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Insights from the Network

Beyond the Lab: Preparing Cell Therapies for Investors, Regulators, and Patients

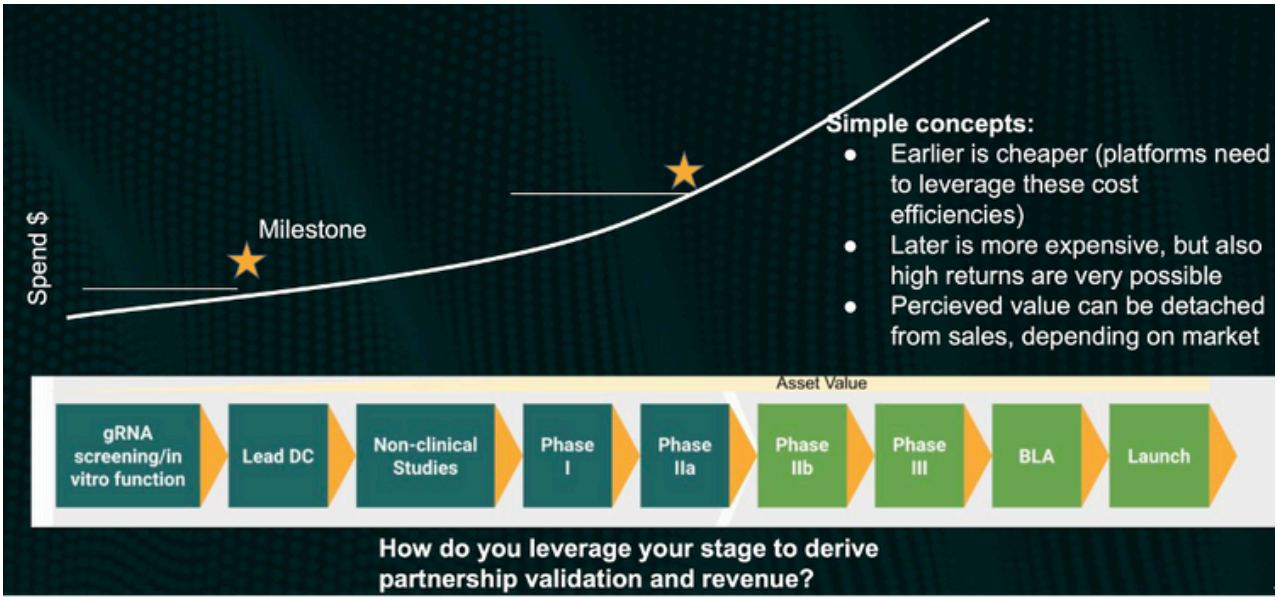
By Gregory Block, Matthew Jeffers and Chantal Martin

You've made a startling discovery in the lab and believe that your technology could generate revenue in the future. You've protected the intellectual property in consultation with the technology transfer office and have ensured that you have followed best practices to prevent disclosure of the invention. It's clear in your mind that this is the next big thing but guess what – no one else is convinced. You are about to enter the world of fundraising in a competitive environment and almost nothing you've learned in your academic career will have prepared you for it.

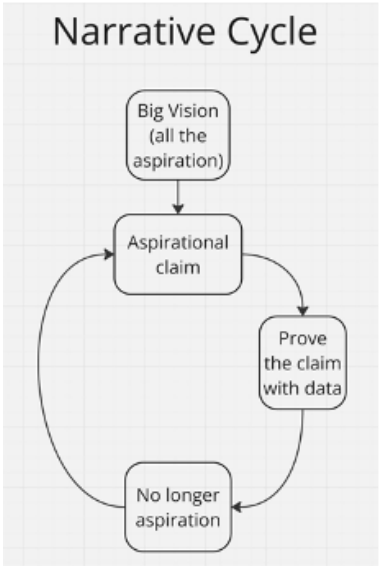
You sit down at your PowerPoint to create your pitch deck. Where do you even begin? How do you pitch? What font do you use? You've followed the advice from below, understand exactly where you sit on the technology development roadmap, and expect others to understand as well. What are the investors looking for? What will convince them to put their partners money to work in anticipation of returns?

