

**CLINICAL  
TRIAL BLAZERS**

## **Faster paths to proof of concept:**

How biotech companies  
approach acceleration

**parexel**<sup>®</sup>  
**BIOTECH**



Accelerating clinical development is a priority across the biopharma industry, but the pressure for speed is often most intense among small and mid-sized biotechs. While their leanness allows them to respond with agility to shifting landscapes, it also makes them susceptible to significant financial risk. By reaching proof of concept as efficiently as possible, biotechs can make the best use of their resources, putting expertise and funding behind the products most likely to succeed in the market.

Recently, Parexel asked biotech leaders across the U.S. and Europe about strategies to accelerate their development work. We surveyed 120 decision makers at small and mid-sized companies with an annual R&D spend of up to \$200 million and \$1.5 billion, respectively. We also held in-depth conversations with vice presidents and C-level executives at 10 U.S. and European biotechs.

Through this research, we've identified the strategies biotechs believe are most important to their success as well as the challenges that most significantly impact them.

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### Research methodology

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**120** decision makers surveyed



small and mid-sized biotechs  
(R&D spend up to \$1.5B)

**10** in-depth executive interviews



U.S. and European companies



**Key strategies for accelerating clinical development according to 120 biotech leaders.**

	Biotechs currently using this strategy	Biotechs planning to use this strategy	Is implementing this strategy a major or critical challenge?
Pursuing regulatory pathways for accelerated review	54%	41%	27% said yes
Implementing innovative trial designs or biostatistical approaches	51%	37%	20% said yes
Expanding research to regions beyond the U.S. and Western Europe	47%	38%	33% said yes
Incorporating real-world data and evidence into clinical trials	33%	51%	14% said yes
Incorporating AI into clinical trials	26%	59%	39% said yes

When asked about the circumstances shaping biopharma development, the 10 biotech leaders with whom we spoke at length named the same trends and challenges identified in our larger survey.

Figure 1

**Trends or challenges most impacting clinical development, according to 10 biotech leaders.**

	What level of impact does or will this trend have on clinical development timelines?	
	Now	In the future
Expanding research to new geographic regions	High	High
Changes in the regulatory landscape	Moderate	High
Adoption of innovative trial designs	Moderate	Moderate
Use of real-world data and evidence in clinical trials	Low	High
Use of AI or advanced technology in clinical trials	Low	High

In addition to an overview of our findings, this report offers insights into the strategies on the rise among biotech innovators.

Figure 2

## Global expansion: Capitalizing on opportunities outside of the U.S. and Western Europe

While the U.S. and Western Europe continue to be centers for biotech development, regulatory uncertainties and slower startup times are pushing sponsors to expand into other global regions. This allows biotechs to:

- › increase their access to patients.
- › select regions based on speed of regulatory processes and operational execution.
- › reduce spending by working in lower-cost regions.
- › secure access to sites with less competition.

According to our survey, small and mid-sized biotechs are currently prioritizing **Australia** for early-phase trials, thanks to relatively short regulatory timelines and ample access to key opinion leaders. Biotech leaders are also interested in locating studies in:

- › **China**, where a large general population often leads to faster patient recruitment. The cost of running a trial in China is also significantly lower than in the U.S.
- › **South Korea**, a scientifically and technologically advanced country in which regulators are often aligned with the FDA.
- › **Japan**. Conducting trials in Japan allows biotechs to prepare for commercial opportunities in the country and protect future value.
- › **Eastern Europe**, as countries in this region are eager to support biopharma development.

