THE Q1 2020 BIOPHARMA CONFIDENCE INDEX: **WHERE BIOPHARMA IS HEADED AND HOW CROS CAN HELP THEM GET THERE**

WORLDWIDE CLINICAL TRIALS

KINETICOS LIFE SCIENCES

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THE 2020 BIOPHARMA CONFIDENCE INDEX

In its recent cosponsored survey of the biopharmaceutical industry, Worldwide Clinical Trials and Kineticos Life Sciences solicited companies' self-assessed capabilities, perceived market risks, anticipated areas for growth, and trends affecting the industry. Together, the participants have provided their opinions of the current state of the biopharmaceutical industry, as well as where they see the industry heading in the next 1, 3, or 5 years.

Who participated?

Respondents included C-suite and executive leadership at companies in North America, Europe, Asia-Pacific, Latin America, and elsewhere around the world. Most respondents (54%) were from privately held companies, with the second largest respondent pool (33%) from start-up biopharma (<$250M market cap), followed by emerging biopharma ($250M to <$1B market cap) with 9% of respondents, and large ($5B+ market cap) and midsized biopharma ($1B to <$5B market cap). Therapeutic focus of respondents largely favored oncology and immunology, followed by infectious or rare diseases, neuroscience, and other fields.

Statement concerning COVID-19.

Please note, the data in this survey were collected prior to the COVID-19 global pandemic. The survey results reported herein are based on the survey's time of issue and reflect pre-pandemic expectations for market performance and company beliefs and activities. In limited cases, this document provides expert predictions for the impact of the pandemic on some of the survey's key topics. And thank you for your interest in making these partnerships work at the highest level!

Internal Capability Shortcomings in Key Industry Functions

Respondents were asked to rate their confidence in their own company's ability to perform in three key areas: clinical development, chemistry manufacturing controls (CMC), and patient recruitment.

Companies of all sizes reported high confidence in their clinical development capabilities, which could perhaps reflect access to CROs for companies of any size. Among start-ups, high confidence in clinical development abilities may also reflect their optimism and enthusiasm for their new products.

In CMC and patient recruitment, however, private companies reported the highest confidence and start-ups the lowest, which may reflect the experience and resources present in bigger firms. Patient recruitment—a top pain point for most clinical trial programs—should be grounded in broad recruitment projections based on thoroughly vetted projected data. Start-ups ranked their own confidence in this area poorly, likely due to inexperience in or the increasingly small patient populations affected by many new therapies under study.

In terms of key commercial activities—field sales, field reimbursement specialists, and health economics and outcomes research (HEOR)—broad gaps emerged between belief in importance of a skill and perceived internal ability (see Figure 1).

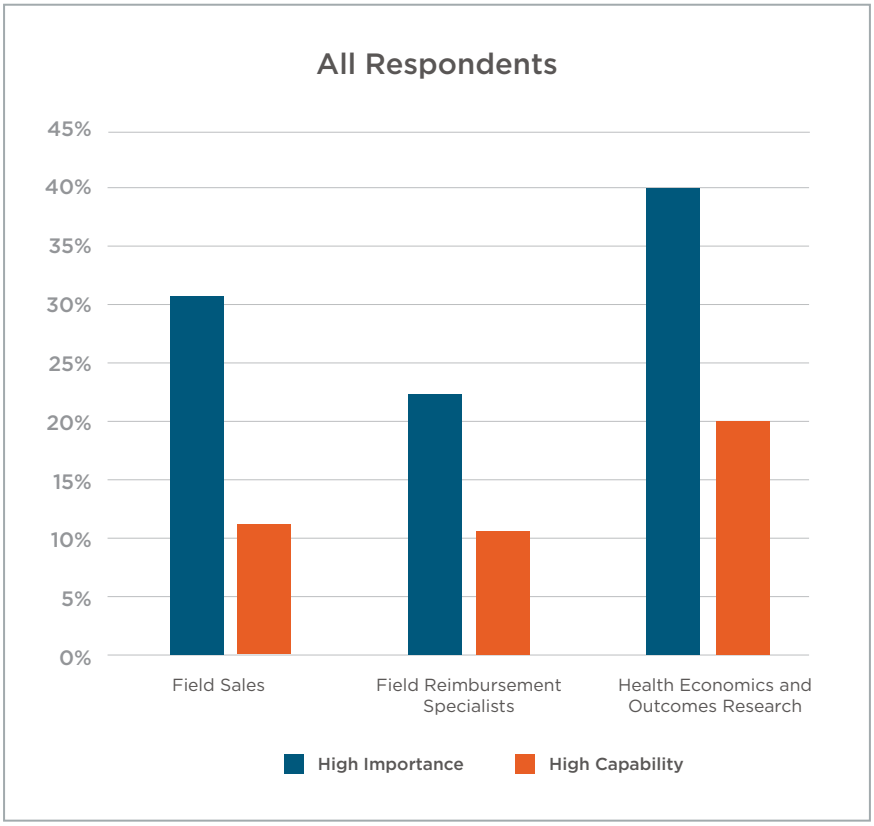


Figure 1. *Companies (of any size) reporting high importance of vs. high confidence in internal capabilities in selected activities.*

All companies reflected a much higher belief in the importance of these activities than in their own internal capabilities to perform in those areas. This may explain the shift in the past decade toward more extensive post-launch and field strategy support from CROs, in addition to the traditional assistance sought for the operational aspects of trials.

