February 2025

Advancing mRNA: Executive Insights and Future Directions

Conversations with 27 Senior Pharmaceutical & Biotechnology Leaders

Alliance for mRNA Medicines

Sponsored by: danaher

About The Study

Research Overview

This research initiative, conducted by the Alliance for mRNA Medicines (AMM) in collaboration with UserCue's AI-driven research platform, involved interviews with 27 senior executives—18 of those from companies over $5B in market cap—to gain real-world insights into the evolving mRNA landscape. Designed to capture real-world insights on the evolving mRNA landscape—spanning investment strategies to commercial readiness—the study arises at a pivotal moment when mRNA's vaccine success highlights potential in oncology, rare diseases, and beyond, while questions remain around scalability, regulation, and public perception. Findings from this targeted study will help inform AMM's 2025 initiatives by guiding resources, research, and advocacy efforts to address emerging challenges—with a more extensive Phase 2 data project planned to further advance AMM's mission of supporting mRNA-based technologies in healthcare.

Objectives

- **Understand mRNA's role in future therapeutics** by capturing executive perspectives on its importance and potential.
- **Assess company involvement and investment** in mRNA, identifying current commitments and strategic directions.
- **Pinpoint key challenges and opportunities** within mRNA development and commercialization.
- **Evaluate industry needs** for advocacy, education, and regulatory support to advance mRNA adoption.
- **Examine the impact of misinformation** and public perception on mRNA's trajectory.

Target Population & Methods

This study focused on senior executives (mostly C-Suite, SVP & above) from pharmaceutical and mRNA-focused biotechnology firms, all directly involved in mRNA development or strategy.

We employed a dual quantitative/qualitative approach, leveraging **UserCue's advanced AI research platform** for dynamic, in-depth interviews:

- **Quantitative Data Collection:** Structured questionnaires gathered metrics on organizational mRNA involvement, investment interest, and strategic priorities.
- **Qualitative Insights:** Open-ended discussions allowed executives to elaborate on delivery mechanisms, manufacturing scalability, regulatory hurdles, and the influence of public perception and misinformation on mRNA progress.

Interviews with 27 executives across Pharma and Biotech about the current and future state of mRNA technology

Participant Mix

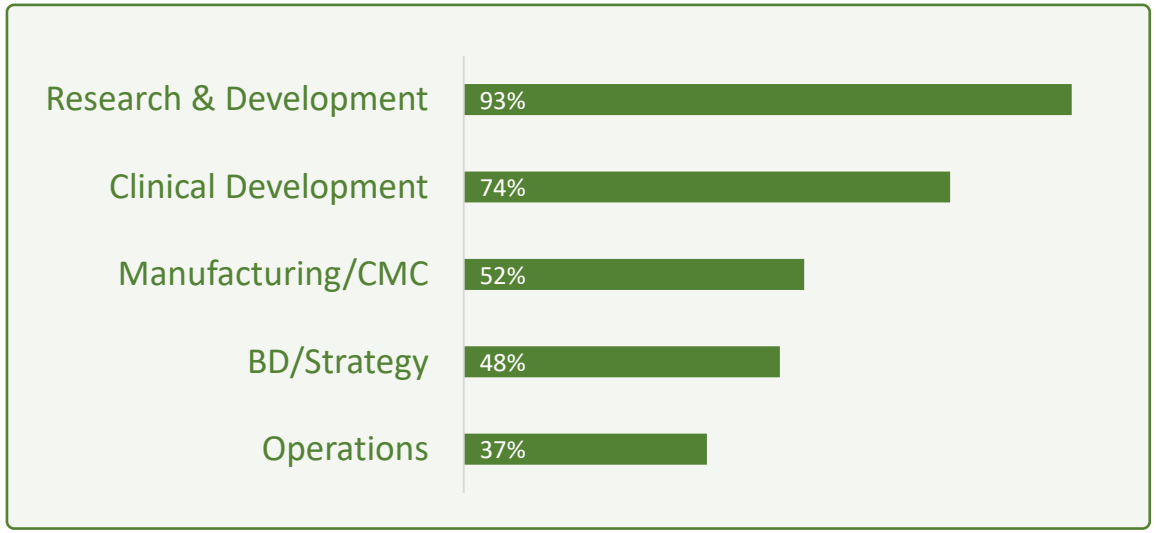
27 Senior Executives

Pharma & Biotech Companies*

By Market Cap

>$10B	$5-10B	<$5B
N=12	N=6	N=9

Functions Directly Responsible For



Experience

480+ Years

of Total Pharma & Biotech Experience

270+ Years

of Total mRNA Experience & Leadership

*For reporting purposes, interview participants are categorized into executives at Large Pharma (>$5B market cap companies) and Smaller Biotech (<$5B market cap companies)

Participant Insights

- Executives cover a range of titles, including **41% who work in the C-Suite**, **26% who are Presidents or Senior Vice Presidents**, **26% who are Vice Presidents**, and **7% who hold Senior Director roles**
- **55% of executives have over 10 years of experience** with mRNA technologies
- **59% of executives dedicate 50% or more of their professional time** to mRNA-related activities
- All executives, representing **100% of participants, are directly involved in their company's decision-making** regarding mRNA technologies

Alliance for mRNA Medicines

THIS STUDY IS

PRESENTED IN PARTNERSHIP WITH

USERCUE

Alliance for mRNA Medicines

DATA COLLECTED DECEMBER 2024 – JANUARY 2025

Topline Summary

Executive Summary

- **mRNA is Emerging as a Primary Therapeutic Modality:** 93% of executives consider mRNA “extremely” or “very” important for future therapeutic development, reflecting a shift from viewing mRNA solely as a vaccine platform to recognizing it as a versatile treatment technology.

- **Expanding Beyond Vaccines:** Over 50% of executives identify opportunities in **cancer treatments and personalized therapies**—including targeted vaccines for lung and pancreatic cancers—and in **infectious diseases beyond vaccines**, where mRNA could provide prophylactic or therapeutic proteins for conditions like HIV, hepatitis, or antimicrobial resistance. There is also strong interest in **gene therapy/editing, rare diseases, autoimmune disorders, and neurological applications** as additional growth areas *(all mentioned as future areas with large potential by >20% of executives).*

- **High Innovation Perception:** Over half (56%) identify mRNA as the most innovative current therapy in the pharma/biotech industry right now, citing its potential to transform oncology, rare diseases, and beyond.

- **Significant Industry Investment:** 93% of executives agree that large pharma companies are making significant investments in mRNA and view its future with considerable interest, driven by new technology advancements (32%), proven success following COVID-19 (28%), and strong market growth potential (24%).

- **Robust Funding and Confidence:** 78% of organizations report being “extremely” or “very” interested in investing in mRNA, driven by mRNA’s breakthrough potential to meet unmet needs (43%), strategic innovation leadership (33%), the potential for mRNA to provide solutions that address current technological limitations (29%), and improved drug safety and efficacy (19%).