### Why biotechs are expanding globally



Increase access to patients



Select regions based on regulatory speed



Reduce spending in lower-cost regions



Secure access to less competitive sites



### Survey respondents indicate regions of interest for clinical development expansion.

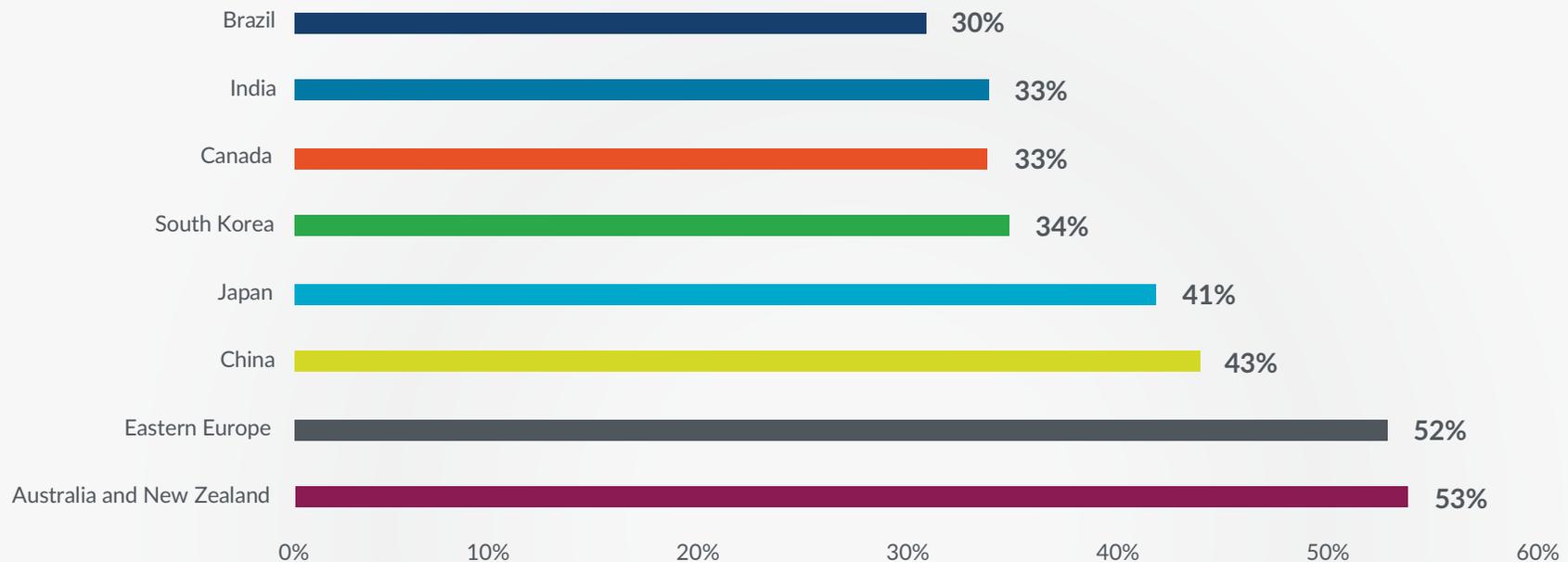


Figure 3

As biotech leaders consider their options for expansion, they need greater understanding of region-specific operational and logistical challenges as well as broader insight into patient recruitment strategies and enrollment rates. Many were also interested in working with CROs that have staff in these regions as they were hesitant to execute trials in a country in which they had no local presence.

In addition, survey responses point to a clear knowledge gap around region-specific regulatory and submission requirements. We found that mid-sized companies often need more information on which regions are best suited to specific therapeutic areas or indications.

**70%**

of biotech leaders say accelerated regulatory pathways have high or significant impact on clinical development timelines

## **Regulatory strategy:** Pursuing accelerated pathways and engaging in scenario planning as early as possible

In our research, 70 percent of survey respondents aligned on accelerated regulatory pathways as a major driver of faster development. Within the FDA in particular, these pathways shorten overall review and approval timelines, reduce the likelihood of trial design modifications and allow biotechs to prioritize studies with the fastest potential path to market.

Most biotech leaders we spoke with pursue accelerated regulatory pathways whenever appropriate – most commonly through the FDA’s Breakthrough Therapy or Orphan Drug Product Designations. “Once we have clinical data [to support our position], we’ll certainly apply for Breakthrough [Therapy] Designation, which is important,” one U.S. C-level leader told us. “And we’re evaluating all these [additional regulatory] approaches to see if we can really utilize all [of them].” Like this executive, many biotech decision makers seek to combine accelerated pathways when possible.

Survey responses indicated that biotechs need to better understand how much time can be saved through specific regulatory programs as well as regulatory designations and programs for specific therapeutic areas or indications. U.S. biotechs need more insight into regulatory benefits and hurdles in other countries.