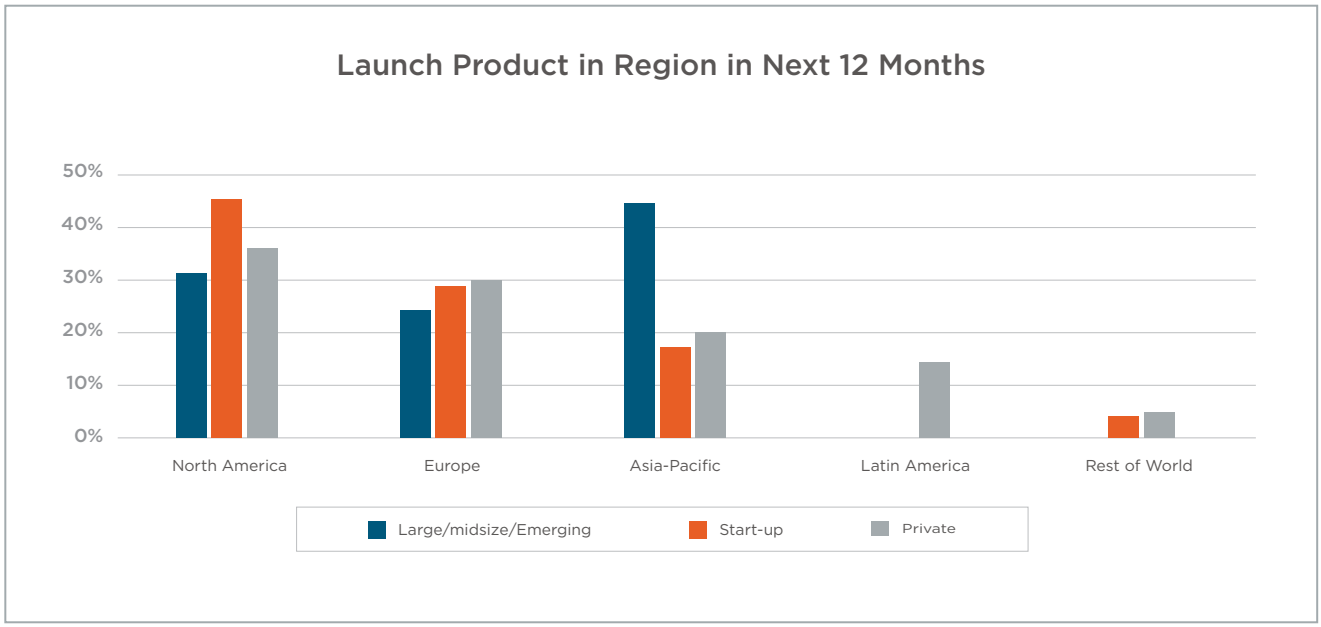
Impact of Global Markets and Politics

The ability to strategically and proactively manage the regulatory process is crucial to any drug development program's success. In general, companies expressed their greatest level of confidence with the regulatory processes in the United States, followed by the European Union, Asia-Pacific, and Latin America. However, companies with a regional presence in Asia-Pacific and Latin America reported high levels of confidence in these regulatory processes.

The disparity in regional confidence is, not surprisingly, strongly linked to having "boots on the ground"—local experts in place who understand how things operate and what is required for success. It also reflects which regions have the most well-established, protocolized regulatory processes. This regional variation in confidence may also explain why CROs with global networks are highly sought out by the biopharmaceutical industry.

When asked where they intend to conduct clinical trials in the coming 12 months, respondents selected regions (for the percent of trials expected in each region) in the same order in which they had expressed confidence in the regulatory process. Interestingly, having a regional presence was not correlated with intended product launches. The greatest activity is planned in North America and Europe, with a considerable number of large companies also planning launches in Asia-Pacific (see Figure 2).

