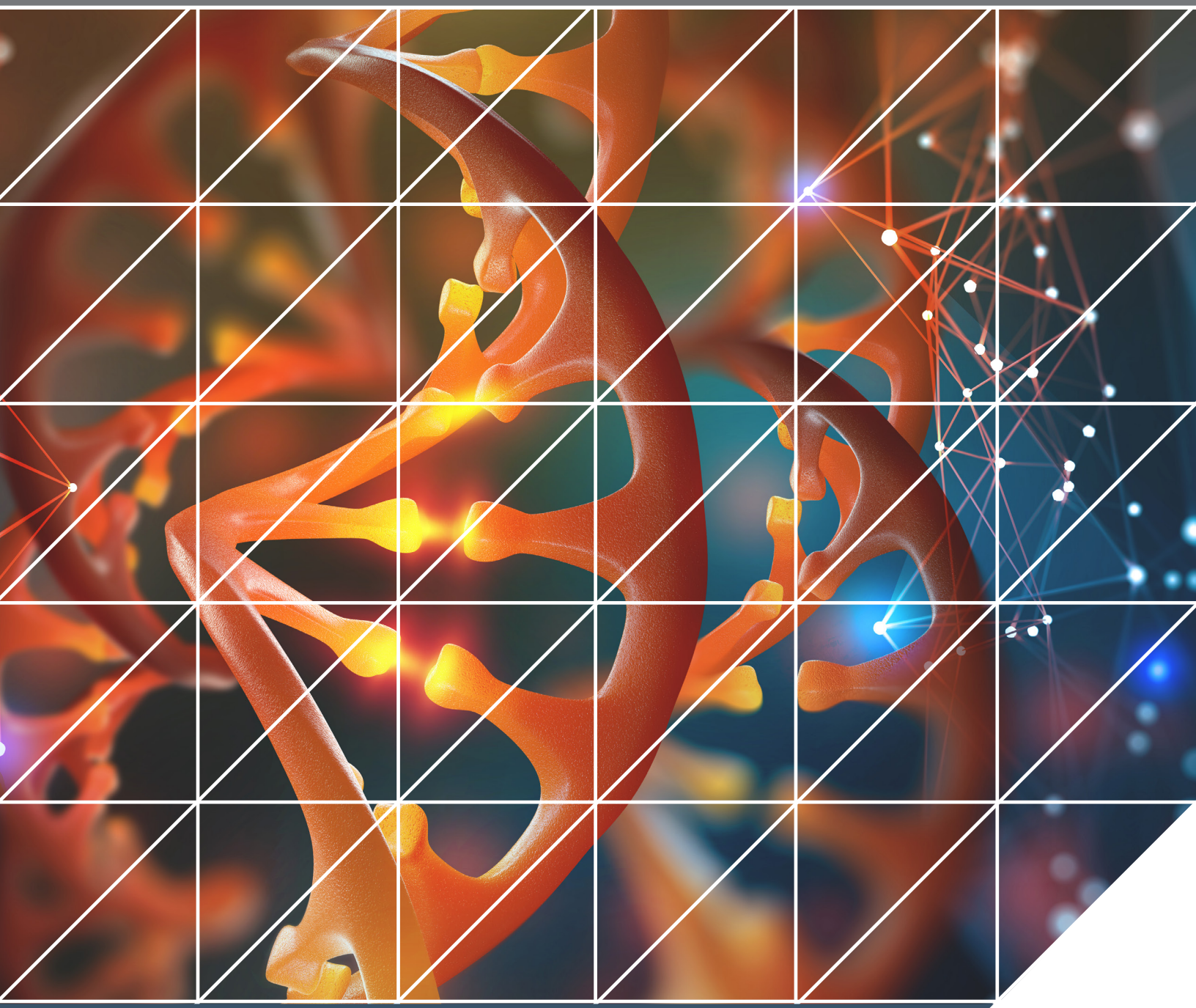

Considerations for Early Stage Gene Therapy Start-ups: From Clinical Development to Manufacturing to Commercialization

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In 2020, nearly $20 billion of funding poured into the advanced therapy sector, reflecting the enthusiasm of researchers and investors in the transformative potential of these novel treatments.

