ThermoFisher SCIENTIFIC

GROWING YOUR BIOPHARMA

TEN QUESTIONS YOU'RE LIKELY TO FACE FROM INVESTORS—AND HOW TO RESPOND TO THEM

- API
- BIOLOGICS
- VIRAL VECTOR SERVICES
- EARLY & LATE PHASE DEVELOPMENT
- CLINICAL TRIAL SOLUTIONS
- LOGISTICS SERVICES
- COMMERCIAL MANUFACTURING

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INTRODUCTION

There are many important considerations to address in building your new drug program. Without a doubt, one of the most critical is funding to get you to your next milestone. As you grapple with funding issues, it can be useful to consider the point of view of a potential investor.

Why? Because investors are betting on you to manage development effectively and to move quickly. In assembling this guide, we asked investment veterans and biopharma executives what questions they ask (and have been asked) when both parties sit down together. Answer these questions well and your potential investors are that much more likely to support your company's vision.

We've also included a few "red flags" that you should avoid. Sometimes knowing what gives potential funders pause is just as important as knowing what they are likely to ask.

BUSINESS MODEL AND STRATEGY QUESTIONS

1. What's your hypothesis?

Why are they asking this question?

To secure funding for your new or emerging pharma company, it's critical that you articulate your core idea. If you can describe your company's strategy succinctly, investors will be able to make a clearer call on whether your hypothesis has merit. You may only have limited information at this point in your evolution, but you need to make your potential investors excited about investigating further.

Why is it relevant and important?

Investors are asking themselves two questions when they sit down with you: Is this a scientific bet worth making, and is there a market for it? The more specific you can be about your company's core idea and why it has a ready market, the more likely you are to find funders that can get behind the idea.

What's the best way to respond?

Be clear and concise. Write out a response and memorize it. If your hypothesis is that a particular antibody works in such a way that it may address a rare disease, for example, state this in as few words as possible.

Red Flag: No elevator pitch

Pharma and biotech investors regularly attend conferences where they hear pitches from dozens of aspiring companies in short, timed presentations. These are often referred to as "speed dating" sessions. If you are unable to articulate in one or two sentences why your treatment or technology is important and why it will matter, you will inspire doubt rather than confidence. Most investors aren't scientists, so if you can't simply and clearly articulate the benefits your company will bring to the world, they are likely to pass. Best advice? Nail your 15-second elevator speech before you approach investors.

2. What's your business strategy?

Why are they asking this question?

Venture capital and large pharma companies are accountable to their investors and shareholders and need to know how quickly they can see a return on their investment. They understand that most of their investments may not pan out, so they want to "fail fast" and move to the next promising company, if that's appropriate. They also will tend to fund one major milestone at a time for your company and want to know how you plan to get there.

Why is it relevant and important?

It's critical for any investor to know what their risks are over a prescribed time frame. If it will take $5 million to get to a yes/no answer based on your hypothesis, your investor will want to know how your plan of action breaks out and how quickly you can achieve results.

What's the best way to respond?

Expect to provide details about not just your current strategy but also your strategy over the next three to five years—including your strategy for an exit scenario. Investors find it helpful to hear strategy in bite-size pieces so they can get excited about a stepwise investment.

For example, you may plan to take an asset, move it to the clinic and sell it to a pharma company or other acquiring entity based on how it works in progressively larger human populations. On the other hand, you may plan to build a sustainable, integrated biopharma company and move to an IPO. Or you may plan to partner with a large pharma for a commercial launch. All three directions are legitimate—the point is to make sure that you clearly articulate your envisioned future.

Red Flag: Unrealistic timeline or budget

Although they may not be scientists themselves, most investors employ scientific experts and rely on consultants to provide them with counsel on candidates they are considering for funding. Confidence is essential to making a good impression, and investors realize that companies tend to present best-case scenarios for timing, but too much confidence about when you will be ready for the clinic can backfire.

Since you're likely to be in the company of business experts, it's best to stress what you know and admit what you don't. Budgets to get you to clinic that underestimate costs such as specialty ingredients, API formulation, viral vector production or manufacturing redundancies may get you into hot water. In the same way, spending too much up front on space, equipment and expensive staff can raise warning flags.

TEAM AND COMPETITION QUESTIONS

3. Who are your founders and management team?

Why are they asking this question?

