From Platform Promise to Commercial Reality: Accelerating Gene Therapy Commercialization Through Structured Data

Despite rapid advancements in vector design and transgene biology, downstream bioprocessing is often constrained by operational bottlenecks, including slow tech transfers, fragmented data, prolonged onboarding, and escalating cost of goods (COGs). For rare disease programs in particular, where patient populations are small and timelines are critical, inefficiencies at the manufacturing and CMC level can threaten program viability.

In response, the push for platforming processes has become a common refrain across the gene therapy ecosystem; however, in practice, these efforts are frequently limited to shared equipment, cell lines, or high-level process similarities. Yet true platform leverage requires more than technical standardization—it demands a way to systematically capture, reuse, and evolve process knowledge at scale. Viralgen, a CDMO specializing in recombinant adeno-associated virus (rAAV) manufacturing, has taken a differentiated approach by pairing a deeply standardized production platform complemented by a structured, digital CMC backbone. The results—data-driven execution across more than 1,500 batches—underpin a future where better data and more comprehensive platforms pave the way for commercial success.

The Hidden Friction in Gene Therapy Technology Transfer

Despite advances in automation and analytics, gene therapy tech transfers are still often underpinned by manual, document-centric workflows. Sponsor knowledge arrives in spreadsheets, slide decks, and PDFs; CDMO platform knowledge lives in separate internal systems; and reconciliation depends heavily on individual expertise and repeated review cycles. Version control challenges, siloed information, and reactive updates introduce both delay and compliance risk.

These inefficiencies compound as programs scale and, in turn, each new sponsor onboarding becomes a bespoke exercise, even when underlying platform processes are highly similar. The extended timelines—often six to twelve months—that ensue slow the journey to GMP execution, increasing development costs and delaying patient access.

Viralgen's Platform, Operationalized

Viralgen's manufacturing platform Aava™ is built on a consistent technical foundation: an exclusive Pro10™ cell line, standardized raw materials, harmonized equipment trains, and validated process definitions spanning 250 L to 2,000 L single-use bioreactor scales. Across more than 30 AAV serotypes, this platform has supported over 1,500 batches with a 100% success rate for regulatory interactions linked to produced material.

What differentiates Viralgen's approach is not simply the existence of this platform, but how it is operationalized. Rather than treating platform knowledge as static documentation, Viralgen has digitized it into a structured data model using QbDVision, creating a single source of truth that integrates historical performance, critical quality

attribute (CQA) risk assessments, process FMEAs, control strategies, and scale relationships. This allows institutional platform knowledge to be reused, adapted, and expanded—not reassembled—for each incoming program.

This platform maturity is reflected in outcomes, not projections: Viralgen reports a 100% regulatory success rate for agency interactions linked to produced material, alongside support for more than 60 global sponsors and over 40 clinical trial authorizations. These metrics underscore a critical distinction: the platform is not theoretical—it is executed continuously across clinical and commercial programs, with measurable results.

Turning Data into Acceleration

In a recent onboarding case of a sponsor seeking gene therapy commercialization, Viralgen demonstrated how this approach can materially compress timelines without compromising quality-by-design (QbD) rigor. Historically, onboarding a new sponsor for Process Performance Qualification (PPQ) was a fragmented process, burdened by document exchange, data reconciliation, and siloed approvals across multiple systems. Even with a mature platform, translating sponsor-specific knowledge into Viralgen's framework remained a bottleneck.

Prior to sponsor engagement, Viralgen had already digitized its entire rAAV platform within QbDVision, including evidence-based Critical Quality Attribute (CQA) risk assessments informed by a comprehensive literature review of more than 4,500 papers. Approximately 150 relevant publications were identified, with 57 key citations incorporated directly into the structured platform model.

This prework fundamentally changed the onboarding dynamic. Rather than starting from blank documents, the sponsor and CDMO worked from a validated, data-rich baseline.

In two focused workshops, the teams completed the core QbD deliverables:

- A finalized, product-specific CQA risk assessment generated within three days by adapting the platform assessment
- A comprehensive process Failure Mode and Effects Analysis (FMEA) completed in one week by leveraging existing parameter impact, occurrence, and control data

Because all information lived in a shared, structured system, updates were immediately visible to all stakeholders, eliminating redundant review cycles and minimizing the risk of inconsistency. Within two weeks, Viralgen completed all core QbD onboarding activities, enabling the overall onboarding timeline to be reduced from six to twelve months down to just three.

Reducing COGs Through Reuse, Not Reinvention

Beyond speed, this approach has meaningful implications for cost of goods. By standardizing data and automating scaling calculations, Viralgen reduces manual effort, rework, and late-stage deviations—all major contributors to COGs in gene therapy manufacturing. Gap assessments between sponsor-specific processes and platform definitions identify differences early, enabling targeted mitigation rather than broad requalification.

Over time, each completed program enriches the platform. Proven scale transitions, validated control strategies, and prior risk learnings accumulate, allowing future programs to start from a stronger position. This creates a virtuous cycle: faster onboarding, fewer surprises, and more predictable execution across the portfolio.

A Scalable Model for Rare Disease Commercialization

For rare disease developers, where resources are limited and timelines are unforgiving, this model offers a pragmatic path forward. By combining a technically robust rAAV platform with a structured digital CMC system, Viralgen demonstrates how platform strategies can move beyond abstraction to deliver measurable commercial impact.

The broader implication is clear: accelerating gene therapy commercialization is no longer just a matter of better science or bigger facilities. It requires rethinking how process knowledge is captured, governed, and reused. Structured data, not simply documents, is the foundation for scalable, efficient manufacturing. As gene therapy programs continue to advance toward commercialization, approaches like Viralgen's show how innovation at the data and execution layer can be just as transformative as innovation at the bench, helping promising therapies reach patients faster, at lower cost, and with greater confidence.