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"What do you want waiting for you?"
A qualitative study of patient advocate involvement in cancer research

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Bachelor of Arts
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2009

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An abstract of
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Abstract

"What do you want waiting for you?"
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By Grace Ellen Schroer

Background: Patient advocates have been involved in guiding and advising the cancer research process for more than two decades. In recent years, advocate involvement in research has increasingly been required. Despite their historic and expanding presence, little research has been done into the activities and impacts of patient advocates in research. The goal of this qualitative study was to thoroughly document the origins and context of patient advocacy before conducting interviews designed to answer two primary research questions: 1) What is the role of the patient advocate in cancer research? 2) How does the work of patient advocates impact cancer research?

Methods: Semi-structured one-on-one phone interviews were conducted with 13 self-identified patient advocates in cancer research. Purposive and snowball sampling techniques were used. Interviews were recorded and transcribed verbatim, and annotations and memos created throughout the data collection and analysis process. Inductive and deductive codes were arranged hierarchically to create a coding tree and codebook. Each interview was coded in MAXQDA 11. Cross-case comparisons were performed for relevant codes.

Results: Participants said their primary role as patient advocates in research was to provide the patient perspective to researchers. By bringing a patient perspective to researchers, advocates hope to have an impact by ensuring that research meets the needs of patients as much as possible. Participants described several approaches used to establish their credibility and strategies used in their work with researchers to meet their goal of aligning research with patient needs, but their work is hampered by significant barriers. Advocate work with researchers is issue-driven and includes activities such as reviewing grant applications, supporting grant applications, providing advice on clinical research, and providing advice on basic research.

Conclusions: The lack of research around patient advocacy is troubling. As advocates, researchers, and the research system invest time and resources in involving advocate perspectives, it is important to have a clearer understanding of the effects of that involvement. Furthermore, it is essential to better define what activities are most likely to have positive impacts so that advocate involvement can be as effective as possible.

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CHAPTER ONE: INTRODUCTION

Defining Patient Advocacy in Research

This thesis is concerned with issues of patient advocates in research, henceforth referred to as patient advocates. For the purposes of this paper, patient advocates will include lay members of the public who are invested in a specific cancer cause, often because either they or a loved one has had cancer, and who work to advance medical research within the research system.

However, the term "patient advocate" is used in various ways across the healthcare system, and as a result, patient advocacy can mean very different things to different people (Morreim, 2004). Outside of clinical research, patient advocates may work in hospitals to convey patient concerns, or in HMOs to process managed care decisions. Within clinical research, research advocates or activists may lobby for research funding or work to influence the direction of research (Morreim, 2004). A more specific definition of research advocacy describes a research advocate as a patient advocate who works to expand research participation options and contributes to the design of research projects and clinical trials (Dresser, 2001), although this may be overly restrictive.

Patients, Consumers, and Users

Navigating the various meanings of "patient advocacy" is difficult enough, but the myriad other terms that have been used to describe similar concepts add an additional element of confusion (Boote, Telford, & Cooper, 2002). Disagreement over proper and preferred terminology can be so extensive as to make up entire discussions about the appropriate roles of "consumers," "users," "lay persons," "clients," "service users," or "patients" in health care and medical research (Bastian, 1998; Boote et al., 2002). One effect of this lack of clear terminology is that major health databases do not have a keyword or subject heading to

classify articles that discuss patient and public involvement in research, which makes it difficult to conduct a thorough review (Boote, Baird, & Beecroft, 2010).

Among the controversies are disagreements over terms such as "patient," "cancer survivor," "consumer," and "service user." The term "patient" may not apply to a person who has completed treatment, which is typical of many people involved in patient advocacy (Staniszewska, Adebajo, et al., 2011), and a patient may imply a passive entity (Baggott & Forster, 2008). Use of "cancer survivor" is controversial, with different groups applying conflicting definitions. Some patients dislike the term, and others question whether it should include not only patients, but also family members affected by a loved one's diagnosis (Twombly, 2004). The term "consumer," meanwhile, may not apply in health care systems where patients do not purchase health insurance coverage or health care services (Baggott & Forster, 2008). "Service user" may be more empowering and has roots in emancipatory research (Barber et al., 2012), but is rarely used. Ultimately, every term has been found to have various negative connotations, and no term has perfectly described the complexities at play (McLaughlin, 2009).

Preferred terms have shifted over time, particularly in the United Kingdom, with shifting definitions of consumerism and of the patient since the 1970s in response to patient-consumer organizations (Mold, 2010). More recently, patient advocacy in the United Kingdom has been generally encapsulated by the term "patient and public involvement," or PPI (Department of Health, 1999). However, this term has not been widely used in the United States, where there is less focus on public involvement (see Historical Origins section).

Assessing Advocacy

Advocacy has been described as "selfish benevolence" on behalf of an individual case, or, in the form of class advocacy, as an organized action on behalf of a group. Whether case or class, this benevolence includes supporting, maintaining, or defending a cause (McCabe,

Varricchio, Padberg, & Simpson, 1995). In the case of patient advocacy, this cause is the patient perspective in the research system.

Dimensions of Advocacy: Involvement

In the current research system, lay involvement may often be trumpeted but not actually meaningful (Gibson, Britten, & Lynch, 2012). One popular definition used by INVOLVE, the United Kingdom's government-funded program to support public involvement in their National Health Service (NHS) and health research, is that public involvement in research is "carried out 'with' or 'by' members of the public rather than 'to', 'about' or 'for' them" (INVOLVE, 2012). While emphasizing active involvement, this does not capture the variety of possible activities and engagement levels within public involvement or patient advocacy. Patient advocacy includes actions across a continuum of engagement and collaboration, as the NHS has emphasized by calling for "active involvement" of the public in research, particularly patients, service users, and carers (Department of Health, 2005).

Many approaches to qualifying the varying levels of patient involvement and engagement build on the work of Arnstein (1969), who developed an eight rung "ladder of citizen participation" in an attempt to describe citizen participation in government. Arnstein was particularly interested in differentiating between "empty ritual" and "real power," emphasizing that "participation without redistribution of power is an empty and frustrating process for the powerless" (p. 216). Historically, patient advocacy has included both empty ritual and real power, and researchers and academics have applied a variety of names to these shades of involvement, as seen in a table published in a recent Patient-Centered Outcomes Research Institute (PCORI) review which ranked the preferred terms for patient involvement from four journal articles on a scale from "passive" to "engaged" (Garces et al., 2012).

Dimensions of Advocacy: Initiation

Of course, dimensions of advocacy beyond involvement have also been identified. Robinson, Newton, and Dawson (2012) suggest a two-dimensional conceptual model with four quadrants of public involvement that introduces the concept of initiation. In this model, "public ignored" indicates that the public has no involvement and thus no impact on the research process, "public engaged" indicates that the public has the most impact, and "public acknowledged" and "public advised" are areas with medium impact. Higher impact projects are likely to be more collaborative with more advocate control, while advocates in lower impact projects have less power, fewer collaborative opportunities, and are less likely to be driven by grassroots, bottom-up action. This scale of "top-down" to "bottom-up" research builds on a framework proposed by Oliver et al. (2006), which focused on the professional researchers' degree of engagement, which could range from initiating public involvement to responding to responding to public activity, or even being completely absent from public work.

Higher levels of involvement are generally seen as favorable, with Gibson, Britten, and Lynch (2012) arguing that true public involvement requires that lay contributors are seen as participating on an equal footing with the professional researchers and that their input is valued and incorporated. However, greater involvement can be difficult. For example, one focus group study found that patient involvement in the health care system rarely involved engagement beyond patient satisfaction questionnaires, due in part to a lack of clarity about what involvement could entail and how it could be operationalized (Forbat, Cayless, Knighting, Cornwell, & Kearney, 2009). In fact, as can be seen by the variety of scales and frameworks discussed here, researchers still struggle to define what active involvement truly is.

Where in "Research" Does Patient Advocacy in Research Occur?

Just as patient advocacy can occur with varying levels of engagement and forms of initiation, it can also take place during various stages of the research process, including study

development and design, data collection, data analysis, and publication or result dissemination. Koay and Sharp (2013) published a particularly striking visual representation of the stages in which patient advocacy can take place along the research continuum, in addition to providing examples of the forms this advocacy can take. Their examples draw specifically from patient advocacy organizations (PAOs), but advocates acting independently or tangentially through a group can be involved in similar activities. In this model, research begins with the concept and design stage, where PAOs can help define the problem and design the study, among other activities. PAOs can then help with activities such as recruitment, data management, analysis, and interpretations during the data collection and analysis stages. Finally, PAOs can assist during the publication and dissemination stages by developing publications and disseminating the results to scientific and lay audiences, including the media.

