Insight Brief

# Beyond the Benchmark: How Innovative Trial Designs are Transforming Clinical Research

## *Biotech trends and strategies that shape therapeutic development*

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# Introduction

The biotech market is undergoing a dynamic reset that is reshaping not just its landscape, but also the strategic path toward drug development. Companies are contending with uncertainties in both funding and investments, while also having to navigate the complex, ever-changing regulatory environment.

One way biotech and biopharma companies can gain a competitive edge and push their therapies to market faster is to optimize their R&D spending while delivering robust scientific data. The importance of innovative trial design as well as leveraging strategic partnerships is a critical path forward in accelerating R&D processes.

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# Emerging trends in biotech

*Murray Aitken shares insights from the 2024 Annual Global Trends in R&D report significant to biotech sponsors.*

The 2024 Annual Global Trends in R&D report provides an insightful look into the ongoing evolution within the biotech sector, revealing key trends that are reshaping its landscape and setting new directions for future growth and innovation. Among these trends, the report identifies several critical areas:

## Volatile funding

The biotech funding landscape has been highly volatile, experiencing a downturn in 2022 and 2023 following the COVID-19 pandemic, before rebounding with an 18% increase in investment in 2023 (Figure 1). This momentum continued into early 2024, with $47 billion in funding, sustained by moves like Bristol Myers Squibb's $13 billion investment offering, suggesting that the industry is undergoing a period of adjustment and renewal.

**Biopharma funding levels per quarter Q1 2018 - Q1 2024 ($Bn)**

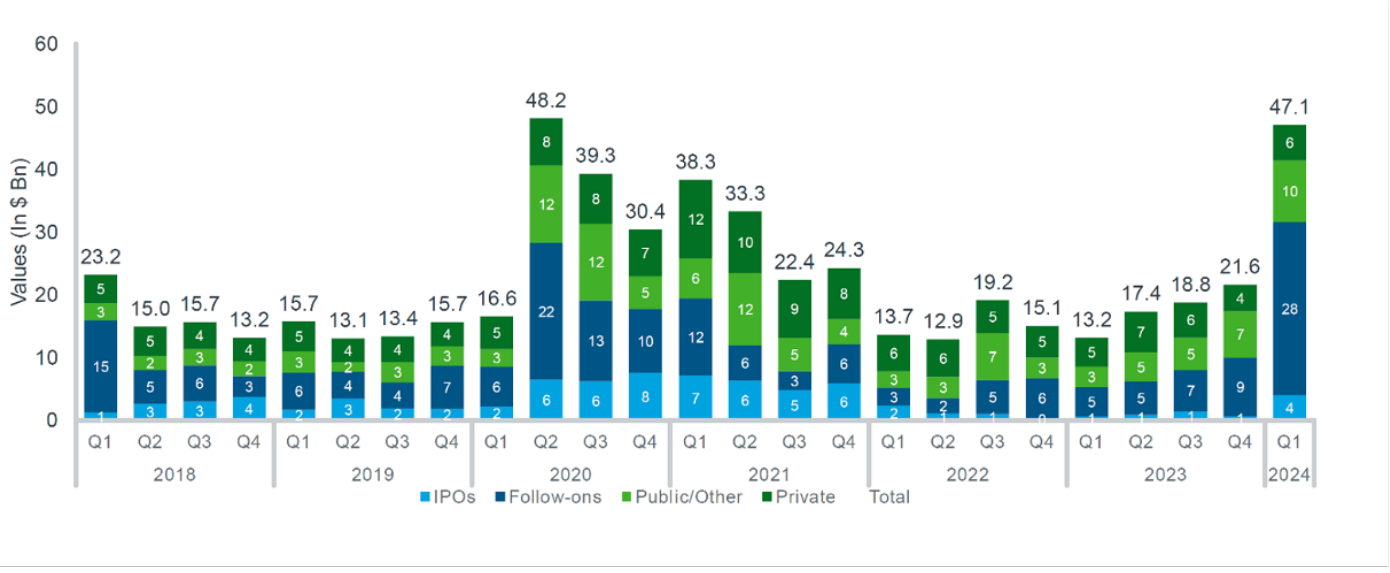


**Figure 1. Quarterly biopharma funding levels have fluctuated widely over the past four years.**

**Source: BioWorld**
**Global Trends in R&D: Overview through 2023. Report by the IQVIA Institute for Human Data Science**

## Reduced clinical trial activity

Clinical trial activity dropped by 22% in 2023 compared to 2021, partly due to a reduction in COVID-19 vaccine and therapeutic trials (Figure 2), as the frantic pace of pandemic-related research slowed down. Emerging biopharmaceutical companies (biotechs), responsible for nearly two-thirds of clinical trial starts, initiated far fewer trials, mainly in early-phase studies.

