2021 Industry Trend Report

The Future of Emerging Biopharma and Biotech

An In-Depth Look at the Clinical Development Market and Trends Driving Innovation

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The Inflection Point of Emerging Biopharma and Biotech

Emerging biopharma and biotech companies have reached an inflection point: After a heightened period of dealmaking, capital building and regulatory approvals, small to midsize biotechs are now confronting new challenges and opportunities in a post-pandemic era. While clinical development has inevitably changed, the transformation has also made space for new possibilities within a sector the whole world is now watching.

Importantly, emerging players were already on pace for record-setting growth: By 2018, of the more than 8,000 active programs underway, about four in five of them originated from small or midsize sponsors.[1] In 2020, these trajectories accelerated with increased backing from venture capitalists, Wall Street and acquirers as big deals pushed forward emerging biotech. Biotech venture transactions, for example, grew more than 50% from 2019 to 2020. Some IPOs commanded more than $200 million in value.[2]

As this exciting growth took shape, biotech leaders expanded their clinical footprint to new areas. In addition to legacy disease states like immuno-oncology and rare diseases, innovations in Parkinson's and Alzheimer's have put neuroscience biotechnology on the map. These and other diversifying developments mean there's more to watch for among regulators, too.

New challenges facing emerging companies include:

- trial complexity[3]
- increased drug development costs
- need for data collection and management
- demand for experienced personnel
- regulatory scrutiny
- heightened competition
- pressure to deliver therapies quickly

As sponsors forge their way through these barriers, optimizing their clinical trial supply strategy — including enrollment efforts and delivery — in a cost-effective, patient-centric way has now become mission critical.

Facing a growing demand for speed, agility, affordability and inventory, emerging biotech companies that optimize the use of experienced supply chain managers, advanced technology platforms and efficient global logistics will likely see the most success in this moment.

Each sponsor, study and clinical need require an individual assessment, including examination of recruitment projections, patient dosing, randomization requirements, analysis of clinical markets and timeline expectations. Building the design, packaging and distribution strategy that responds to those needs — combined with an IRT setup and more — can add layers of complexity, regulatory risk and added costs.

That's why many sponsors are carefully evaluating their selection of contract development and manufacturing organization (CDMO) partners to an even greater extent than they did before. Together with an adaptable partner experienced in these changing market conditions, emerging biopharma and small to midsize biotech players can make sure their therapy advances efficiently and effectively through protocol to patient delivery.

Optimizing that CDMO relationship involves a great many challenges. Detailing what emerging biotech companies should know as they journey toward commercialization, we'll explore the current landscape of emerging biotech — as well as the opportunities and challenges ahead. We'll also discuss what makes a good partner and how to find the right one in this changing biotech environment.

After all, new possibilities await. It's simply a matter of navigating to them.

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Current Industry Landscape

Market Size and Projections

Among the most significant recent drivers of the $752 billion biotechnology industry and its nearly 16% projected growth from 2021 to 2028[4] are those companies now known as "emerging." The term is generally applied to biotech and biopharma players that have a yearly research and development (R & D) spend below $200 million or revenue under $500 million.[1]