Recently, Precision ADVANCE hosted ***Considerations for Early Stage Gene Therapy Start-ups: From Clinical Development to Manufacturing to Commercialization***, an expert panel discussion moderated by Anshul Mangal, President of Project Farma and Precision ADVANCE. Precision ADVANCE is a collection of interconnected services and complementary teams uniquely focused on the complexities of gene therapies and the resources needed to bring these advanced therapeutics successfully to market.

$20 BILLION

The amount of funding invested in the advanced therapy sector in 2020

Thinking About Safety

Andrew Bellinger, Chief Scientific Officer at Verve Therapeutics, opened the panel with an overview of his company's pipeline of novel gene editing therapies aimed at lowering lifetime risk of cardiovascular disease. Given the size of the addressable market for these therapeutics, Bellinger described the need to make decisions early on in the product development cycle regarding approaches that would not only be scalable, but also have a safety profile compatible with treating broader patient populations.

Palani Palaniappan, Chief Technical Officer at Aruvant, continued on the topic of safety in describing his company's approach to treating sickle cell disease. Aruvant has developed autologous stem cells modified with a proprietary fetal hemoglobin variant that enables reconstitution with reduced intensity conditioning.

Managing External Manufacturing

Palaniappan also highlighted a challenge that many early stage gene therapy start-ups face—being fully outsourced and not feeling fully in control of their own destinies.

"Managing CDMOs [contract development and manufacturing organizations] is a challenge, especially in newer modalities, because there is still so much we don't know," explained Palaniappan. "What I've found very useful in CDMO management is being as specific and as clear as possible in the contract stage about expectations."

Tony Khoury, Executive Vice President of Project Farma, expanded on the effort required to achieve the level of collaboration, alignment, and communication necessary for successful tech transfer.

"The upfront evaluation and assessment of a CDMO should be more robust than just an audit for quality. It's also critical to find the right fit and the right culture," emphasized Khoury.

With novel therapies, it is also rare to find one vendor that has all of the facility capabilities and technology needed. Consequently, advanced therapy developers may find themselves tasked with managing multiple vendors while trying to keep timelines integrated.

Overcoming Bottlenecks

When asked about common challenges he has seen in the field, Khoury pointed out that manufacturing is not the only hurdle with gene therapy. Science, analytical, and technical development capabilities, and even warehousing, can be bottlenecks for these advanced therapeutics. There is also a talent shortage in this rapidly growing space. Most of the panelists agreed, however, that previous experience may not exist and the keys to recruitment are finding people with the ability to develop themselves and investing in robust training programs.

"Finding people [who] are able to put the pieces together to find connections between what appear to be disparate bits of the development and commercialization continuum is particularly important," added David Parker, Senior Vice President of Diagnostic Solutions at Precision for Medicine.

Commercializing Gene Therapies

Collecting evidence-based data is a crucial step in the commercialization of gene therapies. Phil Cyr, Senior Vice President at Precision Value & Health and Precision ADVANCE, stressed the importance of developing early economic or health economic models to inform clinical trial design and data collection. He also discussed the value of building a thorough understanding of the cost of illness and the differences among clinical paradigms across different countries.

Another challenge of commercialization is the potential need for companion diagnostics for patient stratification. For most rare disease gene therapies that require companion diagnostics, developing an assay that is accessible within the clinical and economic constraints can be an expensive and complicated endeavor.

Having been a partner to over 75 advanced therapy organizations, Precision Medicine Group's interdisciplinary teams—including Precision for Medicine, Project Farma, and Precision Value & Health—understand that delivering a gene therapy to market requires a comprehensive and integrated approach.

To learn more about considerations for early stage gene therapy start-ups, watch the full webinar on demand.

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Precision ADVANCE, a collection of interconnected services and complementary teams, uniquely focuses on the complexities of clinical, regulatory, manufacturing, and commercial needs to successfully bring cell or gene therapies to market.

Connect with one of our experts. Contact us at **precisionadvance@precisionmedicinegrp.com.**
To learn more about Precision ADVANCE, visit **precisionmedicinegrp.com/advance.**

2 Bethesda Metro Center
Suite 850
Bethesda, MD 20814

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