Other researchers have attempted to describe the opportunities for public collaboration within specific stages of research. For instance, one 2010 review attempted to synthesize what public contributions in the area of research design alone looked like, arguing that a focus on this area is appropriate because members of the public "have the opportunity at this stage to maximize their influence and impact" (Boote et al., 2010, p. 222). Only seven papers from diverse research areas met their extensive inclusion criteria, and in each study, public or patient involvement was obtained through group meetings. Public input was gathered for various aspects of research design, including ensuring the relevancy of the research question, outcomes, and data collection instruments, as well as ensuring that involvement issues (such as consent) are considered and planning the involvement of the public throughout the study.

A recent systematic review and environmental scan took a more comprehensive approach, seeking to describe patient advocacy across research stages and engagement levels (Shippee et al., 2013). This review utilized various studies, grey literature, and previous

reviews to analyze patient and public involvement in across health research, and 202 sources were included. The authors drew on these sources to synthesize a two-part framework including a description of "patient and service user engagement" (PSUE) at various research stages along the preparatory, execution, and translational phases of research.

New Directions for Patient Advocacy in Research

With new advances in basic and biomedical research come emerging opportunities for patient advocates and increasing uncertainty about how they can be best used (Koay & Sharp, 2013; Landy et al., 2012). In one recent review, the authors noted that translational medical research bringing discoveries "from bench to bedside" is increasingly popular, but that public involvement has only been utilized in the later phases. These authors propose that stakeholder or service user input could instead be utilized throughout the process, beginning with how research questions should be prioritized. The article also includes a table of research on service user involvement with a focus on genomics and biomarkers, which is most relevant to the translational science perspective (Callard, Rose, & Wykes, 2012).

As the field of patient advocacy continues to expand, an understanding of its history is increasingly important. A key element of this history is the many names, definitions, and roles that have been applied to patient advocates over time. Researchers have proposed a variety of models for patient participation, but all generally agree that greater engagement and meaningful involvement produces a greater impact, regardless of the stage of the research.

Current Patient Advocacy Programs in the United States

Because patient advocacy in the United States can take place across the research spectrum and involve a variety of activities, a number of diverse opportunities to incorporate patient input have developed. Several of these programs are listed in Figure 1 and are described in this section. They can generally be grouped into three categories: federal and

state governmental programs, non-governmental research programs, and non-profit and advocacy programs.

Governmental Programs

Foremost among the governmental programs are those available through the Department of Health and Human Services (DHHS). The first advocate appointed to a National Institutes of Health (NIH) intramural board was approved by NIH director Harold Varmus 18 months after the initial application with the handwritten note, "Just this once – as an experiment" (Collyar, 2005, p. 73). Since then, their presence has grown significantly. Varmus later established the Director's Council of Public Representatives (COPR), and there are various other patient engagement opportunities throughout the Institutes. Those offered through the National Cancer Institute (NCI) are highlighted in Figure 1, including the Director's Consumer Liaison Group (DCLG) and the Consumer Advocates in Research and Related Activities (CARRA). Patient involvement in peer review has a long history in cancer research, and NCI responded to the trends with the creation of its CARRA program (Gilkey, 2014). The Specialized Programs of Research Excellence (SPORE) program has also brought advocate perspectives to translational oncology research (Collyar, 2005).

In 1955, NCI established the National Clinical Trials Cooperative Group Program to conduct large-scale studies of chemotherapy. Since then, cooperative groups have been well-positioned to conduct studies that industry has less incentive to take on, including evaluations of combination therapies and comparative-effectiveness trials. In 2010, the program was comprised of more than 3,000 institutions and 14,000 investigators, who enrolled more than 25,000 cancer patients in cooperative group clinical trials annually (National Cancer Institute, 2009). Through the Coalition of Cancer Cooperative Group Patient Advisory Board and positions in individual cooperative groups, advocates have led and participated in various specific activities to support clinical research in cooperative groups since 1994 (Collyar, 2005).

Although the cooperative group program is currently in transition, patient advocates, whether for cooperative groups or for the new National Clinical Trials Network, will continue to advise collaborating researchers on study questions and design and assist with recruitment plans.

Even beyond NIH, nearly every agency in DHHS has its own patient input opportunities, including the Agency for Healthcare Research and Quality (AHRQ), Centers for Medicare and Medicaid Services (CMS), Food and Drug Administration (FDA), and PCORI, as shown in Figure 1. For instance, AHRQ has the National Advisory Council for Healthcare Research and Quality, made up of a private-sector panel that includes consumers. The Medicare Evidence Development and Coverage Advisory Committee (MEDCAC) offers guidance to CMS on clinical issues and includes patient advocates, as well as various medical ethicists.

With the fifth authorization of the Prescription Drug User Fee Act, the FDA is required to systematically assess patient-reported outcomes and seek patient perspectives on unmet therapeutic needs as part of a new Patient-Focused Drug Development Initiative. Meetings are being held from 2013-2015 for several conditions, including one that was held for lung cancer in June 2013 (Food and Drug Administration, 2013b). The FDA also involves advocates as patient representatives during advisory committee meetings, where they provide feedback and often have a vote during the review of therapies and products. The cancer liaison program facilitates input from additional advocates for cancer products. Patient consultants are brought in earlier, during the drug development process, to provide input as companies create and test new drugs.

In a description of the origins and actions of PCORI, which was established as part of the Affordable Care Act in 2010, top PCORI leaders describe their approach toward integrating the input of patients and other stakeholders throughout their research process. They emphasize the importance of involving stakeholders at points all along the process "to

Figure 1. An overview of organizations in the United States that work with or support patient advocates.

generate topics for research, help the institute prioritize those topics, select topics for funding, and ensure patients' involvement in the design of research projects" (Fleurence et al., 2013, p. 393). PCORI's mission is to focus research on the areas and outcomes that matter most to patients, rather than arbitrary endpoints or treatment options that patients perceive as irrelevant. At the same time, the authors acknowledge the patient-centered work that other agencies have focused on, including the involvement of patients in the systematic review process at AHRQ. Among the processes that PCORI is using to integrate patient input are techniques already popular in the United Kingdom, where more work has been done evaluating patient involvement in health and research; these include using the nominal group technique as part of a deliberative prioritization and peer review process (Fleurence et al., 2013).

Beyond DHHS, one key area for patient advocates within the federal government is the Department of Defense (DOD). What began in 1992 as $25 million in funding for the Breast Cancer Research Program has become the Congressionally Directed Medical Research Program (CDMRP), funding more than $7 billion in research awards for various disease types and involving consumer reviewers throughout the award process (Department of Defense, 2014). There may also be opportunities for advocates within state governmental programs, with California offering the most prominent example: California's Breast Cancer Research Program requires patient advocate involvement in all research it funds (California Breast Cancer Research Program, 2014).

Non-Governmental Programs

Outside of the government, researchers in both industry and academic settings may seek the advice of patient advocates. University researchers, for instance, may be interested in advocate assistance with grant applications, especially as some funders require advocate

involvement. Within the pharmaceutical industry, researchers may request advocate assistance in accruing patients to clinical trials.

Whether they work in industry or academia, cancer researchers are typically members of at least one professional association: either the American Association for Cancer Research (AACR), which focuses more on basic research, or the American Society of Clinical Advocacy (ASCO), which focuses on research with clinical applications, as well as other issues in clinical oncology. AACR was founded in 1907 to "further the investigation and spread the knowledge of cancer." More than 34,000 scientists, working in academic, governmental, and industry research programs from around the world, are members (American Association for Cancer Research, 2013). ASCO was founded by members of AACR who were interested in focusing on clinical oncology; as a result, their more than 34,000 members include representatives of all oncology specialties and are not necessarily researchers (American Society of Clinical Oncology, 2014a, 2014b).

Both ASCO and AACR have formal mechanisms to engage patient advocates. ASCO's annual meeting offers a reduced patient advocacy registration rate, patient advocacy booths in the exhibit hall, a patient advocate lounge where advocates can interact, and research review sessions specifically designed for lay advocates where experts discuss some of the research advances being presented at the meeting (American Society of Clinical Oncology, 2013). Similarly, AACR offers the Scientist↔Survivor Program, which includes a competitive application process and brings accepted advocates together for a special programming throughout their annual meeting (American Association for Cancer Research, 2014).