Executive Summary (cont'd)

- **Broad Advantages of mRNA:** The top advantages cited by executives include **rapid development and production (52%)**, **innovative mechanisms of action (37%)**, **targeted precision (37%)**, and **versatility across therapeutic areas (37%)**, with **platform-based adaptability (15%)** also highlighted. These features promise faster timelines, minimized off-target effects, and broad applicability, reinforcing mRNA's appeal across multiple disease indications.

- **Growing Pipeline Across Company Sizes:**

    - **Large Pharma:** Pursues expansive external pipelines ("shots on net"), maintains a strong focus on M&A, and leverages robust infrastructure to build a diversified mRNA portfolio. Early engagement with payers and strategic risk mitigation—particularly in light of reduced COVID-19/flu vaccine sales—ensure a broad commercialization strategy spanning multiple therapeutic areas.

    - **Smaller Biotech:** Centers on specialized modalities (e.g., gene editing, cell therapy) and focuses on high-impact diseases such as oncology and rare disorders. Commercial launches are largely partnership-driven, with co-development agreements and early reimbursement discussions helping these smaller firms scale operations and bring mRNA solutions to market.

- **Vaccines Still Dominate:** Vaccines remain the primary mRNA use case (today), with both generalized and personalized versions leading current development; however, despite over 50% of executives citing cancer and infectious diseases as the top future opportunities, only 30% of organizations are actively innovating in these areas today. There is an urgency to further invest in these areas—seen as the largest future use cases for mRNA technologies.

Executive Summary (cont'd)

- **Key Barriers to mRNA Adoption:** Major challenges include **manufacturing complexity and scalability (67%)**, **delivery/targeting (37%)**, and **regulatory hurdles (37%)**, compounded by safety/efficacy concerns, high production costs, stability and storage demands, and public skepticism—all of which must be addressed to ensure widespread implementation of mRNA therapeutics.

- **Regulatory Uncertainty Persists:** While 63% of executives have encountered or anticipate hurdles tied to mRNA's novelty, long-term safety/toxicity, and manufacturing consistency, a notable 37% perceive minimal barriers—citing established frameworks and confidence gained from mRNA vaccines as potential facilitators for future approvals.

- **Prioritizing Key Investments to Advance mRNA:** Executives emphasize the need for expanded R&D (44%) to extend mRNA's reach beyond vaccines, improved manufacturing and scalability (26%), advanced delivery systems (19%), and clinical validation (15%) as top priorities for industry-wide progress. Additional focus areas include stability, storage, education, and streamlined regulatory pathways to fully realize mRNA's therapeutic potential.

- **Misinformation's Limited but Varied Impact:** While 78% of executives report no **direct impact** from misinformation on their own mRNA efforts, 33% indicate broader industry impact due to skepticism and misinformation, fueling public and investor hesitancy. 26% specifically mention that COVID-19 vaccines have impacted public skepticism. Additionally, 15% discuss how misinformation has impacted mRNA vaccines more than therapeutics, where urgent unmet needs and more informed patient populations help maintain momentum.

- **Role of Advocacy Organizations:** 74% believe the Alliance for mRNA Medicines should combat misinformation, suggesting targeted campaigns, close collaboration with regulatory bodies, and transparent scientific data to build trust.

Detailed Findings

mRNA technology considered top innovative therapy in pharma / biotech today

What do you see as the most innovative therapy, modality, or technology in the pharma/biotech industry right now? (N=27, Open End, % = % of participants who mentioned each theme)

1. **mRNA Technologies** (56%)
   Beyond COVID-19 vaccines, mRNA is advancing in cancer, gene editing, rare diseases, and autoimmune conditions. Its versatility, scalability, and adaptability make it a promising tool for personalized medicine.
2. **Gene Therapy and Editing as Curative Solutions** (30%)
   Advances in in vivo delivery and technologies like CRISPR/Cas9, base, and prime editing offer precise, one-time treatments without causing permanent DNA damage in specific instances.
3. **Advancements in Cell Therapies (CAR-T and Allo-CAR-T)** (22%)
   Significant progress in personalized cancer treatment, with therapies tailored to individual patient needs.
4. **Personalized Therapies Shaping the Future** (19%)
   Customizable treatments based on genetic profiles and disease specifics, particularly for cancer and rare disorders.
5. **Expanding Potential of RNA Therapies** (19%)
   siRNA and miRNA therapies are emerging as promising options for novel treatments beyond mRNA.

Other: Antibody-Drug Conjugates (ADC) Transforming Cancer Treatment, AI Revolutionizing Drug Discovery and Personalization, Non-Viral Nucleic Acid Delivery Enhancing Safety, Modular Manufacturing Platforms Filling Therapeutic Gaps

*"**The most innovative therapy in pharma industry is mRNA technology**, particularly its application beyond covid vaccines. Companies are exploring its use for personalized cancer rare disease and even autoimmune conditions. The ability to program mRNA to produce specific proteins in the body opens **unprecedented opportunities for precision medicine**."*
(Interview #17, Large Pharma executive)

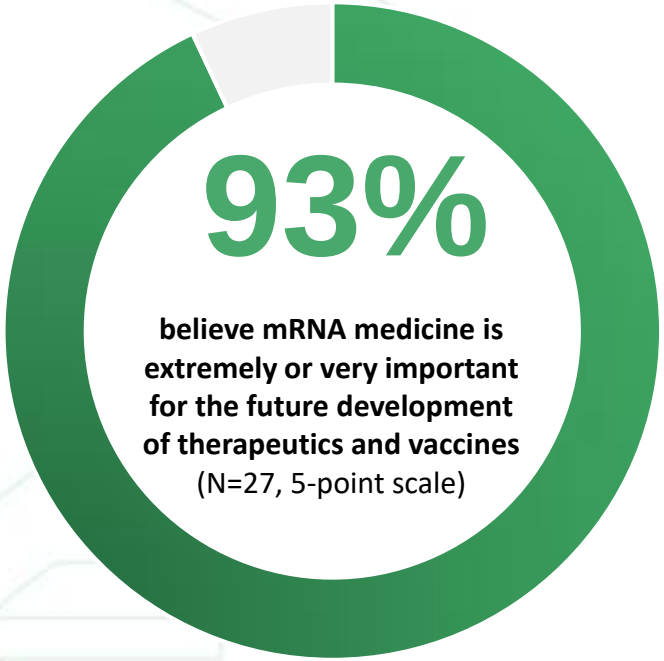
*"**The success of Covid-19 mRNA-based vaccines** served as a prototype for RSV and influenza to come."*
(Interview #2, Large Pharma executive)

"In vivo mRNA delivery for gene editing. There are thousands of diseases that could be amenable to gene editing using mRNA delivery of CRISPR-Cas9, base editing, or prime editing."
(Interview #5, Smaller Biotech executive)

"mRNA technology has the potential to significantly accelerate drug development and reduce costs with more effective therapies."

