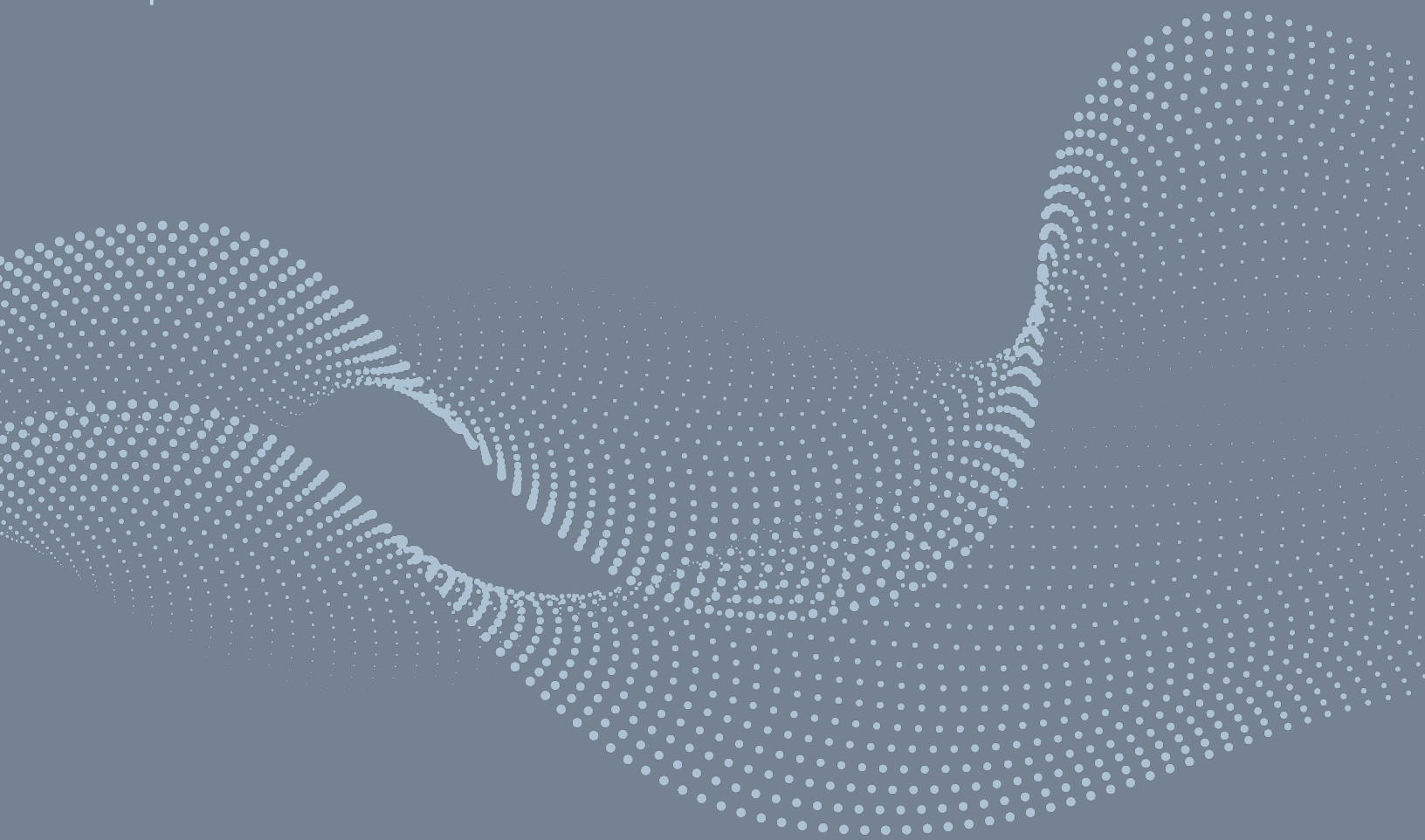


FUTURE
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INITIATIVE
BY ABU DHABI

The Future of Therapeutics

How Abu Dhabi can help translate
the next generation of science into
patient care



strategy&

Knowledge Contributor *Part of the PwC network*

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Executive Summary

Scientific and technological advances are expanding our understanding of disease and the mechanisms that drive it. Genomics, multi-omics, and longitudinal health data are revealing biological differences that broad diagnoses can miss, while AI is helping researchers interpret those data and identify new therapeutic targets. New therapeutic approaches, including cell and gene therapies, are widening the range of ways in which disease biology can be addressed. The harder challenge is increasingly how those advances are translated into therapies that can be developed, validated, and delivered reliably and affordably to patients. More precise treatments often require narrower populations to be identified, practical constraints around delivery or manufacturing to be solved earlier, and evidence to continue accumulating after treatment.¹⁻¹⁵

This puts more weight on the connections between research and care. Data and biospecimens need a route into clinical development, while regulatory and operational questions have to be addressed early enough to influence the program. What happens after treatment is also important, because those outcomes later shape clinical and coverage decisions.

Abu Dhabi has invested heavily to build much of the foundation needed to handle that complexity. Population genomics and biobanking sit alongside longitudinal health data, clinical research and world-class specialist care, with regulatory capability and life-sciences partnerships developing around them. The aim is to make those assets work together around selected therapeutic programs rather than reproduce every part of the global value chain locally. Different institutions can own different parts of the pathway as long as the hand-offs between them work.¹⁶⁻¹⁹

The paper follows that pathway in the emirate from discovering better targets, through designing and validating therapies, to preparing health systems to identify, deliver, and follow them. The future of therapeutics will be shaped by what science makes possible and by how effectively health and life-sciences ecosystems translate that possibility into measurable patient outcomes.

