Roundtables - Session 1 – Table 9 - Platform Applications Outside of AAV Gene Therapy Field

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ABSTRACT

While there was a strong appetite to develop AAV Gene therapies applying a platform approach, such a development strategy is not limited to AAVs, and other product types (CAR-T, CAR-NK, LV, and iPSCs derived Cell Therapies) would benefit from lessons learned on platform applications.

- Scientists, Industry experts and Regulatory experts are welcome to contribute to this roundtable to better understand the ins and outs of Platform Applications Outside of AAV Gene Therapy Field, with a focus on:
- Definition of platform (technical, regulatory…).
- Which platform approaches outside of AAV are being explored?
- Lessons learned: What can a Cell and Gene Therapy leverage from applying a platform approach (expectations vs reality)?
- How and When to discuss platform applications with Regulatory authorities, including different mechanisms for FDA and EMA?
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ROUNDTABLE DISCUSSION NOTES

Industry and regulator perspectives were provided by attendees on the utility and perceived advantages of platform technologies and associated designations. The platform approach is evolving in both US and Europe. The following points were discussed:

- Definition/types of platform technology applications:
    - Manufacturing platform systems: minor changes only between products
    - Analytical platforms: similar analytical testing applies across more than one product with minor changes for product-specific testing
    - Examples of platforms that have been around for some time and are accepted by regulators to use prior knowledge vs complete package for every product: mAbs, vaccines
    - Examples of platform approaches being considered by the roundtable attendees (non-AAV): other viral vector processes (gammaretrovirus), crispr-based gene therapies which are individualized/customized for one patient's mutation, analytical platforms
- Regulator perspective for platform technology
    - Similar to use of prior knowledge during review

    - EMA: this determination is made on a case by case basis at the moment although legislation is being discussed for platform technologies.
    - EMA: Because master files don't exist for biological substances in EU, leveraging a platform technology is a mechanism to shorten review or de-risk submission
    - FDA: new draft guidance for platform designation was discussed
    - Potential opportunities for platform discussion with health authorities:
    - ITF, QIG, CAT, Interact
- Industry perspective: what does platform designation provide that doesn't already exist?
    - De-risk aspects of CMC for multiple products (i.e. lean on Agency's acceptance of original platform process)
    - Can be an incentive for investors (investing in a platform technology with potential for numerous products vs single product)
    - Leverage prior knowledge for safety to streamline development
    - Inform process validation approach
    - Leverage data across platforms (viral clearance, PV data)
- Considerations for manufacturing platform:
    - QTPP for production capacity is critical
    - Appropriate scale for dosing schema needed
    - Examples from AVV show that platform manufacture may not be able to be leveraged for all products