Also important are non-profit advocate organizations that devote significant resources to supporting advocates. Historically, one organization that has been at the forefront of patient advocacy is the National Breast Cancer Coalition (NBCC) and its sister entity, the National Breast Cancer Coalition Fund (NBCCF). Founded in 1991, NBCCF is dedicated to bringing

consumer voices to breast cancer research. One of their strategies has been Project LEAD, which stands for leadership, education, and advocacy development. Started in 1995, Project LEAD's in-person training educates activists in scientific language and processes, as well as the key issues in breast cancer (Dickersin et al., 2001; Visco, 2005). In addition to various other nonprofit organizations, advocates representing all cancers have been able to interact in online communities and forums, and through local organizations, including several discussed in the literature review.

Other Forms of Patient Advocacy

While the above have all been descriptions of patient advocacy in research, it is important to note that the most commonly understand meaning of patient advocacy in the United States is an individual – whether a friend or family member, a nurse, a trained professional, or even the patient – who represents the patient's interests in health care decisions and often helps guide the patient through the health care system. As a result, there are a number of organizations that include "patient advocate" or "patient advocacy" in their name, but whose actions do not include research advocacy. Perhaps most prominent of these is the non-profit Patient Advocate Foundation (PAF), which has case managers available to help patients with serious illnesses negotiate with insurers, employers, and creditors (Patient Advocate Foundation, 2013). PAF's sister organization, the National Patient Advocate Foundation (NPAF), focuses on legislative issues including access and reimbursement for care (National Patient Advocate Foundation, 2013). Similarly, the University of Wisconsin-Madison has established a Center for Patient Partnerships, which offers advocacy services for patients and trains professionals to work as patient advocates. It also conducts research on the effects of patient advocacy, but again, this is advocacy that helps an individual navigate the health system (Center for Patient Partnerships, 2014).

There are also a number of for-profit organizations within this space. Patient or health advocacy is promoted as a "growing career choice," and a number of online resources are available to help individuals learn more about the field and "start and grow a thriving practice" (DiagKNOWsis, 2013). The Center for Patient Advocacy offers to match clients to registered nurses after a free phone consultation (Center for Patient Advocacy, 2013). Many advocates, however, operate independently. Instead of working for a larger company, they may become members of a patient advocacy professional association, such as the Alliance of Professional Health Advocates (APHA) or the National Association of Healthcare Advocacy Consultants, as an indication of legitimacy or a way to advertise their services (Alliance of Professional Health Advocates, 2013b; National Association of Healthcare Advocacy Consultants, 2013).

The business plan for the Patient Advocate Foundation was developed in 1995 (Patient Advocate Foundation, 2013). APHA considers Ken Schueler the "Father of Private Patient Advocacy," and he began practicing in 2000 (Alliance of Professional Health Advocates, 2013a). Thus, patient health advocacy, like patient research advocacy, is a relatively new field. However, in many ways, patient health advocacy has acted more comprehensively to standardize the field. While there is no nationally recognized certification, both NAHAC and APHA have developed ethical codes to which advocates can subscribe as an indication of their integrity and adherence to best practices (Alliance of Professional Health Advocates, 2012).

Theory: Social Ecological Model of Medical Research

Social ecological models in public health have traditionally focused on promoting or reducing specific health behaviors, such as promoting physical activity and reducing smoking. These models propose multiple levels of factors that influence a behavior. For smoking, these might include intrapersonal level influences such as individual genetic factors that affect addiction, interpersonal level influences such as peers' or family members' smoking, community level influences such as availability and marketing of tobacco, and policy level

influences such as cigarette taxes or bans on smoking in public places. Together, these types of levels and factors make up a comprehensive framework of what influences a behavior such as smoking (Sallis, Owen, & Fisher, 2008).

Sallis et al. (2008) propose four core principles underlying different ecological models for health behavior: (1) they include multiple levels of factors, (2) the factors interact across levels, (3) they should be specifically targeted to a specific behavior, and (4) interventions to change a behavior will be most effective if they work at multiple levels. While public health has primarily used the social ecological model as a basis for understanding and designing interventions in health behavior, the underlying concepts are applicable in other situations. In this case, a social ecological model provides a useful description of the context and influences on medical research, which includes multiple levels, with factors interacting across levels, all of which specifically influence a particular issue: medical research (Figure 2).

The fundamental issue of interest is what influences the research being done, and thus, research occupies the central circle. Just outside, at the intrapersonal level, the individual actors are the researchers. Their research can be influenced by several individual factors, including their interests and training.

The interpersonal level addresses relationships between researchers to form research collaborations, as well as the relationships between researchers and patients that are necessary to conduct clinical research. The availability of collaborations, whether formal or informal, as well as the willingness of patients to participate in a specific area of research, can both influence the research that is done by researchers.

The community level is perhaps the most active. Research directions are clearly influenced by how review boards administer funding, whether from governmental or private sources. Research institutions, whether academic or in industry, may have requirements of their researchers that further influence the research that is done. Journals and professional

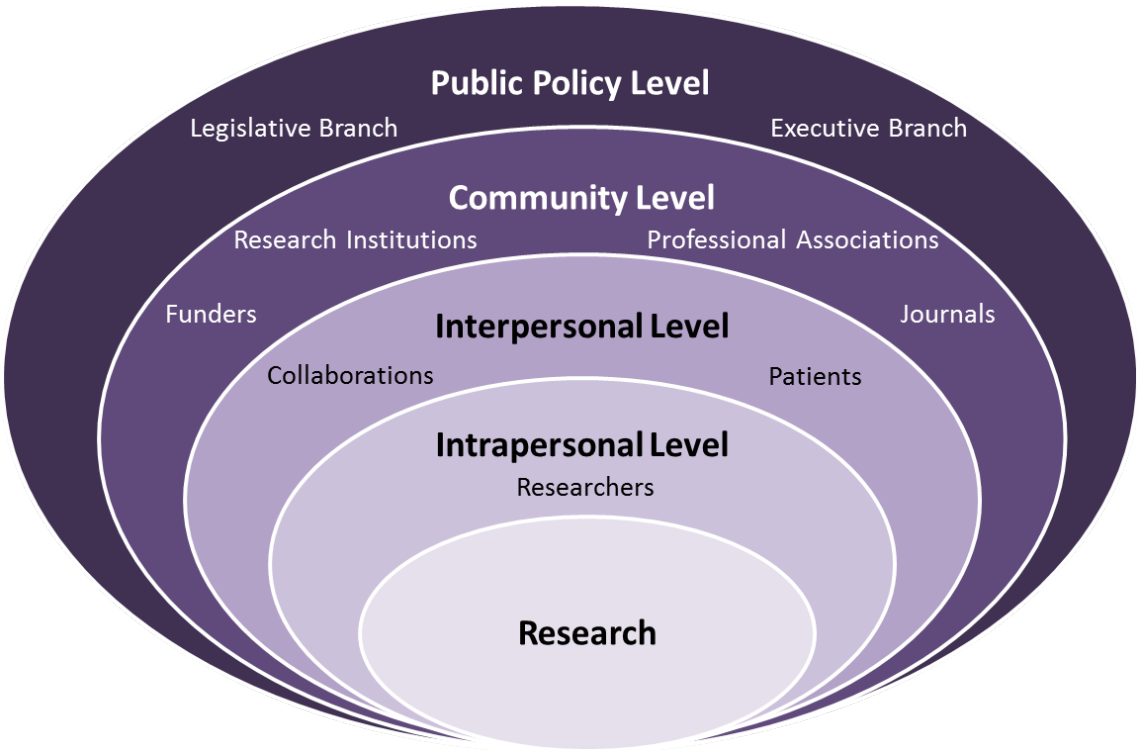

Figure 2. Social ecological model of medical research.

associations make decisions about which articles and abstracts should be accepted for publication or presentation. If certain areas of research are perceived as more likely to be accepted, this can influence research choices, especially in conjunction with funders and institutions that value publications and presentations.

Finally, at the societal level, the legislative and the executive branches work to establish agency budgets for research funding, which is then distributed by funders. More or less research funding for a particular agency or disease area changes what research is ultimately done. Congress and executive agencies can also regulate the research process.

Patient advocates for research ultimately seek to affect what medical research is being done in the hopes that the research produces outcomes relevant to patients and is conducted in a way that is acceptable to patients. While they may not affect every factor described in the model, patient advocates can and do act at multiple levels and on multiple factors. These activities include working directly on research with researchers (intrapersonal level), engaging

patients in research (interpersonal level), providing advice to funding organizations (community level), and lobbying public policymakers for research funding and regulations (public policy level). As was previously discussed, patient advocate actions are not centrally coordinated or organized, so it may be difficult to comprehensively describe what is being done and at what level. However, this model could provide a useful approach to characterizing their work.

