Northern California Regional Forum

Fast-to-Market CMC Strategies

South San Francisco Conference Center
South San Francisco, CA

Wednesday, November 13, 2019

Co-chairs:
Trevor Swartz, *Genentech, a Member of the Roche Group*

Shelley Suggett, *Themistry, Inc.*

Acknowledgements

A special thanks to the program committee who helped develop this program!

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Scientific Program Summary

Wednesday, November 13, 2019

08:00 – 09:00	**Registration and Continental Breakfast** in the Lobby
09:00 – 09:15	**CASSS Welcome & Introductory Comments** in Salon F
09:15 – 09:45	**Accelerated CMC Development and Strategies to Meet Fast to Patient Access** Tura Camilli, *Amgen Inc., Thousand Oaks, CA, USA*
09:45 – 10:15	**Accelerated CMC Development for Polatuzumab Vedotin** Andy Lin, *Genentech, a Member of the Roche Group, South San Francisco, CA, USA*
10:15 – 10:45	**Leverage CMO's Capacity, Technology and Flexible Facility Design to Support Speed-to-Market** Min Zhu, *Boehringer Ingelheim Pharmaceuticals, Inc., Fremont, CA, USA*
10:45 – 11:00	**Break**
11:00 – 11:30	**Panel Discussion** Tura Camilli, *Amgen Inc., Thousand Oaks, CA, USA* Andy Lin, *Genentech, a Member of the Roche Group, South San Francisco, CA, USA* Min Zhu, *Boehringer Ingelheim Pharmaceuticals, Inc., Fremont, CA, USA*
11:30 – 12:30	**Networking Lunch** in Salons G-J Join speakers and committee members for casual table discussions.
12:30 – 13:00	**Navigating the Unique Challenges of Allogeneic Cell Therapy Products** Farah Fawaz, *Allogene Therapeutics, Inc., South San Francisco, CA, USA*
13:00 – 13:30	**CMC Development Strategies for Expedited Gene Therapy Programs** Philip Ramsey, *Sangamo Therapeutics, Inc., Richmond, CA, USA*
13:30 – 14:00	**Networking Break**

14:00 – 14:30 **Panel Discussion**

Farah Fawaz, *Allogene Therapeutics, Inc., South San Francisco, CA, USA*
Philip Ramsey, *Sangamo Therapeutics, Inc., Richmond, CA, USA*
Mimi Roy, *BioMarin Pharmaceutical Inc., Novato, CA USA*

14:30 – 15:00 **Closing Remarks**

Program Abstract

Fast-to-Market CMC Strategies

Companies are driven by clinical development plans to rapidly bring drugs to patients. Efficient CMC strategy is a part of your company's success! In this forum we will look at different perspectives and molecules, and evaluate the different deliverables required throughout development through initial licensure. CASSS is committed to bringing job-relevant education and regulatory trends for biopharmaceuticals to CASSS members through meeting programs Content may include information from other regional, national or international forums organized by CASSS and speakers from industry and FDA. The program will also provide a platform for networking with both peers and regulatory authorities.

Learning Objectives:

1. What CMC strategies can be applied to enable fast-to-market drug development.
    a. Employing these strategies while staying resource lean.
    b. Gating accelerated CMC strategies to clinical readouts.
2. BTD what CMC work packages can be negotiated, e.g., what is non-negotiable, can some work packages be PMCs.
3. Explore various situations that necessitate unique and fast-to-market approaches, such as:
    a. CMC strategies to "catch-up" when surprised by stellar clinical results.
    b. Challenges of new modalities (i.e., gene therapy, T-cell therapies) in fast-to-market scenarios.
    c. Unique challenges for functional areas in fast-to-market scenarios (cell-culture, purification, analytical, and formulation).

The CASSS Northern California Regional Forum is committed to bringing job-relevant education and regulatory trends for biopharmaceuticals to CASSS members through meeting programs. The November forum will focus on CMC strategies to enable fast to market applications. The discussion will include content from other regional, national or international forums organized by CASSS. The program will also provide a platform for networking with both peers and regulatory authorities.

Speaker Abstracts

Accelerated CMC Development and Strategies to Meet Fast to Patient Access
Tura Camilli, *Amgen Inc., Thousand Oaks, CA, USA*

Increasingly, Sponsors are pressured to expedite the availability of drugs for the treatment of unmet medical needs. Acceleration of drug development to meet clinical timelines often results in CMC becoming rate limiting to overall program development, prompting the need to develop appropriate risk-based solutions/strategies to ensure that CMC is not the limiting factor for new marketing applications. In this presentation, we describe some of the CMC acceleration challenges and present strategies considered to ensure fast to patient access.

Accelerated CMC Development for Polatuzumab Vedotin
Andy Lin, *Genentech, a Member of the Roche Group, South San Francisco, CA, USA*

Polatuzumab vedotin (POLIVY) is an anti-CD79b antibody drug conjugate used to treat B cell lymphoma . In order to bring POLIVY to the market as fast as possible after receiving positive Ph 2 clinical results, the team developed an accelerated filing strategy and successfully launched the product 2 years ahead of the original schedule. In this talk, we will describe streamlined validation opportunities, regulatory tools and the risks/benefits to be considered in developing an accelerated CMC strategy.

Leverage CMO's Capacity, Technology and Flexible Facility Design to Support Speed-to-Market
Min Zhu, *Boehringer Ingelheim Pharmaceuticals, Inc., Fremont, CA, USA*

Innovative companies often apply an accelerated CMC strategy when surprised by remarkable clinical readouts. When internal resources and manufacturing capacity are limited, CMO can play an important role in supporting the company's timeline. In this talk, we will describe how a pharmaceutical company can partnership with a CMO to co-develop an efficient CMC strategy, and leverage CMC's capacity, knowledge, flexible facility design, as well new technology to support a fast-to-market strategy.

Speaker Abstracts (continued)

Navigating the Unique Challenges of Allogeneic Cell Therapy Products
Farah Fawaz, *Allogene Therapeutics, Inc., South San Francisco, CA, USA*

Allogeneic CAR T products are emerging as a new modality in immunotherapy and may hold great promise to address unmet medical needs. In an environment where no precedents are available, we propose to leverage the CAR T Quality Target Product Profile (QTPP) as a tool early in development to help implement a robust process and enable modifications/optimizations throughout the product life cycle while supporting comparability strategies.

CMC Development Strategies for Expedited Gene Therapy Programs
Philip Ramsey, *Sangamo Therapeutics, Inc. Richmond, CA, USA*

There are numerous gene therapy product candidates in clinical development for rare diseases that offer an opportunity for accelerated approval. While clinical data may provide a rapid path to approval, there remains a huge challenge in providing a robust CMC package that supports this accelerated approval. In this talk, we describe challenges in implementing gene therapy programs including manufacturing capacity, formulation, analytical characterization, regulatory requirements, and the risks and benefits in pursuing an accelerated CMC strategy.