To ensure alignment on approval pathways, the leaders we surveyed are increasing their proactive engagement with regulatory authorities – interactions that also help hold regulators to agreed-on timelines and standards. Taking initiative with regulators will continue to be critically important for biotechs, which develop novel products that often have greater safety commitments. Biotech leaders will also, however, need to conduct evidence-based regulatory scenario planning as early as possible – long before protocols are locked and capital is committed.



## Survey respondents name countries into which they'd like greater regulatory insight.

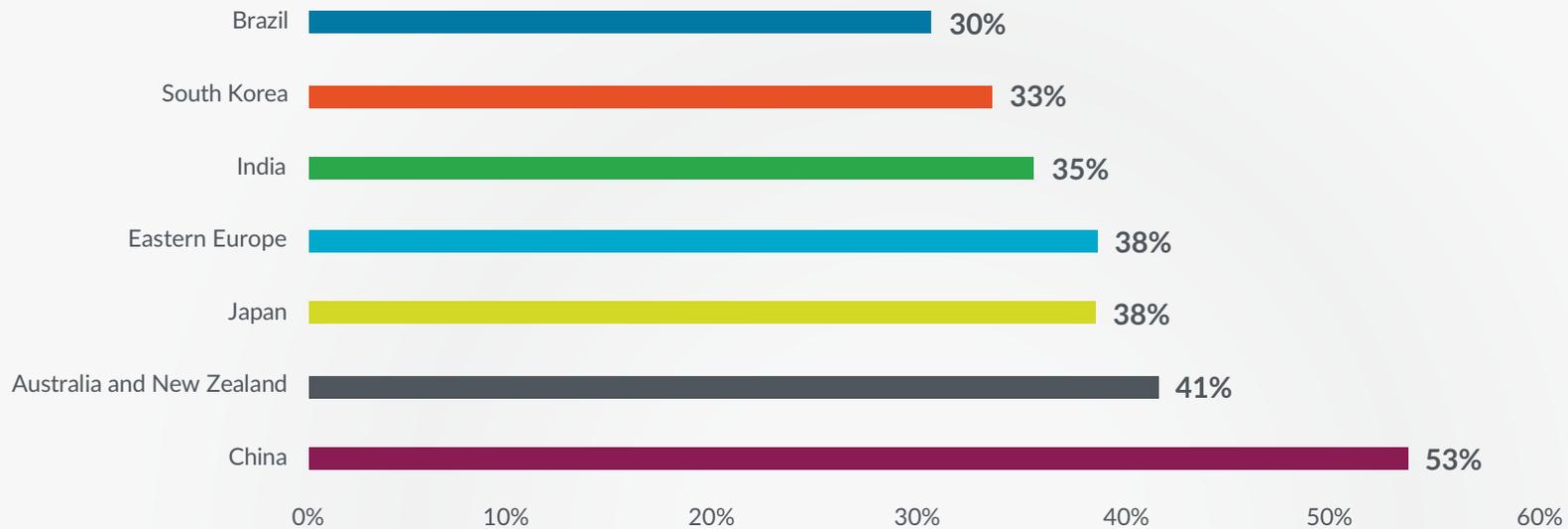


Figure 4

When asked about challenges regarding regulatory strategy, biotech leaders were most concerned about the variability and complexity of regulations across global regions as well as the perceived difficulty of navigating evolving regulatory landscapes. In such situations, biotechs can partner with a CRO that offers global regulatory expertise and on-the-ground specialists in the regions in which biotechs need to operate.



## Operational innovation: Leveraging adaptive and seamless study design, RWE and AI

According to our research, biotechs are increasingly exploring the use of innovative trial designs and statistical approaches. Biotech leaders are particularly interested in innovative methodologies that allow them to predict the probability of success as early as possible in development. Sixty-three percent of survey respondents said that innovative trial designs and biostatistical approaches have a high or significant impact on development timelines.

Biotech leaders say they are currently:

- › Using interim data from adaptive trial designs to adjust sample size, dose levels and endpoints.
- › Using seamless trials to quickly transition between phases.
- › Taking note of innovative trial designs that are proven in one therapeutic area, such as oncology, and modifying them for other areas.

Biotech leaders told us they want to learn more about regulatory expectations and requirements for innovative designs and

about synthetic or external control arms that can reduce patient enrollment needs. Regulatory uncertainty and lack of clear regulatory guidance are the top challenges to implementing this kind of innovation.