Raising money is not a success metric

The first thing to remember, is that raising money is not necessarily a success metric. Raising equity capital is not a grant. You are selling a piece of your company to a group that may or may not truly understand what you are doing and may push you in directions you may or may not want to head. It's important to realize that your job is to raise money to push the technology forward. If you don't need $100M to move a platform to a lead, don't take it because money loses efficiency with scale. As the technology advances, the money need for a single asset changes. Make sure you understand how that impacts the value to your early investors so you can convince them to invest in you!



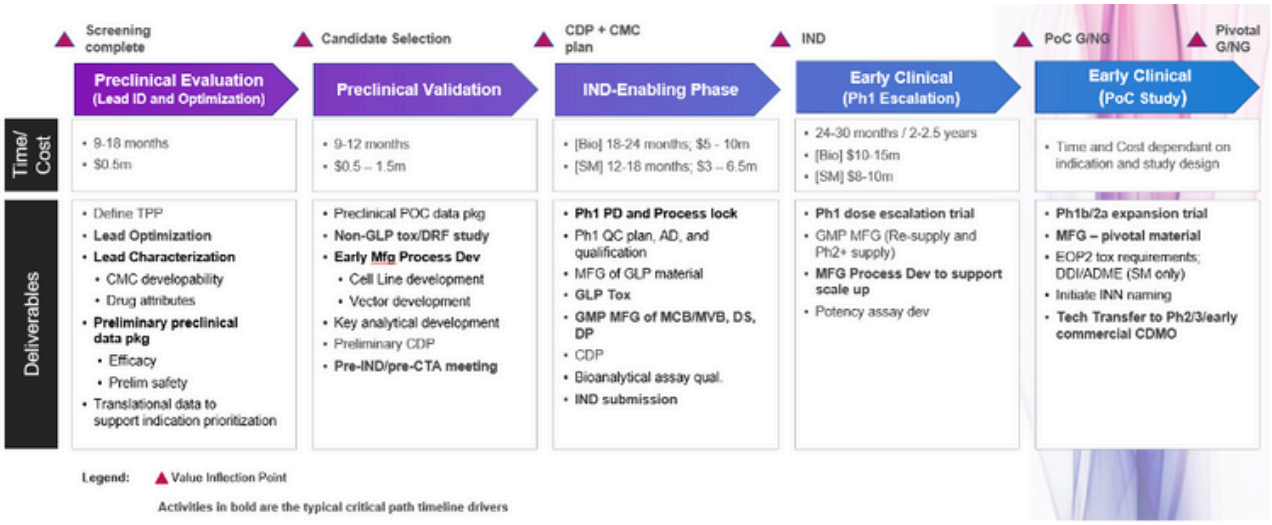
Balance aspiration with reality



Communicating your story is challenging. You'll be pressured to steer away from big vision thinking to providing a granular take on exactly what's going on. Or the opposite. The trick is to find a balance between communicating the big picture with the aspirations of the future. I've found it helpful both internally and externally to use a narrative cycle to drive communication of the company's achievements. These aspirations can align to the commercialization roadmap described below but can also be used to educate stakeholders about interim milestones that may not align directly to the roadmap. Educate about the challenge and highlight the achievement. Rinse and repeat.

Introduction to the commercialization roadmap

The commercialization of a biological drug product is a long, complex journey that blends science, regulation, manufacturing, and investment strategy. It's not just about having a great therapeutic idea—it's about mapping out each stage from preclinical research through to pivotal trials with a clear understanding of the activities, timelines, and costs involved. In the early phases, decisions made can have profound downstream effects on success, speed, and budget. A well-planned roadmap allows you to anticipate critical path drivers, identify value inflection points, and align scientific milestones with investor and regulatory expectations, ultimately increasing the probability of reaching patients efficiently. Two early areas where this alignment is most critical are preclinical evidence and CMC strategy.