Four Implications Frame the Paper

1. **Precision can matter as much as scale.** Population genomics and linked clinical information can make narrowly defined cohorts easier to identify and follow, particularly in rare disease, prevention, and biomarker-led studies
2. **Abu Dhabi will need to choose where to build therapeutic depth.** Disease priorities should guide where established modalities remain highly relevant and where newer approaches offer distinct advantages, with reusable capabilities, a specialist workforce, and global partnerships supporting both

3. Evidence generation is moving closer to biology and care. Human-relevant models, computational approaches, biomarker-led studies, real-world evidence, and longitudinal follow-up place greater demands on data quality, validation, regulatory coordination, and lifecycle oversight
4. The care pathway is increasingly part of the therapeutic proposition. Patient identification, diagnostics, site readiness, reimbursement and access, and post-treatment follow-up can determine whether advanced therapies deliver value

Introduction: A Changing Therapeutic Model

Advances in genomics, multi-omics, and molecular profiling are revealing biological differences that sit beneath broad clinical diagnoses. Patients who appear to have the same disease can be driven by different mechanisms, and those mechanisms can change over time. Therapeutic options are expanding alongside that understanding, with RNA medicines, engineered cells, genetic therapies, and new biologic formats providing additional ways to act on the biology.^{4,5,8-10}

That changes both what a therapy can look like and what has to sit around it. Three shifts are particularly important:

1. Find the right population. Biomarkers and molecular testing can narrow the group most likely to benefit, making identification, and recruitment part of the development challenge. Abu Dhabi's Halia study provides a local example, using Emirati Genome Program data to identify potential participants before symptoms develop.¹⁷
2. Build around the modality. Some therapies bring delivery, manufacturing and site-readiness requirements into development much earlier. Abu Dhabi's advanced-therapy programs, including its work on gene editing and CASGEVY, illustrate how much of the pathway can sit around the product itself.^{20,21}
3. Follow the evidence for longer. Smaller cohorts and potentially durable effects put more weight on trial design, longitudinal follow-up, and evidence collected after treatment.^{11,22}

The paper therefore asks what a health and life-sciences ecosystem needs in order to translate increasingly precise and complex therapeutics into patient care. It follows that question across the therapeutic journey, using Abu Dhabi as a practical lens, and returning to its emerging capabilities in genomics, research, regulation, and clinical care.

Chapter 1: Discover: Turning Biological Insight into Therapeutic Opportunity

Discovery increasingly depends on connecting biological signals to the data, samples, and research capabilities needed to test them.

Drug discovery increasingly draws on a wider range of biological information. Genetics can identify associations with disease, multi-omics can reveal where and how those signals are expressed, longitudinal data can show how they relate to disease progression or treatment response, and AI can help integrate evidence across these sources. Human genetics provides a particularly strong starting point: across clinical programs, mechanisms backed by genetic evidence have been approximately 2.6 times as likely to succeed as those without such support.^{1,6} Functional experiments then test causality, tissue context, and therapeutic relevance.

Bringing these capabilities together is increasingly important to move efficiently from a biological signal to a validated disease mechanism and credible therapeutic target. Strong discovery ecosystems therefore provide governed access to genomics, multi-omics, longitudinal health data and biospecimens, alongside the bioinformatics and experimental capabilities needed to test and translate the insights they generate. As those datasets deepen, advances in translational medicine are improving our understanding of disease mechanisms, opening up new therapeutic targets and potential mechanisms of action (MoAs), while also changing how established targets are interpreted.^{6,7}

From Population Data to a Therapeutic Hypothesis

Population sequencing becomes far more informative when researchers can connect a variant to the tissue in which it acts and to the patient's course over time. Biobanked samples make it possible to revisit the biology, while transcriptomic, proteomic, metabolomic, and single-cell studies help trace the route from genetic signal to cell function. Longitudinal records then show whether the signal appears before disease, changes with progression, or has any bearing on treatment response. The aim is to assemble a plausible causal story across these layers.

Representation also shapes what can be discovered. Many genomic resources remain weighted towards populations of European ancestry, which limits the transferability of risk models and leaves biology in other populations under-characterised. The gap extends into clinical research; a 2025 analysis found that only 1.3% of 50,000 oncology trials registered between 2005 and 2023 included a MENA site. A well-phenotyped regional cohort can improve the relevance of research locally and add evidence that global datasets have missed.^{23,24}

A statistical association is only a starting point; functional work still has to show that the signal is causal and therapeutically relevant. In oncology, that can mean understanding several interacting drivers rather than looking for a single target, which is why molecular subtyping and rational combinations have become increasingly important.⁵

The importance of target discovery remains visible even in a field experiencing rapid innovation in therapeutic modalities. A 2026 review of 93 first-in-class oncology approvals found that 56% were driven by new biological targets, compared with 25% driven by new modalities or mechanisms, and 15% by multi-target strategies.⁷

Where AI is Beginning to Show Value

In discovery, AI is most useful when it sharpens a defined choice. It can help connect evidence across genetics, molecular profiles, scientific literature and experiments, surfacing possible disease mechanisms or targets for further work. Structure-prediction systems can then suggest how biological molecules interact, while generative models explore candidate designs that would be impractical to enumerate by hand. The laboratory remains the arbiter, but better predictions can focus effort on the questions most likely to change a program.^{25,26}

AI-native developers are beginning to show what that scale can mean in practice. Generative models can also widen the option space by proposing new candidate molecules and biologics, including antibody designs, before laboratory testing narrows the field. Insilico Medicine, for example, uses computational platforms to search across large biological and chemical datasets before candidate molecules move into laboratory testing. The important change is the speed at which researchers can search, prioritize and design, which has the potential to transform development timelines even if experimental validation and clinical development still take substantially longer.^{26,27}

Idiopathic pulmonary fibrosis illustrates both the opportunity and the limit. Its biology remains heterogeneous and only partly understood, so each new target hypothesis still has to survive a difficult clinical test. Rentosertib, which emerged from an AI-enabled program, produced encouraging results in a randomized phase 2a study. It is too early to generalize from one program, but the trial moves the discussion from computational promise towards clinical evidence. Self-driving laboratories are also beginning to automate tightly bounded tasks with measurable objectives; open-ended biological discovery remains a longer-term ambition.^{28,29}

Abu Dhabi is Linking Data, Samples, and Development Capability

Several of these assets already sit within the same operating platform. M42 operates the Emirati Genome Program and Abu Dhabi Biobank and connects them with broader health data and clinical research capabilities. Together with Abu Dhabi's longitudinal health records, that gives researchers a population-specific base from which to study disease mechanisms and identify well-defined cohorts for further research and clinical development.^{16,17}