Figure 2. *Intent to launch product in coming 12 months, by region and company size*



Worldwide Tip: *A consulting CRO's global footprint and diverse locations are important. More crucial, however, is a CRO's ability to solve problems in an integrated manner, using experts from all functional teams to advise proactively about the inevitable hurdles a drug will face before, during, and after clinical development. This can reduce risk and save money—no matter where or when the journey takes place.*

Political and Economic Risk. When asked which global economies would have a positive impact on the biopharma industry over the next 12 months, companies expressed the most confidence in the United States, followed by Asia-Pacific and Europe at confidence levels considerably lower but still strongly leading over Latin America. However, a country-specific ranking of potential risk to investments caused by political pressures cited the United Kingdom and the United States as having the most potential risk, followed by China. It is likely this reflects uncertainty in the regulatory processes and political changes that could be produced by Brexit and the U.S. presidential election.

These two factors may have also affected respondents' expectations for what aspects of the industry they felt likely to be impacted in the coming year by political climate. The highest risk level was reported for approaches to pricing and new pricing models. Overall expectations for growth strategy and capacity strategy are expected to remain unchanged compared to the past 12 months (see Figure 3).

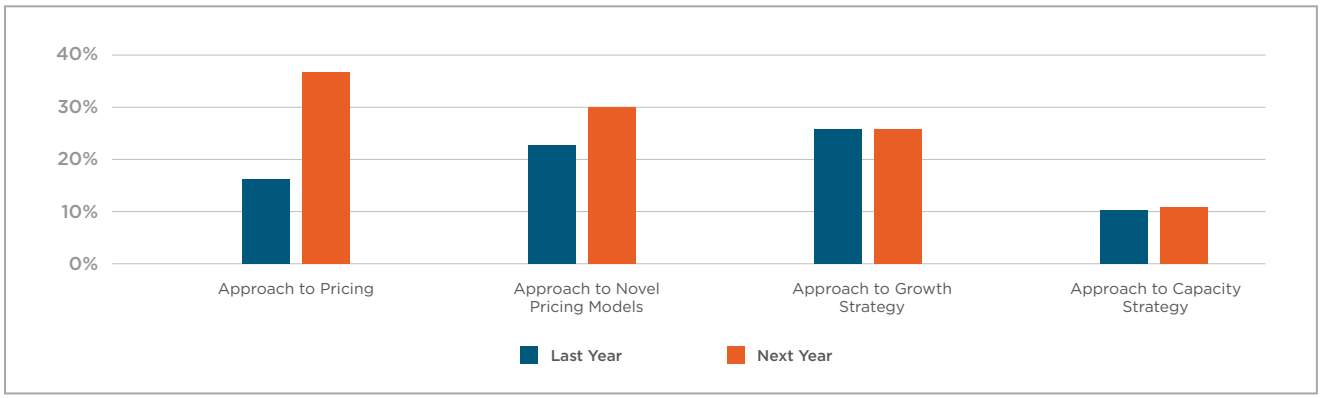


Figure 3. *Percent of respondents expecting high impact of political climate on biopharma planning for last 12 months and next 12 months.*

High Confidence in Raising Capital and Mergers & Acquisitions

Despite uncertainties regarding global economies and the political climate in the U.S., respondents expressed fairly high confidence in capital markets in the coming 12 months. However, large, midsize, and emerging companies had a confidence rate almost half that of start-ups, which may reflect their greater experience with fluctuating funding environments.

When asked about the expected percent change in valuations of biopharma companies in private markets, such as venture capital, private equity, and angel investors, respondents reported a positive expectation for the coming 12 months, often with an anticipated increase of at least 10%.

This optimism carried over to the expected percent change in merger and acquisition (M&A) activity in the coming 12 months, with many respondents predicting increases of 10-20% or more. Companies singled out acquisition of pipeline assets, followed by acquisition of new technology, as their top reasons for M&A activity (see Figure 4). Large companies are not looking as much for global expansion as their smaller counterparts, likely because they already have extensive networks.