Need for this Study

As has been shown throughout this chapter, patient advocacy in research is a broad and relatively undefined field. Significant research funding and resources are dedicated to reducing the growing burden of cancer in the United States and around the world, and patient advocates are increasingly contributing to this research. However, there is minimal information about the various roles that patient advocates play, much less the impact that they have. Thus, this qualitative study attempts to describe the landscape of patient advocacy in the United States, forming a basis for future evaluations.

Cancer Research as a National Priority

In the United States, there were an estimated 1.7 million new cases of cancer in 2013, and 580,000 cancer deaths (SEER Program, 2013). Cancer is the second leading cause of death in the United States, just after heart disease (Murphy, Xu, & Kochanek, 2013). Cancer is the leading cause of years of life lost, with 5.6 million years of life lost each year due to premature deaths from cancer, compared to 3.8 million years of life lost due to premature deaths from heart disease (National Heart Lung and Blood Institute, 2013). The overall costs of cancer were estimated at $216.6 billion for 2008, including $86.6 billion in direct medical costs, and $130 billion in indirect costs due to premature death (National Heart Lung and Blood Institute, 2013). More than 40 percent of people will be diagnosed with cancer at some point in their

lives (SEER Program, 2013). For all of these reasons, cancer research is a national priority in the United States.

Minimal Evidence Base for Advocacy in Cancer Research

Although the United States, like other countries, promotes patient advocate involvement in the research process, there has been minimal research on their impact (Mockford, Staniszewska, Griffiths, & Herron-Marx, 2012; Nilsen et al., 2006; Staniszewska, 2009). Of that, only a small portion focuses on advocacy in cancer research (Hubbard, Kidd, Donaghy, McDonald, & Kearney, 2007). Furthermore, much of the research in this area has been conducted in the United Kingdom, and its transferability to the United States has not been determined (Boote, Barber, & Cooper, 2006).

There have been calls for more rigorous assessments of patient involvement in health research, both to justify the investment in the process and to enhance the way in which patients are utilized (Staniszewska, Adebajo, et al., 2011; Staniszewska, Brett, Mockford, & Barber, 2011; Staniszewska, Herron-Marx, & Mockford, 2008). Most recently, prominent researchers in the areas of patient and public involvement urged a new focus on evaluation in a Comment published in the September 12, 2013 issue of Nature (Petit-Zeman & Locock, 2013). The creation of new government agencies such as PCORI and new mechanisms such as the FDA's Patient-Focused Drug Development Initiative highlight the increasing importance of patient advocacy in the United States and the relevance of research in this area.

Research Questions

Given how much uncertainty surrounds the concept of patient advocacy, there is need for a qualitative exploration that can lay the groundwork for future studies to measure the impact of patient advocates on research processes and participation. Patient advocates are particularly active in cancer research, making it an appropriate focus for this thesis. Thus, with

the proposed social ecological model of research in mind, this study explored the following two research questions:

1) What is the role of the patient advocate in cancer research?
2) How does the work of patient advocates impact cancer research?

CHAPTER TWO: LITERATURE REVIEW

This chapter begins by providing an overview of the origins and historical context for patient advocacy in the United States, with many examples drawn from the breast cancer and AIDS activism movements. There follows a discussion of issues around the background and legitimacy of patient advocates. The chapter concludes with a description of the arguments put forth for and against patient advocacy and the current state of the research from the United States, the United Kingdom, and internationally on patient advocacy and its impact.

Patient Advocacy in the United States

Origins of Consumer Health Movements

In a review of different approaches to clinical research and the different ways in which that research has been regulated, Emanuel and Grady (2007) suggest that there have been four eras with distinct paradigms of biomedical research. First, from World War II to the early 1970s, there was a period of "researcher paternalism" in which the researchers were expected to use their judgment to design appropriate studies, and subjects were seen as passive participants in research. Then, from the 1970s into the 1980s, revelations of harmful research practices such as those seen in the Tuskegee Syphilis Study ushered in an era of "regulatory protectionism," in which vulnerable subjects were protected through IRB reviews and extensive informed consent processes. Beginning in the 1980s, however, activism from the AIDS and breast cancer communities led to a less restrictive period of "participant access" based on individual rights to the highest quality care. More recently, the focus has turned to "community partnership" with active participants in research, rather than passive subjects.

Clearly, modern conceptions of patient involvement in clinical research are a recent phenomenon. Of course, factors other than the activist movements that Emanuel and Grady mention also contributed to this new paradigm. One instrumental figure was Paul Starr, whose 1982 Pulitzer Prize-winning book *The Social Transformation of American Medicine* captured

the movement toward the "generalization of rights" in healthcare (p. 388). This included the extension of rights in healthcare to previously underrepresented and underserved groups, including women, the handicapped, those with mental health issues, and – most relevantly – participants in medical research. Starr argues that this generalization of rights grew out of the American political environment: "Every society shapes the demands made against it. In the United States, the two-party system, the absence of a socialist tradition, and the distinctive role of the judiciary in interpreting the Constitution encourage the dissatisfied to organize in social movements outside the political parties and to present their demands as claims under the Bill of Rights" (Starr, 1982, p. 388).

In this case, the dissatisfied formed the social and civil rights movements of the 1970s. These movements coincided with a new distrust of doctors, hospitals, and the medical establishment. Physicians were no longer assumed to be acting in the best interests of the patient, and reformers wanted more control. Physicians did not respond well to this attack on their authority; American Medical Association president Russell Roth remarked, "Passengers who insist on flying the airplane are called hijackers!" (Starr, 1982, p. 402).

However, the trend toward activism in health persisted, and there are now a variety of approaches toward classifying the diverse activist movements in health. Bastian (1998) focused on identifying the motivation behind group formation. From this perspective, consumer health advocacy groups can take various forms, including groups among people sharing the same health condition or experience, groups among people with a shared experience of being harmed by a product, groups protesting particular practices on an ideological basis, groups with a shared identity, and groups formed for generic advocacy on behalf of a population or as a coalition.

Brown et al. (2004) applied a more sociological approach, suggesting multiple categories of health social movements, including embodied health movements (EHMs). EHMs

closely align with our understanding of patient advocacy, because they are defined as movements that "address disease, disability or illness experience by challenging science on etiology, diagnosis, treatment and prevention" (p. 50). This involves incorporating the embodied experience of patients, challenging existing medical and scientific practice, and ultimately collaborating with scientific and medical representatives. Science is seen as central to EHMs, because these movements often arise due to dissatisfaction with current research and medical practice, but the changes they seek depend on the work of researchers. Breast cancer typifies the politicized collective illness identity that Brown et al. argue is essential to an EHM, and its clearly defined diagnosis, high prevalence, and link to the women's health and AIDS movements have all helped it to become the efficacious social movement it is now. However, even fairly recently, breast cancer was unmentionable in polite society.

Origins of Breast Cancer Activism

Changing Attitudes around Breast Cancer

For most of the twentieth century, cancer was an unmentionable disease associated with shame, guilt, and fear (Holland, 2002). In the 1950s, one breast cancer survivor sought to place an advertisement for a breast cancer support group in the New York Times. Her request was denied because "the *Times* cannot publish the word *breast* or the word *cancer* in its pages" (Mukherjee, 2011, p. 17). Breast cancer was too personal to disclose publicly, and cancer in general was often referred to simply as "the Big C" (Corbett & Mori, 1999). Doctors had little to offer patients in the way of treatment. Until the 1950s, the only care they could provide was surgery or radiation, and this was typically palliative, not curative (Holland, 2002).

Furthermore, patients were not expected to have opinions or make decisions about what treatment they did receive. The standard treatment for breast cancer was a one-step procedure: biopsy and mastectomy were performed in a single surgery. Women would not

know until they woke up if they had breast cancer; if they did, their breast had already been removed (Anglin, 1997).

These attitudes and practices started to change in the 1970s. In 1952, breast cancer patient Terese Lasser was discharged after a biopsy and mastectomy with no instructions other than to exercise her arm. In response, she developed her own rehabilitative exercises and educational materials and began visiting hospitalized women after surgery to share this information. At her persistent urging, the American Cancer Society established the Reach to Recovery program as a formal initiative in 1969, training volunteers nationwide to answer women's questions and support their rehabilitation (Holleb, 1989).

Around the same time, well-known women began to reveal their breast cancer diagnoses, including First Lady Betty Ford in 1974 (Burklow, 1991), and women began to fight for more active roles in their treatment. Rose Kushner, a prominent women's health activist, objected to the one-step procedure for breast cancer. She and other early activists pushed for treatment alternatives and sought to require informed consent before life-changing surgeries. One strategy was the introduction of Breast Cancer Informed Consent bills in 22 state legislatures in the 1980s; none of these bills were carried out, but they raised awareness of the issue (Anglin, 1997).