Although most investors will have researched this topic in advance, they will want to hear in your own words about your track record, how successful the members of your leadership have been in their careers and how they are executing in your new company. It's unlikely that you're a public company with a board of directors at this stage, but investors still want to understand who your key opinion leaders (KOLs) are: Who is guiding you on your journey? How will they help you stay on course if your program has issues, or if it succeeds? Investors are also looking for consistency in what they hear. A lead investor from a large pharmaceutical company was once quoted as saying, "When you're interviewing, ask the same question of everyone. You want to make sure you're getting the same answer."

Why is it relevant and important?

It's essential that your investors believe you have the right team in place, including operational expertise, insightful scientific minds, entrepreneurial business leadership and, increasingly, CMC know-how. Depending on the investor's funding model, they may also want to determine your openness to working with them to pull together a more complete team, often complementing your existing scientific expertise with business acumen. This may include a CEO suggested by the investor or VC firm.

What's the best way to respond?

Stress the successes you and your team members have had in the past, because funders believe that success breeds more success. Also remember to point out the strength of your network, both scientific and financial. It increases an investor's comfort level to know that they won't be the sole party who might have to invest more money down the line because you haven't secured other investors.

Red Flag: Too much ego

As a successful academic researcher, you may have an MD-PhD and have served as principal investigator of a prestigious lab. You may have discovered a molecule and formed your own company. You may have headed up an entire therapeutic area at a large pharma company. In short, you may be accustomed to being seen as the smartest person in the room. A confident ego can inspire funders to see and share your vision, but an arrogant ego does not sit well with many venture funders, especially if you haven't been back on the bench in 15 years.

Investors are looking for scrappy entrepreneurs who know how to get things done and ideally have a track record of doing so. On the other hand, investors will turn a wary eye on start-ups that claim they can do everything themselves with little help from outside partners. New generations of biotech VC firms focus more on a collaborative, hand-in-hand company development and creation process. Reputable firms also are committed to helping you succeed because your therapy or medicine could save lives. Arrogance does not mix well with these settings. A good strategy is to focus on humility.

An investor would much prefer to hear, "I don't know the answer to that, but I know it's important, and here's how I'm thinking about it."

4. What's your competition?

Why are they asking this question?

Every funder wants reassurance that you're not simply copying an idea that's already in the market. And even if many competitors are working in your area, funders want to know what your advantage is in meeting these competitive headwinds. For example: What are the key differentiators about your product—durability, efficacy, dosing or something else? Why will your program be clinically meaningful in ways that others aren't?

Why is it relevant and important?

If ten companies have the same hypothesis and approach to a problem, it will dilute the value of your company if your competitors are further along. Investors want to make educated decisions.

What's the best way to respond?

Before you ask for funding, conduct the most thorough competitive audit you can. If someone is pursuing your hypothesis already, be prepared to describe what's unique about your approach. Take the time to conduct your due diligence and be prepared to defend your unique qualities, be they therapeutic modality, use (and redundant supply) of raw materials, CMC requirements, scale-up process or anything else that presents your company as a sound scientific and business investment.

Red Flag: "We have no competition"

Some innovators feel that their innovation is novel or a one-of-a-kind solution. Unless this therapy is a gene therapy and there is no other innovator working on this specific indication, there is competition.

INFRASTRUCTURE AND PARTNERSHIP QUESTIONS

5. How much are you investing in infrastructure?

Why are they asking this question?

Much of what you need to do in drug discovery research can now be outsourced, so if you plan to purchase a significant amount of equipment or hire large numbers of scientific staff to answer your business's core go/no-go question, that strategy is likely to be challenged by an investor.

Why is it relevant and important?

Investors are looking for new and emerging companies to run lean, without the infrastructure baggage of legacy organizations that may have spawned or inspired them. Just as the CRO industry grew up to complement—and in many cases replace—pharma companies' conducting their own trials, the CDMO industry offers a more financially and scientifically favorable model for new and emerging drug development and manufacturing. If you are making in-house infrastructure a priority beyond a base level of internal infrastructure and expertise to evaluate data and track progress, you may need to make a very strong case for the value this will deliver.

And don't forget: With a reputable CDMO, your team will act as an extension of your company, not as a "set it and forget it" capability that you never see.

What's the best way to respond?

Be honest and make a strong case for why you must maintain in-house control over certain elements of your discovery and development program. If you think you can take risk off the table by having an integrated CDMO as part of your strategy, consider this approach.

Red Flag: Too much focus on efficiency, not enough on innovation

Striving for higher efficiencies and eliminating costs in areas such as engineering probably have utility in large, mature organizations, but do not tend to drive value in new and emerging biotechs. If you come out of a large organization with a cost-saving culture and make claims about driving efficiency in a start-up, it will sound upside-down to investors. No one wants to be the VP of CMC who has to approach their board and say, "I thought I was going to save $500,000, but now the program is six months behind."

