PharmaED RESOURCES, Inc.

Inhalation Drug Delivery Virtual Summit 2021

Strategic Development, Commercialization & Emerging Applications

December 7–8, 2021, Online, PST

Featuring Lessons Learned and Case Studies from Industry Experts

Ju Du
Scientist
Formerly AstraZeneca

Guenther Hochhaus
Professor
University of Florida

Sara Waxberg-McNew
Chief Research Officer
Design Science Consulting

Mick Hurrey
VP Product Development
InCarda Therapeutics

Francois Bidet
VP Business Development
Aptar CSP Technologies

Vladimir Malinin
Executive Director of Research
Insmed Inc.

Hugh Smyth
Professor
University of Texas, Austin

George Cusatis
Director
Merck

Marco Laackmann
Dir. of Inhalation Technology
Harro Höfliger

Michael Eakins
Founder & Principal
Eakins & Associates
(Event Chair)

David Cipolla
VP of Research
Insmed, Inc.

Larry Brown
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Noveome Biotherapeutics

Donovan Yeates
CEO
KAER Biotherapeutics

Jacqueline Green
Business Development Manager
H&T Presspart

Pavan Muttil
Assoc. Professor
University of New Mexico

And Comprehensive Coverage On:

- Rapid Development of Inhaled Antiviral Therapy Against Coronavirus Infections
- Overcoming Challenges in Bringing Inhalation Delivery Products to Market
- Challenges and Potential Alternative Approval Pathways for Generic Suspension-based Nasal Sprays
- Why is Patient Usability Key to Inhalation Drug Product Development?
- Challenges in Formulation Design and Development: Development and Characterization of Treprostinil Palmitil Inhalation Aerosol
- Plasma Technology and pMDIs: A Sustainable Solution
- Rethinking Active Packaging: New Material Science Solutions to Old Challenges
- Non-viral Pulmonary Delivery of RNA Via Nebulization
- Pulmonary Delivery of a Tuberculosis Vaccine: Can Altering the Route of Immunization Resurrect an Age-Old Vaccine?
- Pathway to the Clinic: Intranasal ST266 Delivery for Neuroprotection in Optic Nerve Disease and Trauma
- Solid-phase Surfactant Aerosol Therapies for Patients with Severe Hypoxemia
- And Much More!

Featuring Representation From:

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Tuesday, December 7, 2021

8:50

Welcome & Opening Remarks from Chairperson, Michael Eakins

From Lab Bench to Market—Challenges, Opportunities, Pathways

9:00

Overcoming Challenges in Bringing Inhalation Delivery Products to Market

Dr. David Cipolla, Vice President of Research, Insmed, Inc.

It may come as a surprise that many of the early therapies developed to treat asthma and chronic obstructive pulmonary disease (COPD) were initially given orally or by injection and were later repositioned as inhaled products. This includes epinephrine, corticosteroids and the selective beta-2 agonists. In hindsight, the rationale for this change in delivery format appears obvious. Very little of the dose administered orally or by injection typically reaches the lung; thus, higher doses must be administered to be effective. Pharmaceutical development rarely follows the linear path that was originally outlined, and the target product profile often changes due to external factors or internal challenges. This presentation will focus on the surprises that arise in development and how inspiration, innovation and perseverance are required to achieve the goal of product approval.

9:45

Non-viral Pulmonary Delivery of RNA Via Nebulization

Dr. Ju Du, Research Scientist, Formerly AstraZeneca

Pulmonary drug delivery has made significant progress and shown promising benefits over other administration routes to treat local lung diseases. The aerosolized drug can achieve high local concentration and low systemic exposure. Also, RNA therapeutics have shown clinical success, e.g., RNA vaccine approval to treat COVID-19, delivered via injection. With this trend, there is a growing interest in treating genetic lung diseases using aerosol droplets containing RNA via nebulization. However, the nebulized RNA formulation has many obstacles to address, such as 1) formulation design to protect the biological activity of RNA post nebulization and 2) the efficient dose delivery by nebulizer. This presentation will review the non-viral formulation design for pulmonary RNA delivery, and selection and evaluation of the nebulization device.