"Population genomics creates real value for patients only when it becomes a route into discovery and clinical development. By connecting the Emirati Genome Program, the Abu Dhabi Biobank, and longitudinal health data with clinical research, we can help partners find the right patients, test the right targets, and follow outcomes over time," shares David Kavanagh, General Manager, M42 Omics.³⁰

Insilico Medicine completed the discovery programme for ISM0387 in the UAE, moving from target identification through molecular design and optimisation to the nomination of a preclinical candidate. It provides a local example of AI-enabled discovery carried out across a full preclinical workflow. With population data, biospecimens, computational research and clinical expertise increasingly connected, Abu Dhabi is building the foundations of an integrated discovery environment.²⁷

Strategic implication



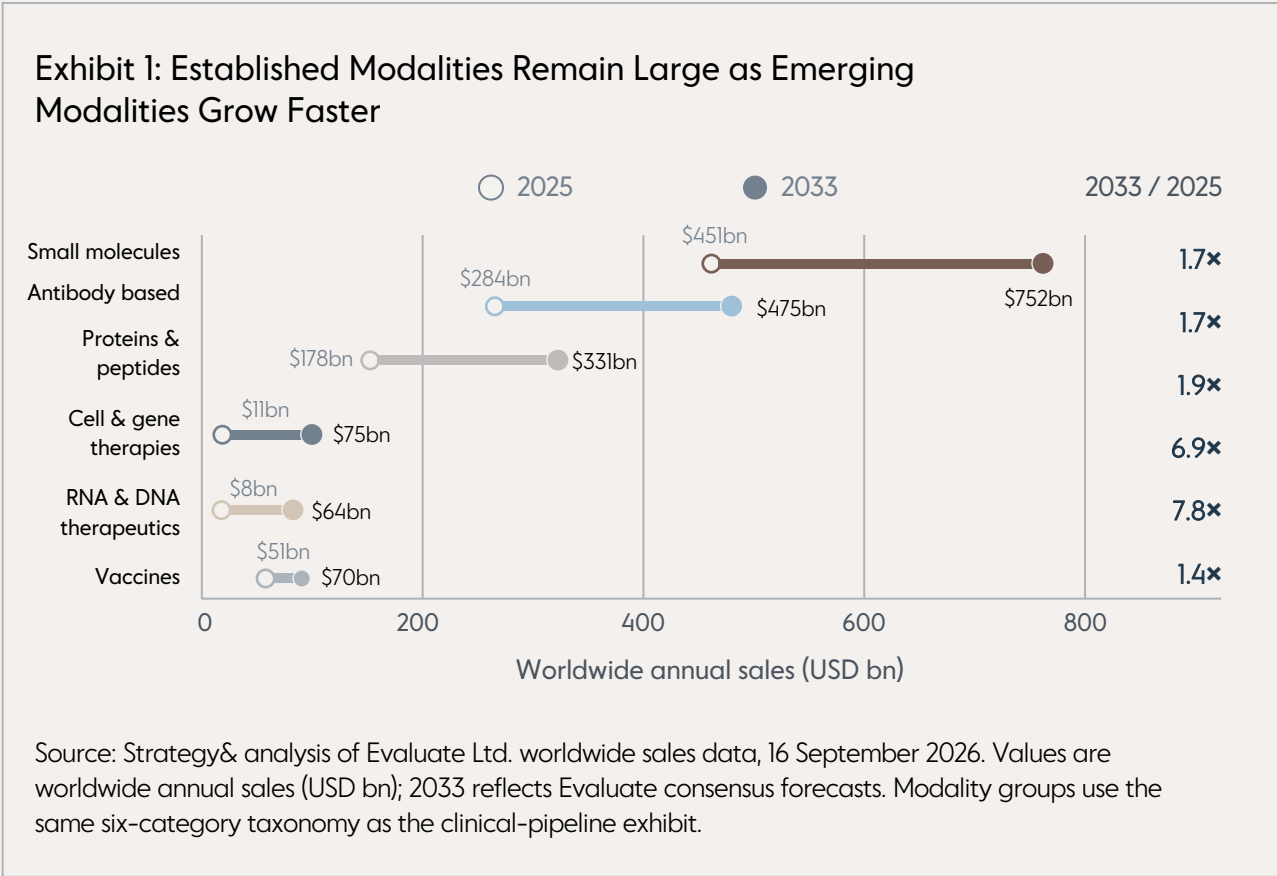
Population data become more valuable when they provide a repeatable route from biological insight into clinical development.



Chapter 2: Design: Choosing Where to Build Therapeutic Depth

A broader therapeutic toolbox raises a strategic question: where to build depth, and which capabilities can be reused across programs.

Therapeutic design is broadening as established modalities continue to improve and newer ones make different kinds of intervention possible. The choice of modality depends first on the biology, but whether a program is practical also turns on delivery, manufacturing, and the capabilities that can be shared across products. That makes selective depth more credible than trying to build every modality locally. A hub can specialize where it has a genuine scientific or clinical advantage. The surrounding CRO, CDMO, analytical, and trial base can serve more than one program, while partnerships with other centers provide capabilities that are better sourced elsewhere. Commercial forecasts tell a similar story from another angle. Small molecules and antibody-based medicines are still expected to be the largest markets in 2033, and both grow materially from today's base. Cell and gene therapies and RNA/DNA therapeutics start much smaller but are forecast to expand roughly seven- to eight-fold. For an ecosystem building therapeutic depth, that argues for capabilities that remain relevant to established modalities while leaving room to build depth in newer areas where the field is still taking shape.³¹

