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The pharma trends you need to know: A CDMO's perspective

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Abstract

The market momentum of novel therapies targeting unmet needs is creating a new landscape for pharmaceutical drug manufacturers. As the focus on these smaller patient pools grows, so does the complexity of drug development.

Companies must understand how the molecules filling today's pipeline are changing our business, as they are causing a dramatic shift in how we plan for and execute drug development and manufacturing. As a CDMO, our customers' needs give us a unique perspective on the evolution of the industry and how it is shaping today's market.

The market impact of increased competition and CDMO growth

A deeper understanding of diseases and the science needed to treat them is helping replace the old model of blockbuster and "me too" drugs with one that focuses on disruptive innovations that can offer better value and benefits to its patients. It is no longer large pharma companies leading the way with an army of resources. Small, emerging companies—defined by having less than 500 employees—are being credited with developing more than half of the novel drugs approved by the FDA over the last decade[1]. Outsourcing has allowed these entrepreneurs to leverage the facilities and expertise of a strategic partner rather than invest too much of their own capital before they know the future of their drug. In the last five years, half of new drugs were manufactured by CDMOs[2]. This reflects the critical role of CDMOs in supporting the development of clinical candidates as well as the registration of these products, marketing authorization, and manufacturing for global markets.

Smaller companies utilizing CDMO resources and companies facing increased competition must find ways to stand out and, more importantly, get ahead. This has led to an exploration of new territories in drug development, such as new mechanisms of action including targeted therapy, which is transforming the make-up of today's drug development outlook.

Now, a third of global value comes from the following five therapy areas, which contribute over 55% of global growth[3]:

- oncology
- antidiabetics
- autoimmune conditions
- pain
- respiratory

Oncology has had the most significant impact, as the way the body responds to an attack on its cells can cause cancers for which there are no available drugs. A recent report by American Cancer Society researchers shows that about one in five cancer diagnoses in the U.S. is for a rare cancer, which are defined as those with fewer than six cases per 100,000 diagnosed each year[4].

As more targeted therapies are needed, we have seen an increase in complex molecules being investigated in clinical trials. These "niche buster" drugs are driving renewed research efforts for many rare diseases, as they have higher probability of approval, thereby a greater return on investment. According to a recent orphan drug report from EvaluatePharma, orphan drugs will represent over a fifth of the world prescription market by 2024[5]. Despite the benefits they serve to the industry and especially the patient, the targeted therapy of rare/orphan drugs come with significant challenges for any company trying to bring one to market.

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The challenges of treating rare disease

The potential orphan drugs have for treating rare disease has not gone unnoticed. This is evidenced by the 1.4 orphan drug designation requests the FDA received per day in 2017[6]. However, the molecules that make up these orphan drugs are not only complex, but they are also highly potent. High-potency active pharmaceutical ingredients (HPAPIs) require specific infrastructure and capabilities. These include but are not limited to containment procedures, engineering controls, and personnel handling during development and manufacturing at both clinical and commercial scale. A facility manufacturing HPAPIs must also meet regulatory and environmental requirements, be segregated from other facilities producing pharmaceutical products, and comply with occupational exposure limits for handling potentially hazardous compounds.

Unfortunately, securing these measures requires a significant capital investment and several years to get a compliant facility up and running. Even if a company already possesses some of these capabilities, they could potentially be outdated. The equipment and engineering techniques used for highly potent compounds 5 to 10 years ago are now more sophisticated. Any company, including a CDMO, that invests in containment procedures and capabilities to handle these types of compounds will be better positioned to support the industry.

Biologics are on the rise, but don't count out oral solid dose drugs

Another trend the industry has seen grow over the last decade is the rise of biologic drugs due to their success in treating major chronic diseases in the areas previously mentioned, such as cancer, autoimmune diseases and others. Research shows the revenue valuation for the global biologics market could reach nearly $480 billion dollars by 2024, with North America claiming nearly half of the market share[7]. The rise in large molecule drugs does not mean, though, that we will see fewer small molecule products in the pipeline. So far in 2018, small molecule drugs make up 58 percent of the industry's pipeline. They have also seen more success with the FDA, as 49% of approved new drug applications (NDAs) in 2017 were for tablets[8].