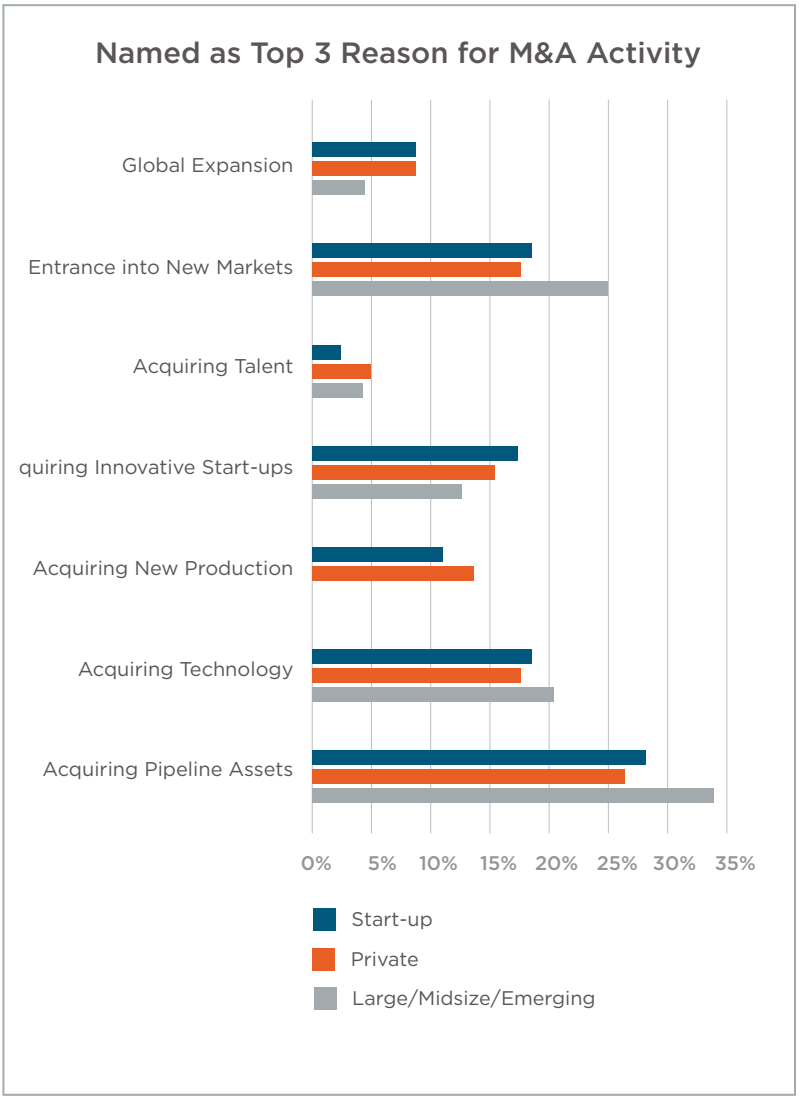


***Figure 4**. Selections of activities as a Top 3 reason for mergers and acquisitions, by company size.*

In general, it is clear that companies want robust pipeline health that enhances their company's financial standing, and they have a fairly positive outlook for the coming year's investment potential.

"If coronavirus causes a substantial downturn in what the current markets look like, there is too much data out there to suggest anything other than that this will be a short-lived downturn. These companies understand the markets that they're in and how to weather these short-term issues and move forward."

- Peter Benton, MBA, President and Chief Operating Officer, Worldwide Clinical Trials

Licensing Confidence Variations Throughout the Developmental Process

As would be expected based on the comparatively large number of trials consistently occurring in this therapeutic area, oncology is, by far, the most popular indication in which companies expect to have a candidate for licensing in the coming year (see Figure 5). When companies chose their top three indications in which they were most likely to have a potential candidate for licensing, rare diseases and immuno-oncology were also popular choices. The fact that rare diseases were not often selected as a top response for licensing may reflect the difficulty in commercializing a product for a very small population.