6. What's your development and manufacturing strategy, and who are your partners?

Why are they asking this question?

Especially in high-complexity areas like cell and gene therapy, funders will want to understand your strategy in clinical development based on the high cash burn rate and the supply chain capacity you'll need in order to produce a reliable supply of product. CMC skills are increasingly important as well, because most investors understand that excellent clinical data on its own is not sufficient to scale manufacturing of your product and get it approved by regulatory agencies. Characterizing and understanding your process and product quality are essential.

Investors know that most small companies have neither the size nor the resources to develop and manufacture their own drugs, so they also need to know what your outsourcing strategy is. Large-molecule drugs are more difficult and expensive to manufacture than small, so having a strong manufacturing relationship with a respected CDMO that brings a strong track record can make a difference with funders. It stands to reason that investors will be more likely to approve of CDMOs with which they've had positive relationships in the past.

Why is it relevant and important?

Funders want to know that you will be following a proven path to a commercially realized product. New and emerging companies need credible, high-quality partners that will not only perform development and manufacturing work, but also help interpret the results of pathology reports and DMPK studies. Good partners also anticipate the downstream impacts from upstream development and manufacturing decisions and can help you present your data package to regulatory bodies like the FDA as they want to see it.

What's the best way to respond?

Do the math. If you'll need 15,000 square feet of manufacturing capacity to make enough of your product to realize its revenue potential, make sure that you've checked your numbers. Investors want you to demonstrate that you understand your supply chain requirements, regardless of how you plan to address them: internal build, outsourcing or a hybrid strategy.

Choose your CDMO partners carefully based on a set of key factors affecting performance outcomes. Be ready to share with investors why you've made your decisions, based on:

- **Track record**
  - Has the company taken a product and commercialized it?
- **Quality processes**
  - Is there any risk associated with choosing this company?
  - Might it have inspection issues based on past programs?
  - Has it previously had FDA actions?
  - How has it performed in pre-license inspections?
- **Reputation**
  - Is it a recognized company or relatively unknown?

Think about the best way to convey that you're focused on the most efficient path to the next milestone, but that you're also looking to make key foundational investments that will set you up for success faster in the next phase. This is a difficult balance to strike, so work out the response with your team and your partners before pitching to an investor.

Red Flag: Overclaiming or "getting out over your skis"

Having confidence and conviction in settled science is good. Conveying the same level of confidence in areas where you're out of your depth is not. For example, if you haven't really built out your CMC strategy, you're better off acknowledging it than trying to talk your way through it. If you start to claim that "We're really going to think out of the box on CMC and do this differently" or "Yes, we can make the product; don't worry about that," expect some raised eyebrows. VC investors don't expect a molecular biologist to have all the answers. The important thing is to be capable of engaging with other people who do have this expertise.

7. Are you working with one CDMO or many?

Why are they asking this question?

Every investor wants to future-proof their investment as much as possible. If your company has contracts with multiple CMOs or CDMOs for drug substance, drug product, clinical trial and other services, it's likely that each new team will be restarting at each step.

Why is it relevant and important?

Investors would prefer that you remain focused on reaching the next milestone rather than on educating new vendors and managing multiple relationships, especially if new and existing vendors compete with each other.

What's the best way to respond?

Keep in mind that as a new and emerging biopharma, you have to move fast with a limited amount of budget and resources to get to first-in-human trials followed by proof of concept. If you currently have multiple partners working on your program, this can be seen as adding risk to your program, so put together a strong argument for why this isn't the case. You might also consider collecting proposals from larger, integrated CDMOs with which you'll spend less time negotiating terms, overseeing day-to-day details or transferring processes between partners.

Red Flag: Scattered focus

Investors would prefer that you stay focused on science and development rather than acting as program manager for multiple CDMO relationships. Partnering with multiple CDMOs potentially adds risks and delays as well. If your attention appears spread across too many outsourced partners, expect to be asked why you haven't considered a larger, integrated partner that can handle program oversight on your behalf.

8. How are you thinking about the data package you'll be submitting to regulators?

Why are they asking this question?

Although it may seem to be a downstream consideration, many cell and gene therapy start-ups are surprised by how quickly their Phase I results lead to requests from the FDA to submit for accelerated designations. With the FDA predicting that it will be approving 10 to 20 cell and gene therapy products a year by 2025,[1] it's important to think about the regulatory submission earlier.

