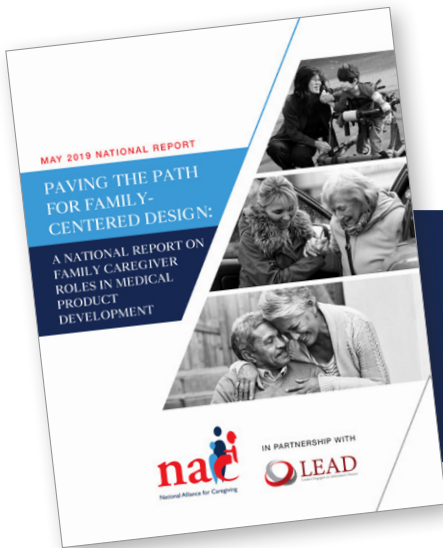




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PAVING THE PATH FOR FAMILY-CENTERED DESIGN: A NATIONAL REPORT ON FAMILY CAREGIVER ROLES IN MEDICAL PRODUCT DEVELOPMENT

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At conferences and in articles related to patient-centricity and patient-focused medical product development, the term “patient” is often described as referring to patients, caregivers, advocates, and others concerned about the patient’s wellbeing.

Yet, the perspectives of patients and caregivers may differ and should be considered distinct from one another. “Paving the Path for Family-Centered Design: A national report on family caregiver roles in medical product development,” explores the vital roles that family caregivers can play in shaping biomedical research and development, regulatory decision-making, and healthcare delivery. It presents recommendations for leveraging the enormous – and largely untapped – reservoir of information and learned wisdom of caregivers about the conditions their care recipients experience and the health outcomes that matter most.

The nature and prevalence of unpaid family caregiving is changing dramatically. With longer lifespans, a widening care gap, and changing family structures, the demand for family caregivers is growing exponentially, while the supply is shrinking. For at least a subset of caregivers, identifying a productive outlet for their close-up observations and learned wisdom would provide added meaning to their lives – and potentially better health outcomes for those in their care.

A national summit on Family Caregiver Roles in Patient-Focused Medical Development, held November 1, 2018 in Washington, D.C., convened 50 professionals representing a variety of disciplines and stakeholder groups to explore ways that family caregivers can contribute to the development and delivery of medical therapies. With regulatory agencies in the U.S. and Europe setting new expectations that patients’ priorities and preferences will be integrated from the earliest stages of medical product development, they agreed that it is time to illuminate a new dimension in the science of patient input by distinguishing the role of caregiver as a vital resource and stakeholder.

“Paving the Path” provides background on ways in which patient perspectives are beginning to inform and shape key decisions about biomedical research and development. The report establishes working definitions for potential caregiver roles, acting as observers, reporters, surrogates, and proxies, as the needs and desires of the care recipient, his or her age, intellectual capacity, and other factors may warrant. Conceptual models depict the interplay of patient and caregiver voices in various conditions, using examples drawn from Alzheimer’s, pediatric rare disease, and mental health. The challenges of more fully engaging caregivers are acknowledged and addressed as well.



PATIENT-FOCUSED MEDICAL PRODUCT DEVELOPMENT (PFMPD)

Formalized under the 2012 Food and Drug Administration (FDA) Safety and Innovation Act (FDASIA), a series of 24 meetings hosted by FDA provided FDA staff, R&D sponsors, and the public opportunities to hear directly from patients about symptoms, impacts of their condition on daily life and long-term health, outcomes of greatest priority, burdens of available therapies, and other unmet needs. Through these meetings and activities hosted by others at FDA, FDA has recognized that “what patients care most about may not always be factored into clinical trials or approved labeling.”

This program has also fostered tool development by numerous multi-stakeholder initiatives and life science companies are initiating or expanding activities to better understand patient perspectives. The capacity for generating and making good use of patient experience information varies widely, but there is more interest than ever in scaling up.

The report includes more detail about PFMPD and ways in which it is already making a difference.

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A NATIONAL REPORT ON FAMILY CAREGIVER ROLES IN MEDICAL PRODUCT DEVELOPMENT

The report examines each stage of medical product R&D, building on existing patient engagement models to highlight where caregiver insights might be most useful:

Discovery & Pre-Clinical Phase: Building a holistic understanding of the natural history and unmet medical need; informing design features of a product such as dosing and acceptability of mode of administration; and development of patient-centered and caregiver-relevant clinical outcome assessment tools.