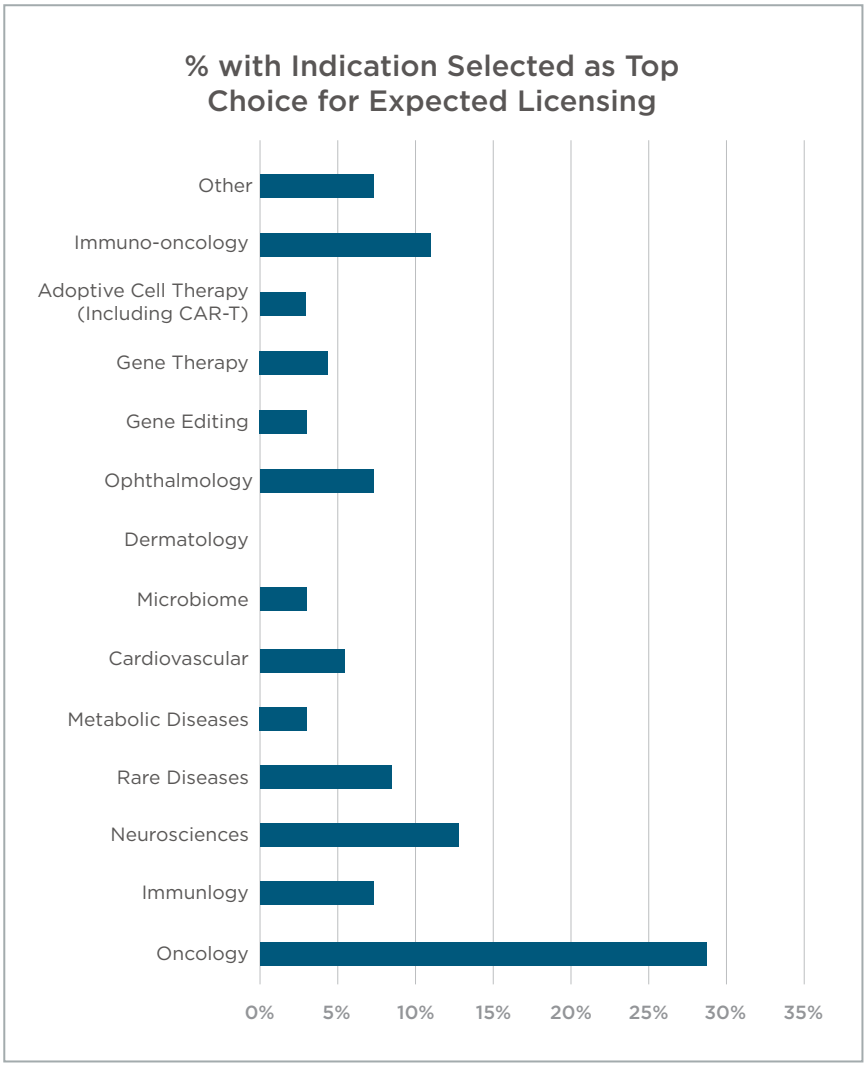


Figure 5. *Percent of respondents (all companies) selecting a given therapeutic area for their number 1 licensing candidate.*

The further along in the development process, the lower the risk and the greater the ability to out-license. Importantly, however, this does not always translate to a higher valuation.

Not surprisingly, most companies expressed increasing confidence in the ability to out-license a product the further along in the development process it is (~75% reported high confidence in their ability to out-license a commercial asset). This confidence is in stark contrast to company reports of high intent to out-license at each of these stages. Start-ups, for example, report the greatest intent to out-license at preclinical and clinical stages.

Developing the product is only one aspect. Do you have a regulatory strategy plan? Are you thinking about reimbursement strategy? What about technology assessments outside of the U.S.? You need to think about all of these puzzle pieces and make your transitions when there is a suitable inflection point."

- Aman Khera, Global Head of Regulatory Strategy, Worldwide Clinical Trials

Increasing ROI for Artificial Intelligence in Specific Therapeutic Areas

Survey respondents were asked several questions about their predictions for the importance of a range of emerging technologies in the near future. Of particular interest was response by therapeutic area regarding the expected importance and return on investment (ROI) for artificial intelligence (AI) and machine learning technologies. Companies focused in cardiovascular diseases (CVD) reported the greatest confidence in the importance and positive ROI of this type of technology, perhaps because of the early shift toward wearable technology in this field and the large number of easily monitored, relevant clinical indicators (such as blood pressure, heart rate, oxygen saturation, and more) (see Figure 6).

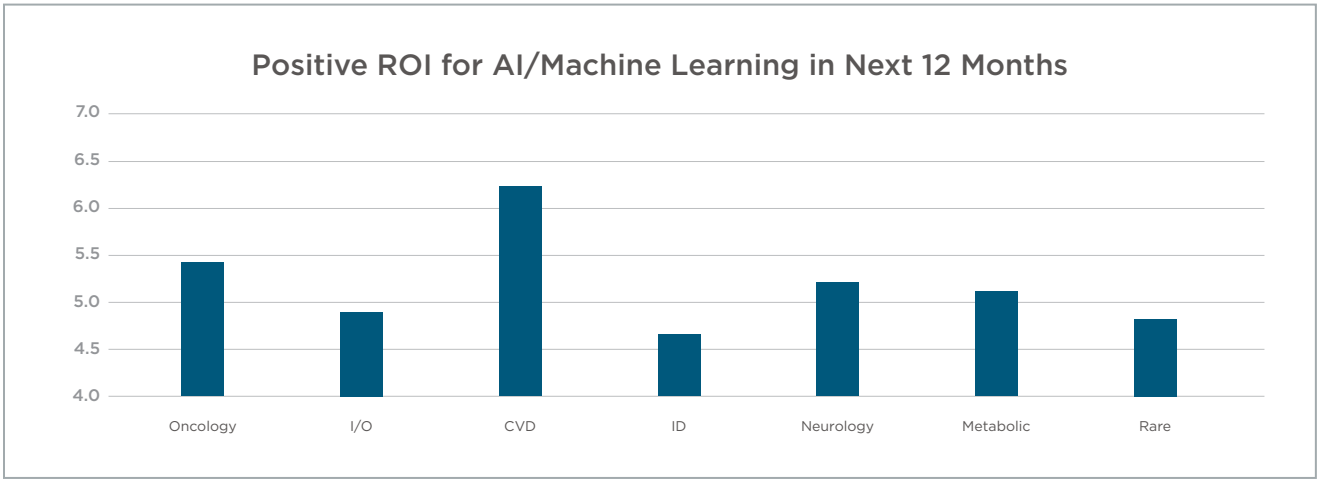


Figure 6. *Confidence (out of 10) in a positive ROI in the coming year for AI/machine learning, by respondent therapeutic area.*