**Share of clinical trials starts by phase and company segment, 2014-2023**

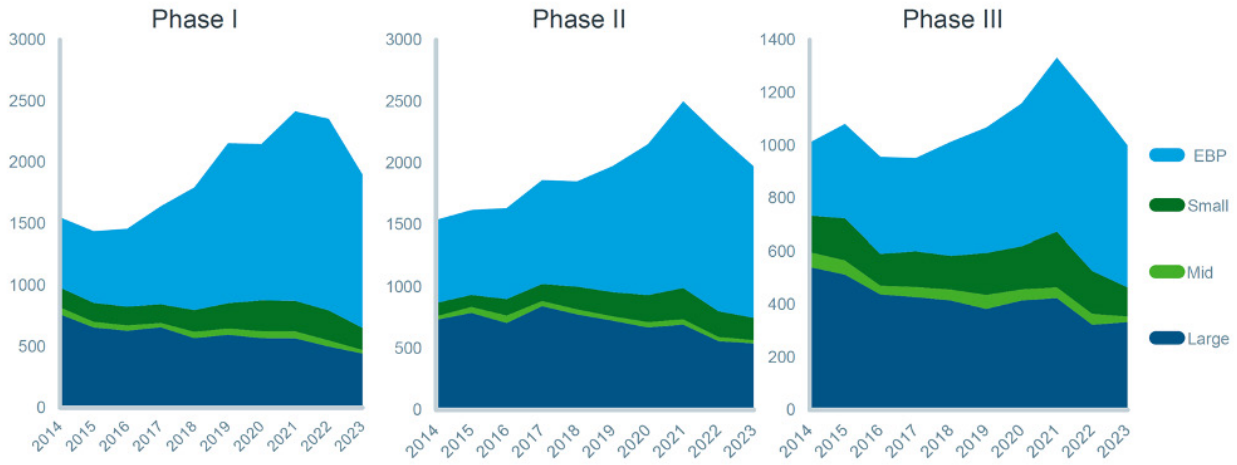


**Figure 2. Emerging biopharma companies are responsible for two-thirds of trial starts, but declined the most since the peak in 2021.**

**Source: Citeline Trialtrove, Jan 2023; IQVIA Institute, Jan 2024**
**Global Trends in R&D: Overview through 2023. Report by the IQVIA Institute for Human Data Science.**

## Growing biotech contributions to therapeutic launches

Biotech sponsors play a critical role in the clinical research that is underway today. These organizations account for approximately two-thirds of trial starts in Phase I and 60% in Phase II. Despite reduced activity over the past two years, they remain at the center of research and development efforts. Large pharma companies have seen a decline in activity, although their Phase III involvement increased slightly in 2023. This reaffirms the biotech community's role in pushing the boundaries of clinical development and scientific discovery.

Biotech sponsors also drive pharmaceutical innovation and regulatory approvals. Data shows that in 2023 alone, they were responsible for 56% of all novel active substance (NAS) and drug approvals, consistently maintaining a majority share. Furthermore, many of these biotech-originated drugs are managed and submitted for regulatory review by the sponsor themselves, showing their control over the development process from inception to market (Figure 3).