(Interview #3, Large Pharma executive)

mRNA viewed as important due to versatility, adaptability, efficacy, commercial impact, and potential to treat previously untreatable diseases



believe mRNA medicine is extremely or very important for the future development of therapeutics and vaccines
(N=27, 5-point scale)

89% (16/18) of executives at **Large Pharma companies specifically** believe mRNA medicine is **extremely or very important** for the future development of therapeutics and vaccines.

Please elaborate on why you believe mRNA medicine is extremely/very important
(n=25, those who indicated 'extremely' or 'very' important, % = % of participants who mentioned each theme)

- ✓ **Versatility** (60%): Broad applications in vaccines, cancer, genetic disorders, personalized medicine, and more.
- ✓ **Adaptability** (32%): Rapid platform development proven during COVID-19.
- ✓ **Proven Efficacy** (32%): Demonstrated safety and success of mRNA medicines.
- ✓ **Commercial Impact** (20%): mRNA has attracted significant investment as a key driver of innovation.
- ✓ **Transformative Potential** (20%): mRNA is unlocking treatments for previously untreatable diseases.

Please elaborate on why you believe mRNA medicine is somewhat important
(n=2, those who indicated 'somewhat' important)

- mRNA considered on par with other modalities (1 mention)
- mRNA seen as still new and limited in terms of therapeutic applications (1 mention)

No one indicated they believe mRNA medicine is slightly or not at all important.

*"mRNA medicine is important because of its **versatility**; it can be applied to a vaccine development, therapeutic protein gene editing via CRISPR, vaccines, etc."*
(Interview #20, Large Pharma executive)

*"Rapid development of COVID-19 vaccines demonstrated mRNA platforms can **adapt** to emerging pathogens within weeks."*
(Interview #17, Large Pharma executive)

*"mRNA has been proven to be **safe** in a vaccine **tested** in billions of people."*
(Interview #5, Smaller Biotech executive)

*"These drugs will have **commercial implications**... This is a multi billion-dollar industry."*
(Interview #7, Smaller Biotech executive)

*"We are venturing in **new areas** where traditional medicines have failed."*
(Interview #6, Large Pharma executive)

mRNA’s advantages include speed, innovation, precision, versatility, and adaptability

Key Benefits/Advantages of mRNA medicine

In your opinion, what are the key benefits or advantages of mRNA medicine?
(N=27, Open End, % = % of participants who mentioned each theme)

Rapid Development & Production (52%)

mRNA medicines enable significantly accelerated drug development and production timelines. Their rapid design and testing processes lead to quicker clinical trials, reduced costs, and faster time-to-market, which is crucial for addressing urgent health needs and emerging diseases.

Innovative MOA (37%)

Utilizing the body's own cells to produce therapeutic proteins, mRNA medicines reduce the need for external protein production and potentially lower toxicity. This innovative mechanism harnesses natural biological processes, leading to effective treatments with fewer side effects compared to some traditional therapies.

Targeted Precision (37%)

mRNA medicines offer high specificity by targeting particular proteins or cells, minimizing off-target effects and enhancing therapeutic efficacy. This precision enables personalized medicine approaches, allowing treatments to be tailored to individual patient genetics and disease profiles for improved outcomes.

Versatility Across Therapeutic Areas (37%)

mRNA technology is highly adaptable, allowing for therapeutic applications across a wide range of diseases and conditions. Its flexibility extends to vaccines, oncology, genetic disorders, metabolic diseases, autoimmune conditions, and regenerative medicine, making it a valuable tool for addressing diverse and unmet medical needs.

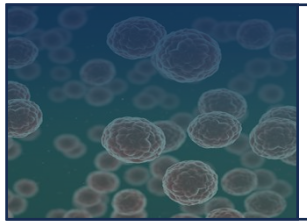
Platform-Based Modularity and Adaptability (15%)

mRNA technology operates on a platform basis, wherein modifying the mRNA sequence can adapt the therapy to produce different proteins for various indications without significant changes to the development and manufacturing processes. This modularity enhances efficiency and allows for rapid response to new therapeutic targets or emerging health threats.

mRNA unlocks new frontiers across a wide range of Tx areas, including oncology, infectious disease, oncology, gene therapy, and immunology

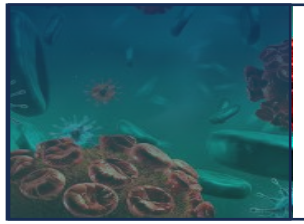
What are the potential emerging opportunities you foresee for mRNA medicine beyond its current use in vaccines?
(N=27, Open End, % = % of participants who mentioned each theme)

Tier 1 (>50%)



Cancer Treatments & Personalized Therapies

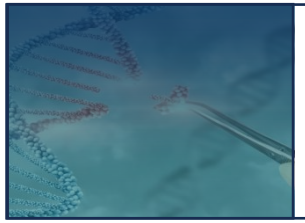
mRNA enables personalized cancer vaccines and targeted therapies for cancers like lung and pancreatic cancer by enhancing immune response.



Infectious Diseases Beyond Vaccines

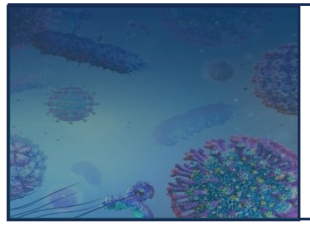
mRNA therapies to provide prophylactic or therapeutic proteins for diseases like HIV, hepatitis, or antimicrobial resistance.

Tier 2 (>20%)



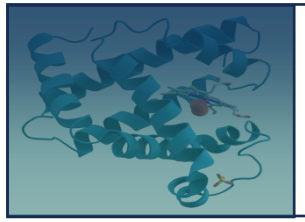
Cell Therapy & Gene Editing

mRNA supports gene and protein replacement therapies, offering potential cures for inherited diseases.



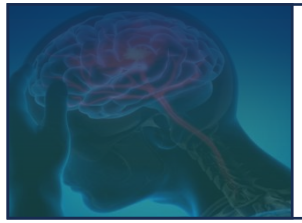
Autoimmune Diseases

Modulating immune responses for conditions like multiple sclerosis, rheumatoid arthritis, and type 1 diabetes



Rare Disease

mRNA therapies for protein replacement or correction of defective genes in rare diseases like cystic fibrosis and muscular dystrophy.



Neurological Disorders

mRNA therapies show promise for neurodegenerative diseases like Alzheimer's, Parkinson's, and spinal muscular atrophy.

Other mentioned applications for mRNA therapies include veterinary medicine, diagnostics, allergy treatments, and synthetic food development. Key modalities highlighted include metabolic diseases, such as mRNA-based therapies targeting insulin production in diabetes, cholesterol modulation, and GLP-1 production. Cardiovascular diseases also surfaced, focusing on heart repair, diabetes management, and treatments for conditions like CHF and ESRD. Protein deficiency disorders were noted for their potential in protein replacement therapies, addressing conditions like hemophilia and storage diseases.