Confidence in positive ROI for this technology is surprisingly lower for some fields, such as metabolic conditions, but perhaps reflective of the tendency for some therapeutic areas to be trailblazers and others to be followers. Use of AI and machine learning is still a fairly new approach to patient care, clinical trial data collection, and postmarket monitoring. As early advances work their way through new regulatory processes, generate data, and demonstrate their utility, it is likely other fields will see the value in these emerging technologies and their increased potential for drug development and commercial success.

This may be why companies across therapeutic areas of focus believe these approaches will provide breakthroughs in delivering new therapies and drugs in the next 3 to 5 years. Companies in most fields also predict the growing importance of these technologies over the next 5 years, in both preclinical and clinical development and commercial planning and execution.

Worldwide Tip: *As AI and machine-learning technology advances, the commercial-clinical feedback loop could transform the clinical development process through improved data collection. This has the potential to transform the pace and value of clinical work, provided companies develop ways to meaningfully classify and interpret the influx of data.*

Mixed Expectations for Alternative Sales and Marketing Channels

Participants were asked how confident they were in their expectations for increased integration of alternative sales and marketing channels to emerge in the next 1 or 5 years. The reported focus and confidence in increased integration of alternative sales and marketing channels varies by therapeutic area, with highest long-term confidence in integration in immunology and oncology, with slightly less anticipated in CVD, neurology, and rare diseases. Furthermore, only immunology, oncology, and neurology anticipate growth in integration by 5 years from now.

Overall confidence in increasing integration of alternative sales and marketing channels is also not high for now or the future for any company type, although midsize and large companies were slightly more confident than start-ups that this will occur. This may reflect that start-up companies are less focused on sales and marketing compared to larger companies, which commercialize and market more.

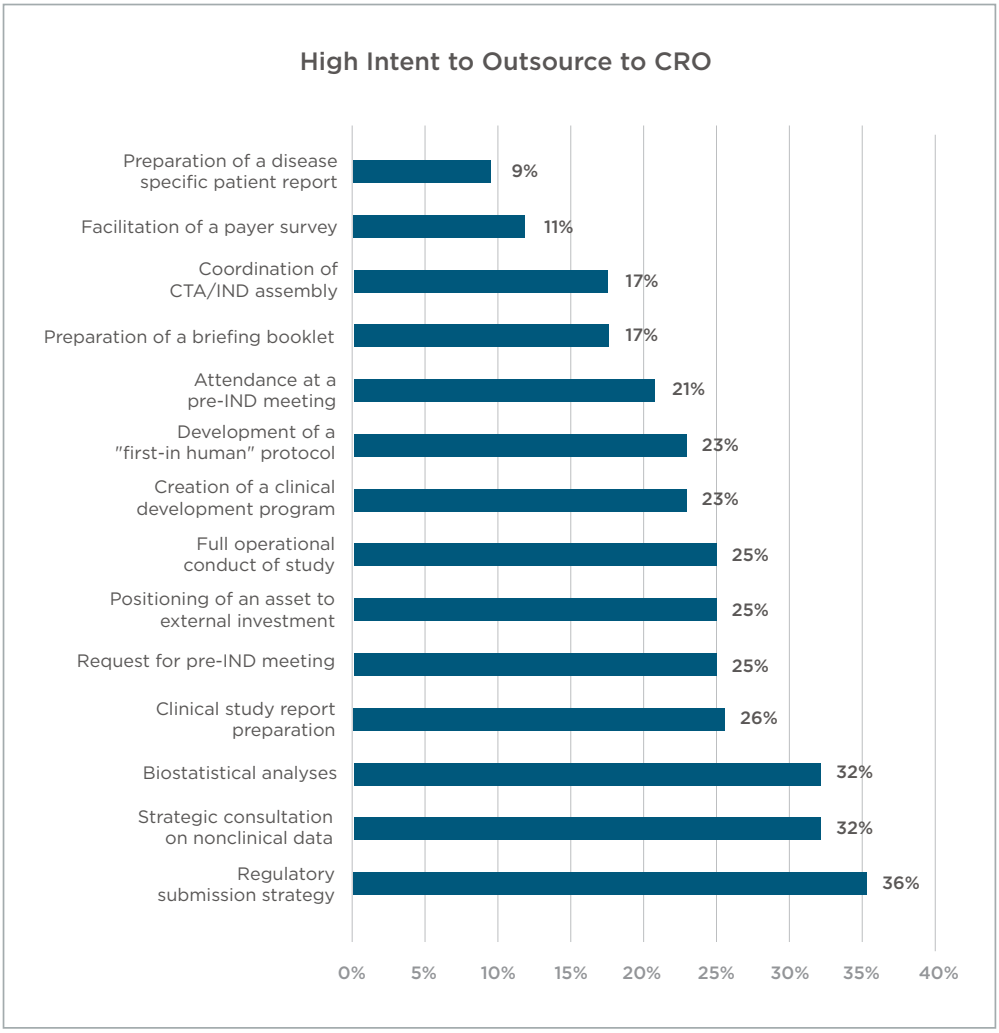
Almost across the board, companies expect the need for medical science liaisons (MSLs) to go up and the need for field sales to diminish. Large companies expressed the most confidence in the increased demand for MSLs and diminished need for field sales, both in the short- and 5-year term. Among therapeutic areas, only companies in ID anticipated the reversed trend over time.