Why is it relevant and important?

A strong, high-quality data package is key to a fast, successful regulatory submission. When you work with a single integrated CDMO, information and learnings are shared across the teams focused on your program. If your asset is split among multiple CMOs or CDMOs, investors may have to invest time and money in helping to pull the data package together. Investors do not consider this a good use of their precious resources.

What's the best way to respond?

Before you pitch to an investor, talk to some experts about what the best route to a clean, integrated data package will be for you. If working with a single integrated CDMO is part of the solution, incorporate this into your planning process.

RISK MITIGATION AND PROBLEM-SOLVING QUESTIONS

9. What will you do when problems occur?

Why are they asking this question?

Investors are unlikely to ask you about responding to highly specific problems if you are pre-IND. With a question like this, the investors are likely trying to determine how you think about approaching a problem or creating a Plan B, both of which are important considerations to establish confidence. Given the current global pandemic, new considerations also arise. For example, if you can't visit your CDMO in Europe due to travel restrictions, how will you ensure that quality stays high enough?

Why is it relevant and important?

Problems and challenges are part of the nature of the biopharma business, so investors want to get inside your thought process. From their perspective, they also want to avoid getting pulled in to contribute more money (and even more important, more time) to get your program across the finish line.

What's the best way to respond?

Think in advance about different categories of problems your program might face and how you'd work to mitigate them. For example, you might consider building in manufacturing redundancy if manufacturing falls on the critical path for your R&D program. You might also consider a manufacturing partner that has a proven ability to identify the root cause of manufacturing process deviations and a broad network that can be called on to get you back on track. When the question comes, it's ideal to be able to say, "The CDMO we want to work with is the best fit for our product. It has the ability to expand capacity. It can leverage a broad network. We can leverage its resources here for distribution. It has strong regulatory experience. And given all this, our CDMO can help us manage any problem that may arise."

10. How are you positioning yourself for the next step in your program?

Why are they asking this question?

Drug development presents many options for a young biopharma company, including the partners you've chosen and the decisions they may lead you to make. For example, what is the development route you'll choose for your API, if you are pursuing a small-molecule drug?

Why is it relevant and important?

Investors want to know that you've taken off the table every risk that you can control for today. In the API example above, you may choose purity over yield in your formulation instead of fully characterizing your molecule and investigating formulation and solubility. This can be a shortsighted decision in terms of next steps if it compromises downstream bioavailability and increases cost. If your CDMO combines drug substance and drug product capabilities under the same roof, however, this is much less likely to happen.

What's the best way to respond?

Make sure that you have explored the downstream implications of your development decisions as well as the foresight of your potential partners. Investors will be impressed that you have thought through these issues before you sit down together.

CONCLUSION

Obviously, there is a lot to think about when it comes to making a case to investors. Get the answers to the basic questions before you pitch, and you'll have a greater chance of succeeding:

- What is my concept?
- What could it be good for?
- How much will it cost to get to an answer?
- How can I make developing and manufacturing my drug as fast and successful as possible?

Also ensure that you are working with a CDMO partner that has the right skills and experience, gives you the right level of attention and project management, integrates as many steps of development and manufacturing as possible, is clear about how you'll work together, and can help you think strategically about commercial supply.

Some investors will be more willing to give you a higher valuation and provide more capital if you can show you've thought seriously about manufacturing issues. Once you have, you'll be a lot closer to the funding and positive patient impact you envision.

"When you're interviewing, ask the same question to everyone. You want to make sure you're getting the same answer."

— Lead investor, large pharmaceutical company

Funding Flavors

Seed money:
Friends and family money to get your idea off the ground

Series A:
VC funding to launch your company, ideally to get you to the discovery finish line and into the clinic

Series B:
VC funding to move your programs through clinical testing for safety, efficacy and large-scale patient efficacy

Mezzanine funding:
An additional level of VC funding to make up for a budget shortfall or to fund extra necessary research to reach your goal

IPO:
Public funding to bring your company to the world of investors at large

[1] FDA, "Statement from FDA Commissioner Scott Gottlieb, M.D. and Peter Marks, M.D., Ph.D., Director of the Center for Biologics Evaluation and Research on new policies to advance development of safe and effective cell and gene therapies," White Oak, MD, January 15, 2019.

ABOUT US

With unwavering commitment to service, science and process engineering, Thermo Fisher Scientific is powered by people with an exceptional commitment to quality, deeply instilled ethics of personal responsibility and unrivaled expertise.