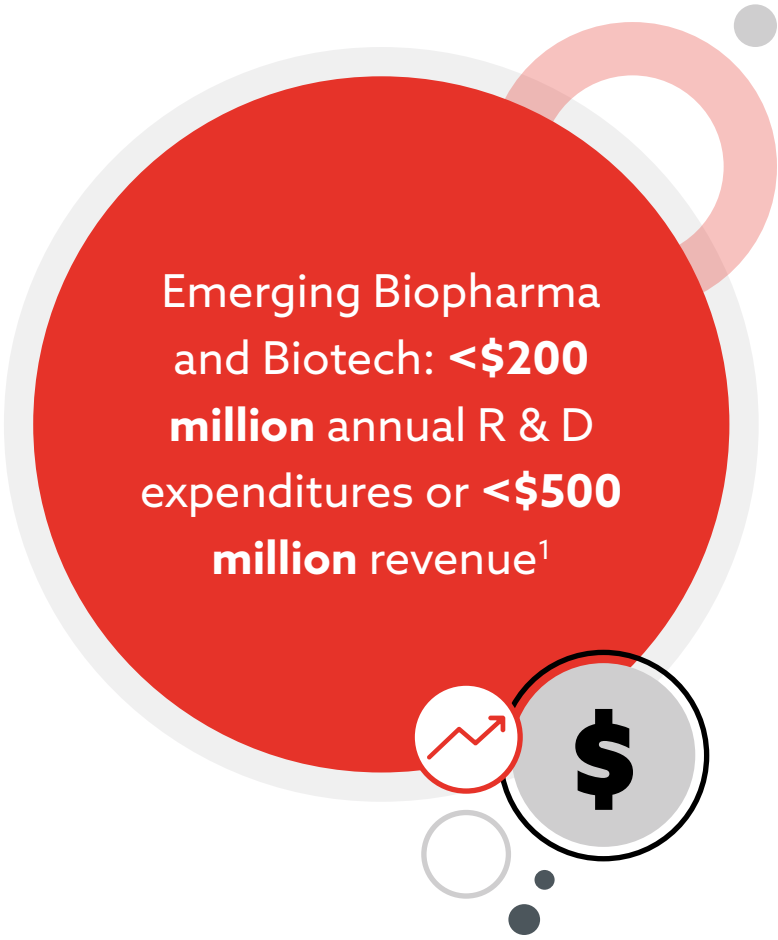
Despite their small size, these up-and-comers have an outsized influence over clinical development, with 70% of clinical trials conducted by emerging biopharma companies.[5]

That growth has been a long time in the making: From 2000 to 2005, for example, Phase 3 compounds from large pharma reduced by 23% while biotech companies grew by 60%. After that market share repositioning, newer entrants were responsible for nearly 66% of Phase 3 therapies by the mid-2000s.[6]

By 2018, more than 3,000 companies were considered to be emerging. They not only maintained a majority of Phase 3 compounds, but also of early-stage research, of which 84% is attributed to emerging biopharma. All told, such players account for more than eight in 10 of all therapies in development.[1]

Since then, the trends have continued going up despite the challenges of 2020. Jonathon Calderwood of Almac Group calls this most recent upswing the "biotech boom."

"The game changer just in the past 12 or 18 months has been the rate of acceleration of development for biologics based treatments," says Calderwood, a Commercial Development Director at Almac Group. "You see that happening in tandem with all of these other factors, like increased funding and even COVID-19 putting clinical trials and development of novel treatments into the spotlight as a catalyst for increased activity. As a result, we're seeing quite a significant trend of shifting priorities that really carves out more opportunity for emerging biopharma and strengthens their foothold long-term."



This accelerated growth aligns with the expansion of precision medicine, which is expected to reach nearly $100 billion by 2026,[7] nearly doubling the $58 billion valuation of 2020. Drug development pipelines are filling with personalized therapies that give new hope to cancer patients, with the Food and Drug Administration (FDA) expecting an additional 10 to 20 approvals of cell and gene therapies per year atop the 800 active investigational new drugs (INDs) on file.[8]

"One of the major market indicators I always look for is the shift in where the clinical trial market is going," says Natalie Balanovsky, MBA, PMP, Manager of Strategic Innovation at Almac Group. "And one of the major drivers right now on the biotech side is in personalized cell and gene therapies. The whole sector is growing at a more rapid rate than pharma itself."

The growth is particularly evident in oncology: Anticancer compounds from emerging companies went up by 74% from 2013 to 2018.[1] These innovations span the frontiers of immuno-oncology thanks to new interest in complex programs like immune checkpoint inhibitors, monoclonal antibodies and CAR-T research.

