April 2020

“Changes” in sourcing R&D in pharma and biotech: the old made new again?

The pharma and biotech industries have reached significant R&D milestones in the past decade, including the broad adoption of immunotherapy and the advent of gene therapies. Despite these breakthroughs, the productivity of research and development efforts among large pharma/biotech remains low.¹ The tried-and-true models of sourcing innovation internally and externally maintain a strong position, although with the technology breakthroughs, the focus and key targets of innovation have shifted.

*Evolving disease focus: From primary care to specialty medicine to rare disease*

As pharma/biotech addressed the “low hanging” unmet medical needs in general medicine areas (e.g., statins in dyslipidemia, ACE inhibitors and ARBs in hypertension, GLP-1s in type 2 diabetes), the focus has shifted to smaller populations with higher unmet needs. These new technical challenges (e.g., large molecule and antibody R&D) have been offset by commercial reward – higher price points for bringing new technologies to patients in underserved/overlooked diseases, as well as longer exclusivity (i.e., orphan drug designation) and/or access to expedited regulatory pathways (e.g., fast track, breakthrough therapy).

Cue the era of mid-sized, specialty pharma companies: Cephalon’s “wake” franchise (Provigil, Nuvigil); Genzyme’s enzyme replacement therapies for lysosomal storage diseases/inborn errors of metabolism (Cerezyme, Fabrazyme, Lumizyme); Amgen’s supportive cancer care drugs (Epogen, Neupogen, Aranesp, Neulasta, Nplate); Endo Pharmaceutical’s pain portfolio (Lidoderm, Percocet, Opana).² This has been accompanied by reorganizations in big pharma to bring focus on specialty disease areas. GSK, Novartis (e.g., Novartis Oncology), BMS, Pfizer (e.g., Specialty Care Business Unit; Rare Disease Business Unit; Oncology Business Unit) and many others built fully fledged immunology and oncology portfolios to capture the opportunity from these new disease areas. As the environment has continued to evolve, both large and small companies have now shifted focus to rare diseases as the new frontier for growth (e.g., Shire/Takeda, Actelion/J&J, Genzyme/Sanofi, Alexion, Amicus, BioMarin, Sarepta, Vertex).

---


Innovative R&D sourcing models and the increasing role of technology

As in the past, large pharma/biotech have augmented their own R&D efforts by acquiring technologies from smaller innovators. For decades, big pharma and biotech have supplemented their internal pipelines using a variety of approaches for sourcing external innovation, including collaborations with key academic research centers – both (1) from a disease area perspective (e.g., Memorial Sloan Kettering Cancer Center) and (2) geographically (e.g., La Jolla/San Diego, Boston/Cambridge) and also through external contract research organization (CRO) relationships (e.g., Covance, Wuxi). Pharma/biotech have also relied on hybrid internal VC/incubators (e.g., GSK’s SR One, Johnson & Johnson Innovation Lab’s JLABS, Novartis’ Venture Funds, MSD’s Merck Research Venture Fund). The more recent “cycles” of this R&D sourcing model have focused on acquisition of companies that have developed CAR-T therapies (e.g., Kite/Gilead, Juno/BMS, Spark/Novartis, Avexis/Novartis).

With ever-increasing pressure to innovate in this new era, companies will seek new tools to help reduce their R&D key performance indicator (KPI): total spending per FDA-approved drug. Artificial intelligence in R&D models, advanced analytics and big data are expected to play a larger role in the future of R&D. Such changes are already apparent with collaborations among data and pharma giants (e.g., Google with Novartis, Qualcomm with Roche). With increasing data analytics capacities and broader adoption in pharma, research technologies that heavily depend on data (e.g., RNAi, CRISPR, microbiome) will increase their share in R&D pipelines. Furthermore, such developments are expected to improve the efficiency of research (discovery/translational) and catapult the current R&D model into an era of increased productivity and improved R&D economics.

The path forward for pharma and biotech

Moving forward, as novel methods of development become more commonplace and the areas of focus shift from larger disease areas to more focused, niche diseases, pharma companies will need to expand on existing capabilities across the product lifecycle. The first challenge posed by the evolving models will be developing the ability to accurately assess opportunities in rare/niche diseases, where forecasting and business case development requires a nuanced understanding of the disease dynamics and patient treatment pathways. For companies that have acquired/developed assets, clinical development teams will be operating in niche areas where requirements for regulatory approval must be created collaboratively with key stakeholders more so than previously. Field teams will find themselves in novel settings of care where there will be higher demands to help coordinate patient access to novel therapies.

In this new complex world, companies large and small need to tread the R&D waters carefully. External partners can help companies by providing a disciplined, thorough, and efficient approach to assessing the commercial opportunity of external products or portfolios and how well they fit with companies’ internal portfolios. At a franchise level, therapeutic area strategies can help to inform decision-making for inorganic and organic growth by identifying future growth areas – within and across diseases in the therapeutic area – and by building a complementary portfolio of products. Given the unique challenges and considerations for any company at a particular stage of growth, no single solution will fit all needs; expert insights and thoughtful approaches can help guide and customize solutions to each company’s challenges.

About CRA and the Life Sciences Practice

CRA is a leading global consulting firm that offers strategy, financial, and economic consulting services to industry, government and financial clients. Maximizing product value and corporate
performance, CRA consultants combine knowledge and experience with state-of-the-art analytical tools and methodologies tailored to client-specific needs. Founded in 1965, CRA has offices throughout the world.

The Life Sciences Practice works with leading biotech, medical device, and pharmaceutical companies; law firms; regulatory agencies; and national and international industry associations. We provide the analytical expertise and industry experience needed to address the industry’s toughest issues. We have a reputation for rigorous and innovative analysis, careful attention to detail, and the ability to work effectively as part of a wider team of advisers. To learn more, visit crai.com/lifesciences.

Authors

**Emre Vural**  
Associate Principal  
+1-415-205-9270  
evural@crai.com

**Kishore Gangangari**  
Consulting Associate  
+1-212-520-7268  
kgangangari@crai.com

**Joanna Lee**

The conclusions set forth herein are based on independent research and publicly available material. The views expressed herein are the views and opinions of the authors and do not reflect or represent the views of Charles River Associates or any of the organizations with which the authors are affiliated. If you have questions or require further information regarding this issue of CRA Insights: Life Sciences, please contact the contributor or editor at Charles River Associates. Detailed information about Charles River Associates, a trademark of CRA International, Inc., is available at www.crai.com.

Copyright 2020 Charles River Associates