SUPPORTING SMALL AND EMERGING PHARMA THROUGH COLLABORATION AND SPECIALIZED EXPERTISE

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Growth in the pharmaceutical industry is no longer exclusively driven by large pharmaceutical companies. The switch in the marketplace from a focus on blockbuster drugs to an emphasis on therapies that treat orphan and rare diseases has led to the rise of small and emerging pharma companies founded to advance novel medicines for specialized patient populations. These innovators require the support of contract service providers that recognize their unique requirements.

THE RISE OF SPECIALTY PHARMA

Advances in genomics have lead to ongoing discoveries about the role of genetics and genetic mutations in disease mechanisms. The identification of novel drug targets that enable the development of highly specialized – and even personalized – medicines is behind many new drug candidates currently in the pipeline. These drugs – often with orphan drug, priority review, breakthrough therapy and other accelerated approval designations – are in many cases, based on small molecule APIs developed by small or emerging companies, sometimes in collaboration with charitable foundations and/or universities and institutes.

According to the Tufts Center for the Study of Drug Development, smaller pharma and biotech firms developing small molecule drugs have higher clinical approval success rates than large companies.[1]

ONE NICHE: SMALL MOLECULE INJECTABLES

While most small molecule drugs are formulated for oral administration, for some chemical APIs, there are advantages to developing parenteral products – and demand for small molecule injectable drugs is increasing.[2] Drugs delivered parenterally into the bloodstream, including heart medications, antibiotics and analgesics, provide an immediate therapeutic effect, which is often crucial for patients being treated in hospitals, particularly in emergency rooms. Patients who cannot take medications by mouth also benefit from the administration of drugs by injection or infusion. Parenteral administration is also a desirable alternative for drugs that degrade in the stomach or intestines.

Parenteral formulations can be prepared as admixtures that must be manipulated into the correct dose before administration, or they can be prepared in premixed bags. Using premixed bags reduces the risk of medication error and microbial contamination, and producing these premixed bags in highly automated GMP facilities offers an even higher level of quality.

THE NEED FOR IN-DEPTH EXPERTISE

The thousands of small and emerging pharma companies across Europe and North America developing novel small molecule drugs often have limited resources, personnel and expertise with regard to the full drug development and manufacturing process. They often rely heavily on contract development and manufacturing organizations (CDMOs), outsourcing much of their process and formulation development, validation, regulatory compliance and manufacturing activities.

CDMOs that provide integrated services across all phases of the drug development and commercialization cycle provide the best support to these firms. For small companies looking to advance small molecule injectables, the ideal CDMO will also have extensive knowledge and the specialized expertise required to rapidly develop robust, high-quality manufacturing processes that are readily scalable and ensure the highest product quality and consistency.

THE VALUE OF QUALITY

The processes involved in the manufacture of sterile parenteral products are highly complex. The stability of the parenteral solutions must be assured, which requires the completion of extensive compatibility and stability studies. Products packaged in plastic must also undergo extractable and leachable testing to ensure that contamination of the drug product does not occur. Sterility must be confirmed via microbial contamination studies. Cold-chain management is also required for some parenteral products. These issues can only be addressed on an ongoing basis if the CDMO has a well-established culture of quality and effective quality systems in place.

FLEXIBILITY AND UNDERSTANDING MATTER

With many new drug candidates receiving designations allowing for accelerated approval pathways, CDMOs supporting small and emerging pharma companies developing small molecule injectable drugs must have the process understanding and physical capability to implement projects within dramatically shortened timelines. They must also have flexible capacity to support projects as they move from the clinic to commercialization.

In many cases, specialized products developed as orphan drugs or therapies designed to treat rare diseases, will target small patient populations. The quantities of these drug products required at the clinical stage, and even for commercial production, can be much smaller than those for more traditional medications – as low as 100,000 units, for instance. The ideal CDMO for clients developing these products welcomes these smaller volumes but also has the ability to expand production to millions of units if or when the volume demand increases.

THE IMPORTANCE OF RELATIONSHIPS

Smaller pharma companies that rely heavily on outsourcing require CDMO partners that are willing to establish strategic partnerships based on open communication and transparency. It is also essential that the relationships between the different departments within the CDMO are strong, because there is much less time to develop and validate manufacturing processes and analytical methods. Good relationships with FDA representatives are equally important, particularly for drugs developed under accelerated approval pathways.

THE GRIFOLS DIFFERENCE

Grifols was one of the first companies in Europe to obtain approval for the parametric release of parenteral solutions. Parametric release is authorized for companies that have historically shown excellent sterility test results and high consistency in their overall quality systems. It also facilitates reduced timelines for product release, allowing our clients to get their products to the clinic and market more quickly.

Collaboration is emphasized at Grifols through the use of an integrated project management strategy. Cross-functional teams with representatives from R&D, quality assurance, manufacturing, quality control, regulatory and sales and marketing collaborate to consider all potential consequences before implementation of even the smallest changes. The result is the avoidance of unexpected problems and the need to make corrections, preventing unwanted delays and keeping projects on schedule. P

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