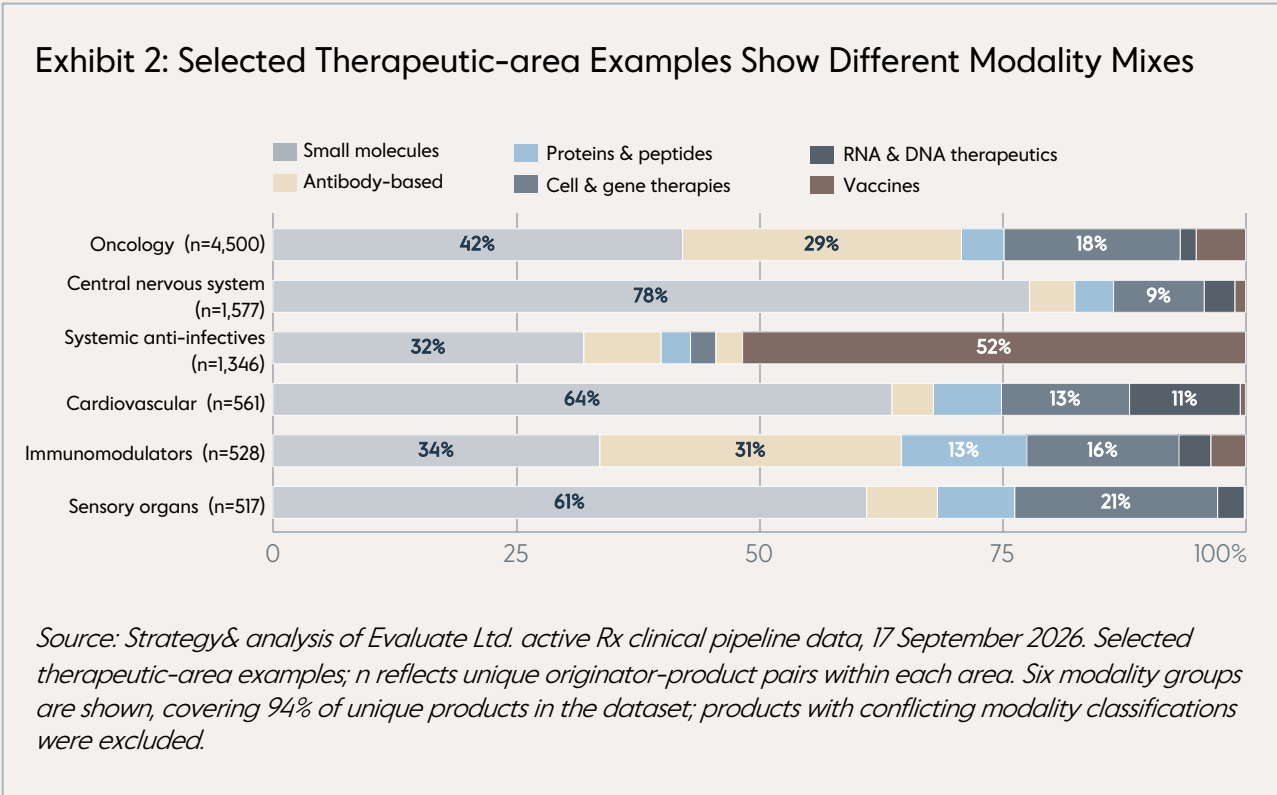


The Therapeutic Toolbox is Expanding

The science explains why both sides of that market remain relevant. Small molecules and antibodies continue to reach new targets as chemistry and biologic formats improve, while RNA, cell, and gene therapies become more useful when the biology calls for replacement, silencing, or reprogramming. Clinical maturity also varies widely: CRISPR-edited blood stem cells have reached approval, whereas individualised mRNA vaccines and in-vivo editing remain earlier in development.^{8-10,32-39}

As disease models become more detailed, combinations can also be selected around complementary mechanisms. One component may address the main disease driver while another changes the immune or tissue environment in which it operates. In oncology, that can bring several modalities into the same treatment strategy, for example a targeted small molecule alongside an antibody-based immunotherapy, with therapeutic vaccines adding another option in selected settings. The benefit still has to be shown clinically; adding more components does not automatically create a better therapy.^{5,37}

The chart is more useful as a portfolio lens than as a ranking of modalities. Oncology draws on a broad set of approaches, while CNS remains overwhelmingly small-molecule and anti-infective development is shaped by vaccines. Sensory disease shows another pattern, with a much larger cell-and-gene share. For Abu Dhabi, the question is which of these global patterns line up with capabilities already being built locally. Disease-area priorities should therefore drive where modality depth is developed, rather than the growth rate of a modality on its own.⁴⁰



Delivery and Production Shape What Can Scale

Programmability does not remove the physical problem of delivery. The carrier largely determines tissue distribution and cellular uptake, and it often decides whether repeat dosing is possible. Progress has been strongest in a limited number of organs, especially the liver, while protected tissues such as the brain remain difficult to reach.⁴¹

For autologous cell therapy, manufacturing begins with variable patient material and ends with a product that must return to the same patient on time, under an unbroken chain of identity. Vectors and lipid nanoparticles present a different challenge because potency and purity have to remain stable as production scales. In both cases, process development shapes the product long before launch.

"As medicines become more complex, the requirements for local production extend far beyond the manufacturing facility itself. It requires internationally recognised quality standards, the ability to absorb technology transfer, specialised talent, and integrated logistics capabilities that ensure products reach patients in the same condition in which they were produced. Together, these capabilities form the foundation of a resilient and competitive life sciences ecosystem," explains Mubadala Bio.⁴²

The workforce requirement extends beyond research scientists. More complex production requires expertise in bioprocessing, quality, and technology transfer. As therapies move through development and into care, the capability base also needs to include clinical-trial operations, regulatory science and health-technology assessment, alongside specialist clinicians, pharmacists, and teams equipped to support long-term safety monitoring.^{12,13,42}

The field is also moving toward approaches that could reduce some of this patient-specific manufacturing burden, including in-vivo cell engineering and more standardised, potentially "off-the-shelf" cell-therapy formats.³⁶

Some of this work can be reused across related products. A validated delivery vehicle or manufacturing process may support several therapies, together with the analytical methods needed to assess them. The sequence, guide or cell configuration still changes, as does the clinical setting. Efficiency comes from carrying forward what is truly shared without assuming that the new product carries the same risk.

