Pre-conference Workshop of the CC-CRS

Date: May 22, 2018 8:00 am – 12:30 pm

Title: Formulation and Delivery of Biologics: Vaccines, Peptide/Protein, and Gene Therapeutics
Location: Leslie Dan Faculty of Pharmacy, University of Toronto, 144 College Street, Toronto, Ontario

Organizing committee: Afsaneh Lavasanifar, Marc A Gauthier, Emmanuel Ho, Shirley Wu, Todd Hoare, Larry Unsworth

Co-Chairs: Afsaneh Lavasanifar, Faculty of Pharmacy and Pharmaceutical Sciences, University of Alberta; Marc A Gauthier, EMT Research Centre, Institut National de la Recherche Scientifique

Sponsors: NSERC, Quest Pharmatech, Feldan Therapeutics, Alberta Research Chemicals, Janssen, GSK Vaccines, Leslie Dan Faculty of Pharmacy

Sponsorship opportunities are still available!

Description:
The use of biologics as therapeutics for different diseases and/or disorders has been on an exponential rise in recent years. Production of effective formulations that can ensure enhanced delivery of these compounds to their biological target is still a significant challenge. This workshop will provide an introduction on different aspects of the formulation and delivery of biologics, with focus on therapeutics and preventive vaccines, protein/peptide drugs, as well as gene therapeutics. Experts from academia and industry will provide an overview of the scientific basis of the formulation of biologics, and then present related examples of applications and pharmaceutical cases. This will be followed by a panel discussion.

Time	Topic	Speaker
8:00 – 8:20	Registration, continental breakfast and networking	
8:25 – 8:30	Introductions and welcome address (co-chairs)	
8:30 – 9:00	Fundamentals of Vaccine Formulation and Delivery	Dr. Afsaneh Lavasanifar Dr. Emmanuel Ho
9:00 – 9:30	Formulating potent and Safe Delivery System for a Broad Range of Molecules from Small Molecule Adjuvants, to Proteins and RNA Vaccines	Dr. Derek O'Hagan Global Head of Discovery Support and New Technologies, GSK Vaccines
9:30 – 9:50	Coffee break – Networking	
9:50 – 10:20	Gene delivery technologies	Dr. M. Foldvari Professor, University of Waterloo
10:20 – 10:50	The Feldan Shuttle Technology: a peptide-based method to deliver proteins in living cells	Dr. Thomas Del'Guidice Research Specialist Feldan Therapeutics Inc.
10:50 – 11:20	Large Molecule Drug Product Development & Manufacturing – Janssen Practice	Dr. Kai Zhang Associate Director of Drug Product Development, Large Molecule, Janssen Inc.
11:20 – 11:40		Andy Sinclair Manager, NSERC Ontario Regional Office
11:40 – 12:30	Lunch and Panel Discussion	

Registration (free): https://www.ShirleyWuLab.com/Pre-Conference; for further information, please contact gauthier@emt.inrs.ca

WORKSHOP ORGANIZING COMMITTEE

Prof. Afsaneh Lavasanifar (Co-chair)

University of Alberta

Prof. Marc Gauthier (Co-chair)

INRS

Prof. Shirley X.Y. Wu

University of Toronto

Prof. Emmanuel Ho

University of Waterloo

Prof. Larry Unsworth

University of Alberta

Prof. Todd Hoare

McMaster University

ACKNOWLEDGEMENTS

Director Board of the Canadian Chapter of controlled release society

(CC-CRS)

Workshop Sponsors

Student volunteers

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Ricky Chang (University of Toronto)

Tian Zhang (University of Toronto)

HoYin Lip (University of Toronto)

PRESENTATION ABSTRACTS AND SPEAKERS' BIOGRAPHY

Fundamentals of Vaccine Formulation and Delivery

Prof. Afsaneh Lavasanifar
Faculty of Pharmacy and Pharmaceutical Sciences, University of Alberta, Edmonton, Canada

Prof. Emmanuel Ho
School of Pharmacy, University of Waterloo, Waterloo, Canada

Abstract

Vaccination has been recognized as one of the ten greatest public health achievements of the 20th century, because of its success in the eradication and/or controlling the spread of life-threatening infectious diseases. Vaccination is the process of induction of immunity against foreign pathogens, diseased cells or disease-associated molecules. Immunization strategies are now being explored or clinically used to prevent or treat many diseased conditions. This includes bacterial as well as viral infections, cancer or Alzheimer's disease. In this presentation, we will first review the fundamentals of vaccine function and development, different types of vaccines, formulation components and delivery technologies for the stimulation of the right immune response for specific disease conditions. Route of vaccination is a critical factor for immunization success. Although intramuscular and subcutaneous injections are the most common routes of vaccine administration, other routes such as intradermal, oral, and intranasal used for vaccine delivery, as well. In this presentation, we will compare the advantages and disadvantages of each route, examine the specific formulation requirements, and present current advancements in vaccine delivery technologies.