10:30 ***A Word from our Sponsors***

Keynote: Inhaled Antiviral Therapies for Coronaviruses

10:45

Rapid Development of Inhaled Antiviral Therapy Against Coronavirus Infections

Dr. Hugh D. Smyth, Alcon Centennial Professor, & Professor of Molecular Pharmaceutics & Drug Delivery, University of Texas at Austin

KEY NOTE

Repurposing existing drugs is a promising strategy for inhaled therapeutics and even more so in the case of antivirals against SARS-CoV-2, the causative agent of the COVID-19 pandemic. Niclosamide (NIC) is a FDA-approved drug but this oral formulation produces systemic drug levels that are too low to inhibit SARS-CoV-2. Development of an inhaled formulation of NIC to target the respiratory tract as an aerosol could target the primary site of for SARS-CoV-2 acquisition and spread. We developed a NIC system suitable for delivery via dry powder inhaler, nebulizer, and nasal spray. This novel formulation exhibits potent in vitro and in vivo activity against coronaviruses such as MERS-CoV and SARS-CoV-2. This presentation outlines the rapid development of this formulation.

11:30 ***A Word from our Sponsors***

11:35

Solid-phase Surfactant Aerosol Therapies for Patients with Severe Hypoxemia

Donovan B. Yeates, Ph.D. CEO, KAER Biotherapeutics Corporation

Inhaled infective agents, irritants and toxic agents can lead to inflammation, depletion of pulmonary surfactant and severe hypoxemia. In the USA, ~15,000 patients are admitted to hospitals each day resulting from the COVID-19 pandemic. In addition, 2.2 million non-COVID-19 respiratory impaired patients are admitted each year. Aerosol treatments with aqueous and solid-phase surfactant replacements have been proposed, but none has reached sufficient efficacy to be approved by the FDA. The need for delivery of sufficient mass of fine particle surfactant aerosols deposited in the peripheral lungs is the major limitation to achieving effective treatment. Solid-phase surfactant aerosols contain 20 times higher surfactant mass than aqueous surfactant aerosols of similar diameter. Solid-phase surfactant aerosols can be generated from aerosolizing aqueous surfactant suspensions, evaporating the water from the droplets, and removing excess gas from the resultant particles using virtual impactors. This technology has been shown to achieve high concentrations of respirable fine particle aerosols. When such solid-phase surfactant aerosols are generated using heliox rather than air, the diameter of the particles is smaller and can be delivered with improved efficiency. It has been shown that aerosols entrained in heliox can have greater peripheral penetration and higher deposition in the alveolar regions

of the lungs. Using the SUPRAER aerosol generation and delivery system, the aerosol diameter, concentration and flow rates may be adjusted enabling the use of the same technology for animal efficacy, toxicology and clinical studies. This technology opens new potential to effectively deliver aerosols generated from non-viscous and viscous liquids, which can contain proteins, antibodies and high molecular weight moieties, for the treatment of severe respiratory ailments.

12:20 *Lunch Hour*

Critical Issues—Human Factors in Inhalation Product Development

1:20 **Why is Patient Usability Key to Inhalation Drug Product Development?**

Sara Waxberg-McNew, Chief Research Officer for Design Science, and Mick Hurrey, Vice President, Product Development at InCarda Therapeutics, Inc.

CASE STUDY

Many users today do not receive their full dose of medication due to mistakes made during the administration or handling process with their inhalation devices. With the numerous types of inhalers on the market, there are nuances to the use steps and external attributes of each. When developing drug products that are planned for inhaled use, it is important to use user-centered design approaches and iterative human factors methods to reduce the potential for errors while also increasing usability and patient satisfaction. This presentation will discuss the development of the user interface of inhalation products that demonstrate to regulatory bodies safe and effective use and the challenges that must be overcome.

Regulatory Considerations

2:05 **Demystifying the Regulatory Environment for Drug-Device Combination Products**

George Cusatis, Director, Device & Digital Health, Merck

Over the past several years, combination products (which include inhalation delivery devices) have become more of a household name both from an Industry and Health Authority perspective. It is only fitting that as the number of combination products available on the market continues to increase from previous years, so does the available guidance and scrutiny from regulators around the world. Legislation such as the 21st Century Cures Act, and the European Medical Device Regulation (MDR), has shed light on the direction that the regulation of combination products is heading. In this presentation, we will take a look at the evolving global regulatory landscape for combination products, best practices for regulatory filings, identification of regulatory opportunities and challenges, and future regulatory considerations for combination products as we move forward.

