupts resilience of these neurons to ischemic, mechanical and other injuries. Mechanistic insights into pathophysiology of pannexin-1 activation suggest that neuroprotective effect strongly correlates with blocking ionic dysbalance and cytokine production. Our results indicate that pharmacological control of pannexin-1 activation can protect against various types of retinal pathologies triggered by ischemic injury, optic nerve stroke and glaucoma.

3:00 Tear Fluid Analysis as a Window into Ocular Health & Disease

Mark W. Duncan, Ph.D., Professor of Medicine, Anschutz Medical Campus, University of Colorado Denver

We are exploring the potential of tear fluid analysis, especially of peptides and proteins, in defining ocular health and disease by using both targeted and non-targeted analysis strategies. Tear samples (n=10) were collected from subjects (n=40) directly onto a polymeric support following photorefractive keratectomy (PRK). Target proteins were assayed by ELISA (both multiplexed and individual assays) and the samples were also examined by matrix-assisted laser desorption/ionization mass spectrometry (MALDI-MS). The relationships between the tear constituents and their quantitative changes following surgery will be discussed.

3:30 Breakthrough Solutions for Ocular Diseases

Guillaume Pfefer, Ph.D., President & CEO, Kala Pharmaceuticals

Kala is building a diversified pipeline of ophthalmic product candidates: a best-in-class treatment of ocular inflammation and a first-of-a-kind, completely non-invasive wet age-related macular degeneration (AMD) treatment that will transform the industry. We’ll look at the role of technology to create powerful therapies with significantly less cost and risk.

3:45 Refreshment Break with Exhibit & Poster Viewing

4:30 Treating Ocular Disease with Therapeutic Mirror Proteins

Dana Ault-Richie, Ph.D., CEO, Reflexion Pharma

Vascular endothelial growth factor (VEGF) is a protein that when aberrantly expressed stimulates the formation of new blood vessels that contribute to tumor growth and macular degeneration. Reflexion’s novel approach uses mirror-image protein technology. Antibodies, like all natural proteins, are “left-handed,” meaning that they have a particular orientation in their structure. We have created an unnatural “right-handed” VEGF protein. The beauty of this process is that the “right-handed” end product protein can’t be so readily metabolized by the body. Thus lower and less frequent doses are possible, and a reduced side-effect profile (such as immune reactions) is likely.

5:00 Cell-Based Therapies for Ocular Disease

Mohammed El-Kalay, Ph.D., Research and Development, EyeCyte, Inc.

5:30-6:30 Welcome Reception with Exhibit & Poster Viewing

Present a Poster

CHI encourages attendees to gain further exposure by presenting their work in the poster sessions.

- Your poster will be exposed to our international delegation
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To secure a poster board and inclusion in the conference materials, your abstract must be submitted, approved and your registration paid in full by August 16, 2013.
retinal diseases. Research has demonstrated atypical PKC (aPKC) acts as a common signaling molecule for vascular permeability induced by VEGF, as well as TNF, CCL2 and thrombin. Novel, specific aPKC inhibitors effectively prevent combined VEGF and TNF induced permeability in vivo and may provide an effective means to treat a wide range of retinal diseases.

11:30 Targeted Nanoparticle Therapy using Intraceptor Inhibition of Choroidal Neovascularization
Michael Burr, Ph.D., Research Scientist, Moran Eye Center, University of Utah
Here, we show that a single intravenous injection of targeted, biodegradable nanoparticles delivering a recombinant Fit23k intraperitoneal plasmid homes to neovascular lesions in the retina and represses CNV in murine models. Moreover, this treatment suppressed subretinal fibrosis, which is currently not addressed by clinical therapies. We found no evidence of ocular or systemic toxicity from nanoparticle treatment. These findings offer a nanoparticle-based platform for targeted, vitreous-sparing, extended-release, nonviral gene therapy.

12:00 pm Luncheon Presentation (Sponsorship Opportunity Available) or Lunch on Your Own

LOOKING TO THE FUTURE

1:45 Chairperson’s Remarks

1:50 Lens Epithelium-Derived Growth Factor Fragment (LEDGF1-326), a Novel Therapeutic Protein: Biosynthesis, Characterization, and Efficacy in Retinal Degenerative Diseases
Uday B. Kompella, Ph.D., Director, Kompella Lab, University of Colorado Anschutz Medical Campus
We identified lens epithelium derived growth factor fragment (LEDGF1-326) as a novel protein therapeutic. We biosynthesized, purified, and characterized LEDGF1-326. Eight weeks after single intravitreal injection in Royal College of Surgeon (RCS) rats, LEDGF1-326 increased the b-wave amplitude significantly from 9.4 ± 4.6 to 57.6 ± 8.8 μV for scotopic electroretinogram (ERG) and from 10.9 ± 5.6 to 45.8 ± 15.2 μV for photopic ERG. LEDGF1-326 significantly increased the retinal outer nuclear layer thickness from 6.34 ± 1.6 to 11.7 ± 0.7 μm. LEDGF1-326 is a potential new therapeutic agent for treating retinal degenerative diseases.

2:00 Inhibition of Cytokine Signaling to Treat Ocular Diseases
Eric Furline, Ph.D., President of R&D, Eleven Biotherapeutics

2:50 Treatment for Dry Form AMD in a New Mouse Model
Haoyu Mao, Ph.D., Department of Molecular Genetics and Microbiology, University of Florida College of Medicine
By developing a new dry form AMD mouse model, a new treatment with a potential therapeutic reagent is investigated by up to 4 months daily treatment. Monthly monitor of ERG, OCT and fundus showed improved retinal function with treatment comparing with that of control saline group.

3:10 Concluding Remarks

3:30 Close of Conference
CHI offers comprehensive sponsorship packages which include presentation opportunities, exhibit space and branding, as well as the use of the pre and post-show delegate lists. Customizable sponsorship packages allow you to achieve your objectives before, during, and long after the event. Signing up early will allow you to maximize exposure to hard-to-reach decision makers!

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4 | Targeting Ocular Disorders Healthtech.com/Targeting-Ocular-Disorders
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Discounted Room Rate: $289 s/d
Discounted Cut-off Date: August 26, 2013

Please call the hotel directly to reserve your sleeping accommodations. You will need to identify yourself as a Cambridge Healthtech Institute conference attendee to receive the discounted room rate with the host hotels. Reservations made after the cut-off date or after the group room block has been filled (whichever comes first) will be accepted on a space-and-rate-availability basis. Rooms are limited, so please book early.

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Special discounts have been established with American Airlines for this conference.
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- Call Hertz 1-800-654-3131 and use our Hertz Convention Number (CV): 04KL0004
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- Complimentary wireless internet access
- No commute since meeting takes place across the street from host hotel
- Lots of new restaurants within walking distances.
- Seaport is the up-and-coming area of Boston!
- “Pure” rooms for those with allergies
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**Pricing and Registration Information**

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<td>Registrations after August 16, 2013, and on-site</td>
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### CONFERENCE DISCOUNTS

**Poster Submission - Discount ($50 Off)**: Poster abstracts are due by August 16, 2013. 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.*

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If you are unable to attend but would like to purchase the **Targeting Ocular Disorders CD** for $350 (plus shipping), please visit healthtech.com/Targeting-Ocular-Disorders. Massachusetts delivery will include sales tax.

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

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