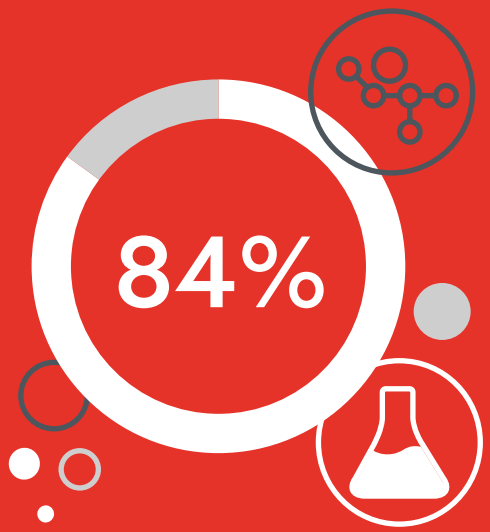
Other emergent areas include orphan drug programs, driven in part by new regulator trends to encourage rare disease therapeutics. CRISPR, neuroscience developments and other fields also add to the expanding and exciting role of personalized therapies in the future of biotechnology.

Transaction Activity

The growth story of emerging biotech and biopharma is also reflected in the amount of public and private funding pouring into the sector.

By 2019, life sciences had been seeing deals averaging $200 billion a year. That number dipped considerably in 2020, down to $159 billion. But many emerging biotech programs were buffered against those reductions and even saw gains: Venture deals in biotech approached $25 billion in 2020, growing more than 50% from 2019. That activity gave smaller companies a distinct advantage: On average, many managed to raise enough funding to stay independent.[9]

Eighty-four percent of Phase 3 compounds are attributed to emerging biopharma



Regulatory Outlook

The heightened activity in emerging biotech and biopharma leads to more regulatory scrutiny in a time when new therapies already have a less than 10% chance of approval on average after clinical trials. However, emerging players do have an edge on traditional pharma when it comes to success rates, at around 17%.[10]

In part, these trends are indicated by an uptick in requests for more data from the FDA for cell and gene therapy products, as well as unexpected rejections.[11] After a series of high-profile setbacks and regulatory reluctance, this could indicate changing conditions for products in development and the need for emerging biotech and biopharma players to preempt regulator concerns.[12]

Profit and Labor Implications

With increased venture funding, the pressure's on for small to midsize biotech companies to deliver palpable returns, yet more than 90% of biopharma programs are not profitable.[5]

As companies aim to get more products across the finish line profitably, they're also competing for talent in a labor market held back by shortages. In many areas, demand for life sciences talent is well exceeding available workers. According to a report from the Massachusetts Biotechnology Education Foundation, for example, demand for graduate and undergraduate talent soared above 100% between 2010 and 2020 but without commensurate growth in available candidates.[13]

International Reach

The global footprint of emerging biotech and biopharma is evident, with growth markets across the United States, Europe, Australia, China, South Korea, Japan and elsewhere. That international reach has pushed worldwide expansion to the forefront of commercialization plans, adds Calderwood.

"Similar to here in the U.S., where we started to see amazing activity from small players in Boston and the West Coast, you're seeing similar parallels in proportional terms across other places like China and Korea for these more nimble organizations. Many of these companies have been founded by pioneers that have spent time in the US or have a similar commercial ethos and structure. There's a real interest in global play for biotech right now."

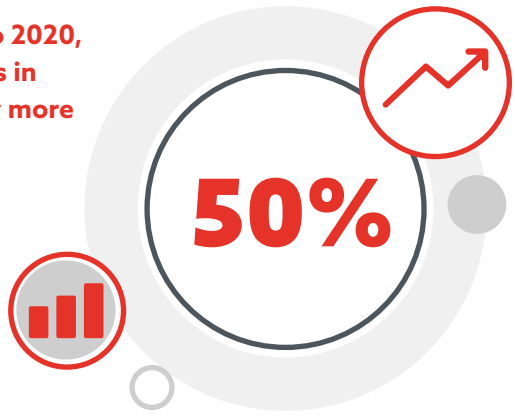
Effects of COVID-19

The aftershocks of the coronavirus pandemic will have a lasting and reverberating effect on the biotechnology and biopharma sector, particularly within trial management. The acceleration of decentralized trials in tandem with remote technologies, telemedicine and direct-to-patient drug delivery has created new opportunities for emerging players, as well as a few new challenges. Moving forward, experts suggest the emphasis will be on finding cost-effective solutions that meet today's needs while accommodating for future unknowns.