Regulators are beginning to formalise that distinction. FDA draft guidance published in 2026 describes circumstances in which knowledge from related genome-editing products may inform chemistry, manufacturing, nonclinical, and clinical work. The approach is particularly relevant to ultra-rare disease, where a family of related therapies may be viable even when a stand-alone program for each mutation is not.³

Abu Dhabi is Building Capability Around Selected Platforms

Abu Dhabi is also exploring capability in areas that could support future programs across more than one product. DoH - AD, UCSF, and the Innovative Genomics Institute are exploring Centers for Genome Surgery focused on highly personalized editing for severe rare and inherited diseases, while DoH - AD and Sanofi are exploring a Vaccine Innovation Centre intended to include mRNA research, AI-enabled vaccine discovery, clinical development and clinical manufacturing. These initiatives remain at an earlier stage, but they illustrate the type of platform capability that can be built around selected therapeutic areas rather than individual assets alone.^{43,44}

The collaboration among DoH - AD, M42, and Mammoth Biosciences sets out a development pathway for MB-111, including local site qualification, clinical capability transfer and plans for GMP lipid-nanoparticle manufacturing. The Abu Dhabi Biobank partnership with Human Life CORD Japan is pursuing a parallel route for umbilical-cord-derived cell therapies, with plans spanning local development, manufacturing and clinical translation. Both are prospective programs, but they show how Abu Dhabi is using global partnerships to build capabilities that could support later advanced-therapy programs.^{20,45}

Mubadala Bio is following a similar partnership-led route in pharmaceutical manufacturing, moving progressively from essential medicines towards more complex products. "Since our launch in May 2025, we have introduced more than 40 SKUs to the UAE market, including the first locally manufactured hemodialysis solutions in the country. In December 2025, we opened a high-potency facility for oncology and hormone products, and our agreement with Biosidus is expected to support the localisation of biologics at DiabTec across endocrinology, nephrology, oncology, and immunology," outlines Mubadala Bio.

Strategic implication



Build selectively, connect broadly. Abu Dhabi's choices should reflect where disease priorities create an advantage, including in established modalities such as small molecules that will remain large and innovative, as well as newer platforms where the field is still taking shape. Reusable capabilities can be built locally, while specialist capabilities can be accessed through global partnerships where that is more efficient.

Chapter 3: Validate: Precision Can Matter More than Scale

As therapies become more precise, evidence generation has to become more precise as well. Precision changes the evidence problem as well as the therapy. Human-relevant and digital models can help narrow what should be tested and clinical studies increasingly depend on finding molecularly defined patient groups. Those patients may need to be followed for years. A strong evidence ecosystem keeps that thread intact from preclinical work into trial design and routine care, with regulators involved early enough to shape requirements while a program can still adapt.

Human-relevant and Digital Models Sharpen Early Decisions

Human-relevant models are valuable when they answer a well-defined question. An organoid derived from a tumour might help compare drug response, while a tissue chip can add blood flow or mechanical forces that conventional cell culture cannot reproduce. The same platform may be poor at predicting a different type of toxicity or a whole-body response. Regulators therefore need evidence that a model performs reliably in the particular decision for which it is proposed.⁴⁶

FDA's 2025 roadmap specifically addresses preclinical safety studies, beginning with areas where validated alternatives can reduce or replace particular animal studies. The direction is not to remove the requirement for nonclinical safety evidence, but to allow human-relevant methods to carry more of that evidence where their performance has been demonstrated. This is likely to happen incrementally, with human-relevant systems taking on a larger share of the preclinical package as their performance becomes established.⁴⁷

The same direction is visible in the UAE. In 2026, the federal Emirates Drug Establishment showcased an Organ-on-Chip initiative that simulates human-organ functions on micro-engineered chips for medicine testing, alongside an insilico project using digital models to assess drug efficacy earlier in development. The initiatives signal growing regulatory interest in human-relevant and computational evidence approaches. For newer modalities, that early dialogue can be particularly important because questions about the evidence package, manufacturing controls, or clinical delivery may still be taking shape while a program is adaptable.⁴⁸

Digital models face the same need for validation. Single-cell foundation models and virtual-cell concepts are opening new ways to choose perturbations and anticipate response, although independent testing has not yet shown a consistent advantage over simpler methods. Their value will become clearer as predictions are tested prospectively in the laboratory.⁴⁹

Computer simulation can also bridge preclinical work and the first human studies. Model-informed development, sometimes described more broadly as *in silico* trials, uses existing biological, pharmacological and clinical data to test assumptions about dose, exposure and likely patient response before or alongside a trial. It does not replace human evidence. Its value is to make the clinical study more focused and to state clearly which predictions are being tested.⁵⁰

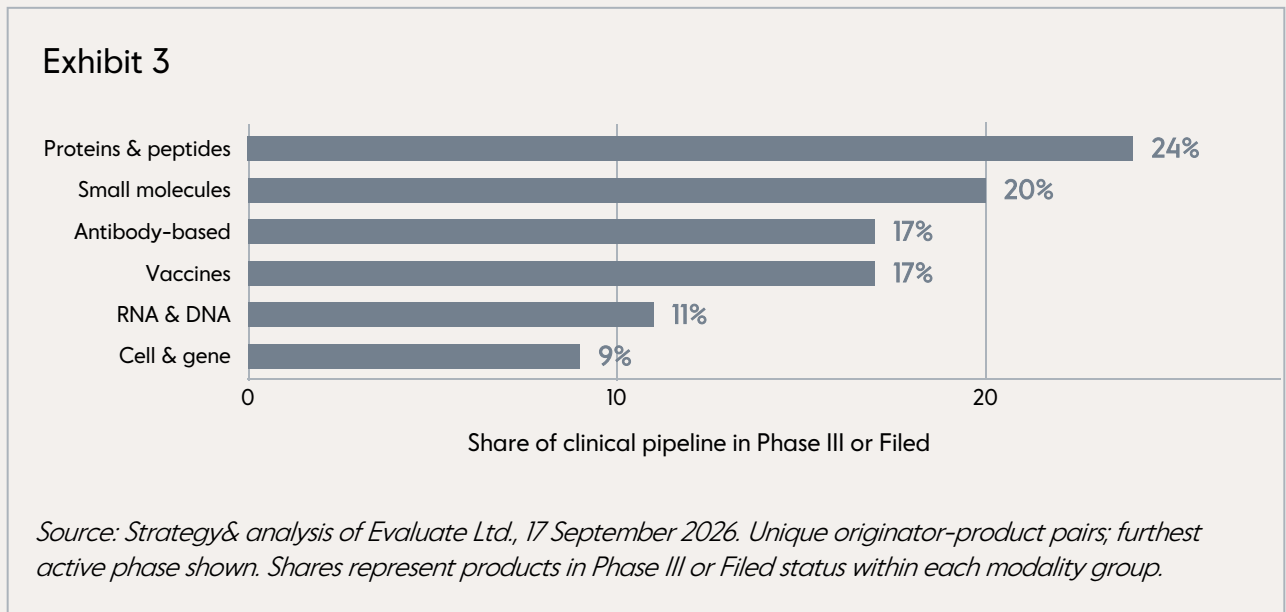
Trials are Being Built Around Biological Definitions