**Companies originating and filing U.S. Food and Drug Association (FDA) regulatory submissions for NASs and percent of launches by NAS launch year, 2014-2023**

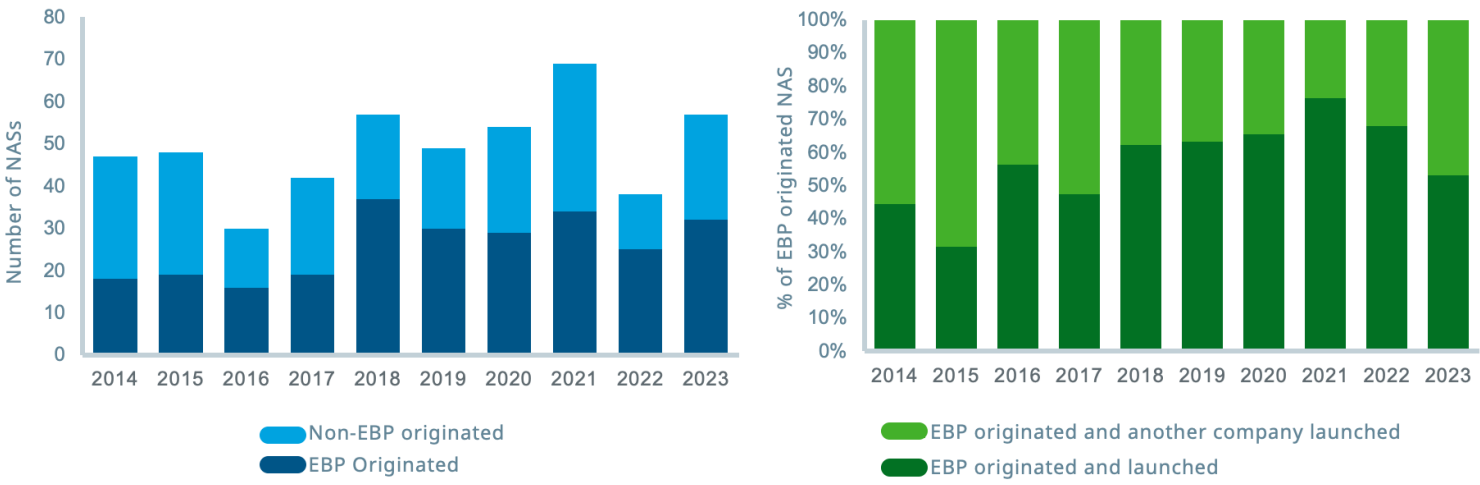


**Figure 3. Emerging biotech companies originated 56% of all new drugs in 2023 and launched 53% of them, less than in recent years.**

**Source: IQVIA Institute, Jan 2023**
**Global Trends in R&D: Overview through 2023. Report by the IQVIA Institute for Human Data Science.**

## The evolving regulatory landscape

Many initiatives are being introduced to improve development efficiency and accelerate access to medicines.

These efforts include closer collaboration with developers, fast-tracking approvals, and granting exclusive marketing rights for treatments targeting rare diseases and children's health issues. The Accelerating Clinical Trials in the EU (ACT EU) initiative seeks to optimize trials, offering National Authority scientific advice and quicker trial-specific guidance, similar to the U.S. model.

In the U.S., the FDA's Center for Drug Evaluation and Research (CDER) launched the CDER Center for Clinical Trial Information (C3TI) to improve trial efficiency and increase participation from diverse populations. This aligns with the FDA's draft guidance on master protocols and the push for diversity plans by the end of Phase II. The U.K. is also moving to include more underrepresented groups in studies.

Project Optimus in the U.S. highlights the FDA's focus on optimizing doses for new cancer treatments, addressing the unique toxicities of modern immunotherapies compared to traditional chemotherapy. This initiative exemplifies international regulatory collaboration, with significant global participation and more than 70 product approvals since 2019.

Regulatory incentives have significantly invigorated rare disease and pediatric-focused clinical research. Initiatives such as market exclusivity for promising therapies encourage quicker approvals, pivotal to advancing pediatric drug development.

# Optimizing delivery of clinical development programs

In an era where biotech companies are at the forefront of clinical development, two-thirds of all trial initiations originate from small biotechs. However, this coincides with a period of funding volatility, during which investors demand more data to bolster their confidence in investments and company valuations to ultimately raise capital.

Organizations need a compelling value story, intelligent solutions and expert insights to successfully transition from preclinical to commercialization in a competitive environment. Additionally, there's mounting pressure to accelerate R&D processes and timelines to get to proof of concept or top-line results faster.