There are increasingly pinpointed, strategic ways to market. Emerging therapies tend to have smaller target populations than the blockbuster drugs of the past, making specialized, alternative marketing techniques more economically effective than field sales in many cases. Companies that adopt the most efficient, flexible strategies will reap the greatest ROI for their sales and marketing efforts.

A CRO'S ROLE IN BIOPHARMA CONFIDENCE AND SUCCESS

As evidenced by respondents' replies regarding internal capabilities, it is clear that no biopharmaceutical organization can do everything alone. By bringing in talent, experienced strategists, and full-service partners, biopharma can improve their ROI, streamline their development process, navigate regulatory hurdles, and ease market entry. Survey respondents expressed high intent to outsource certain activities, key among which were regulatory submission strategy, strategic consultation on nonclinical data, and biostatistical analyses (see Figure 7).

Figure 7. *Companies (of all sizes) reporting high intent to outsource certain activities to a CRO.*



This finding fits with the trends seen by Worldwide experts, as clients increasingly ask for advice on preclinical or postmarket strategy, regulatory support, and sophisticated approaches to small but difficult trials. Patient engagement and retention, portfolio valuation, preclinical planning, clinical development decision-making, and other inflection points along the development trajectory are additional strong areas in which a thoughtful, engaged CRO can make a big impact on a company's success.

Identifying Opportunities to Maximize Success

Worldwide believes there are underappreciated opportunities for pharmaceutical companies of any size to partner with CROs to achieve other key – perhaps unrecognized – goals.

1

CROs can help companies bring an asset to market that is attractive to many buyers.

By advising small or start-up pharma about early differentiation of their products, CROs can help companies determine if the end goal should be partnering in development or selling an asset sooner rather than later. This proactive planning also helps remove hurdles and maximize the number of opportunities available for development and sales.

As companies reach critical decision points, face ethical quandaries, and contemplate widely varied and complex financial alternatives, a CRO can help consolidate information and simplify the options.

2

CROs can balance short- and long-term strategies to support business sustainability.

Early consultation can identify the best ways to obtain preclinical data required for first-in-human programs, the paths of least resistance for obtaining regulatory approval, and methods to maximize the amount of information obtained from a single study—before a company even initiates its first clinical trial plan.

CROs should strategically reduce risk for a burgeoning program through proactive strategies and an informed, responsive relationships with pharmaceutical clients. This can dictate a drug's appeal to payers and its market performance in many respects.

3

CRO positioning insights and diligence reporting can maximize an acquisition's ROI.

For companies on the purchasing side of a potential deal, well-rounded CROs can offer valuable research and insights into the likely consequences—both positive and negative—of acquiring a particular asset. Not only does this reduce risk and enable defensible, strategic decision-making, it also ensures that money spent on acquisitions or alliances will help companies be well prepared for success.

CROs can explore any asset's potential value in different indications or real-world payer scenarios through advanced analytics. In addition, a CRO can identify possible synergies with a company's existing assets for clinical programs, indication opportunities, payer bundling, and more.

"Small companies need to define the value of their product very early. We can answer questions like, 'Which target should we go for?' We can support companies through inflection points to make a decision to further develop or to sell."