"These drugs will have commercial implications...

This is a multi billion-dollar industry."

(Interview #7, Smaller Biotech executive)

Large Pharma/Biotech significantly interested in mRNA – driven by COVID-19 vaccine success, mRNA tech advantages, Tx applications, and more

Please indicate whether you primarily agree or disagree with the following statement.
(N=27, Close End, Answer Options: 'I agree with this statement', 'I disagree with this statement)

93%

Agree with the statement:

"Many large pharma/biotech companies are making significant investments in mRNA and have expressed significant interest in the future of the industry."

93%

Agree with the statement:

"Large pharma/biotech is shifting, or will be shifting, more focus and resources towards mRNA, especially therapeutic modalities."

You mentioned you agree with this statement. Please expand upon why. (n=25, those who agree with statements, Open End, % = % of participants who mentioned each theme, responses to both statements combined below.)

- ✓ **Technological Advantages** (32%)**:** mRNA offers scalability, rapid development, and cost-efficiency
- ✓ **Proven Success and Investment Growth** (28%)**:** COVID-19 validated mRNA's potential, driving increased confidence and funding.
- ✓ **Broad Therapeutic Potential** (28%)**:** Addresses unmet needs in oncology, autoimmune, chronic, and rare diseases.
- ✓ **High Market Growth Potential** (24%)**:** Rising R&D budgets, collaborations, and strong market prospects.
- ✓ **Pandemic Preparedness & Global Health** (12%)**:** mRNA's role in tackling emerging infectious diseases.
- ✓ **Shift From Small Molecules toward mRNA Therapies** (12%)**:** Expected to replace small molecules in the long term.

***"High efficacy rate** in clinical trials, exceeding 90%."*
(Interview #2, Large Pharma executive)

*"I see a huge increase in investments and mRNA wherein mRNA technology is being used for **treating oncology and rare diseases**. The investment in rare disease and cancer treatment is increasing across all top ten pharma companies."*
(Interview #26, Large Pharma executive)

***"The R&D budget is growing across the board**... Increase of 15% per year."*
(Interview #7, Smaller Biotech executive)

***"Small molecules have reached a limit and have diminished returns....** mRNA technology hits the sweet spot between all this with much potential, few failures, and great safety."*
(Interview #14, Large Pharma executive))

"I see a huge increase in investments and mRNA wherein mRNA technology is being used for treating oncology and rare diseases. The investment in rare disease and cancer treatment is increasing across all top ten pharma companies."

(Interview #26, Large Pharma executive)

"At least 8 of the top 10 pharma companies in the world are working on mRNAs as therapeutics."

(Interview #4, Smaller Biotech executive)

Executives' personal experience in mRNA includes expanding applications beyond vaccines and clinical development of mRNA technologies

58%

Average percent of professional time and responsibilities involving mRNA; no differences by large and small companies

Large Pharma

1,173

Average number of employees employed in roles involved mRNA in their organization

Smaller Biotech

143

Average number of employees employed in roles involved mRNA in their organization

Shifting focus to mRNA technologies, what is your personal involvement, interest, and experience in mRNA?
(N=27, Open End, % = % of participants who mentioned each theme)

Executives' mRNA experience includes…

Strategizing Expanding mRNA to Therapeutics Beyond Vaccines (41%)

Including dementia, cardiovascular diseases, rare genetic disorders, neuromuscular diseases, wound healing, and skin conditions

*"I have worked with my company for the last fifteen years on helping to try to identify programs that could be of interest **expanding the window around mRNA technologies beyond vaccines."***
(Interview #15, Smaller Biotech executive)

Working on Solutions for mRNA Delivery (15%)

Advancing delivery mechanisms (e.g., novel lipid nanoparticles) to improve efficacy and safety of mRNA therapies

*"I've been involved in conducting research and on identifying potential opportunities for commercializing mRNA for cancer treatment across the population including **the advanced delivery systems for such technologies."***
(Interview #26, Large Pharma executive)

Background in Clinical Development of mRNA Technologies (33%)

Including leading numerous clinical studies, overseeing development programs, and contributing to both therapeutic and vaccine development

***"I have developed mRNAs that have been used in about 35 clinical studies.** We have used them to produce peptides and proteins that act as agonists."*
(Interview #4, Smaller Biotech executive)

Advancing mRNA Technologies Specifically in Oncology (15%)

Specifically developing mRNA-based cancer treatments, exploring immunotherapies, and advancing programs in immuno-oncology

*"The experience of using mRNA post-COVID has forced many companies to look forward to how this can be used… **In cancer, especially, presently immuno-oncology is the most important area."***
(Interview #25, Smaller Biotech executive)

Large Pharma: making sweeping strategic investments in mRNA across therapeutic areas

How specifically is your organization involved in mRNA? What specific mRNA modalities or use cases is your organization involved in? In what ways is your organization involved in capital, partnership, or M&A activities related to mRNA?
(n=18, executives at large pharma companies, Open End)

Broad strategic investments

Investment spans R&D, manufacturing, and technology across exploratory research, early-stage development, and preclinical trials to drive innovation across various stages of the pipeline.

Across diverse mRNA applications

Engagement in vaccines, therapeutics, gene editing, epigenetic testing, cell therapy, and diagnostics demonstrates the versatility of mRNA technology.

Among various therapeutic areas

Prioritization of oncology, infectious diseases, neurology (e.g., Alzheimer's, Parkinson's), rare genetic disorders, cardiovascular diseases, immunology, gene editing, etc.

Focus on advanced technology

Strategic focus on advanced delivery mechanisms and platforms, such as lipid nanoparticles and ex vivo applications, to enhance efficacy and expand mRNA applications.

Strategic collaborations and acquisitions

Strategic collaborations with biotech startups, academics, and industry players and active pursuit of acquisitions to secure mRNA platforms, intellectual property, or expertise.

"There are already a lot of applications in infectious disease vaccine development and rare diseases... the current portfolio consists of one vaccine already available on the market with more vaccines in regulatory steps and lot of researchers are devoted to this."
(Interview #24, Large Pharma executive)

"Working on multiple aspects of mRNA modalities and use cases such as gene editing, protein replacement, and personalized therapy."
(Interview #27, Large Pharma executive)

"Organic growth as well as select BD deals in technology access and IP rights."
(Interview #9, Large Pharma executive)

"Aim to acquire one to two mRNA-based platforms in the next five years."
(Interview #19, Large Pharma executive)

"For M&A we are looking to purchase small firms with IP/patent - approx. $100 M purchase."
(Interview #6, Large Pharma executive)

Smaller Biotech: making specific strategic investments in mRNA across specific therapeutic areas

How specifically is your organization involved in mRNA? What specific mRNA modalities or use cases is your organization involved in? In what ways is your organization involved in capital, partnership, or M&A activities related to mRNA?
(n=8, executives at smaller biotech companies, Open End)

Specific investments

Such as clinical development, delivery and modification technologies, vaccine development, etc.