Key questions companies must ask as they advance their clinical development program include:

- **What are the right clinical endpoints to meet regulatory and patient needs?**
- **How do we demonstrate value to payers, prescribers and patients alike?**
- **What is our strategy to navigate the complexities of the healthcare system?**
- **What is the optimal design for a clinical trial?**

## Implementing innovative trial design

The traditional, single-arm randomized clinical trial has long been the foundation of drug development. However, the landscape is changing. New trial designs, such as adaptive trial design, are emerging, providing greater flexibility and adaptability to meet the complex challenges of clinical and commercial development.

Adaptive trial design allows changes based on participant data, improving statistical efficiency, ethical standards, and treatment insights. The FDA values these benefits, which can lead to faster delivery of therapies.

Innovative designs also use external data to create robust control arms, strengthening evidence for marketing and reimbursement. Decentralized clinical trials enhance operational efficiency by improving patient participation through telemedicine, home-based treatments, and digital data collection, which can speed up patient enrollment by 78%.

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The FDA's guidance on digital health technologies for remote data collection signals growing acceptance of these approaches. Embracing these advancements will make clinical trials more efficient and productive, accelerating the delivery of new treatments to patients.

# Generative AI: Balancing innovation with caution

Generative AI is transforming clinical trials by optimizing operations and enhancing data management, from planning to regulatory outcomes. By rapidly analyzing protocols and managing data, AI streamlines trial processes. However, its adoption comes with challenges such as data privacy, security concerns, bias, inaccuracy and ensuring accountability. Addressing these requires robust data lineage, transparent validation processes and integrating AI within standard business operations, with ultimate responsibility resting on humans. With FDA approval of AI-driven digital therapeutics, the industry

acknowledges AI's revolutionary impact. Success in this evolving landscape depends on partnering with experts in technology, science and analytics, especially for emerging companies navigating unique development paths.

### Core principles

- Productivity increase
- Relief of burden
- Optimized trial outcomes
- Safeguards and consistency

### Areas of opportunity

- Patient safe data review
- Compliance monitoring
- Inspection readiness
- Data quality
- Operational fluidity
- Patient experience

### Strategic GenAI solution mapping

- Design
- Delivery
- Monitoring & compliance
- Patient engagement

### Maximized validation

- Consistent engineering in a safe and careful manner
- Continuous improvements and fact-checking

## Conclusion

The biotech industry is navigating a dynamic landscape that is shaped by funding fluctuations, evolving regulatory frameworks and innovative trial designs. In recent years, funding has experienced volatility with a downturn then followed by a rebound in investment. Regulatory bodies have also remained steadily current to the growth by creating initiatives that help aid trial activities. While clinical trial activity has decreased, partly due to reduced COVID-19 trials, biotech sponsors remain pivotal in drug development, driving research in Phases I and II.

IQVIA Biotech is a biotech-specialized CRO delivering flexible clinical development solutions for biotech and emerging biopharma companies. Our clinical solutions are built on 25 years of unmatched experience with therapeutically aligned expertise, uniquely designed to deliver full-service solutions on a global scale. Learn more at iqviabiotech.com

# About the authors

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Meg Hooton serves as President of IQVIA Biotech, a specialized business unit within IQVIA dedicated to providing clinical development solutions for biotech and emerging biopharmaceutical customers. With more than 30 years of experience in managing global research and development operations across pharmaceutical and biotech sectors, Meg brings a wealth of operational and cross-cultural expertise to her role. Her remarkable journey—from nursing to biotech leadership—reflects not only her wealth of operational insights but also her cross-cultural expertise and unwavering commitment to advancing healthcare.

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Keith McDonald is a seasoned expert with over 25 years in regulatory affairs and drug development. His extensive regulatory authority expertise in clinical trials, marketing authorization approvals and policy development makes him an expert in our industry. His many contributions include providing regulatory authority scientific advice to leading the UK Covid Therapeutics Taskforce, where he shaped policy and secures marketing authorizations.

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As head of the IQVIA Institute for Human Data Science, Murray Aitken provides policy setters and decisionmakers in the global health sector with evidence, analysis, and insights that contribute to the advancement of Human Data Science to improve human health outcomes.

Murray is tasked with creating and managing a research agenda that leaders in global governments, payers, providers, academia, and the life sciences industry use to accelerate the understanding of global trends in disease patterns, data science, and technology. This research is used to foster innovation critical to evidence-based decision-making and the advancement of human health.

He holds an MBA, with distinction, from Harvard University and a Master of Commerce from the University of Auckland in New Zealand.

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