CGTP Fall Virtual Summit 2024

Schedule

Tuesday, 19 November, 2024

09:00-09:10 Virtual

CASSS Welcome & CGTP Fall Virtual Summit 2024 Introduction

JR Dobbins
V - Virtual
CGTP Summit Oral

Summit Chair: JR Dobbins, Eli Lilly and Company

Accelerating the Development of Adeno-Associated Viral Vector Gene Therapies: Innovations in Chemistry, Manufacturing, and Control

Gene therapy using adeno-associated viral (AAV) vectors has emerged as a transformative approach for treating a variety of genetic conditions. Many of these novel therapies are being developed to treat rare diseases with unmet medical needs, necessitating the rapid advancement through development and commercialization. The inherent complexity of AAV vectors has historically presented challenges in advancing the chemistry, manufacturing, and control (CMC) aspects commensurate with the pace of clinical development.

This virtual summit will bring individuals from academia, industry, and health authorities together to discuss innovative CMC approaches. Attendees will gain insights into CMC strategies, challenges, and opportunities to accelerate the development and commercialization of AAV therapies. The summit will be comprised of two sessions that focus on development of the process and product control strategy along with commercialization topics such as technology transfer and process performance qualification strategies. Each session will consist of multiple presentations and a panel discussion providing attendees the opportunity to learn from and interact with experts in the field.

By attending this summit, you will connect virtually with a relevant global network and gain tangible knowledge to help you more rapidly and robustly advance these transformative AAV vector therapies to patients who need them.

09:10-10:35 Virtual

Session I - Mastering the Art of Control: CQAs and Considerations for Control Strategies for AAV-based Therapeutics

Andrea Challand, Leslie Nash, Christiane Niederlaender
V - Virtual

Session Chairs: Andrea Challand, F. Hoffman La Roche Ltd, Leslie Nash, Health Canada, and Christiane Niederlaender, Parexel International

The relatively safe product profile has catapulted AAV-based gene therapy products to the forefront of desirable therapeutic strategies to treat genetic conditions with high unmet need. However, the rapid clinical advancement of AAV products frequently outpaces CMC development, often due to the limited manufacturing process and product characterization. Developing a phase-appropriate control strategy that consistently achieves the desired product quality and performance attributes required to support safety and efficacy throughout the product life cycle can be challenging. This need is made more pressing by limited material availability due to low process yields and batch numbers, as well as continual evolution and advancement of analytical technologies.

This session aims to explore how product development can be streamlined based on thoughtful CQAs, combined with solid process and product understanding. We will explore how development of robust and standardized analytical methods can facilitate this integrated approach further, enabling rapid and effective commercialization of AAV therapies. Control strategies for unique regulatory challenges, such as rare diseases, will be discussed.

Session Speakers:

Release Quality Attributes of rAAV-Based Gene Therapy Medicinal Products
Laura Rodríguez, *Spanish Agency of Medicines and Medical Products (AEMPS)*

AAV Critical Quality Attributes: Comprehensive Analytical Control Strategies from Release to Characterization
Thomas Powers, *Pfizer, Inc.*

Control Strategies using CQAs for Method Changes and Comparability for Gene Therapies
Phillip Ramsey*, Sangamo Therapeutics, Inc.*

CQAs and Strategies to Accelerate the Development of Gene Therapies
Catherine Campbell, *4D Molecular Therapeutics*

10:35-10:40 Virtual

Mini Break

10:40-12:00 Virtual

Session I - Panel Discussion - Questions & Answers

Andrea Challand, Leslie Nash, Christiane Niederlaender
V - Virtual

Session Chairs: Christiane Niederlaender, Parexel International, Leslie Nash, Health Canada, and Andrea Challand, F. Hoffman La Roche Ltd

Additional Panelists:

Tania Rosen-Cheriyan, *CBER, FDA*

Francis Galaway, *Medicines and Healthcare products Regulatory Agency (MHRA)*

12:00-13:00 Virtual

Break

13:00-14:20 Virtual

Session II - Development and Manufacturing Strategies Enabling the Commercialization of AAV Therapies

JR Dobbins, Darius Pillsbury, Jen Wellman
V - Virtual

Session Chairs: JR Dobbins, Eli Lilly and Company, Darius Pillsbury, ValSource Inc., and Jennifer Wellman, Akouos, Inc.

Historically, the rapid advancement of AAV therapies through clinical development has resulted in the development of the manufacturing process to be on the critical path, potentially impacting the CMC readiness for commercializing these innovative therapies. Thus, it is imperative early in the development lifecycle to consider the commercialization strategy. As AAV products progress into late-stage development, programs are often presented with challenges related to comparability necessitated by process, scale or site changes, process performance qualification and stability strategies to support commercialization. This session will build upon the morning session by exploring effective process development and manufacturing strategies along with regulatory considerations to support the rapid and effective development and commercialization of AAV therapies.

Session Speakers:

The Journey Towards Commercialization
Scott Cross, *Dark Horse Consulting*

Late-Stage Tech Transfer and Comparability Considerations for AAV-based Gene Therapy: A Case Study
Shengjin Jin*, Sarepta Therapeutics, Inc.*

Navigating Complexities of Late-Stage Analytical Development for Ensuring Comparability of Manufacturing Process Change in a Mutation-Agnostic Gene Therapy
Samar Mohanty, *Nanoscope Therapeutics, Inc.*

14:20-14:25 Virtual

Mini Break

14:25-15:50 Virtual

Session II: Panel Discussion - Questions & Answers

JR Dobbins, Darius Pillsbury, Jen Wellman
V - Virtual

Session Chairs: JR Dobbins, Eli Lilly and Company, Darius Pillsbury, ValSource Inc., and Jennifer Wellman, Akouos, Inc.

Additional Panelists:

Martin Nemec - *Health Canada*

Anurag Sharma, *CBER, FDA*

Fraser Wright, *Kriya Therapeutics, Inc.*

15:50-16:00 Virtual

CGTP Fall Virtual Summit 2024 Closing Remarks

JR Dobbins
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CGTP Summit Oral

Summit Chair: JR Dobbins, Eli Lilly and Company