Opportunities Ahead

As we enter what some have called the Biotech Century,[14] the sheer volume of first-to-market treatments coming from emerging companies is growing by the day. This surge of activity brings with it new opportunities, including that of clinical trial participation and data collection inherent of the changed trial landscape.

From 2019 to 2020, venture deals in biotech grew more than 50%.



Patient Centricity

In this new environment, recruitment, enrollment and trial experience are adapting to more virtual and decentralized models supported by technology. And emerging biotech and biopharma leaders can use those platforms to differentiate and accelerate their programs to more meaningfully serve more people.

"For years, companies had talked about patient centricity but the pandemic really forced that innovation instantly," says Balanovsky. " It's propelled our industry into developing and using these patient-focused solutions like telehealth that we've been talking about for so many years."

Further enabling these technologies are new direct-to-patient shipment models, which have made home-based administration more practical moving forward. Such models may have originated out of public health necessity, because patients couldn't be physically present in an environment that would expose them to COVID-19, but they will no doubt have long-term applications.

"You're always going to need investigators and sites, but the hybrid model with remote participation has clear benefits," Calderwood says. "But sponsors will need to prepare for how these kind of changes affect supply chains and data collection."

Sluggish enrollment can be a problem, but so can unexpectedly high demand. Read how one small biotech company supplemented its limited internal resources with Almac when patient recruitment was three times faster than anticipated.

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Adaptive Research

In light of biotech's recent regulatory setbacks and the larger tapestry of clinical failures across pharma, adaptive trials hold potential. These models build flexibility into protocols to allow for more pivots from one treatment to the next.

"Traditionally, trials are run one at a time in a linear fashion where we wait for the results of one before moving into the next one, but that will have to change if we want failure rates to improve," says Balanovsky. "There will need to be a higher emphasis on early phase assessment and real-time data so that you can make adaptive changes along the way."

Partnerships

While the sector had already embraced collaboration leading up to COVID-19 — with 43% of small biotech companies partnering up with others for clinical trials[5] — the pandemic taught the value of co-development and sharing intellectual property to an even greater extent.

In this new environment, the ability to co-opt strengths among different collaborators worldwide helps to build a global presence and asset base that can facilitate recruitment and trial success and, ultimately, bring therapies to market faster.[15,16] Plus, when funding is a challenge, many other small sponsors are choosing to co-market products with established pharma companies.

The Clinical Supply Value Chain

An important undercurrent of these trends, the clinical supply chain represents another opportunity for emerging biopharma and biotech — particularly amid the pressing demand for more agility and responsiveness to changing market conditions.

As more drugs are developed faster in a hypercompetitive market, more small to midsize companies are turning to proven vendors, including CDMO partnerships, to streamline efforts, reduce wasted costs, bolster supply chain management and make clinical development and distribution more efficient and effective end-to-end. These effects can in turn overcome recruitment gaps and challenges inherent in stock-limited studies.

Challenges to Overcome

Despite (and perhaps because of) the fast-paced growth of biotech and the opportunities ahead, many challenges remain. Small and midsize companies are competing for labor, funding, investigators and sites — and they're also under the clock, having to fill pipelines, appease investors, get approvals, scale up and hit the market before competing therapies while not compromising safety.

"What we saw from COVID-19 was that the dynamic has changed in terms of timing expectations — companies aren't going to wait 10 years to get a drug to market anymore," Calderwood says. "The business model is starting to change, and that's putting pressure on companies to work faster and more efficiently."

These challenges invite a radical change to the way clinical development is done. As a result, developers are realizing the value of CDMOs to navigate the many barriers and transform their trial pipeline approach.

