Uniting Discovery and Care: The Role of Pharmaceutical Companies in Research, Clinical Studies, and Patient Care
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In an era of increased complexity of clinical research, a demand for personalized medicine, an increasing value of diversity, a focus on digital health, and a call for patient centricity, the discovery and development of new medicines, more than ever, is dependent on collaboration between multiple stakeholders.

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The attribute in the clinical research ecosystem that is nearly unique to pharmaceutical research is its relationship to novel therapies, generally created to disrupt a disease mechanism in an unprecedented way—a novel molecule driving a novel mechanism. This novel-novel characteristic of pharmaceutical research has proven to be a very effective wedge to crack open new research areas by playing two vital roles in clinical trials and research direction, both deliberate and indirect. Intentionally, sponsored clinical trials stimulate innovation in clinical investigative methods to robustly test scientific hypotheses, typically borne out of basic research insights. Indirectly, results of clinical investigation that provide guidance to future experiments are equally impactful, as they often disprove a hypothesis and possibly abort future research in a failed mechanism. The IL-17A clinical trials in psoriasis and inflammatory bowel disease illustrate these aspects. Although clinical development of anti–IL-17A medicines redefined the pathogenesis of psoriasis (Nestle et al., 2009), despite coherent preclinical evidence supporting a potential benefit of anti–IL-17 treatment, the negative trial in Crohn disease revised the field of research for the role of IL-17 in gut hemostasis (ClinicalTrials.gov, 2016; Targan et al., 2016).

Patient centricity
During the past decade, patients have become key decision makers for an increasing number of health care decisions. In response to the current healthcare environment, both researchers and industry will need to focus their efforts on initiatives that will maximize impact and value for patients. Patients and their families, more than ever, are active participants in healthcare dialogues with regulatory agencies, academia, and industry (Stergiopoulos et al., 2019). To design more patient-oriented clinical trials with flexible approaches and solutions aimed to enhance knowledge gathering, sharing, application, and retention associated with clinical development, a holistic shift of mindset in clinical research is necessary.

Patient expectations and needs can differ dramatically from the aims and objectives of healthcare professionals. Thus, patient experiences will increasingly drive the development and delivery of medicines and medical devices. For instance, immune-mediated diseases like rheumatoid arthritis, psoriasis, and atop dermatitis generally have negative impacts on patients’ lives. Despite available therapies that work for some or for a while, unmet treatment needs remain. Moreover, these needs can differ among patients with the same diagnosis as the immune disorder evolves. As disease heterogeneity becomes better defined, increased efforts in translational research and biomarker science that correlate with differing disease paths will be critical in both clinical research and care. Furthermore, patient journeys and associated patient needs can be discerned through direct engagement. Patient-centered strategies will lead to solutions that will better treat patients and their medical conditions (Figure 1). It is already common for pharmaceutical research to involve patients and families in clinical trial design to opine on study measures that are important to them and are gathered in ways that are more sensitive to their needs as persons.

Digital health and connected care to better generate evidence. Electronic tools are widely recognized as complimentary aids in clinical trials. Today, researchers use digital technologies to
objectively capture and measure clinical biomarkers and performance outcomes as primary and secondary study endpoints. Patients in clinical trials serve both as trial participants and data collectors. When surveyed about their preferences and willingness to participate in trials that used digital technologies, patients reported improved convenience and perceived accuracy of data collection with mobile clinical trials versus traditional trials (Perry et al., 2019). Although using technology may enrich and simplify the trial experience for the patients, further research and sharing of practical experiences across pharmaceutical companies and clinical research communities are needed to optimize effective use of digital technologies in clinical trials. Digital transformation of healthcare has the potential to meet the growing expectation of greater personalization. However, companies will need to invest heavily to create electronic tools that are reliable, accurate, and secure.

**Momentum of translational medicine**

With growing applications of patient-centered trials and therapies, dermatological conditions can clearly contribute to, and benefit from, the acceleration of translational medicine. As patients contribute samples or participate in trials, clinicians and academicians work to advance our understanding of the disorders. In turn, industry innovates around this new information with urgency to provide remarkable treatments to patients to achieve improved health outcomes. These bench-to-bedside exchanges hasten the translation of discoveries into therapies (Figure 2). By sharing bio-samples and pooling data across companies and academic investigators, we have an opportunity to better understand disease pathogenesis and better characterize the natural histories of disease, and the accumulated knowledge can be incorporated into future clinical trial designs.