As women grew more aware of the issues around breast cancer treatment and more willing to discuss "the big C," they formed organizations to offer support, share information, and fight for treatment changes (Anglin, 1997). Now-widespread grassroots organizations such as the Susan G. Komen Breast Cancer Foundation were formed in the 1980s and 1990s (Collyar, 2005). Kushner's creation of the National Alliance of Breast Cancer Organizations in 1986 was one of the first attempts to organize otherwise independent groups (Burklow, 1991). It was this alliance that established the National Breast Cancer Coalition (NBCC), first called the

Breast Cancer Coalition (BCC), in 1991 to more explicitly advocate for breast cancer research, rather than focus on treatment and support (Gillon, 2004).

Origins in Women's Health and AIDS Treatment Activism

Breast cancer activists were not the first to advocate for issues around health and medical research. The women's rights movement was leading similar efforts to transfer power from "paternalistic" medical doctors to increasingly empowered female patients in other settings. Women wanted more female doctors and more control of their medical experiences, particularly during childbirth (Starr, 1982).

More recently, the gay community conceptualized health research activism in ways that are still seen today. Steven Epstein is well-known for his descriptions of the origins and actions of the AIDS treatment activism movement, and particularly of the AIDS Coalition to Unleash Power (ACT UP) (Epstein, 2004). ACT UP was founded in 1987 and brought a radical approach to activism, including massive protests at FDA and NIH to attract attention to AIDS research and treatment issues. The gay community's fight to increase investments in medical research and expand access to experimental treatments for AIDS planted seeds that would grow into active patient advocacy movements across disease areas.

Many advocates in the breast cancer movement in the early 1990s had a history in the women's rights movement. As NBCC President Fran Visco noted, "We were the antiwar advocates of the '60s. We were in the women's rights movement in the '70s. We're now getting breast cancer and we're not used to being quiet" (Gillon, 2004, p. 198). Advocates had also been involved with the gay rights movement, where they were familiar with ACT UP's efforts to improve access to AIDS research. As Visco said in a 2001 newspaper article, "The AIDS movement was something we looked to when we formed the National Breast Cancer Coalition. A lot of us had been antiwar activists and then we saw what the AIDS activists had been able to accomplish" (Collins, 2001).

Advocacy at a Local Level

Breast cancer research advocacy took place at both local and national levels. Much like in the AIDS movement, northern California, and particularly San Francisco, was a hot spot for breast cancer activism (Anglin, 1997). Key groups included NORCAL in Northern California and Breast Cancer Action (BCA) in San Francisco, both research advocacy groups founded in 1990 that sought to apply tactics from ACT UP and the women's health movement to breast cancer activism.

NORCAL was the focus of an ethnographic study in the early 1990s, which highlighted a number of important factors common across early breast cancer advocacy groups (Anglin, 1997). One important characteristic of NORCAL, and of many breast cancer patient groups, was its demographics. Between 1992 and 1994, the NORCAL board included just one woman of color, and one woman on disability payments; other members were white and middle-class. This had implications for the types of activism NORCAL's members focused on. While members had often been diagnosed using mammograms and now wanted even better diagnostic measures, women of color were less likely to have had the benefits of early mammography and wanted to focus on increasing mammography accessibility. Similarly, cultural perceptions of the importance of medical research and the value of participating in clinical trials have differed between white and African American populations.

The Fight for Access to Research: From Local to National

NORCAL and BCA were also extremely active on the issue of access to experimental drugs for compassionate use. The concept of "compassionate use" was developed in response to AIDS research, and its applicability to breast cancer developed in response to advances in the scientific understanding of breast cancer. In the 1980s, researchers discovered that there are many different subtypes of breast cancer, each caused by different genetic mutations. One common mutation is in the HER2/neu gene, causing overexpression of the protein HER2 (or

human epidermal growth factor receptor 2) (Burstein, 2005). Breast cancer tumors that are HER2-positive, and produce too much HER2 protein, tend to be more aggressive than tumors that are HER2-negative, and between 25 and 30% of breast cancer tumors produce too much HER2 (Baselga et al., 1996).

HER2-positive tumors can be identified by testing a tumor sample. However, identification alone is not enough – effective treatments must find a way to act and change the course of the cancer. Unlike other proteins associated with cancer-causing genes, HER2 crosses the cell membrane, meaning a drug can target it without having to enter the cell (Mukherjee, 2011). This made it a good candidate for an antibody protein designed to attach to the HER2 receptor and reduce its expression.

Biopharmaceutical company Genentech, founded in San Francisco in 1976 (Genentech, 2012), developed an antibody to test in women with HER2-positive breast cancer in 1990 (Mukherjee, 2011). They called their new drug Herceptin, and began their first trial in 1992. Of the fifteen women who started, one fully recovered from metastatic breast cancer; others had mixed responses. However, rumors of the trial spread among the breast cancer community, and patients were desperate for the drug.

AIDS activists had already fought for expanded access to experimental drugs on a compassionate use basis (Epstein, 1996). Their rallying cry was "drugs into bodies" (p. 222), and their target was the FDA, which was seen as restricting research and access to experimental treatments. In response, in 1987 and 1988, the FDA worked to develop formal procedures for treatments under investigation to be made available for life-threatening diseases (Young, Norris, Levitt, & Nightingale, 1988), but this was not seen as enough. In 1988, protesters carried tombstones reading, "I got the placebo. R.I.P." outside of the FDA headquarters (Epstein, 1996, p. 225).

A key concern from the FDA's perspective was that researchers would not be able to find people to enroll in clinical trials if patients knew they could obtain unproven, but highly lauded, experimental agent through expanded access programs. Without sufficient research participants, new drugs could never truly be proven effective. However, patients in need of treatment and faced with no other options viewed this argument as coercive. Ultimately, AIDS patients accessed many experimental drugs through both official and unofficial means.

In the early 1990s, the newly established breast cancer advocacy organizations wanted to ensure breast cancer patients had the same access as AIDS patients to potentially life-saving treatments (Mukherjee, 2011). Genentech, however, wanted a drug that was tested in appropriate clinical trials and could be approved by the FDA. They did not have a compassionate use policy, and responded slowly – if at all – to patient requests.

Advocates did not wait quietly. In 1994, BCA members coordinated with San Francisco AIDS activists from ACT UP/Golden Gate to send phone and fax "zaps" to the Genentech office. Phone and fax zaps were a common ACT UP technique intended to tie up phone and fax lines with simultaneous callers (Zimmerman & Haggerty, 1999), a lower-tech version of distributed denial-of-service attacks now used by hackers and online protesters (Mirkovic & Reiher, 2004). Here, the goal was to pressure Genentech to complete tests for HER2 for Marti Nelson, a woman with advanced breast cancer (Evans & Peterson, 1995). Following Nelson's death in December of 1994, ACT UP and BCA led further protests at Genentech's headquarters. NORCAL objected to these tactics but continued to work with BCA to fight for expanded access (Anglin, 1997).

Widespread access to treatment did not come quickly. In 1995, Genentech negotiated with leading national activists, including NBCC's Fran Visco. Visco agreed that NBCC would help enroll patients in the next phase of Herceptin's clinical trials if Genentech would create an expanded access program (Mukherjee, 2011). In 1996, the BCA newsletter announced that

"Breast Cancer Action and ACT UP Golden Gate Breast Cancer Committee claimed victory recently when they learned that two Chicago area women received HER2/neu antibody treatment because of activists' year-long negotiation with Genentech," the first time patients had accessed experimental therapy through the compassionate use program (Breast Cancer Action, 1996).

From Research Discovery to Approved Drug: Cancer Activists and the FDA

Ultimately, the work of breast cancer, AIDS, and other disease activists prompted FDA to develop a variety of mechanisms intended to improve the drug approval process. One of the first of these was the "fast track" designation, intended for drugs treating serious conditions where there is no available therapy or where the drug under review has significant advantages over existing therapies (Food and Drug Administration, 2013a). Herceptin was given "fast track" designation by the FDA and in 1998, in response to initial results from a Phase III trial that was ultimately published in 2001 (Slamon et al.), FDA approved Herceptin (with the generic name Trastuzumab) for HER2-positive patients with metastatic breast cancer (Food and Drug Administration, 1998). Because this approval was based on preliminary findings, Genentech was required to submit additional study results as they were completed.

Another new mechanism developed by FDA in the early 1990s was the "accelerated approval" process. This allows the FDA to approve drugs for an unmet need in a serious illness when research shows an effect on a "surrogate endpoint" which is thought to predict a clinical benefit, rather than requiring that studies show a measurable clinical benefit (Food and Drug Administration, 2013a). Genentech product Avastin had been approved through the standard mechanism for metastatic colorectal cancer in 2004 but was given accelerated approval for metastatic breast cancer in 2008 (Allison, 2010). Although early results in clinical trials found that Avastin had an effect on the surrogate endpoint of tumor growth, long-term clinical trials

found no clinical benefit because the survival rate was unchanged. As a result, FDA had to decide whether to revoke the accelerated approval (Couzin-Frankel & Ogale, 2011).