The Expanding Role of CDMOs

Given the complexity, urgency and highly competitive nature of emerging biotech and biopharma, many entrants struggle to gain a foothold without the support and backing of a trusted CDMO. Such organizations act as a fully functioning extension of a sponsor's team to provide dedicated support, from trial design and management to supply chain and distribution strategies.

And with modern solutions, the right CDMO can give emerging players a leg up in this crowded market, overcoming the many challenges they face, including labor shortages, regulatory scrutiny and competition for funding and sites.

"A key differentiator for any biotech coming into this environment is their ability to partner with a CDMO, particularly one who utilizes real-time data and telehealth technologies for trial management," says Balanovsky. "You can more effectively enroll and retain patients in trials with these digital solutions, and in addition to improving the patient experience, it also makes things easier for the healthcare provider."

Rapid scale-up is table stakes in this new era. Read how one small biotech company worked with Almac to ready its supply chain for unprecedented demand while ensuring cost-effective continuity for all patients.

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You have a physical supply chain, but what about a digital supply chain? Read how emerging technologies are powering modern-day trials to assure compliant, timely and cost-effective supply to patients.

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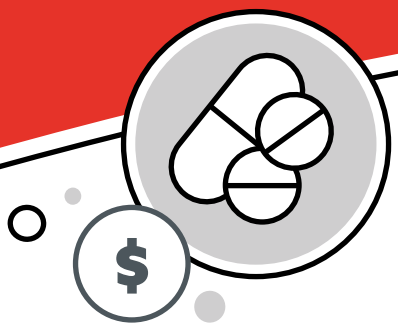


"Despite the advantages of engaging with a CDMO, however, some sponsors have historically opted not to outsource that support. While that method may seem like the best choice in the short term, it can be problematic later on," Balanovsky adds.

"Traditionally, some firms have started off doing preclinical on their own, and they stay that way throughout the program duration," she says. "But as these companies enter Phase 2 and Phase 3 trials, the challenges of understanding the global regulatory environment and other program components can become quite overwhelming."

That's why the best time to engage with a CDMO is as early as possible within the preclinical period. It's during that critical time that sponsors and vendors can optimize protocol development and future projections while staying responsive and proactive to unexpected changes.

"Activities set in motion during those early phases can either positively or adversely impact the future management of trials," Balanovsky says. "Without proper planning, programs may need to make corrections along the way, which cost time and money. So, the more effort you put into the front end, the more successful you're going to be."

Many factors go into choosing a CDMO, starting with finding a partner with the resources and reach to collaborate across every stage of development — from preclinical through commercialization. A demonstrated experience and expertise in the target disease area is also beneficial, as is the use of a proven software platform to support trial management and patient experience. These and other factors have driven sponsor interest in engaging with so-called all-in-one vendors to create a more seamless path to commercialization.[17]

You also want a partner who can meaningfully augment your team's existing strengths with global coverage, but who also understands the unique challenges of an emerging company. Look in particular for a CDMO with proven experience working with small to midsize sponsors but who also has an expansive global network and footprint. This can ensure the partner understands the nuances and requirements for building compliant but efficient infrastructure, teams and processes for target markets.