**Interorganizational collaboration**

Feeding best practice information back into the profession closes the loop of...
patient centricity. The nonprofit organization TransCelerate Biopharma facilitates collaboration within the biopharmaceutical research community to accelerate the delivery of novel therapies to patients. Through participation in TransCelerate, companies can share information and employ the combined resources of member organizations to advance the greater scientific community (Gill, 2014).

**Personalized care evolves to customized care and treatment of genetic mutations.** As the science underlying specific disorders becomes better elucidated, currently applicable to certain cancers and monogenic heritable disorders, pharmaceutical companies have moved to create customized treatments for individual patients or small numbers of patients with the same genetic mutations. In this decade, several cell-based oncological therapies have been developed using autologous immune cells from patients that undergo ex vivo modification and subsequent reinfusion to the donor/patient to target and destroy cancer cells (Food and Drug Administration, 2018; Kymriah (tisagenlecleucel) [package insert], 2017; Yescarta (axicabtagene ciloleucel) [package insert], 2017). In these examples, genes encoding chimeric antigen receptors (CARs) are inserted into autologous T cells (CAR-T therapy) that are subsequently reinfused back into the patient and seek and destroy malignant cells. A more recently approved gene therapy approach for the genetic disorder spinal muscular atrophy does not include the ex vivo modification of autologous cells. Instead, the treatment is transduction in vivo and expression of the normal protein (Zolgensma (onasemognene abeparvovec-xioi) [package insert], 2019). In all of these situations, pharmaceutical companies need to create the technology, manufacturing facilities and processes, and logistics to both conduct the clinical research and make these treatments commercially available.

**Diversity and inclusion in personalized health care.** Creating medicines that make life better for people around the world cannot be fully achieved without increasing the diversity of clinical trial participants and including more underrepresented groups such as ethnic and racial minority populations and children. Children are not small adults, and safety and efficacy data from adult clinical trials cannot reliably inform appropriate dosing and safety based simply on extrapolation. Thus, conducting trials in children is an imperative investment for a future of better evidence-based treatments for children (Joseph et al., 2015). Other historically underrepresented groups are minority populations. Although racial and ethnic minorities are underrepresented in clinical trials, strategies to successfully address these challenges have been proposed and should be considered for implementation (Heller et al., 2014). Atopic dermatitis, for instance, is a heterogeneous disease and presents differently among various racial groups. Increased inclusion of minority groups in clinical trials would facilitate the development of targeted therapies for the multiethnic world population (Kauffman et al., 2018).

**Real world evidence (RWE).** Digital health care, electronic medical records, a diminishing number of pharmacy benefits managers, vertically integrated healthcare delivery, government-managed healthcare, and advances in informatics create an extraordinary opportunity for study of RWE. RWE approaches have long been employed as a way of evaluating cost effectiveness and monitoring safety, but the added richness of the emerging RWE environment allows for a greater range of hypothesis generation and testing across geographies and patient populations, including those with subtypes of a disease. Here again, the pharmaceutical industry builds the tools and recruits the collaborators needed to perform this work skillfully to optimize patient and population health outcomes.

**Summary**

Pharmaceutical companies have a mission to help people and promote better lives through better health. Although patients have larger roles in healthcare activity and decision making, innovative pharmaceutical companies continue to be a major catalyst to induce regulation; test scientific hypotheses and disease models; and lead in the creation of new technology, scientific methodology and analysis, and logistical and manufacturing innovation. Discovery science continues to provide novel insights in disease pathogenesis, which very rapidly result in a pharmaceutical company’s design of new molecules to attack new targets. Measures of success go beyond randomized controlled clinical trials and require demonstration of access and utilization that measurably improve patient outcomes analyzed through RWE. Health monitoring, connected devices, and novel delivery systems have emerged as key elements favoring greater treatment success, and we are now at the leading edge of customized treatments made possible by breakthrough science and breakthrough pharmaceutical manufacturing.

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**CONFLICT OF INTEREST**

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**AUTHOR CONTRIBUTIONS**

Conceptualization: LM, JMM; Project Administration: CT; Supervision: LM, JMM; Visualization: LM; CT; Writing - Original Draft Preparation: CT; Writing - Review and Editing: LM, JMM.

**REFERENCES**


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