Many breast cancer patients attended FDA hearings on the topic, arguing that Avastin had helped them and that removing Avastin from the market would be "morally and ethically wrong." Other advocates disagreed, with Visco arguing that "[t]he FDA's role is not to look at those individual stories, it's to look at science." She went on to say that, in the search for any cancer treatment that may do anything, "we've lowered the bar so much" (Couzin-Frankel & Ogale, 2011, p. 143). This echoed statements from an earlier interview, when she said, "We are looking for something that has a major impact, and in no cancer has this drug had a major impact" (Allison, 2010, p. 880).

The FDA's approach to drug approval, and particularly the accelerated approval mechanism, has not been without controversy. Some consider the program successful, but many others have voiced opposing views, either fearing that drugs were still not approved quickly enough, or that drugs were not being sufficiently studied after the initial approval (Richey et al., 2009). Avastin is arguably an example of the system working: it was approved based on initial data, and the approval was quickly revoked when more extensive results were submitted. However, Avastin's approval has had lasting consequences, since it remains listed as a recommended treatment by the National Comprehensive Cancer Network (NCCN) (National Comprehensive Cancer Network, 2011), and Medicare bases coverage decisions for off-label pharmaceuticals in oncology on the NCCN recommendations (Centers for Medicare & Medicaid Services, 2008), so it continues to be prescribed and covered by insurance.

Advocacy and Medical Research Funding

Organization of Medical Research in the United States

Although their work around compassionate use was significant, NBCC's most prominent work was inarguably on research funding for breast cancer. Before World War II,

the federal government had yet to become the leading investor in research and development; instead, "university endowment income, gifts from individuals, and foundation grants" (p. 238) drove advances in medical research (Bush, 1945, p. 238). Federally funded medical research took place, but it was primarily within the Public Health Service and the military. Legislation creating the National Cancer Institute (NCI) had passed in 1937 and included for the first time a mechanism for awarding external "grants-in-aid" for the purpose of "stimulating and creating research institutions outside of those in the federal government" (National Cancer Institute, 1937). This was seen as an opportunity for a council of leading researchers to "decide which applications for such grants-in-aid have merit and should be so furthered, and which are useless and should not receive assistance" ("The National Cancer Institute" 1937, p. 1288).

Coming out of World War II, the country wanted to maintain the momentum of dramatic discoveries that had been made in federally funded research, including well-known examples such as penicillin, radar, and the atomic bomb. Starting in 1940, Vannevar Bush led the military Office of Scientific Research and Development (OSRD) and helped pioneer a new strategy for working with university researchers: instead of inducting top researchers into the military, civilian scientists were awarded contracts and grants (Graham & Diamond, 1997). At President Roosevelt's request, Bush prepared a report outlining recommendations for the future of federally funded research after the OSRD was dismantled, including special reference to "what can be done now to organize a program for continuing in the future the work which has been done in medicine and related science" (p. 231). His report, titled *Science – The Endless Frontier*, was presented to President Truman in 1945 and suggested a central National Research Fund to coordinate all research, including external grants and fellowships (Bush, 1945).

Bush's vision, however, was politically untenable. While Congress debated issues such as patent rights and the accountability of researchers to elected officials, other agencies

stepped in to develop their own programs. By the time the National Science Foundation (NSF) was established in 1950, new, more specialized programs such as the Office of Naval Research and the Atomic Energy Commission had already laid claim to their research areas. The National Institutes of Health had also grown significantly, taking on medical grants previously administered through the OSRD and expanding the successful NCI grants-in-aid program for external research (Graham & Diamond, 1997). The NIH budget had begun to grow, from just $8 million in 1947 to more than $1 billion by 1966 (Harden). While the NSF would play a role in federal research funding, it never became the central, coordinating agency that Bush envisioned (Graham & Diamond, 1997).

Funding at the National Cancer Institute: An Overview

In 1938, Congress provided the new National Cancer Institute with $400,000 in funding, which, adjusted for inflation, would be equivalent to just over $6.5 million in 2012 (Bureau of Labor Statistics, 2013; National Cancer Institute, 2013a; Sahr, 2013). Figure 3 shows the annual NCI appropriations as reported in the NCI Annual Factbooks, adjusted for inflation to 2012 dollars and shown in billions. It also shows the percentage change in adjusted appropriations from the previous year.

Figure 3 tells a story, and the story of the early decades at NCI aligns well with the above discussion of the history of government investment in medical research. Small funding increases had a large percentage impact in the 1930s and early 1940s, when appropriations increased by 43% from $400,000 in 1938 to $570,000 in 1940 (unadjusted), or $6.5 million to $9.3 million (adjusted). The first major increases, however, were in the post-wartime years, with appropriations increasing by more than 600% in 1948. The next major increase, in the early 1970s, reflects the nation's commitment to the new "war on cancer." After America put a man on the moon 1969, Mary Lasker, who transformed the American Cancer Society and was a leader in the fight for research funding, wanted a "moon shot" for cancer. The ensuing

advocacy campaign included a full page ad in 1969 in the Washington Post that began, "Mr. Nixon: You can cure cancer" (Mukherjee, 2011, p. 180). In his 1971 State of the Union address, Nixon took up the campaign, saying, "The time has come when the same kind of concentrated effort that split the atom and took man to the moon should be turned toward conquering this dread disease. Let us make a total national commitment to achieve this goal" (p. 2). Congress held hearings on Nixon's proposals, including an additional $100 million for cancer research, between March and November, and the National Cancer Act of 1971 was passed in December (National Cancer Institute, 1972).

This new investment in cancer research was facilitated by an outpouring of public activism, including the largest letter campaign in congressional history. In April, Ann Landers devoted her entire advice column to a personal request to her readers. Her column read, "How

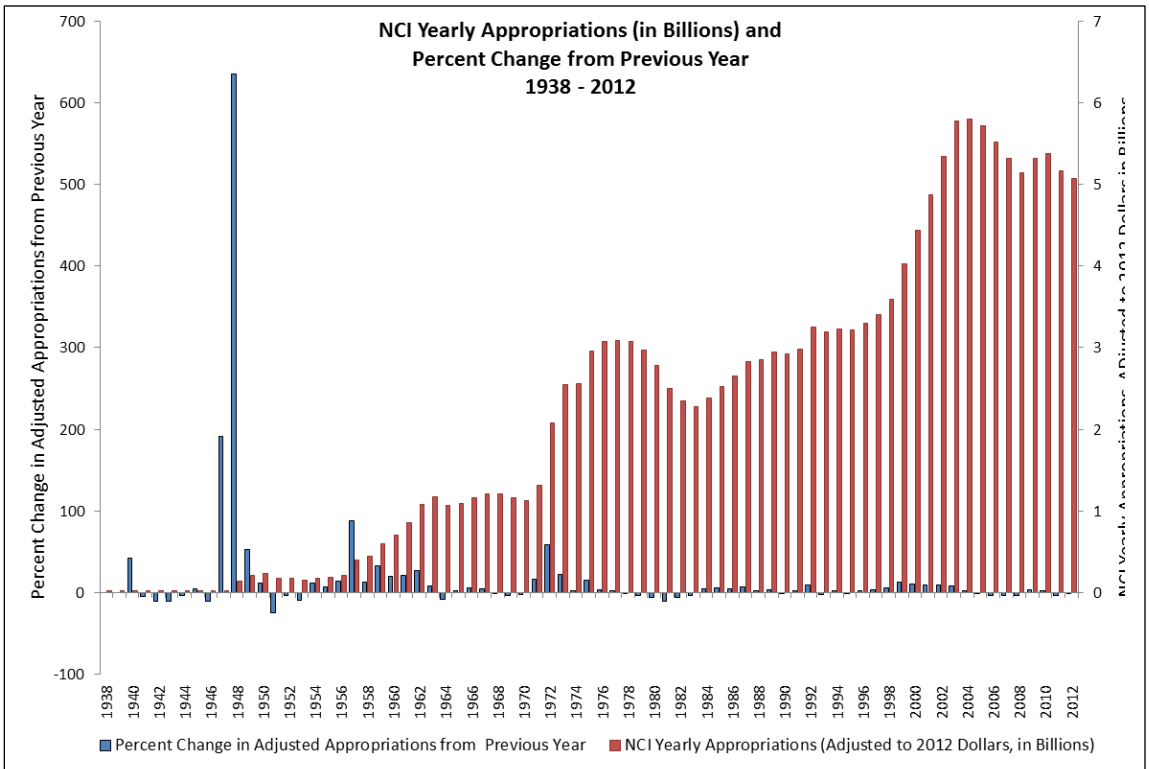

Figure 3. NCI Yearly appropriations (in billions) and percent change from previous year, 1938 - 2012. Appropriations data gathered from NCI Annual Factbooks for fiscal years 1971 – 2012 (National Cancer Institute, 2013a). Adjustment conversion factors from Sahr (2013), based on data from the Bureau of Labor Statistics (2013).

many of us have asked the question, 'If this great country of ours can put a man on the moon why can't we find a cure for cancer?' One reason is that we have never launched a national campaign, a united effort, against this killer disease. Another reason is money." She went on to compare government funding for the Vietnam War, the space program, and foreign aid, each receiving between $19 and $125 per person in 1969, to the funding allocated to cancer research, which was just 89 cents per person (Landers, April 20, 1971). Her request – that her readers write to their U.S. senators in support of the new cancer legislation – was heard across the nation, and Congress received more than one million letters in response (Burklow, 1991).

