Patient Engagement and Involvement in Research and Development

Increasing levels of patient engagement in R&D are driven by scientific advances as well as an increasingly research-focused patient movement, alongside evolving means to understand and validate patient perspectives. Pfizer has been a leader in engagement with patient organizations for over a decade. With a growing appreciation for the benefits that patient-focused research can provide to both patients and to drug developers, there is an increasing need to continue to build out these meaningful relationships with patient groups and to expand their involvement into earlier stages of discovery and the drug development process. Patient engagement poses new opportunities as different archetypes of partnerships begin to develop within an evolving R&D ecosystem.

Background

Science has reached an inflection point that is expected to deliver significant advances in the fight against life-threatening diseases. Consequently, leadership in R&D requires a new way of thinking. One new approach involves pharmaceutical R&D to target investigative therapies at patient subpopulations. This has elevated the need for drug manufacturers and clinical trial sponsors, like Pfizer, to engage with patient communities to better understand the necessary qualities expected of new therapies to address their unmet needs. Furthermore, patient perspectives early on and throughout the R&D process benefits both patients and drug developers. As credible experts, patient communities collaborate with industry to reveal valuable patient perspectives, provide data assets and intellectual capital, and help to drive development programs during early science and discovery. Other benefits of patient engagement may include:

- reducing the risks of early science; patient-relevant research questions and findings; more efficient recruitment, retention, and clinical study optimization; access to experts and pre-competitive consortia/collaborations; study designs that include relevant patient-centered outcomes assessments; alignment of research agendas; avoidance of some costly/time-consuming protocol amendments; input to lay summaries through interpretation and dissemination of study results; alignment of patient-defined value messages that will facilitate payer discussions and to help inform regulatory decision making; earlier insights into market access issues.

Key Facts and Figures

Pfizer has been a leader on patient engagement for over a decade. Various archetypes of partnerships across the continuum of R&D, that begin in early discovery and continue through product registration and beyond, have been undertaken. These partnerships are vast in scope and represent a wide range of goals, such as insights to clinical research, novel funding models, influencing policy and access, and advocacy-initiated guidance for regulatory submissions. Pfizer’s disease area partnerships have been diverse and have included:

- Friedreich’s ataxia (early discovery); cystic fibrosis (nonclinical research); sickle cell disease (ethnographic research); lupus (preclinical research/disease understanding); Parkinson’s disease (patient reported outcomes); Duchenne muscular dystrophy (protocol design insights and benefit/risk assessment); oncology, neuroscience, rare disease (novel funding partnerships)

The importance of enhanced patient engagement in R&D is recognized by a broad coalition of stakeholders. For example, the Clinical Trials Transformation Initiative (CTTI) is a public-private partnership whose aim includes promoting practices to increase the quality and efficiency of clinical trials. In parallel, patients are also increasingly
involved in public policy and the regulatory procedures that impact drug development. To facilitate patient involvement into the regulatory process of drug development, programs have begun in some government agencies including,

- Food and Drug Administration (FDA): Patient-Focused Drug Development (PFDD) program.
- European Medicines Agency (EMA): In addition to the dedicated Patients’ and Consumers’ Working Party (PCWP), patient representatives are present on a range of the EMA’s committees, including its Management Board.

One disease-specific example of patient group-regulatory cooperation is the FDA’s Draft Guidance on Duchenne muscular dystrophy (DMD). To help accelerate development and review of potential therapies for DMD, the Parent Project Muscular Dystrophy (PPMD) and other stakeholders submitted the first ever patient advocacy-initiated draft guidance for a rare disease to the FDA. Patient representatives are also beginning to have influence on reimbursement procedures, as part of Health Technology Assessment (HTA) procedures, allowing them to communicate their real treatment preferences.

Pfizer’s Position

Patient involvement in R&D is a fundamental strategy to delivering on Pfizer’s commitment toward advancing science, delivering needed therapies, and improving patient outcomes by applying emerging science and technology. Pfizer has a wide range of patient partnerships, whose long-term goals include end-to-end integrations of patient experiences and preferences throughout the R&D continuum, to registration, and beyond.

How Patients, Researchers, and the Health Care System Benefits

Patient engagement can help guide drug development toward meeting patients’ needs and preferences. Engagement between organizations encourages the sharing of capabilities and is based upon achieving mutual objectives and goals. Engagement can build stronger alliances and more diverse representation in clinical research. Patient-relevant research findings can be communicated to all stakeholders to optimize treatment plans. More representative participants in clinical trials can potentially influence health equity and broaden market access to therapies. Therapies that better address patient needs may foster drug adherence and reduce associated morbidities and costs.

What It Means for Pfizer

Patient involvement in R&D means a better chance at delivering on our purpose to significantly improve the lives of patients. It means building a patient-centered culture both internally and externally; it means potentially faster, more efficient development of meaningful therapies; it means greater potential for regulatory success, and greater chance at broader access of our medicines.

Related Pfizer Activities

Patient involvement throughout the drug development process poses opportunities for the advancement of life sciences, as well as the need for principles of engagement. Pfizer continues to seek to involve patients earlier and more systematically in the drug development lifecycle. Pfizer’s global principles when working with patient organizations includes neutrality and independence, separation of interests, transparency, balanced and compliant partnerships, and respect for individual patients. This approach reflects our ability to work with various patient groups, whose interests and goals may be quite different, whether it be early research and discovery, or advocacy and access.