2:50 *A Word from our Sponsors*

3:05 **Proposed and Recent Revisions to USP Chapters Addressing Key Quality Attributes for Components and Delivery Systems for Inhalation Drug Products**

Michael Eakins, Principal Consultant, Eakins & Associates

There are a number of official chapters in the USP that address inhalation delivery systems for drug products. This presentation will take a look at those changes that have occurred from 2018 to the present day followed by what new areas may be addressed during the current USP cycle (2020 - 2025). Changes have been made to USP chapters <5>, <601>, <1601> and <1602> while new chapters, <1603> Good Cascade Impactor Practices and <1604> Presentation of Aerodynamic Particle Size Distribution Measurement Data have been proposed in the Pharmacopeial Forum. Other key quality attributes are addressed in chapters devoted to plastic and elastomeric packaging materials that are used to construct inhalation delivery systems and also extractables and leachables from these materials. The chapter on Elastomeric Closures for Injections <381> has been revised and 3 other elastomeric chapters added (<1381>, <382> and <1382>). Looking forward the presentation will review what packaging material chapters will be revised and what new chapters are planned during this current cycle that will impact delivery systems for inhalation products.

Panel Discussion

3:50 **Product Development—Current & Future Prospects**

Moderator: Michael Eakins

Panelists:

- Donovan Yeates, KAER Biotherapeutics
- Mick Hurrey, InCarda Therapeutics, Inc.
- David Cippola, Insmed, Inc.

Discussants:

The Audience

4:25 *End of Day 1*

Wednesday, December 8, 2021

8:55 *Chairperson's Welcome & Opening Remarks*

9:00 **Rethinking Active Packaging: New Material Science Solutions to Old Challenges**

Francois Bidet, VP of Business Development, Aptar CSP Technologies

Dry powders used for inhalation are sensitive to moisture that can cause particle build-up in the delivery channel and impact the stability of APIs. Depending on the device, formulation and markets, the level of moisture needs to be controlled to assure optimal dosage delivery and improve patient compliance.

Active material science technologies enable a portfolio of active packaging and integrated solutions to address these challenges for the development of Dry Powder Inhalers.

This presentation will cover the following key points:

- Activ-Polymer™ platform technology introduction
- DPI moisture challenges and selection of appropriate moisture adsorbing materials
- Protection of capsules for single dose DPIs within blisters
- Protection of powders within reservoir DPIs during shelf-life and use-life

9:45 **Dry Powder Inhalers (DPIs): Process Shortcuts from Lab to Production**

Marco Laackmann, Director of Inhalation Technology, Harro Höfliger Verpackungmaschinen GmbH

This presentation will cover key topics in DPI process and manufacturing, including:

- Powder properties and fill weight results for various dosing systems
- New insight on the correlation between lactose fines, flow characteristics and dosing behavior
- Criticality in up-scaling of DPI and manufacturing and the influence of powder dosing technologies on drug product performance
- Options to improve machine capabilities based on powder characteristics and mechanical treatment to make cohesive powder free flowing for powder feeding and metering processes

10:30 *A Word from our Sponsors*

Research Spotlight: Nasal Delivery—Challenges & Opportunities

10:45 **Challenges and Potential Alternative Approval Pathways for Generic Suspension-based Nasal Sprays—A Discussion**

Dr. Guenther Hochhaus, Professor of Pharmaceutics, University of Florida

Currently, approval of generic nasal spray suspensions is challenging, as clinical endpoint studies are recommended by FDA, mainly because of the challenges in assessing the equivalence in particle size distribution. This presentation discusses the feasibility of potential alternative pathways for approval of suspension-based nasal sprays.

11:30 *A Word from our Sponsors*

11:35 **Pathway to the Clinic: Intranasal ST266 Delivery for Neuroprotection in Optic Nerve Disease and Trauma**

Dr. Larry Brown, CSO & Executive Vice President, Noveome Biotherapeutics

ST266 is a novel, cGMP-produced secretome secreted by Amnion-derived Multipotent Progenitor (AMP) cells. AMP cells are created by culturing a select population of amnion-derived epithelial cells under proprietary conditions. The ST266 secretome produced by these cells is anti-inflammatory, anti-apoptotic and neuroprotective. We have successfully demonstrated intranasal delivery of this complex protein mixture to the brain via the olfactory nerves and the optic nerve. Neuroprotection in animal models has now propelled this research to human clinic studies.

