



Patient Engagement and Involvement in Research and Development

Scientific advances in drug development are increasingly informed by active engagement with the growing research-focused patient population. Increasing levels of patient engagement, alongside an evolving understanding of how to learn from and validate external perspectives, are driving an innovative research and development (R&D) ecosystem. Pfizer has been a leader in engagement with patients and patient organizations for over a decade. We are continuing to build these meaningful relationships and to expand the involvement of patients, patient organizations, and the general public in all stages of the drug discovery and development process. Patient engagement offers new opportunities as different types of partnerships begin to develop within the evolving R&D ecosystem.

Background

Scientific and technological advances are expected to help deliver significant advances in the fight against life-threatening diseases, but challenges remain in the effort to develop innovative drugs. Leadership in R&D requires new ways of thinking. An approach that has emerged over the past decade involves the precise targeting of investigative therapies to patient subpopulations. At the same time, the patient community has shown increasing empowerment, connectedness, and sophistication. The evolving ecosystem has enhanced the opportunity for researchers and clinical trial sponsors such as Pfizer to engage with patients and patient organizations to better understand the characteristics necessary to develop new therapies to address patients' unmet needs. Patient engagement includes dialogue with patients, their care partners and healthcare providers, as well as relevant stakeholders. Each of these communities brings a unique perspective. Early and continued engagement is providing important information and ensuring that patient needs are addressed. As subject matter experts for a specific disease or therapeutic area, patient and patient organizations reveal valuable perspectives, provide access to data, and help to focus research and development programs.

Other aspects of patient involvement in R&D may include: exploration of research questions focused on patient needs; improved clinical trial recruitment and retention; study designs that include relevant patient-centered outcome assessments; more patient-friendly informed consents and other documents; improved study design that avoids or reduces the need for potentially time-consuming protocol amendments; input to plain language summaries for dissemination of study results; patient-focused value messages that may facilitate payer discussions and help inform regulatory decision making; and earlier insights into market access challenges.

Key Facts

Pfizer has been actively engaging with patients and the broader community for over a decade. Pfizer's patient partnerships range across the continuum of the R&D process, beginning in early discovery and continuing through drug development, product registration, and beyond. Patient-centric collaborations enable open dialogue on a range of topics, including unmet needs, clinical research targets, policy and access issues, and regulatory submissions. Pfizer's disease-area partnerships have been diverse and focused on different therapeutic areas including cystic fibrosis, sickle cell disease, lupus, Duchenne muscular dystrophy, oncology, and cardiovascular and metabolic disease.

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.¹ Patients are also increasingly



involved in public policy and the regulatory procedures that impact drug development. To facilitate patient involvement in the regulatory process, programs have begun in government agencies, including,

- Food and Drug Administration (FDA): Patient-Focused Drug Development (PFDD) program.²
- European Medicines Agency (EMA): In addition to the dedicated Patients' and Consumers' Working Party (PCWP),³ patient representatives are present on a range of the EMA's committees, including its Management Board.

One disease-specific example of patient organization-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-initiated draft guidance for a rare disease to the FDA.⁴ Patient representatives have also provided input on reimbursement procedures, for example as part of Health Technology Assessment (HTA) procedures, allowing patients to communicate their treatment preferences.

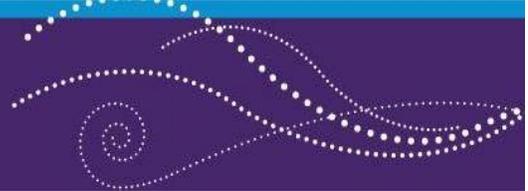
Pfizer's Position

Patient involvement in R&D is a fundamental component of Pfizer's commitment to advancing science, delivering needed therapies, and improving patient outcomes. Pfizer engages in a variety of partnerships and initiatives, whose long-term goals include end-to-end integration of patient experiences and preferences throughout the R&D continuum, to product registration, and beyond. A few examples of how Pfizer is engaging with patients are listed below.

- **Setting the Research Agenda:** Pfizer engages with patients and patient organizations in numerous disease areas to develop research agendas and determine shared goals. Some examples of disease areas that Pfizer works in include: sickle cell disease, muscular dystrophy, and other rare diseases.
- **Clinical Trial Input:** Pfizer engages with members of patient organizations to gather their input on the feasibility and design of clinical trials, including on the development of protocols and endpoints, informed consent, and other clinical trial documents. Patient organization representatives and Pfizer clinicians review patient feedback, discuss how it may be incorporated into upcoming trials, and provide information to the patients regarding which feedback may or may not be incorporated and why.
- **Plain Language Summaries and Return of Individual Results:** Pfizer believes that data collected during a clinical trial should be returned to the study participants, if they wish and where permitted, so that they may better understand the research in which they participated and use the data gathered about their health. Pfizer was the first biopharmaceutical company to return individual clinical trial data to patients. Pfizer is also providing summaries of aggregate clinical trial results in easy-to-read, non-technical language so that study participants can understand why the study was done, how it was done, and the results.

How Patients, Researchers, and the Health Care System Benefit

Patient engagement can help guide development of therapies and vaccines to meet the needs and preferences of patients and the broader community. Discussions with patients and patient organizations that are based upon mutual respect and independence can facilitate the achievement of mutual goals. This exchange of ideas can lead to better research and clinical trial design, improved recruitment for clinical research and more diverse patient population representation in clinical trials, which can help to achieve health equity and increase access to therapies. Therapies that better address patient needs may foster adherence to treatment plans and reduce associated morbidities and costs.



What It Means for Pfizer

Patient involvement in R&D enables Pfizer to deliver on its mission to significantly improve the lives of patients. It means continuing to build a patient-centered culture, both internally and externally; as well as to develop meaningful therapies potentially more efficiently and effectively, with greater regulatory success and improved access to our therapies.

Pfizer Patient Engagement Principles

Pfizer continues to seek to involve patients earlier and more systematically in the drug development lifecycle. Patient involvement throughout the drug development process offers opportunities for the advancement of life sciences, but also creates a need for principles of engagement. The principles that guide Pfizer's work with patients and patient organizations include *mutual respect and independence, avoidance of actual or perceived conflicts of interest, and transparency*, in a manner that is consistent with applicable laws and regulations as well as Pfizer's corporate values. This approach reflects our commitment to working with patients and patient organizations who share our interests in improving health, whether focused on research and development, or access to our medicines.

¹ See: <https://www.ctti-clinicaltrials.org>.

² U.S. Food and Drug Administration. The Voice of the Patient: A series of reports from FDA's Patient-Focused Drug Development Initiative. Available at: www.fda.gov/forindustry/userfees/prescriptiondruguserfee/ucm368342.htm. Accessed August 28, 2017.

³ European Medicines Agency. Patients' and Consumers' Working Party. Available at www.ema.europa.eu/ema/index.jsp?curl=pages/contacts/CHMP/people_listing_000017.jsp&mid=WC0b01ac0580028d32. Accessed on August 28, 2017.

⁴ EndDuchenne.org. FDA Draft Guidance on Duchenne. Available at www.parentprojectmd.org/site/PageServer?pagename=Advocate_fdaguidance. Accessed on August 28, 2017.