Specific therapeutic area focus

Such as oncology, cardiovascular, infectious diseases, rare disease and protein deficiency

Specialized and focused modalities and technologies

Across vaccines, personalized therapies, gene editing, and cell therapy.

Niche expertise

Such as immuno-deficiency prevention programs or diagnostics

Collaborations & raising capital

Raising capital through public and private means, collaborations with larger pharmaceutical companies and investors, considering M&A to strengthen mRNA capabilities

*"**We are in randomized phase 2 studies** for 8 different therapeutic indications. We are spending just under $250M/y on clinical development."*
(Interview #4, Smaller Biotech executive)

*"We are involved in making therapeutics **using this technology in cancer**. Close to $300-350 million dollars yearly."*
(Interview #25, Smaller Biotech executive)

*"I work with two organizations, one that is **developing mRNA-based delivery** through lipid nanoparticles of base editing through CRISPR proteins for the treatment of cardiovascular disease. The other organization is using **antisense oligonucleotides to modify mRNA in the cell** to increase protein production. Both organizations are a hundred percent committed to mRNA-based therapies and are funded for multiple years and are currently in phase two or phase three clinical testing."*
(Interview #5, Smaller Biotech executive)

*"**We have partnerships with outside investors** who have invested in our development programs."*
(Interview #16, Smaller Biotech executive)

"No one wants to be left out of this very promising and novel area."

(Interview #12, Large Pharma executive)

Current focuses still revolve around vaccines, but new modalities are growing

Current and Future Use Cases

Based on analysis of mentions from interviews

- **Vaccines remain the dominant current use case**, with generalized vaccines and personalized vaccine therapies leading the focus.
- While over 50% of pharma/biotech executives mention **cancer therapies and infectious diseases** as the largest future opportunities for mRNA technologies, only **30% mention of organizations mention they are actively working on innovations in these areas today.**
- In contrast, **cell therapy and gene editing**, seen as mid-tier future opportunities (compared to new cancer therapeutics), are **already being pursued by 26% of organizations**, likely due to their current feasibility and alignment with ongoing advancements.
- **Personalized therapies, spanning cancer vaccines, rare diseases, and advanced immunomodulation, are rapidly emerging** as companies move beyond traditional "off-the-shelf" approaches to explore more tailored solutions.

What specific mRNA modalities or use cases is your organization involved in?
(N=27, Open End, % = % of participants who mentioned each theme)

Vaccines (general) (51%): Vaccines, mRNA vaccines, flu vaccines, low-cost vaccines, replacing existing vaccines

Personalized Vaccines (51%): Personalized therapies, personalized medicine, specialized & customized therapies, patient-specific therapies

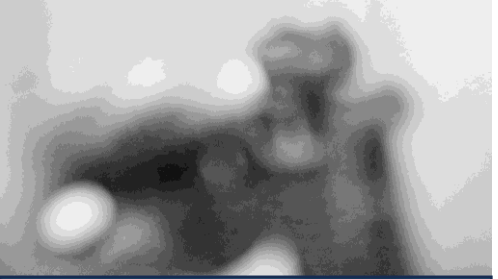
Cancer Therapeutics (30%): Cancer treatment, oncology, cancer therapies, personalized cancer vaccines

Infectious Diseases (30%): Infectious diseases, flu vaccines, immunodeficiency prevention programs, immunology

Cell Therapy & Next-Generation Technologies (26%): Cell therapy, ex vivo cell therapy, next-generation cell technologies, advanced cell technologies

Gene Editing & Gene Modification (26%): Gene editing, gene regulation, gene modification, mRNA as a component of gene editing, and cellular modifications

Additional mentions include rare diseases, immunomodulation and therapy enhancement, diagnostics, protein replacement, and T-cell technologies, reflecting a diverse range of emerging applications for mRNA



How interested is your organization in investing in mRNA and next-generation/advanced therapeutics? (N=27, Close End, 5-point scale)

78%

Indicate their organization is extremely/very interested in **investing in mRNA and next-generation/advanced therapeutics**, such as small activating RNA, circular RNA, epigenomic and gene editing technologies, and regenerative medicine

Pharma/Biotech interested in mRNA to address unmet medical needs and stay at the forefront of innovation

Please elaborate on why your organization is extremely/very interested in investing in mRNA and the next-generation/advanced therapeutics. (n=21, those who indicated 'extremely' or 'very' interested, Open End, % = % of participants who mentioned each theme)

Breakthrough Potential in Unmet Needs (43%): mRNA enables personalized treatments and gene editing, targeting oncology, rare, and genetic disorders.

- *"**mRNA is the future of treatment**, especially in oncology and rare disease space also in genetic diseases and hence the forward-looking perspective."* (Interview #26, Large Pharma executive)

Strategic Innovation Leadership (33%): Investing in mRNA aligns with goals to lead in cutting-edge genetic therapies.

- ***"mRNA is the next chapter in medicine.** We are invested in this field and need to be the best."* (Interview #11, Smaller Biotech executive)

Advancing Beyond Current Limitations (29%): mRNA offers solutions to existing technological challenges.

- *"[We are investing in mRNA] to overcome some of the **limitations of the technologies.**"* (Interview #21, Large Pharma executive)

Improved Drug Safety & Efficacy (19%): mRNA enhances treatment success and safety profiles.

- *"Using gene editing will increase the success of drugs and **improve safety and efficacy issues**."* (Interview #8, Large Pharma executive)

"mRNA is the next chapter in medicine. We are invested in this field and need to be the best."

(Interview #11, Smaller Biotech executive)

Large Pharma: Investments include in-house R&D, manufacturing, technology, strategic partnerships and acquisitions

How, if at all, is your organization planning for the commercial launch and reimbursement of your mRNA products or investments, especially considering the recent low sales of COVID and flu mRNA vaccines?
(n=18, executives at large pharma companies, Open End)

Robust commercialization strategies: Leveraging existing commercialization infrastructure and experience to streamline the launch of mRNA products; tailoring strategies to target unmet needs and personalized approaches.