12:20 *Lunch Hour*

1:20 **Challenges in Formulation Design and Development: Development and Characterization of Treprostinil Palmitil Inhalation Aerosol**

Dr. Vladimir Malinin, Executive Director of Research, Insmed, Inc.

It is not uncommon for an inhalation product to be formulated first in a liquid form for delivery by nebulization to allow expedited development to reach proof of concept. Following verification of the nebulized product concept in those studies, a more convenient formulation delivered by a dry powder inhaler (DPI) or a metered dose inhaler (MDI) may be desired for further development.

Such a transition is often faced with technical challenges to maintain the desired in vitro aerosol properties, and lung deposition and pharmacokinetics in vivo. Treprostinil palmitil (TP) is a prodrug of treprostinil, a pulmonary vasodilator that has been previously formulated for inhaled administration via a nebulizer. TP demonstrates a sustained presence in the lungs with reduced systemic exposure and prolonged inhibition of hypoxia-induced pulmonary vasoconstriction in vivo. Here, we present a case study on re-formulation efforts to develop a more convenient solution-based MDI formulation of TP that matches the pharmacokinetic and efficacy profile of a nebulized TP formulation.

2:05 *A Word from our Sponsors*

2:20

Pulmonary Delivery of a Tuberculosis Vaccine: Can Altering the Route of Immunization Resurrect an Age-Old Vaccine?

Dr. Pavan Muttil, Assoc. Professor of Pharmaceutical Sciences, University of New Mexico

Vaccination has been the most impactful medical intervention ever devised and has been effective to prevent the global spread of infectious disease. However, an estimated 10.4 million individuals develop tuberculosis (TB) globally accounting for 1.3 million mortality annually. The only vaccine against TB, the Bacille Calmette-Guérin (BCG) is a live bacterial vaccine and has been used in humans for the past hundred years. Unfortunately, BCG is highly variable in its protective efficacy, ranging from 0-80% in different regions of the world; the low efficacy of BCG (in high TB burden regions), at least in part, is due to humans being exposed to non-tuberculous mycobacteria or environmental mycobacteria (EM). Chronic EM exposure alters the immune environment in humans making them susceptible to TB infection. Globally, the BCG vaccine is administered by the intradermal (ID) route; however, parenteral immunization does not overcome the immunosuppression caused by EM, thus making the vaccine ineffective. We show, for the first time, that the immune suppression caused by EM exposure could be overcome by delivering the BCG vaccine by the pulmonary route; further, pulmonary immunization induces vaccine-specific immunity in the lungs that protect against aerosol TB infection. In this talk, I will discuss the protective immunity elicited after pulmonary immunization in animal models. I will also discuss the challenges faced with pulmonary immunization, especially in regions of the world that are TB-endemic.

3:05

Plasma Technology and pMDIs: A Sustainable Solution

Jacqueline Green, Business Development Manager, H&T Presspart

With the introduction of new propellants and more complex formulations, the pMDI canister plays a significant role in the efficacious delivery of the drug to the patient. The relationship between propellant, drug and canister has significantly changed, with different technologies and treatments being used to accommodate this relationship. The need for sustainable solutions within the pharmaceutical industry is growing, with some pharmaceutical companies already committing to developing a carbon free pMDI. H&T Presspart's plasma surface treatment for MDI canisters, presents its advantages compared to existing technologies and how it can provide a sustainable solution whilst improving drug delivery performance.

3:50 *End of Program*

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In the Age of COVID, the Show Must Go Online

In response to overwhelming audience and speaker feedback, and in view of the current health and safety concerns involving the novel coronavirus COVID-19, we're taking our events online (at least for the foreseeable future; we do hope to see you again soon in person when all this is over!) ***Inhalation Drug Delivery Virtual Summit 2021*** is an entirely online event, complete with insightful presentations from leading researchers, 1:1 networking opportunities, live question & answer sessions, and sponsored informational presentations that highlight how vendors are tackling some of the key issues facing the Inhalation Drug Delivery market today. Just sit back and enjoy this online learning experience from the safety and comfort of your home or office. Missed a session or two? No worries—the full program will be archived and available for post-conference viewing and download.

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