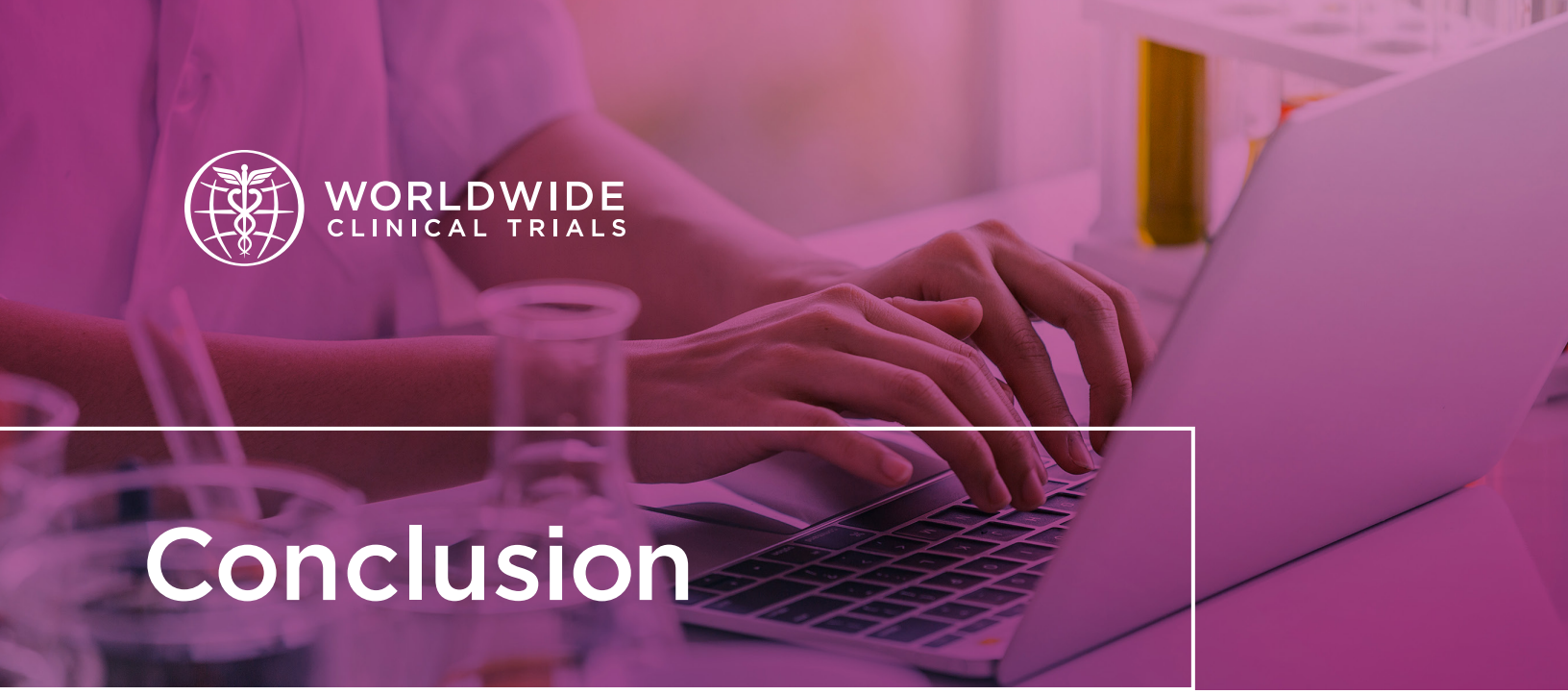
- Michael Murphy, MD, PhD, Chief Medical and Scientific Officer, Worldwide Clinical Trials

"You are adjudicating multiple, usually conflicting, points of interest. It involves acknowledging these diverse demands and balancing, recalibrating—making it work. Worldwide acknowledges the different demands, accommodates them, and finds a solution that satisfies everybody."

- Michael Murphy, MD, PhD, Chief Medical and Scientific Officer, Worldwide Clinical Trials

"You need to know your end game. We can help identify what makes sense as an investment, where more due diligence is required, and where to focus to get the most bang for the investment buck."

- Sherilyn Adcock, RPh, PhD, Executive Vice President, Scientific Solutions, Worldwide Clinical Trials



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Conclusion

Survey respondents have painted an optimistic picture for the future of the biopharmaceutical industry. While acknowledging the importance of strong emerging markets and global economies, companies of all sizes have expressed confidence in the future of drug development and collaborations. In addition, they have identified areas of opportunity: new scientific and technological areas that are likely to change the shape of the industry in the coming years and areas in which their companies require improved capabilities.

Hand in hand with the acknowledged need for improved capabilities comes the biopharmaceutical industry's continued desire to collaborate with CROs to achieve their clinical development, regulatory approval, and statistical and analytical goals. This type of partnership has a strategic value and a purpose that can transcend the typical business relationship to transform the therapeutic landscape, streamline the development and approval process, optimize opportunities for novel molecules, and enable sustainable success for new products.

When it comes time to make great things happen, consider a CRO partner with uncommon expertise, unsurpassed commitment, and unbridled problem-solving capabilities. From preclinical to postmarket, enjoy the benefit of a single, full-service strategic partner.

Worldwide—from start to finish.