When asked about using Bayesian or other statistical models to extract rich information from small sample sizes, biotech leaders acknowledged that this approach can be especially valuable for rare disease research with extremely small patient populations. Decision makers, however, need to better understand how to implement such models. They also need better ways to communicate the value of these models to stakeholders, whose buy-in is critical.

The leaders we spoke with and surveyed also cited real-world evidence (RWE) as one of the factors most influencing innovative study design. Developers can use RWE to:

- › inform endpoint selection and trial comparators.
- › replace control arms in studies with niche populations.
- › support and potentially accelerate regulatory submissions.
- › demonstrate value when negotiating reimbursement agreements.

### Real-world evidence: Four key applications



Inform endpoint selection and trial comparators



Replace control arms in niche populations



Support and accelerate regulatory submissions



Demonstrate value in reimbursement negotiations

## Survey respondents indicate areas of interest regarding RWE in clinical research.

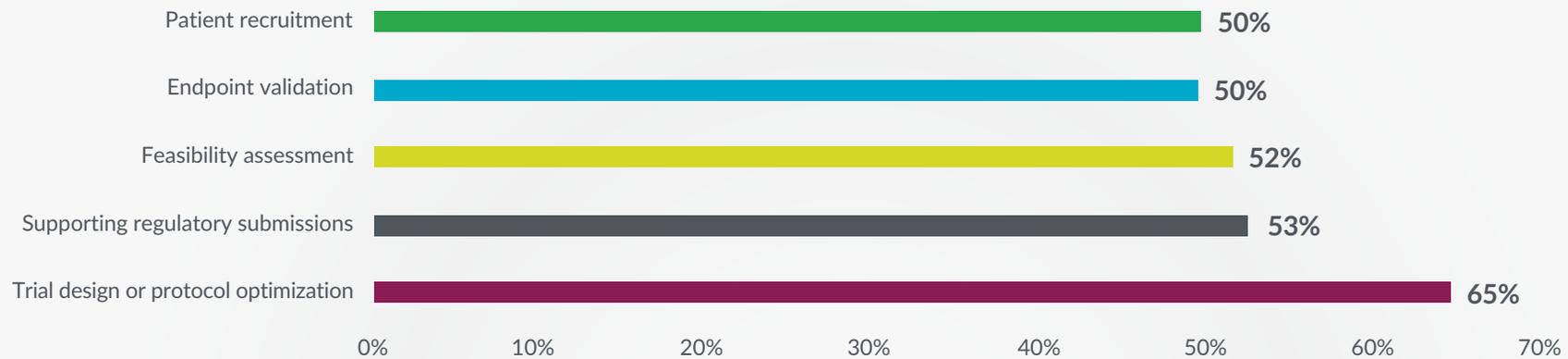


Figure 5



Our research shows, however, that many biotech companies lack experience in adaptive or seamless trial design and RWE and are averse to risk, which may slow adoption of innovation. In particular, they cite concerns about the quality and completeness of RWE.

We also asked biotech leaders about artificial intelligence (AI). While their current use of AI is limited, they believe it could transform clinical development going forward. To facilitate this transformation, CRO partners will need to provide biotechs with specific, compelling use cases for incorporating AI into the development process. “In the future ... [AI] will help us better assess feasibility of sites, the ability to enroll patients, and number of patients in areas. I think AI can help us where there’s large amounts of data that have to be sorted through to find trends or answers,” one C-level U.S. biotech executive told us.

## Well-informed decisions made faster

A biotech's success will be determined, in large part, by its ability to streamline development processes and, as a result, decision making. "We're trying to ... identify early on these assets that will have the highest likelihood to be able to perform – and also make the difficult decision to close programs early on," a vice president at a mid-sized European biotech told us.

Biotechs need data and insights that enable them to make informed decisions as early as possible – from selecting global study locations to setting regulatory strategy to designing studies most likely to lead to faster approvals. In the coming year, Parexel will closely examine what biotechs can do to set up their programs for success.

**Ready to accelerate your path to proof of concept?** Get in touch to learn more. We're always available for a conversation.

Contact us at [Parexel.com](https://www.parexel.com)

For more insights and perspectives from Clinical Trialblazers, [explore our hub](#).