Biography

Dr Lavasanifar is a Professor of Pharmaceutics in the Faculty of Pharmacy and Pharmaceutical Sciences and has a joint appointment at the Department of Chemical and Material Engineering, University of Alberta. She is the Scientific Chief Officer and Vice President of Meros Polymers, a spinoff company established based on the technology developed in her lab. Her research is focused on the development of delivery systems that can increase solubility, modify the pharmacokinetics, reduce toxicity and increase the activity of different therapeutics with a focus on the development of nano-medicine for cancer chemo and immunotherapy. The ongoing research projects in her laboratory include development of polymeric nano-carriers as systemic delivery systems for cancer therapy as well as stimulus responsive nano-gels for regional drug delivery. Dr. Lavasanifar has > 120 peer reviewed papers, 4 book chapters, several abstracts and conference presentations. She is an inventor in 8 filed patents. She has been the recipient of the 2016 and 2013 TEC Edmonton, Innovation Makes Sense award; 2007 GlaxoSmithKline/CSPS Early Career Award; the 2009 Sanofi-Aventis/AFPC award in recognition of outstanding research in Pharmacy. Dr Lavasanifar is the associate Editor of Journal of Pharmacy and Pharmaceutical Sciences and a member of the Editorial Board in Materials Sciences and Applications and Cancers. She has an active teaching program in both undergraduate and graduate levels.

Dr. Emmanuel Ho is currently an Associate Professor at the University of Waterloo, School of Pharmacy and a member of the Waterloo Institute for Nanotechnology and the Centre for Bioengineering and Biotechnology. Previously, he was the Leslie F. Buggey Endowed Professor in the College of Pharmacy at the University of Manitoba. Dr. Ho earned his Ph.D. in Pharmaceutical Sciences from the University of Toronto and was awarded an Industrial Post-Graduate Scholarship from the Natural Sciences and Engineering Research Council of Canada (NSERC). As a post-doctoral fellow at the British Columbia Cancer Research Center, Dr. Ho was awarded the Canadian Institutes of Health Research (CIHR) Post-Doctoral Fellowship along with the Michael Smith Foundation for Health Research Post-Doctoral Fellowship (MSFHR). His current research interests include the development and characterization of nanomedicines and medical devices for imaging, treatment, and prevention of diseases including HIV/AIDS, wound healing, and cancer. He was recently presented the 2013 Rh Award for Excellence in Research, was the recipient of the 2014 GlaxoSmithKline / Canadian Society for Pharmaceutical Sciences Early Career Award, and was awarded the 2015 Association of Faculties of Pharmacy of Canada (AFPC) New Investigator Research Award. Dr. Ho's research program is supported by grants from the Bill and Melinda Gates Foundation, CIHR, NSERC, Canada Foundation for Innovation (CFI), Research Manitoba, Manitoba Medical Service Foundation (MMSF), and Diagnostic Services Manitoba.

Formulating potent and Safe Delivery System for a Broad Range of Molecules from Small Molecule Adjuvants, to Proteins and RNA Vaccines

Dr. Derek O'Hagan
Global Head of Discovery Support and New Technologies, GSK Vaccines, Rockville, MD.

Abstract

Rapid progresses in molecular immunology has advanced vaccine adjuvant discovery efforts, enabling the use of cellular and target based assays to screen large collections of chemical compounds for potential use as immune potentiators. However the question remains as to how to safely and effectively deliver these new adjuvant compounds, particularly for use in existing vaccines which commonly use insoluble aluminium salts as adjuvants. Here we describe a novel approach to enable recently discovered adjuvant active compounds called Small Molecule Immune Potentiators (SMIPs) to adsorb to aluminum hydroxide adjuvant via the mechanism of ligand exchange. A phosphonate group has been chemically linked to the compounds to enable adsorption to aluminium hydroxide and here we present extensive formulation characterization, and an overview of the in vitro and in vivo performance of the new generation adjuvants.

In addition, we will address the challenges for formulation and delivery of a large and complex nucleic acid (RNA) that needs to be protected against degradation and also delivered effectively into cells to enable the induction of potent immune responses. We will compare and contrast the different formulation and delivery strategies for the diverse and complex molecules, from SMIPs, to proteins and to RNA.

Biography

Dr O' Hagan is the Global Head of Discovery Support and New Technology, in the Technical Research and Development group at GSK Vaccines, Rockville. Formerly, he was the Global Head of Vaccine Chemistry and Formulation for Novartis Vaccines. He has co-authored >140 original research publications, >60 book chapters and reviews and he is a named inventor on >60 filed patents. He was awarded the Conference Science medal of the Royal Pharmaceutical Society of Great Britain in 1997, and the Young Investigator Research Achievement Award of the Controlled Release Society in 1999. He is a Fellow of the American Association of Pharmaceutical Scientists.