Precision trials reorganise research around the biology that defines the patient group. Biomarker and pharmacodynamic readouts can show early whether a therapy has reached its target and altered the intended pathway, allowing a weak programme to stop before a large trial and a stronger one to progress without an unnecessary pause. A basket study may follow one alteration across several diseases, whereas an umbrella study tests different targeted treatments within a single disease. Platform and adaptive designs allow cohorts or treatments to change within a common protocol as evidence accumulates. These designs can save time and patients, but only when the diagnostic is reliable and the rules for adaptation are agreed in advance.⁵¹

Small cell- and gene-therapy populations often make a conventional large, randomised trial impossible. FDA draft guidance therefore considers designs that draw on external controls, disease-progression models, Bayesian methods, and shared master protocols. These approaches still have to make the remaining uncertainty visible. Follow-up in routine care can then add information on durability, delayed safety signals, and performance beyond the original study sites.^{11,22}

For advanced therapies, regulatory oversight also continues well beyond approval. Long-term follow-up may be needed to detect delayed adverse events and understand whether clinical benefit persists, with post-market surveillance becoming part of routine lifecycle oversight. Evidence collected after launch can then inform safety requirements, clinical guidance, and future development decisions.⁵²

The maturity profile makes that need immediate. Only about 9% of cell and gene therapies and 11% of RNA/DNA therapies in the Evaluate clinical pipeline have reached Phase III or Filed status, compared with 20% of small molecules and 24% of proteins and peptides. The comparison is not a measure of ultimate success; it shows that a large share of the modalities attracting the most attention still has to pass through the evidence-generation systems being built today.⁴⁰



Clinical Trial Value Can Come from Precision Rather than Scale

The UAE is unlikely to compete with the largest global trial markets on volume alone. Abu Dhabi’s strength is the ability to make specific cohorts easier to find and follow. Population genomics and linked health data can identify people who would be difficult to recruit through conventional site-by-site screening, while specialist procedures can remain concentrated at a small number of experienced centres. This is particularly relevant for rare disease, prevention and biomarker-defined studies, where access to the right participants can matter more than the size of the general population. The region’s historical underrepresentation in global trials gives sponsors a further reason to generate evidence locally.^{17-19,24}

Burjeel Cancer Institute adds a regional rationale for building this capability locally. The UAE’s diverse resident population can broaden the evidence base for clinical research, while locally-led discovery and early development can keep attention on diseases with a particularly high regional burden that may otherwise sit outside global development priorities.⁵³

“Some diseases that carry a particularly high burden in our region are unlikely to become global drug-development priorities unless discovery and early development begin here,” notes Prof. Khaled Musallam, Group Chief Research Officer, Deputy CEO and Chief of Cell & Gene Therapy and Hematology, Burjeel Cancer Institute.⁵³

Abu Dhabi is already testing this approach. The Halia Therapeutics study uses Emirati Genome Program data to identify potential asymptomatic APOE4 carriers for an Alzheimer’s prevention trial, with counselling and clinical-data linkage through Malaffi. In inherited retinal disease, the OPGx-MERTK program brings together DoH - AD funding, a specialist site at Cleveland Clinic Abu Dhabi, and trial operations through IROS. These programs show how population data and specialist care can be connected around small, precisely defined cohorts.^{17,18}

PureHealth's Abu Dhabi Health Research Centre extends the clinical network available to such studies across hospitals, primary care and laboratories, while IROS adds local experience in trial design and regulatory coordination. For sponsors, that creates a route from patient identification into study delivery and then long-term evidence generation within Abu Dhabi's provider network.^{18,19}

Mediclinic Middle East provides a complementary provider view. Its Health Information Management capability and participation in TriNetX are already used to assess feasibility and identify potentially eligible patients across its network, and a dedicated clinical trials unit is being developed at Airport Road Hospital. Mediclinic also points to faster referral and approval pathways and more interoperable health data as practical enablers for complex precision studies.⁵⁴

Strategic implication



For selected studies, the differentiator may be visibility and continuity rather than volume: the ability to identify the right patient, connect that patient to the right study, and retain the evidence generated over time.



Chapter 4: Treat: The Care Pathway Becomes Part of the Therapy

Earlier intervention and advanced therapies are reshaping care pathways, reimbursement, and long-term follow-up.

Reflecting on the role of earlier intervention, Dr. Nicole Sirotin, CEO of the Institute for Healthier Living Abu Dhabi (IHLAD), notes: "Novel therapies will expand what we can treat. Some of the most immediate gains, however, may come from using established interventions earlier and more precisely. In Healthy Longevity Medicine, nutrition, physical activity, and sleep are part of evidence-based care. Research shows healthy lifestyle habits can add up to 20 additional healthy years to someone's life. Some of the most exciting drug discoveries right now involve repurposing already approved drugs for healthspan benefits beyond their original indications. Drug candidates are also being studied for their potential to target biological processes of aging rather than a specific disease. Ultimately, the value of any intervention has to be demonstrated in people's lives through clear clinical outcomes, beyond a change in one biomarker."⁶¹

The treatment spectrum is widening in both directions, earlier intervention and advanced therapies. Risk markers and molecular monitoring can bring intervention forward, while cell, gene, and regenerative approaches seek durable correction in selected diseases. That shift changes the care pathway around medicine. Some patients may enter care before symptoms become obvious, and complex therapies may only be deliverable at specialist sites. Access decisions can also depend on evidence that continues to mature after treatment. The practical challenge is keeping the pathway connected from the first signal through referral and treatment into coverage and follow-up.^{2,32-36}