Oral solid dosage drugs continue to be a mainstay of the pharmaceutical industry, and it is likely due to the advantages they continue to have over biologics, which are often sterile injectable products. From a patient compliance perspective, oral solid dosage forms are a more convenient option when it comes to ease of use. Sterile injectable products typically require a specialist or a hospital environment for administration, as opposed to the more familiar option of taking a pill at home—although oral solid dosage forms can sometimes be administered as a sterile injectable product. Oral solid dosage forms also require less of an investment from manufacturers, which translates to lower costs to the patient.

As we continue to see how the industry takes shape over the next three to five years, drug manufacturers must ensure their company has the resources available to withstand the ebb and flow of the pharmaceutical industry. If they do not, finding a CDMO with the capabilities needed is critical to the success of their products and especially the life of their company.

Finding a CDMO that is prepared for the future of pharma

Selecting the right CDMO for your project is critical. From discovery to commercialization, the drug development process can take many years, so you must choose one that can support a long-term relationship. This requires specific capabilities to support the clinical and/or commercial development of your drug substance or product in order to bring it through the regulatory approval stages and, ultimately, to the patient. The criteria a sponsor—no matter what size—should use to evaluate a partner include technical capability, capacity, experience, quality, client management, reputation, and financial stability.

In addition, continuity of development and clinical manufacturing services at a single CDMO ensures a thorough understanding of deliverables, the ability to avoid delays, and a much smoother project management. Multiple tech transfers and changes of an outsourced partner at various stages of development often leads to the loss of knowledge and trade secrets, which is why it is beneficial to retain the know-how and experience within one organization. Overall, the strength of a company's personnel as well as the organization itself brings stability to the development and clinical programs they offer. Partnering with a qualified CDMO can not only help prepare you for the future of the industry, but, more importantly, it can also facilitate the delivery of safe and effective medicines to patients in a timely manner.

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Thermo Fisher Scientific provides industry-leading pharma services solutions for drug development, clinical trial logistics and commercial manufacturing to customers through our Patheon brand. With more than 65 locations around the world, we provide integrated, end-to-end capabilities across all phases of development, including API, biologics, viral vectors, cGMP plasmids, formulation, clinical trials solutions, logistics services and commercial manufacturing and packaging. We give pharma and biotech companies of all sizes instant access to a global network of facilities and technical experts across the Americas, Europe, Asia and Australia. Our global leadership is built on a reputation for scientific and technical excellence. We offer integrated drug development and clinical services tailored to fit your drug development journey through our Quick to Care™ program. As a leading pharma services provider, we deliver unrivaled quality, reliability and compliance. Together with our customers, we're rapidly turning pharmaceutical possibilities into realities.

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Dr. Kane has more than 25 years of experience in the science and business of taking molecules through the entire drug development process. His extensive knowledge spans early stage development to scale-up and commercial manufacturing, and includes technical transfers between global sites and drug life cycle management. Dr. Kane received his Bachelors, Masters and Ph.D. degrees from the Bombay College of Pharmacy, University of Bombay, India, and served as a post-doctoral fellow at the School of Pharmacy, University of Cincinnati, Ohio. He has also earned an executive MBA from Richard Ivey School of Business, University of Western Ontario, Canada. Dr. Kane is a member of various international pharmaceutical professional organizations, and is often asked to speak about scientific topics on formulation, technology other technical aspects, QbD, etc at major industry events. He has also published many articles in International journals and delivered many talks at meetings and conferences cross the globe.

In his current role, Dr. Kane leads a team of "Subject Matter Experts" to support our clients in developing sound formulation and process development strategies and closely works with the scientific teams at Thermo Fisher Scientific's global sites for execution, provides leadership in the complete development of novel lead compounds and line extensions. He is also responsible for evaluating drug delivery technologies to support the business. Dr. Kane has been an invited speaker at many global conferences, workshops, seminars and training programs and has published several articles, interviews and white papers across the world including North American, European, Japanese and Korean publications.

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