Perspectives on Gene Therapy Advances: Non-viral Gene Delivery Technology for Neuroprotection in Glaucoma

Prof. Mariana Foldvari
School of Pharmacy, University of Waterloo, Waterloo, Canada

Abstract

Gene therapy is at the forefront of medicine with several new therapies recently approved by regulatory agencies. Despite the encouraging successes there is still a long road ahead to achieve a full spectrum of safe and effective clinical gene therapy treatments. One example from our laboratory illustrates the development of a non-viral gene delivery system for overcoming the neurodegenerative aspects of glaucoma. Currently, glaucoma management relies on pharmacological and invasive surgical treatments mainly by reducing the intraocular pressure (IOP), an important risk factor for the progression of visual field loss. Gene therapy with neurotrophic factors (NF) has the potential to provide neuropreventative and neuroregenerative functions for retinal ganglion cells to prevent or restore visual deterioration. We have developed and characterized non-viral gemini surfactant-phospholipid nanoparticles (GL-NPs) as NF gene carriers for intravitreal and topical administration. The overview will reflect on the design and formulation of gemini NPs as well as other gene carriers, NP toxicity and the identification of parameters that require deeper understanding to develop successful topical gene delivery systems targeting the retina.

Biography

Dr. Marianna Foldvari is a Professor of Pharmaceutical Sciences at the University of Waterloo's School of Pharmacy. She received her PhD from the College of Pharmacy at Dalhousie University. Between 1989-2006 she was a Professor at the College of Pharmacy and Nutrition, University of Saskatchewan. Then she moved to the University of Waterloo where she held the Tier 1 Canada Research Chair in Bionanotechnology and Nanomedicine from 2007-2014. She is an internationally recognized expert in nanomedicine. Her research program is focusing on non-invasive protein and gene delivery system design for regenerative medicine in dermatology, ophthalmology and immunology.

Dr Foldvari's contributions include both basic and applied research with a total of 230 publications, 26 patents, 110 invited presentations and about $23M in grant funding. She established the first pharmaceutical company, PharmaDerm Laboratories Ltd., in the province of Saskatchewan in 1991 to commercialize a topical protein delivery system technology, which is in Phase III clinical trials. She currently serves as Editorial Board Member of the *Journal of Controlled Release*, Associate Editor for *Precision Nanomedicine*, *Frontiers in Neuroscience*, *OA Drug Design and Delivery* and served for the past ten years for *Nanomedicine: NBM.*

Dr Foldvari is one of the Founding Directors of CSPS, the American Society for Nanomedicine (ASNM) and the International Society of Nanomedicine (ISNM). Recently she has received The AFPC Pfizer Research Career Award, the Honorary Award for Outstanding Lifetime Achievements in Nanoscience and Nanotechnology in Ontario and was elected as an AAPS Fellow.

The Feldan Shuttle Technology: a peptide-based method to deliver proteins in living cells

Dr. Thomas Del'Guidice

Research Specialist, Feldan Therapeutics, Quebec, Canada

Abstract

Delivery of recombinant proteins to therapeutic cells is limited by a lack of efficient methods. This hinders the use of transcription factors, antibodies or Clustered Regularly Interspaced Short Palindromic Repeats (CRISPR) ribonucleoproteins to develop cell therapies. Here, we report a new protein delivery method, the Feldan Shuttle Technology. This approach is based on the design of amphiphilic peptides with membrane permeation properties that mediate the direct delivery of proteins into mammalian cells including human stem cells, hard-to-modify primary natural killer (NK) cells, and cancer cell models. A less than two-minute co-incubation of Feldan peptide and proteins with cells achieve remarkable biological outcomes including gene regulation, genome editing and cell signaling modulation. The broad applicability and flexibility of this DNA- and chemical-free method across different cell types, particularly hard-to-transfect cells, opens the way for a direct use of proteins for biomedical research and cell therapy manufacturing.

Biography

Thomas Del'Guidice is a Research Specialist and Project Manager at Feldan Therapeutics. He earned his PhD in molecular and cognitive Neurobiology from the Laval University, Québec-Canada. He achieved an industrial postdoctoral fellowship at Feldan Therapeutics in the design and the screening of peptide sequences for the intracellular delivery of recombinant proteins and drug reagents in mammalian cells. His work focuses on the development of the Feldan Shuttle Technology, a new peptide-based method for the intracellular delivery of native and engineered proteins.

Large Molecule Drug Product Development & Manufacturing – Janssen Practice

Dr. Kai Zhang

Associate Director of Drug Product Development, Large Molecule, Janssen Inc.
Malvern, PA, USA

Abstract

Therapeutic proteins have played an important role in the treatment of human diseases due to their high efficacy and relatively low side effect profile. However, during development, manufacture, storage, and shipment of therapeutic protein products, the proteins may be susceptible to physical and chemical degradation, potentially resulting in reduced efficacy and/or unintended side effects. Product formulation and manufacture processes are key to success in the development of therapeutic protein drug products. This presentation will focus on the development and manufacture processes of large molecule drug products based on the practice at Janssen Inc.. Challenges and opportunities in therapeutic protein drug products and their development will be discussed, as well.

Biography

Dr. Kai Zhang has worked in pharmaceutical industry for over 15 years with extensive knowledge and experience in formulation and drug product development. She is Associate Director at Drug Product Development – Large Molecule, Janssen Inc., based in Malvern, PA, USA. Dr. Zhang received her Ph.D. in Pharmaceutics & Controlled Drug Delivery particularly for Proteins and Peptides from the Faculty of Pharmacy, University of Toronto, Canada.