Earlier Signals Need a Clinical Action

Longitudinal biomarkers can reveal biological change before conventional symptoms or imaging. In SERENA-6, serial circulating tumour DNA identified an emerging ESR1 resistance mutation and guided a switch to camizestrant before radiological progression, extending progression-free survival relative to continued standard therapy. The signal was useful because it led to a validated treatment decision.⁵⁵

Burjeel's experience reinforces the operational side of that point. Timely access to molecular testing and a novel therapy still depends on clinical teams being able to interpret the test and select, dose, and monitor treatment appropriately.⁵³

Burjeel Cancer Institute puts it simply: "At the hospital level, however, access must be matched by readiness."⁵³

Cleveland Clinic Abu Dhabi provides another specialist-site example through the OPGx-MERTK inherited-retinal-disease gene-therapy study, where it serves as the clinical site within a DoH – AD supported program.¹⁸

Disease interception moves the decision earlier, sometimes to a point when the person feels well. That can be valuable when damage is irreversible, but it also raises the cost of being wrong. A positive signal therefore needs confirmation before it triggers treatment, and the threshold for intervening should rise with the risk and irreversibility of the therapy. Where uncertainty remains, it needs to be made clear to the patient, with specialist review and close follow-up providing an additional safeguard against unnecessary or harmful intervention.⁵⁶

Before routine use, an advanced therapy has to answer four practical questions:

1. Who is eligible, and how certain is the evidence?
2. Can the product be made and administered safely at the intended site?
3. Will coverage extend to the prevention, diagnostics and supportive care around the medicine?
4. And which outcomes will be followed closely enough to inform later clinical and funding decisions?

Clinical Delivery and Access Need the Same Evidence

Ex-vivo cell therapy shows how heavily delivery can depend on the hospital. The patient's cells have to move through collection and manufacturing before the final product returns to the same person under an unbroken chain of identity. The treating centre must also be ready to manage acute toxicity. Expert centres are therefore likely to retain assessment and administration, while wider provider networks handle referral, rehabilitation, and routine follow-up closer to home.¹²

Mediclinic Middle East reaches a similar conclusion from the provider perspective: "The successful adoption of next-generation therapeutics will depend as much on the clinical infrastructure surrounding these treatments as on the therapies themselves."⁵⁴

The financial pathway has to reflect the time horizon of the clinical benefit. A one-time therapy can carry a very high upfront price, but for a durable or potentially curative treatment the relevant comparison is the lifetime burden of the disease it may replace. This matters particularly for conditions treated early in life, where recurring medicines, hospitalisation, supportive care and disease-related complications can otherwise continue for decades. Public payers therefore need to consider value over a longer horizon than the year in which treatment is administered, while still accounting for uncertainty over how durable the benefit will be.¹³⁻¹⁵

Equitable access becomes more challenging as therapies become more specialised and costly. Differences in access can arise before treatment itself, including whether patients can obtain the required diagnostic testing and specialist assessment, whether they are referred to qualified treatment centres, and whether coverage extends to the supportive care and follow-up required around the therapy. Equitable access therefore depends on both affordability and pathway design, with funding and referral mechanisms that reduce avoidable variation in access for clinically eligible patients.¹³⁻¹⁵

Payment models can also help manage the affordability and uncertainty associated with advanced therapies, but outcomes-based approaches depend on reliable data. The US Cell and Gene Therapy Access Model ties payment to patient outcomes and provides participating Medicaid programs with a common framework for implementation and data collection. Reimbursement can therefore reflect how the therapy performs after treatment, including through rebates when agreed outcomes are not achieved.⁵⁷

Abu Dhabi is Linking Access to Outcomes and Long-term Value

Abu Dhabi has already coordinated these decisions in practice. The ITVISMA case moved from accelerated approval through health-technology assessment and coding to referral and specialist administration. DoH - AD's 2025 introduction of CASGEVY through Abu Dhabi Stem Cells Center adds experience with an ex-vivo gene-editing pathway. Taken together, the cases show how faster access can be paired with assessment, coverage, and specialist delivery when there is a credible case for improved outcomes and long-term value. The same principle can extend upstream to diagnostics and prevention, where earlier action can reduce later disease burden and cost.^{21,58}

The Rare Diseases Centre of Excellence being activated by DoH - AD, PureHealth, and AstraZeneca is designed to connect diagnosis, specialist care, research, and lifelong follow-up in a single pathway. PureHealth's provider and payer capabilities create a strong base for coordinating eligibility, coverage, and outcome tracking. As the centre is activated, it can make those decisions more continuous for patients and create a long-term evidence base for future therapies.⁵⁹

PureHealth sees the connection between provider and payer data as another advantage. Linking clinical outcomes from SEHA with claims and cost data from Daman could allow evidence generated through routine care to support outcomes-based agreements with manufacturers and faster access to new therapies. As Rashed Al Qubaisi, Group Chief Operating Officer, PureHealth, puts it, "real-world evidence becomes a by-product of good care rather than a separate project."⁶⁰

Strategic implication



As therapies become more complex, the pathway around the product increasingly becomes part of the therapeutic proposition. Reusable clinical, regulatory, funding, and follow-up mechanisms can make future adoption faster and more predictable across the therapeutic lifecycle.

Conclusion: Abu Dhabi: Building A Repeatable Path From Science To Patient Impact

Therapeutic progress is arriving at different speeds. Some new modalities already produce clear clinical benefit, while others are still at proof of concept. AI is improving selected research tasks, and its wider effect on development productivity will become clearer as more programs reach the clinic. For health systems, the central question is how to turn a credible scientific advance into a treatment that can be studied, produced and delivered reliably.

Translation is easier when scientific and clinical work are connected before a product is ready to launch. Researchers need reliable routes into governed data and clinical expertise, while regulatory and clinical preparation can begin while development is still underway. Evidence collected after treatment then feeds back into care and coverage, so later programs can build on what the system has already learned.

Abu Dhabi already has elements of this model in place. Population genomics and biobanking are increasingly connected with health data, while local trial operations and specialist care provide routes into clinical use. Early programs are beginning to show how those pieces can work together, adding practical experience while the global therapeutic pipeline is still taking shape.