Phases 1-3: Conveying perceptions of benefit-risk tradeoffs – serving as an observer, reporter, surrogate, or proxy for the patient or from an independent perspective; informing protocol design (especially under conditions where the caregiver is essential to enabling participation in a clinical trial); and developing communications about trials, data analyses, and early or expanded access programs.

Regulatory Review: Supporting key regulatory meetings to articulate the unmet needs of patients, benefit expectations, and tolerability of tradeoffs for side effects or other adverse events; participating in market research to inform packaging, labeling, and patient-facing materials; and developing programs to address Risk Evaluation and Mitigation Strategies (REMS).

Post-Approval: Collaborating on post-marketing study requirements (if any) and ongoing surveillance; developing market access strategies; preparing for health technology assessment and commercial launch; informing the ongoing marketing and patient information materials, including identifying and problem-solving for emergent barriers to access or adherence.

The report's final pages present a suite of potential actions to advance caregiver participation in patient-focused medical product development. Recommendations focus on opportunities to leverage existing policy, enhance emerging practices, and pursue novel possibilities. The National Alliance for Caregiving will convene a multi-disciplinary, multi-stakeholder Caregiver Pathways Task Force to prioritize actions to enlarge the national discussion and galvanize a movement to better integrate caregivers and their perspectives. Venues for potential action include:

Implementation of FDA Reauthorization Act and 21st Century Cures Act: The U.S. Food and Drug Administration (FDA) is working on a series of four regulatory guidances to further shape expectations for and the practice of PFMPD. Public opportunities to inform these guidances should be seized by those interested in advancing distinct roles for caregivers.

Development of a National Family Caregiving Strategy: Under the RAISE Family Caregivers Act, the Secretary of Health and Human Services will identify recommended actions to support family caregivers and promote patient-centered care. This plan must reinforce opportunities for caregivers to inform medical product development as a means of achieving patient-centered care.

Ongoing PFMPD/PFDD Programs: Through greater engagement with FDA and existing collaborative initiatives shaping the science of patient input, deepen understanding of the various roles caregivers play and ways in which they can be adopted into practice. Similarly, caregivers can

be encouraged to participate in disease-specific meetings held through FDA's externally led PFDD program in direction provided by FDA to applicant organizations and across the planning process for those selected to conduct meetings.

Training Opportunities: Training materials designed to educate patients about the process of medical product development could be enhanced with information specific to caregiver roles and distributed through caregiver-rich networks to equip caregivers with knowledge to participate effectively.

Evidence Development: Documenting and disseminating case studies of key initiatives where caregiver perspectives are being utilized to inform medical product development, several of which are described in the report, could stimulate greater awareness of opportunities to engage caregivers, as well as the rationale for doing so. Similarly, conducting a literature review and compiling a resource library of caregiver-related contributions to medical product development would help to advance practice.

AMERICA'S CAREGIVERS

- Approximately 43.5 million people provide unpaid caregiving services to Americans of all ages, 60% of whom have a long-term health condition.
- As a group, caregivers provide \$500 billion in economic value.
- 60% of caregivers are women; the average age of caregivers is 49, although 1 in 4 caregivers is part of the Millennial generation. 21% of U.S. caregivers are Hispanic, 20% are African-American, and 20% are Asian-American; Caucasians account for 17% of caregivers.
- 65% of recipients of care are women and the average age of care recipients is 69.4 years of age; however, 3.7 million people provide caregiving to children under age 18 who have health conditions and/or other special needs.
- Half of all caregivers perform medical/nursing tasks of a type and complexity once provided only in clinical settings; 84% of those caring for a child or adult with a rare disease perform medical/nursing tasks.
- 33% of caregivers dedicate more than 20 hours each week to caregiving, putting them at risk of increased emotional, physical, and financial strains.
- Caregivers derive satisfaction from better understanding their care recipient's condition(s) and assuring the care recipient is well cared for; they experience a sense of purpose as a valued member of the care team.

In "Paving the Path," these and other facts about today's caregiving landscape are summarized from a variety of research reports issued by authoritative sources, including the National Alliance for Caregiving.

The **National Alliance for Caregiving** and the **Leaders Engaged in Alzheimer's Disease (LEAD) Coalition** have committed to pursue actions that align with their missions and capabilities. They strongly encourage others to join them or contribute in other ways to enhance caregiver engagement in biomedical R&D and healthcare delivery.