NCI did not get the promised increase of $100 million in 1972, but unadjusted appropriations did increase from $190 million to $230 million, or 64%, and appropriation increases continued to outpace inflation until 1978. Unadjusted appropriations first reached one billion dollars in 1980, but, as a result of deflation at the time, NCI's spending power was actually reduced by 6% from its 1979 level. Intense lobbying from cancer activists in the early 1990s saw a 13% increase in the 1992 unadjusted appropriations, which was a 9% increase in the adjusted levels. After that, however, appropriations barely kept pace with inflation until 1999. Appropriations topped out in the early 2000s, but have since declined, and adjusted funding levels have never again reached their peak of $5.8 billion in 2004 (National Cancer Institute, 2013a).

Between 1973 and 1985, NCI explicitly attempted to provide an overview of the "National Cancer Program" in its annual factbooks, including estimates of amounts contributed to the cause by other organizations and agencies. (The National Cancer Program shield can be seen in Figure 4.) Figure 5 and Figure 6 show the total resources (unadjusted for inflation) dedicated to the National Cancer Program, with the relative contributions delineated by five sources: NCI, other federal agencies (including NIH), state and local governments, voluntary and private institutions, and labor and industry. Figure 5 sums the amount contributed by

each source to the total national resources, which increased from $812 million in 1973 to $2.2 billion in 1985, while Figure 6 highlights the changes in proportions across different sources. Most interesting is the relative increase in the estimated contribution from labor and industry beginning in 1978, which helped bring the total program resources above two billion dollars in 1984. Unfortunately, this data was not reported after 1985, when the factbooks focused more specifically on the National Cancer Institute (National Cancer Institute, 2013a). Although it is more difficult now to estimate current industry contributions, NCI remains a leading funder of cancer research.

Figure 4. Logo used on annual factbooks after the development of the National Cancer Program as a result of the 1971 National Cancer Act. Image source: National Cancer Institute, 2013a.

The Fight for Cancer Funding

In 1996, NCI began publishing the yearly funding for various cancer types in the annual factbook, including data from preceding years for comparison. As a result, this data is available since 1990. Not all cancer types are included, and information for certain types is only available for later years; for instance, data on pancreatic cancer begins in 1998. As might be expected, the relative distribution of funds across different cancer types can be controversial. Harold Varmus, a Nobel prize-winning cancer researcher, director of NIH from 1993-1999, and current director of NCI discussed the problem in his 2009 memoir:

> "One of the most difficult aspects of the job of running the NIH, or of directing any individual institute, is the designation of research priorities. This is an emotionally and

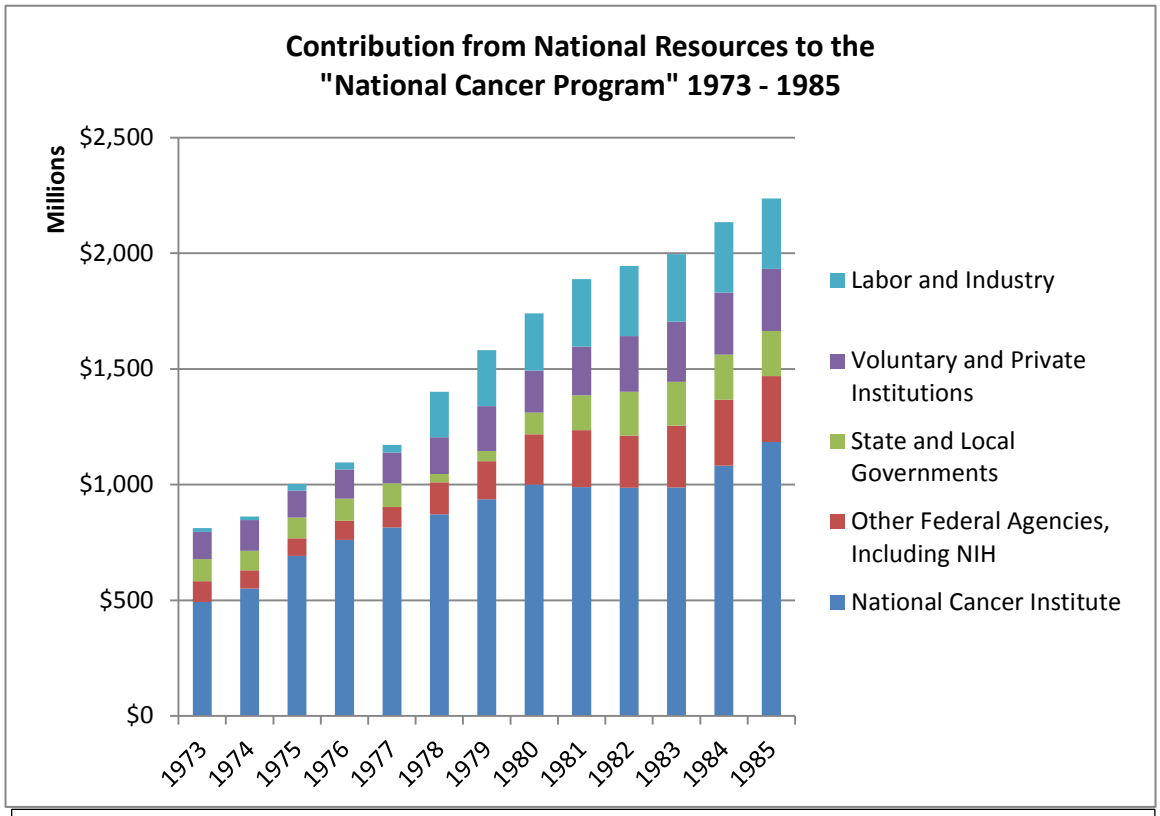

Figure 5. Contribution from national resources to the "National Cancer Program."