Thermo Fisher Scientific provides industry-leading pharma services solutions for drug development, clinical trial logistics and commercial manufacturing to customers through our Patheon brand. With more than 55 locations around the world, we provide integrated, end-to-end capabilities across all phases of development, including API, biologics, viral vectors, cGMP plasmids, formulation, clinical trials solutions, logistics services, and commercial manufacturing and packaging. We give pharma and biotech companies of all sizes instant access to a global network of facilities and technical experts across the Americas, Europe, Asia and Australia.

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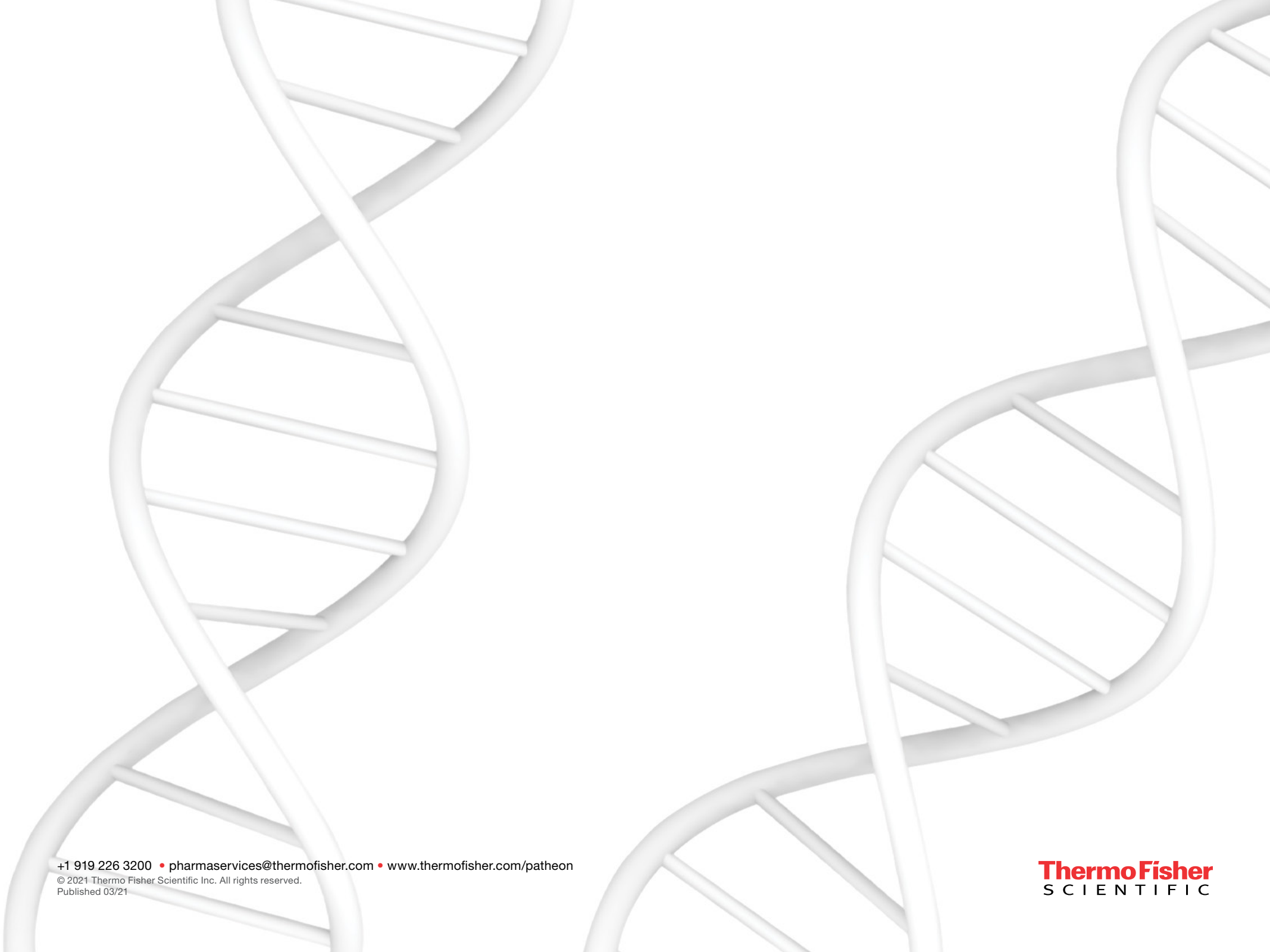

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