Agenda (I)

Set up
9 – 9:30

- **Galen/Atlantica opening:** Introductions, objectives, ground rules
- **Peter Marks:** Call to action and objectives for the meeting
- **Mike Lehmicke and David Barret:** Meeting genesis

Problem definition & impact
9:30-10:30

FDA staff

- How does CGT potency assay challenge show up at the Agency?
- What are developers top 3 mistakes? What is misunderstood or misapplied from the 2011 guidance?

Developers

- How does CGT potency assay challenge show up in your experience?
- What has been your experience as to how the FDA engages on potency assays?

Individual activity: Start/Stop/Continue

- What approaches should companies and regulators start/stop/continue for CGT potency assay development?
- Offer modality-specific or –agnostic comments

Galen/Atlantica synthesis of challenges based on pre-meeting discussions

Break

Detailed issue exploration and solution ideation (I)
10:30 – 1:00

Advancing collective knowledge: Role for pre-competitive and multi-stakeholder action (optional, time permitting)

- Sharing CQAs for established modalities
- Potential fruitful pre-competitive areas (e.g., donor cell viability, AAV)

Breakout: What is best practice/ ideal pathway across lifecycle of development?

- Expectations linked to stage for CAR-T, AAV-delivered gene therapy, and cell therapies
- Role of FDA and Company in ‘commitment in principle’ to an assay matrix design
- Incentives for companies to invest early in PAs
- Role of post-market commitments

Agenda (II)

Lunch (1 pm)

Detailed issue exploration and solution ideation (II)
1:45 – 3:30

Optimizing the assay matrix

- What is the appropriate portfolio of potency assays for a development program?
- How to address highly correlated assays? Do they reveal new information?
- How to engage with FDA in pruning? Can you ever scientifically justify a single validated assay for lot release?
- What mix of assays for lot release vs. for comparability?
- When is it appropriate to use the post-market setting to optimize PAs?

Demonstrating the biological cascade

- What is the appropriate set of assays to demonstrate potency of a multi-step biological process?
- Can we define cases when an expression assay is sufficient (and functional not needed)?
- Or, when functional is sufficient and expression not needed?
- Or, where infectivity is not needed when expression/ functional demonstrated?
- Organize perspectives by modality / therapeutic type

Break (if needed)

Next steps and closing
3:30 – 4:00

- **Peter Marks:** Closing reflections and take-aways
- **Galen/Atlantica:** Opportunities for progress
- **Close**