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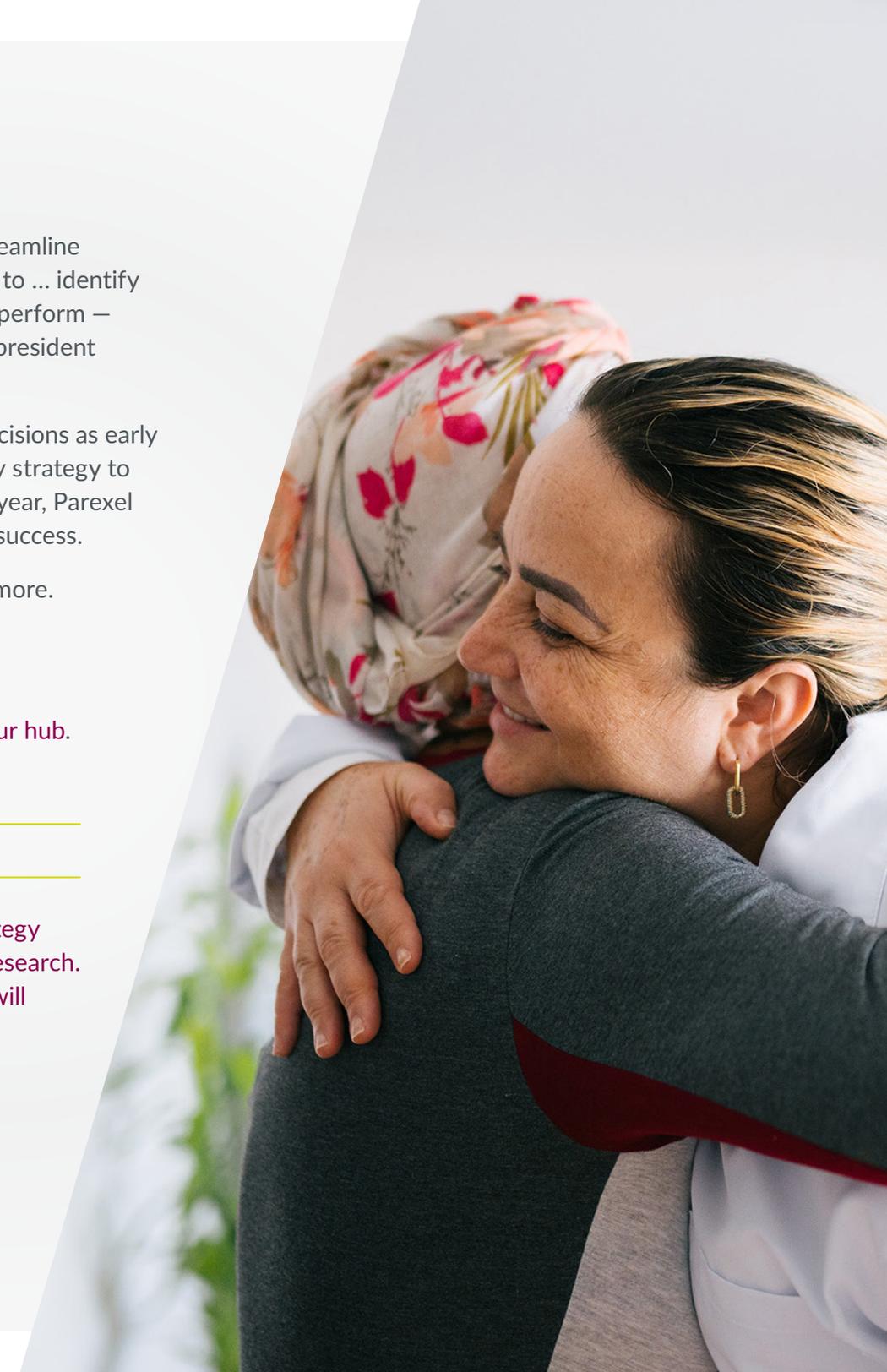
### The path forward

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Success requires taking advantage of every available strategy – from advances in technology to emerging regions for research. Biotechs that combine multiple acceleration approaches will reach proof of concept faster and more efficiently.

*Parexel would like to acknowledge the Life Science Strategy Group (LSSG) for its collaboration on this research.*





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