The next phase is to make that pathway more systematic. Four areas can build on what is already in place:

1. Expand precision clinical trials by using genomic and longitudinal health data to identify defined cohorts, connect sponsors with specialist sites, and make study start-up more predictable
2. Create clearer and more equitable managed-access pathways for innovation so promising therapies, diagnostics, and preventive interventions can reach appropriate patients while remaining evidence is collected. Outcomes-based reimbursement, such as the US Cell and Gene Therapy Access Model, provides one relevant model⁵⁷
3. Deepen selected platform and workforce capabilities in the therapeutic areas where Abu Dhabi has a scientific or clinical advantage, while continuing to connect with specialist capabilities elsewhere
4. Make long-term evidence reusable and strengthen lifecycle oversight so outcomes and safety signals collected after treatment inform future clinical guidance, reimbursement decisions, and the design of subsequent programs

That model creates a distinct proposition for organisations working on the next generation of therapeutics:

For research institutions: access to population biology, biospecimens and longitudinal clinical information with routes into translational and clinical partners

For biotech and biopharma companies: access to identifiable patient cohorts, specialist clinical talent and trial infrastructure that can support faster recruitment and study start-up, with a more efficient route into local clinical development

For the health system: better population outcomes through earlier intervention and more effective treatment, with the potential to reduce avoidable admissions, disease burden, and long-term costs while supporting economic activity in the local life-sciences sector

**Her Excellency Dr Noura Khamis Al Ghaithi,
Undersecretary of the Department of Health – Abu Dhabi.**

"Scientific advances are changing what is possible in medicine, giving us new ways to understand disease and develop increasingly precise treatments. The opportunity now is to ensure that this progress can be translated into therapies that deliver meaningful outcomes for patients. Abu Dhabi has built strong foundations across genomics, health data, clinical research, and specialist care. By connecting these capabilities more closely and working with leading partners globally, we can create a clearer path from scientific discovery to patient care, helping promising therapies reach the patients who can benefit while continuing to build the evidence that will shape the future of treatment."

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Future Health is a year-round platform for global collaboration and transformative health innovation, held under the patronage of His Highness Sheikh Khaled bin Mohamed bin Zayed Al Nahyan, Crown Prince of Abu Dhabi and Chairman of the Abu Dhabi Executive Council.

Bringing together health researchers, policymakers, business leaders, investors and entrepreneurs from across disciplines and geographies, Future Health drives collective action to strengthen health systems and unlock longer, healthier lives for all.

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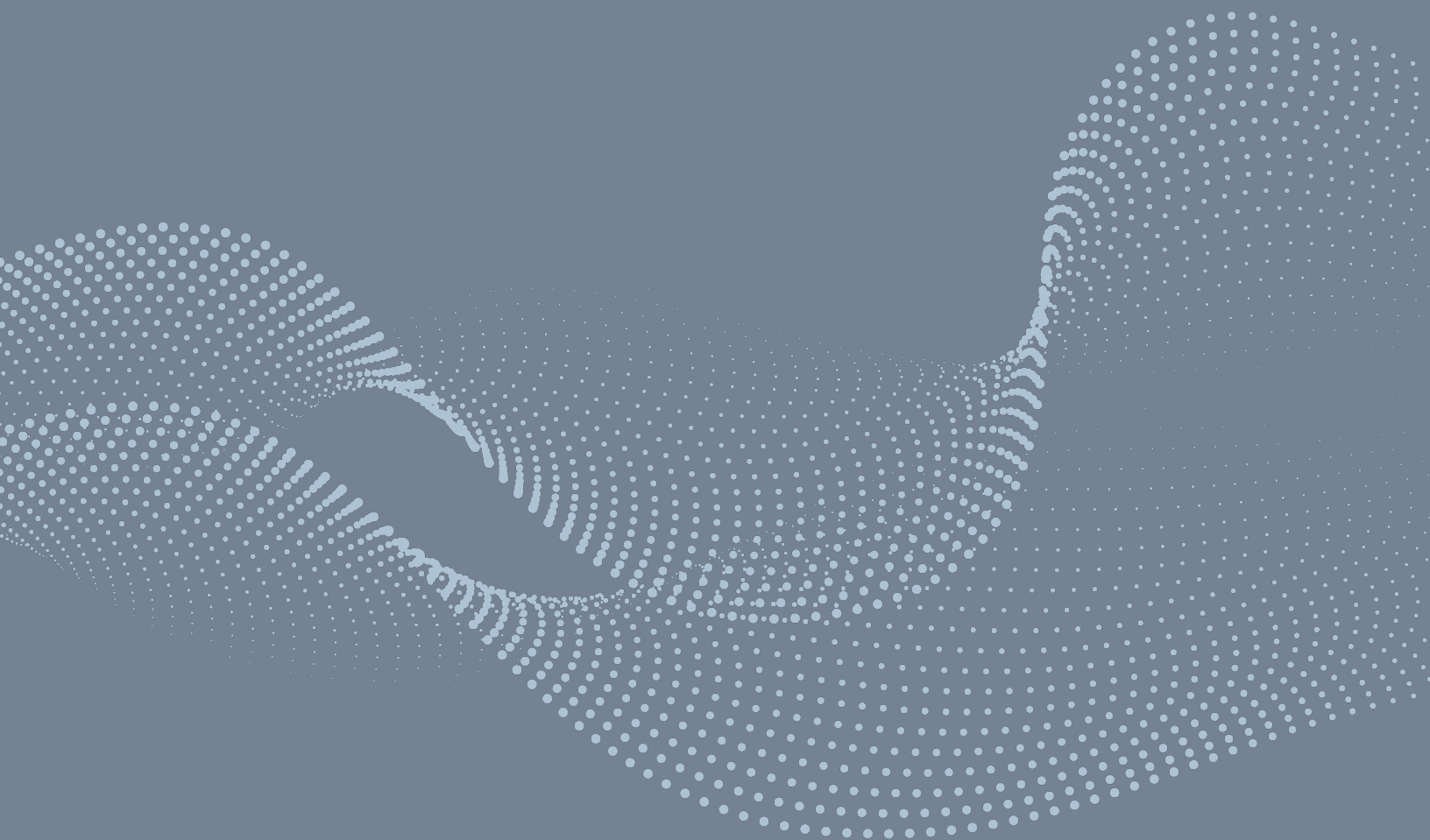
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