Key recommendations for preclinical evidence enabling cell therapy translation

One of the earliest critical milestones in this journey is building a strong preclinical evidence package. For cell therapies in particular, establishing proof-of-concept is essential to overcoming regulatory objections and justifying first-in-human studies. To better align early research with regulatory expectations and reduce delays, global regulatory guidance highlights four key areas for establishing robust preclinical efficacy evidence (1):

1. Mechanism of action – Preclinical studies should demonstrate a plausible mechanism, including evidence that the therapy reaches the intended site of action, exhibits functional properties consistent with the proposed benefit, and elicits a relevant pharmacological response. Establishing these elements early can also help identify critical quality and potency markers.

2. Intervention parameters – Dosing schedules, delivery routes, and co-interventions used in preclinical studies should closely match those planned for clinical trials. Manufacturing processes should be stabilized early, as changes can render preclinical evidence irrelevant.

3. Clinically relevant models – Regulators emphasize using human-derived in vitro systems and disease-relevant in vivo models that replicate the intended clinical population. Use of immunosuppression, humanized models, or large animals may be necessary to achieve clinically meaningful dosing and delivery.

4. Meaningful outcomes – Preclinical endpoints should be directly translatable, such as surrogate biomarkers measurable in both preclinical and clinical studies. Studies should also address the persistence and durability of cell effects over clinically relevant timeframes.

Ultimately, these principles remind us that preclinical studies should not only demonstrate scientific novelty but also anticipate regulatory expectations and clinical realities. This alignment becomes even more important when considered alongside manufacturing strategy. Choices around dosing, delivery, and even model systems intersect with Chemistry, Manufacturing, and Controls (CMC), underscoring the need to plan CMC in parallel with preclinical studies.

Keeping CMC in mind during early development

In early-stage biologics development, overlooking CMC can derail progress. Because for biologics, "the process is the product," it's critical to build your manufacturing strategy from day one. Start by defining your Target Product Profile—what indication are you targeting? How will your therapy be administered and where? These foundational choices influence everything from preclinical design to manufacturing.

Next, choose your starting materials and vectors with care. Changes later—say, swapping cell lines or altering production reagents—can shift product attributes and trigger delays. Establish your production cell line from the same source you'd use for GMP manufacturing, ensuring consistency and traceability.

Your manufacturing process should mimic future clinical production wherever possible. Avoid techniques that won't translate into GMP and embrace analytics early. Monitor impurity profiles, potency, and residual contaminants. Early tracking supports comparability and strengthens regulatory submissions.

Resist the pull of "scientific fascinoma": the temptation to chase every experimental curiosity. Instead, ask whether this study serves a strategic or regulatory purpose. Build a study log, sketch out expected submission content, and keep every activity purposeful.

Select vendors wisely. Whether it's GLP labs or CDMOs, make sure they understand your technology, phase of development, and regulatory expectations. And remember, your team, internal or external, is core to success. Keep your team engaged and focused on corporate and project objectives. Early attention to CMC not only mitigates technical and regulatory risks but also positions your program for smoother progression to market.

For researchers and entrepreneurs ready to dive deeper into product development, the Capital BioVentures Ascent Program launches this Fall. For more information or to apply, email info@capitalbioventures.ca.

References

1. Jeffers MS, Xi CE, Bapuji R, Wotherspoon H, Kimmelman J, Bedford P, McIsaac DI, Lalu MM, Fergusson DA. Synthesizing regulatory guidance for demonstrating preclinical efficacy and translating promising cell therapies to early phase clinical trials: a scoping review. BMC Med 22, 487 (2024). https://doi.org/10.1186/s12916-024-03690-8