Proactive reimbursement planning: Including early engagement with payers, regulators, and stakeholders to address pricing and reimbursement challenges. Additionally, emphasizing tailoring pricing and reimbursement to specific regions and products

Risk mitigation and diversification: Diversifying pipelines, shifting focus from vaccines to therapeutics to reduce reliance on flow-demand vaccine markets, and emphasizing market research to adapt strategies based on changing demand

*"We have a well-built **commercialization strategy and team**."*
(Interview #17, Large Pharma executive)

*"Low sales is due to low demand, thus you have to explore **unmet medical needs."***
(Interview #2, Large Pharma executive)

*"We are **engaging the payers quite early** as well as government agencies and insurance companies."*
(Interview #19, Large Pharma executive)

***"Price and reimbursement to be discussed** with each regulatory authority."*
(Interview #24, Large Pharma executive)

*"We are more **focused on drugs and therapeutics** and not vaccines."*
(Interview #6, Large Pharma executive)

Smaller Biotech: plan for commercial launches through collaborations with partners and handling reimbursement early

How, if at all, is your organization planning for the commercial launch and reimbursement of your mRNA products or investments, especially in light of the recent low sales of COVID and flu mRNA vaccines?
(n=8 executives at smaller biotech companies, Open End)

Partnership-Driven Commercialization: Reliance on partners to handle commercialization and reimbursement responsibilities

*"We expect these to be **responsibility of a partner(s)**."*
(Interview #13, Smaller Biotech executive)

Reimbursement Strategies: Engaging with payers early but many relying on partners or dual reimbursement strategies

*"We are at an early stage of commercial planning since we are far from approval. We plan to **seek reimbursement from both private and public sources."***
(Interview #16, Smaller Biotech executive)

Reassessing Investment Focus: Pivoting to high impact diseases like cancer and chronic conditions to focus on areas with higher unmet medical needs

*”**Life-threatening diseases like cancer may receive much more attention** than the common cold or flu, which are already well-established with lower returns on cost.”*
(Interview #25, Smaller Biotech executive)

mRNA challenges include manufacturing and scalability, delivery and targeting challenges, and regulatory hurdles

What are the main challenges mRNA technology faces concerning the development, adoption, and implementation of mRNA therapeutics and other modalities?
(N=27, Open End, % = % of participants who mentioned each theme)

Manufacturing Complexity and Scalability (67%): Scaling mRNA production while ensuring consistent quality and purity is challenging due to the complexity of manufacturing processes.

Delivery and Targeting Challenges (37%): Effective delivery to specific tissues remains a hurdle, limiting the therapeutic potential of mRNA technologies.

Regulatory and Clinical Trial Barriers (37%): Regulatory uncertainty and complex trial designs delay mRNA development and approval processes.

Safety, Immunogenicity, and Efficacy Concerns (37%): Long-term safety, potential immunogenicity, and efficacy issues must be addressed for broader acceptance.

Cost and Economic Viability (33%): High production costs, reimbursement challenges, and limited affordability impact the accessibility of mRNA therapeutics.

mRNA Stability and Storage (33%): Managing instability and stringent cold storage requirements poses logistical challenges for mRNA products.

Public Perception and Healthcare Adoption (19%): Misinformation and skepticism hinder acceptance, requiring education and advocacy to build trust among the public and healthcare providers.

*"The current main challenge for mRNA technology is **developing methods for delivery** to tissues beyond the liver. Multiple companies are working on delivery to the lung, brain, and muscle, as well as in vivo delivery to circulating immune cells."*
(Interview #5, Smaller Biotech executive)

*"Pricing is a significant challenge. **Overall safety and longevity are also concerns**. There haven't been many mRNA-based technologies that have made it into the clinic or are currently in use, so the long-term safety risk is obviously a concern for patients and the healthcare system. Conducting a budget impact analysis will also be challenging."*
(Interview #19, Large Pharma executive)

*"There are challenges at every stage, from **transcription to capping to purification**, especially when transferring technology across different platforms and sites."*
(Interview #9, Large Pharma executive)

*"One of the biggest challenges with mRNA molecules is their inherent instability. **They can decay or degrade very quickly**, which makes storage and transportation difficult."*
(Interview #26, Large Pharma executive)

"My biggest concern at this point is that while mRNA is a cutting-edge technology with significant opportunities for success, skepticism remains. I am particularly worried about political interference, where political expediency could override scientific judgment. Strong leadership—like what we saw with Kennedy's vision to send a person to the moon—is crucial to overcoming these challenges and ensuring scientific progress in this field."

(Interview #25, Smaller Biotech executive)

Regulatory hurdles for mRNA due to novelty, long-term safety/toxicity concerns, complexity of mRNA, and manufacturing challenges

Have you encountered, or do you anticipate, any regulatory hurdles in advancing mRNA therapeutics or other modalities? (N=27, Open End)

63%

***have* encountered or anticipate regulatory hurdles** *in advancing mRNA therapeutics or other modalities*

Novelty Creates Regulatory Uncertainty Lack of clear pathways for mRNA therapeutics due to its novelty creates challenges, requiring ongoing dialogue with regulators to clarify expectations.

*"The number of mRNA therapeutics is very small that have been approved therefore **the regulatory pathway is still unclear** for most products that are in development."*
(Interview #16, Smaller Biotech executive)

Long-Term Safety/Toxicity Concerns
Proving mRNA therapeutics' long-term safety and managing toxicity requires extensive testing and regulatory scrutiny, especially for complex applications like gene editing.

*"I think overall **safety demonstrating longevity or long-term safety** of these molecules as well as from a drug development consistency standpoint ensuring that we're really manufacturers and reliable and easy to replicate manner."*
(Interview #19, Large Pharma executive)

Need for Tailored Approach
The complexity of mRNA therapeutics requires customized regulatory strategies, emphasizing clear communication of unique attributes and clinical success criteria.

*"The challenges lie in moving beyond conventional CMC and clinical regulatory mindsets. **It's essential to highlight the unique characteristics and differentiation factors of mRNA**, particularly in clinical endpoints and success criteria, when communicating with regulators."*
(Interview #22, Large Pharma executive)

Manufacturing Consistency Challenges
Scaling mRNA production while ensuring GMP compliance and consistent quality poses significant regulatory challenges.

*"...along with **quality control and controlling at the manufacturing standards** for reproducibility and handling the good manufacturing practices that also goes along with all this and which is also the standardization of the manufacturing all this should be clear to the regulator..."*
(Interview #20, Large Pharma executive)

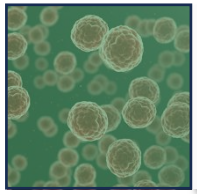
37%

***have not* encountered or anticipate regulatory hurdles** *in advancing mRNA therapeutics or other modalities*

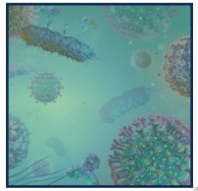
Perception of Minimal Regulatory Hurdles Due to Established Frameworks
Some participants perceive minimal regulatory hurdles, citing the positive reception of mRNA technology by regulatory bodies and established frameworks supportive of its advancement. They believe that the successful deployment of mRNA vaccines has built confidence among regulators, potentially easing the pathway for future therapeutics.