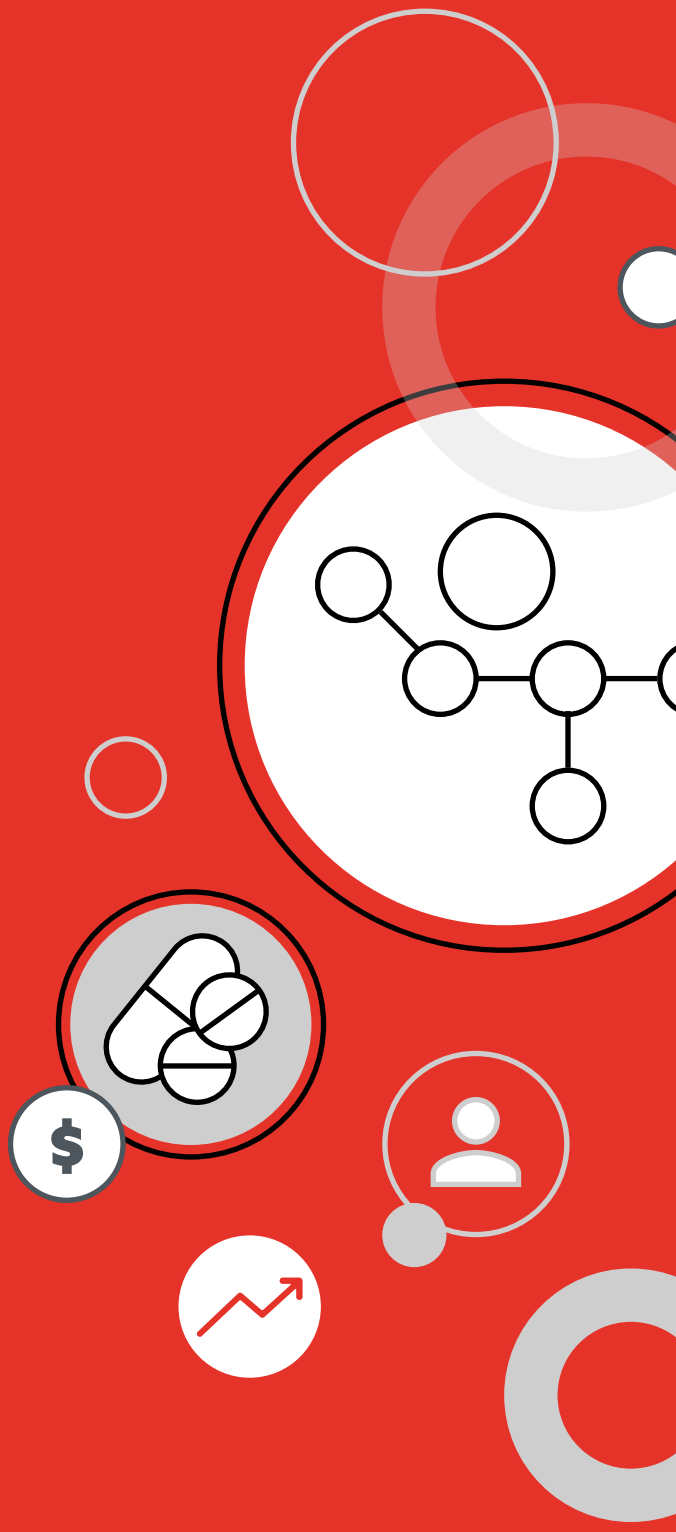
"There's not a corner of the globe that doesn't have a trial being conducted or some aspect of the supply chain," Calderwood says. "It's important to find someone to start to edge in that global application and provide coverage where it's needed for regulatory frameworks and supply chain management, or even basic support like cultural

Make Your Mark in a Crowded Industry

Emerging biopharma and biotech companies are at the pivot point of modern-day research: Maintaining an outsized influence on life sciences as a whole, these small and midsize players have increased their footprint worldwide within and outside of oncology, and they have investor support. As a result, new innovations and therapies are giving patients new hope and access.

However, these opportunities invite new challenges, including competing for money, time and labor while improving the patient experience. Overcoming these barriers requires a new approach to clinical trial management, willingness to try new technologies, and a dedicated partner to help manage the many moving parts of a successful commercialization journey.

By working with a proven CDMO, sponsors can make their mark on this hypercompetitive industry while helping their products see market success. Sponsors should consider the wide range of experience and technology capabilities vendor candidates have, as well as their international reach. Doing so can ensure you find a partner who can help you deliver your innovations to the people who need them most.



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Utilizing our best in-house Supply Chain Management (SCM) expertise, custom-designed services and software solutions, we offer the most flexible approach to support the delivery of your global clinical trial from protocol to patient delivery.

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