2019 ARM CMC Summit

Draft Agenda

Where: North Carolina Biotech Center, Research Triangle, North Carolina

When: December 17th, 2019

What: The ARM S&T Committee is hosting its 3rd annual CMC Summit to explore manufacturing and analytical challenges specific to the regenerative medicine industry. The 2019 CMC Summit is focused on "CMC Preparedness in Regenerative Medicine."

The Summit is a single day event and includes presentations exploring current thinking on key CMC challenges, as well as panels discussing opportunities to proactively address obstacles through education and communal effort.

The event is free to attend for ARM members and will include both a breakfast and lunch for attendees.

Who: The CMC Summit is intended as an education and network development event for those interested in exploring and understanding challenges around CMC for Cell Therapies, Gene Therapies, and Tissue Engineered Products.

Draft Agenda (8:30am-4:30pm ET)

8:30am - 9:00am | Breakfast & Registration

9:00am - 9:30am | ARM S&T Presentation

9:30am - 10:30am | EMA CMC Presentation

10:30am - 10:45am | Coffee Break

10:45am - 11:45am | FDA CMC Presentation

11:45am - 12:45pm | Lunch & Networking

12:45pm - 1:45pm | Considerations in Developing Durable CMC Competency

1:45pm - 2:15pm | Durable CMC Competency Breakout Sessions

2:15pm - 2:30pm | Coffee Break

Alliance for Regenerative Medicine

2:30pm - 3:15pm | Implementing a Strategy for QC Assay Development

3:15pm - 4:00pm | Addressing Questions on CGTx Comparability

4:00pm - 4:30pm | Summary Session & Summit Report Out

Session Descriptions:

1. FDA CMC Presentation (60min w/ Q&A)
    - *Talk will explore the agency perspective on CMC preparedness including where the field stands, what the agency hopes to see, and any recommendations from the agency on how to best prepare a CMC program early*

2. EMA CMC Presentation (60min w/ Q&A)
    - *Talk will explore the agency perspective on CMC preparedness including where the field stands, what the agency hopes to see, and any recommendations from the agency on how to best prepare a CMC program early*
    - **Pending EMA Confirmation**

3. Considerations in Developing Durable CMC Competency (60min, 30min breakout)
    - *Session will explore the key questions and decisions to be addressed by a therapeutic developer with regards to implementation of a CMC program. This session will focus on methods for creating a CMC program which scales as appropriate for clinical phases of development, and which could support the program through an accelerated approval such as RM/AT or PRIME. The panel will also consider how to address the ever-present question of resource investment between R&D and CMC/GMP programs.*
    - *Breakouts to focus on the CMC preparedness question as either a small scale, preclinical developer vs a later stage ph2+ organization*

4. Implementing a Strategy for QC Assay Development (45 min)
    - *This session will focus on addressing key questions around QC assay development during the prolonged course of product development and clinical trials. The panel will address questions such as when to begin validating an assay, the development of potency assays, the role of animal models in human CGTx*

testing, and recommendations on methods and technologies for characterization of a CGTx drug product.

5. Addressing Questions on CGTx Product Comparability (45 min)
    - *A panel of CMC experts from across technology sectors will address questions from the attendees around Comparability. Questions and input will be sourced both in advance from the attendees, and in-real time through audience engagement.*