Advancing mRNA would include investments in R&D across applications, focus on scalability, enhanced delivery, clinical validation, and more

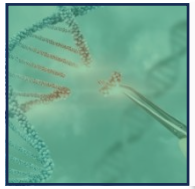
What areas do you believe the industry should focus on and prioritize, to advance mRNA therapeutics and other modalities?
(N=27, Open End, % = % of participants who mentioned each theme)



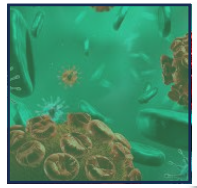
Invest in R&D to Expanding mRNA Applications (44%)
Prioritize R&D to extend mRNA use beyond vaccines into oncology, neurology, infectious diseases, and emerging modalities like circular RNA to address unmet needs.



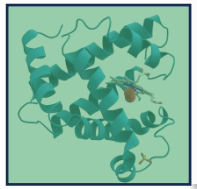
Manufacturing Optimization and Scalability (26%)
Enhance production efficiency, scalability, and cost reduction through automation and flexible, high-volume platforms to ensure consistent mRNA quality.



Advanced Delivery Systems (19%)
Develop targeted, efficient delivery technologies to improve tissue specificity, potency, and stability of mRNA therapies.



Clinical Validation (15%)
Demonstrate mRNA efficacy and safety in human trials to drive development, investor confidence, and commercialization.



Other: Addressing stability and storage challenges, education and outreach to healthcare stakeholders, quality assurance and robust testing methods, and regulatory engagement and streamlined approval processes

*”I think **oncology** is the big area first of all that I think could be very very successful followed in the area around **neuroscience** as well."*
(Interview #15, Smaller Biotech executive)

*“The focus also should be given to the **optimization of the manufacturing process,** especially automation and scalability, because it should be flexible modular and the manufacturing system [should be able to] **handle high volumes with consistent quality and reproducibility**.”*
(Interview #20, Large Pharma executive)

*”The industry should focus on developing new lipid nanoparticle to **allow for delivery to tissues beyond the liver.**"*
(Interview #5, Smaller Biotech executive)

***"Initial demonstration of clinical efficacy in humans** by whatever route of administration. If positive, this will provide impetus for further development."*
(Interview #13, Smaller Biotech executive)

***“Storage is extremely important** and it's better to develop something that could be stored easily at normal temperatures like a fridge rather than at minus one hundred or minus eighty or minus sixty.”*
(Interview #20, Large Pharma executive)

"The focus also should be given to the optimization of the manufacturing process, especially automation and scalability, because it should be flexible modular and the manufacturing system [should be able to] handle high volumes with consistent quality and reproducibility."

(Interview #20, Large Pharma executive)

Misinformation and skepticism present challenges to the mRNA industry overall; however, most indicate minimal/no impact on their organization

To what extent have misinformation and public skepticism about mRNA vaccines affected the broader mRNA therapeutics industry?
(N=27, Open End, % = % of participants who mentioned each theme)

Concerns and Fears Lead to Hesitation (33%)
Concerns about safety, efficacy, side effects, and misconceptions—such as fears of genetic alteration or infertility—lead to hesitancy among the public and investors, hindering progress and adoption.

Influence of COVID-19 on Public Skepticism (26%)
Skepticism is often specifically tied to COVID-19 vaccines rather than mRNA technology itself. The pandemic increased public awareness of mRNA technologies but also amplified misinformation and political controversies. While successes like the COVID-19 vaccines have improved understanding, ongoing challenges highlight the need to separate perceptions of the technology from specific applications.

Greater Impact on Vaccines than Therapeutics (15%)
The impact of misinformation varies across the industry. Some organizations are avoiding vaccine development due to negative public and investor perceptions, while others focus on therapeutic applications, which may be less affected. Therapeutic development often continues because of unmet medical needs and a smaller, better-informed patient population.

*"There were a lot of people that **did not want to take a vaccine** simply because it contained mRNA."* (Interview #19, *Large Pharma executive)*

*"Claims that mRNA vaccines could alter DNA and **cause fertility issues created fear.** This fear complicated the development and distribution of mRNA-based vaccines."* (Interview #20, *Large Pharma executive)*

*"There are a number of people in the media who have no scientific background that are making claims about mRNA vaccines... **but the misinformation persists in the public opinion."*** (Interview #16, Smaller Biotech executive)

*"At the time of the COVID pandemic, mRNA technology was seen as a newly found technology that was put out of the box on the market and **seen by many as a quick and dirty compound**."* (Interview #24, *Large Pharma executive)*

***"It has had an effect on the vaccine space** but not the therapeutic use since there is a real unmet medical need there and is a smaller patient population that can be better informed." (Interview #14, Large Pharma executive)*

Have misinformation campaigns or regional bans on mRNA affected your organization's work in this space? (N=27, Open End)

Majority of executives (78%) reported minimal or no direct impact from misinformation campaigns or regional bans on **their organization's mRNA programs or strategies.** Internal operations and development efforts have largely remained unaffected by these external factors.

“There are a number of people in the media who have no scientific background that are making claims about mRNA vaccines... but the misinformation persists in the public opinion.”

(Interview #16, Smaller Biotech executive)

How AMM can help: Majority support AMM in combating misinformation and potential bans, emphasizing robust advocacy and educational initiatives

Would you like to see the Alliance for mRNA Medicines, as an advocacy group, combat misinformation and potential bans related to mRNA technologies? If so, where and how? (N=27, Open End, % = % of participants who mentioned each theme)

74% express strong support for AMM combatting misinformation

Targeted Efforts Toward Specific Audiences (37%)

Patients, healthcare providers, regulators, Congress, the FDA, political entities, and even philanthropic organizations seen as key groups where targeted education and engagement could be most effective in combating misinformation and influencing policy decisions.

*"Focus on real world evidence on the safety and efficacy of mRNA therapeutics and **share these with regulators and patient groups**."*
(Interview #9, Large Pharma executive)

Target Specific Platforms and Strategies for Combating Misinformation (26%)

Suggestions included leveraging social media, conducting public education campaigns, hosting webinars and podcasts, participating in international and regulatory conferences, engaging with academic centers, and utilizing emerging technologies like AI tools to disseminate accurate information.

"International conferences and regulatory conferences and BARDA platforms and philanthropic organizations."
(Interview #8, *Large Pharma executive)*

Collaborating with Regulatory Bodies (22%)

Collaboration with regulatory bodies such as the FDA, NIH, BARDA, and government entities was highlighted as critical. Participants believed that working closely with these organizations could bolster advocacy efforts, support science-based policy development, and help prevent potential bans on mRNA technologies through informed legislative actions.

*"Work with **congress and FDA.**"*
(Interview #7, Smaller Biotech executive)

Emphasize and Support Transparent and Objective, Unbiased Data (15%)

Building trust through scientific transparency and providing access to clinical data was emphasized as critical, as well as continuing scientific research and generating real-world evidence to demonstrate safety and efficacy.

*"Yes, that would be helpful provided it is done based on **OBJECTIVE and UNBIASED data.**"*
(Interview #13, Smaller Biotech executive)

Unprompted, a few mentioned an opportunity to increase awareness and interaction with AMM:

"Moderna isn't part of the alliance. I know of many companies that haven't been contacted." (Interview #11, Smaller Biotech executive)

"AMM currently has relatively low visibility, even within the biotech industry, and even less among the general public. Despite having prominent members and an important mission, their impact has been relatively limited to date."
(Interview #5, Smaller Biotech executive)

Educational initiatives seen as a top mRNA advocacy issue, followed closely by developing standards for advocacy/policy support

Please rank the following advocacy issues by importance to you, with 1 being 'most important' and 3 being 'least important' (N=27, Ranking)

Please share more why you believe [insert rank #1] is the most important, relative to the others. (N=27, Open End)

Educational Initiatives
For the public, healthcare providers, and government/regulators.

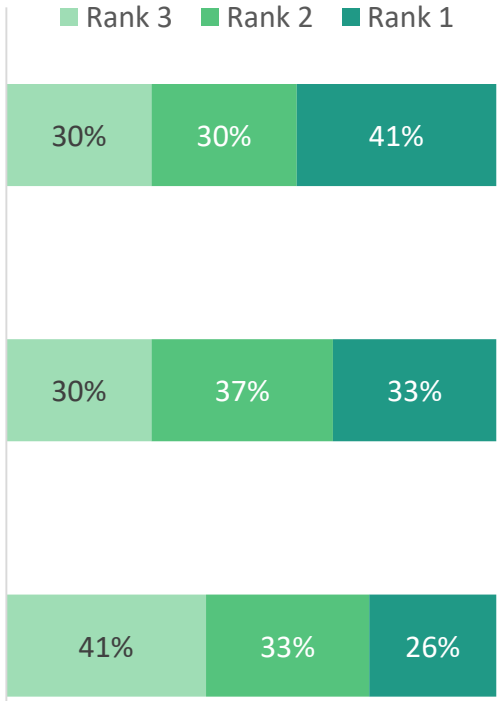
Average ranking: 1.9 of 3

Developing Standards for the Field
To ensure the safety, efficacy, and quality of mRNA technologies.

Average ranking: 2 of 3

Advocacy and Policy Support
Including drafting and influencing regulatory frameworks for therapeutic vaccines.

Average ranking: 2.1 of 3



Educational initiatives ranked as a top advocacy issue for advancing mRNA technologies due to the need for **building public trust, combating misinformation, and empowering healthcare providers** to support patient decision-making.

Developing Standards ranked as a top advocacy issue for advancing mRNA technologies due to the need for **clear guidelines for gaining regulatory approval** and ensuring **consistent product quality.**

Advocacy and Policy Support ranked as a top advocacy issue for advancing mRNA technologies due to the need for a **supportive regulatory environment** and collaborative efforts to **address regulatory resistance.**

Other Advocacy Issues

What other advocacy issues, if any, are important to you beyond these 3? (N=27, Open End)

Pricing, Reimbursement, and Financial Accessibility

Participants identified the **need for effective pricing strategies**, involving pricing and reimbursement parties early in the development process, considering payer perspectives, and conducting cost-benefit analyses.

***"Pricing and reimbursement parties need to be involved early** in the development process to prevent major discussions from occurring too late."* (Interview #24, *Large Pharma executive)*

Addressing Unmet Medical Needs

Participants advocated for highlighting the unique opportunities that mRNA technologies offer in **addressing large unmet medical need**s, such as Alzheimer's and Parkinson's diseases. Demonstrating the potential of mRNA technologies in treating conditions lacking effective therapies can enhance public understanding and support for their development.

*"mRNA vaccines address diseases that also have classic vaccines, so people don't see the advantage. However, in **addressing large unmet medical needs** like Alzheimer's and Parkinson's people may better understand the potential of this technology."* (Interview #14, *Large Pharma executive)*

Collaboration and Knowledge Sharing

Participants highlighted the **value of intercompany collaboration and knowledge sharing**, including partnerships with academic institutions. Promoting communication and sharing of technology and insights between companies and academia drives innovation and accelerates the development of mRNA technologies.

*"The alliance should also promote **intercompany communication** and **technology knowledge share** also **connects university or other academic optimization,** so that new findings in the academic setting can be [translated to the] industry setting."* (Interview #27, *Large Pharma executive)*

Global Access and Equity

Participants emphasized the **need to address disparities** by making treatments accessible to low-income countries and populations. Advocacy efforts should focus on global access to address health inequities

.

*"One very important factor is ensuring global access to mRNA therapies, **especially for low-income countries and populations**, to address disparities."* (Interview #20, *Large Pharma executive)*

Future mRNA Industry Reports

What data, questions or discussion topics would you most like to see addressed in reports on the mRNA industry? Why?
(N=27, Open End, % = % of participants who mentioned each theme)

Safety and Efficacy Concerns (37%)

Long-term safety data, cancer risk assessments, and clinical efficacy evidence are crucial for building trust in mRNA technologies. Comprehensive data on safety, effectiveness, and quality are essential to reassure stakeholders.

***"More safety and effectiveness** and quality data and manufacturing issues and capabilities. Data speaks the truth and results."* (Interview #8, *Large Pharma executive)*

Manufacturing, Quality, and Cost (30%)

Manufacturing challenges for mRNA technologies include cost, scalability, stability, and quality assurance. Key focus areas are processing parameters, product quality, cost-effective practices, and leveraging technological breakthroughs to reduce healthcare costs.

*"I think topics like new support for growing technologies and **the reduction in health care costs."*** (Interview #18, *Large Pharma executive)*

Market Dynamics and Opportunities (30%)

Analyze the mRNA industry's competitive landscape, investment trends, pipeline developments, and market potential. Focus on key players' strategic positioning, investor confidence, and resource allocation to development programs.

***"Industry-wide survey of what drugs have been approved, what's in the pipeline.** This would be important to see what resources are being applied to development programs."* (Interview #16, *Smaller Biotech executive)*

Education and Misinformation Mitigation (19%)

Address public and governmental misconceptions about mRNA technologies to improve acceptance. Increase awareness of mRNA therapeutics, explain clinical trials, address skepticism about rapid vaccine development, combat misinformation, and highlight the positive global impact of mRNA vaccines.

Regulatory and Reimbursement (15%)

Clear reimbursement pathways and harmonized global regulations are needed for approvals and access. Educating policymakers and understanding reimbursement strategies are crucial for overcoming regulatory challenges and ensuring commercial success.

Therapeutic Applications and Unmet Needs (15%)

Focus on mRNA therapies' efficacy and cost-effectiveness for genetic, oncological, and rare diseases. Understand disease biology to enhance technology development and explore new therapeutic indications where mRNA can significantly impact unmet medical needs.