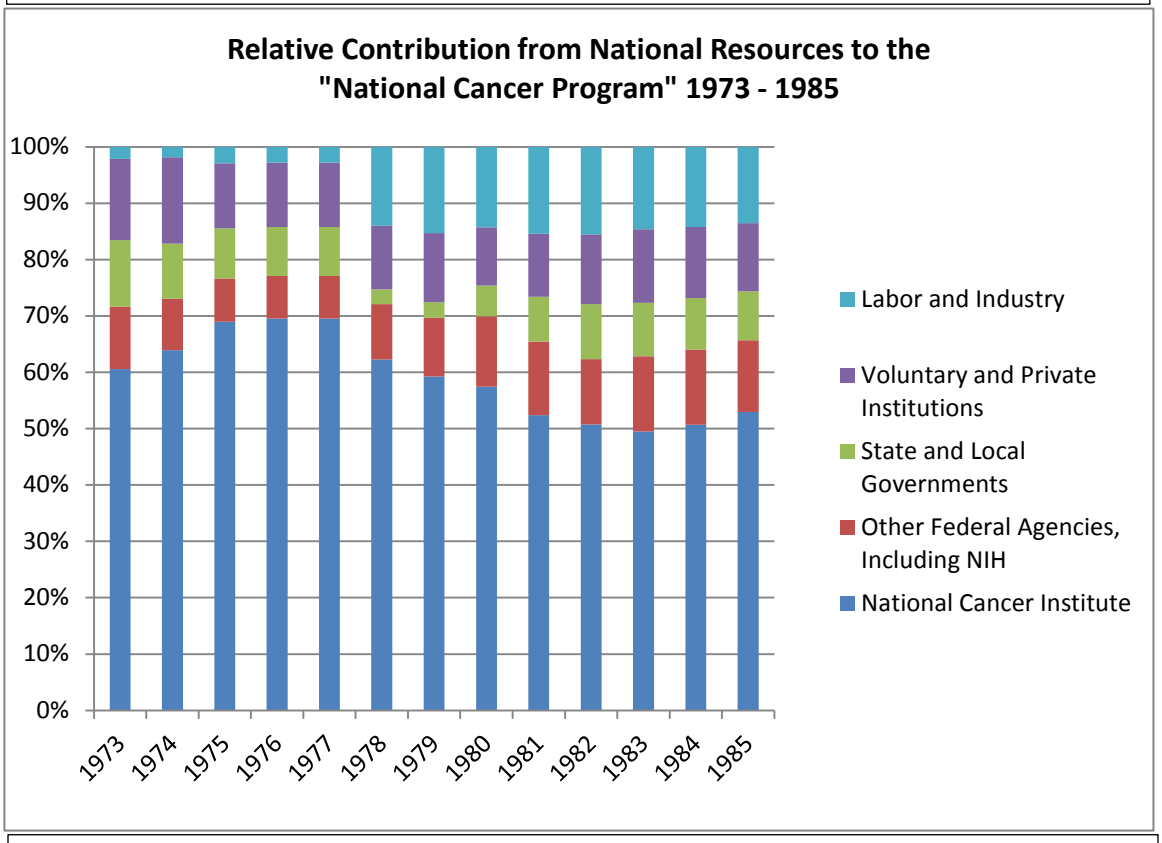

Figure 6. Relative contribution of national resources.

> politically sensitive part of the job because it is closely watched by some of NIH's strongest supporters, who often advocate for the NIH because of a passionate interest in a small fraction of what the NIH does. That fraction is almost always a specific disease or even a subset or facet of that disease" (Varmus, 2009, p. 162).

Even before this type of data was published in the factbook, it was used to inform advocacy positions. In February 1992, the Breast Cancer Coalition's research task force met to determine how much funding breast cancer research should be allocated in the next budget. In this meeting of advocates, physicians, and researchers, one advocate suggested that, based on the incidence and funding levels of AIDS and breast cancer, breast cancer should receive $4.3 trillion, so that equivalent amounts of federal funding would be spent for each new breast cancer case and new AIDS case.

Ultimately, the coalition decided to ask for a $300 million increase in breast cancer research funding levels. This would have more than tripled the previous year's funding levels of $133 million and add up to nearly twice the $220 requested by NCI to cover all reasonable grant applications in their bypass budget ("Breast Cancer Coalition To Congress: 'Find A Way To Fund The War;' Bypass Level Is Not Enough," 1992). The bypass budget was authorized in the 1971 National Cancer Act and allows the NCI director to present the agency's budget requests directly to the president, without changes from NIH or DHHS (National Cancer Institute, 2013b).

When BCC's Visco went to Congress to lobby for the $300 million, she and representatives of other groups were expected to thank members for past support, acknowledge the difficult fiscal situation, and ask for a bit more in the future. Visco had planned to do the same, but on the train down to Washington from Philadelphia, she changed her testimony (Gillon, 2004). She went on to say:

> "When this administration decided to wage a war, you found $7.5 billion to fund it. Women have declared war on breast cancer and you had better find a way to fund that war. Women refuse to fight with other diseases for which no funds are available. That would be going by existing rules, and too many women die under those rules. … We will no longer be passive. We will no longer be polite. We can no longer afford to wait while Congress gets around to significant, decent funding for breast cancer. We implore you: you must find a way to appropriate the additional $300 million for breast cancer research now." ("Breast Cancer Coalition To Congress: 'Find A Way To Fund The War;' Bypass Level Is Not Enough," 1992).

Congress knew that Visco had the support of hundreds of thousands of women; BCC had coordinated a letter writing campaign in 1991 that resulted in over 600,000 letters delivered to the White House. BCC's work resulted in re-appropriations within the NCI budget, increasing the breast cancer budget by $64 million to $197 million. The most significant change, however, was directed outside of NCI (Marshall, 1993).

The U.S. Army's Breast Cancer Research Program (BCRP) began with $25 million in funding allocated by Congress toward breast cancer research in Fiscal Year 1992. This was expected to be a one-time expenditure, but the program was strongly supported by breast cancer advocates, and particularly the Breast Cancer Coalition (Committee to Review the Department of Defense's Breast Cancer Research Program, 1997). For Fiscal Year 1993, Congress appropriated $210 million from general defense funds to this Department of Defense program, more than matching what was appropriated within NCI and nearly achieving the $300 million increase (Marshall, 1993). Allocating the funding through the DOD was politically important, because the domestic budget was hitting its spending limit (Watson, 1992a). What began as the Breast Cancer Research Program has now expanded to form the Congressionally Directed Medical Research Programs (CDMRP), allowing congress to allocate funding to a

number of areas of interest, as can be seen in Figure 7. However, breast cancer has been the largest and most consistent recipient of the $7.5 billion in total funds allocated over various disease types during the past two decades (Department of Defense, 2014).

Comparing funding across diseases has continued to be used to argue for an unmet need, and prostate cancer advocates were the next to build on the work of breast cancer advocates. In a 1997 advocacy effort, the National Prostate Cancer Coalition (NPCC) compared the ratio of research funding to disease deaths per year for AIDS, breast cancer, and prostate cancer. AIDS killed 51,000 per year and was allocated $1.62 billion in federal research funding. Breast cancer killed 44,000 per year and was allocated $550 million. Prostate cancer, in contrast, killed 42,000 per year and was allocated $80 million.

In a newspaper article at the time, NPCC president Bob Samuels argued, "Certainly our lives are just as important" (Burling, 1997). In the same article, NBCC president Fran Visco cautioned against making these comparisons, saying, "Once you start pointing the finger at other diseases, you get involved in disease wars." Visco also worried that the new prostate

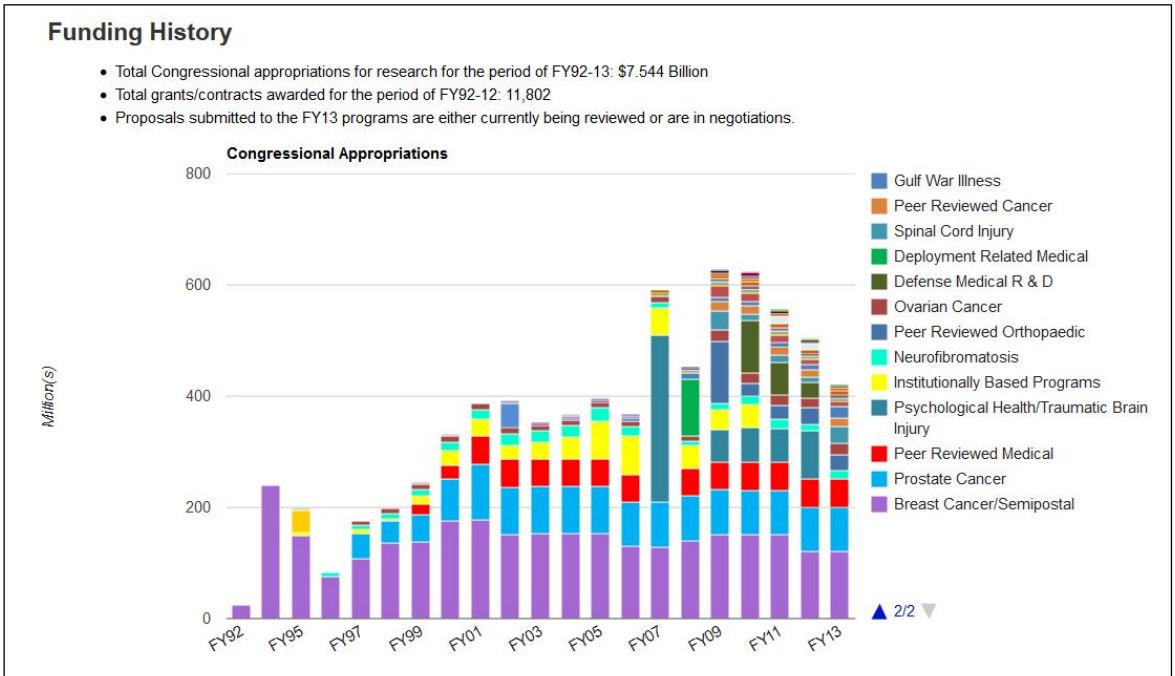

Figure 7. Congressionally Directed Medical Research Programs funding, 1992-2013. Image source: Department of Defense, 2014.

cancer advocacy movement was borrowing too heavily from the tactics breast cancer advocates relied upon. "We're flattered that they have decided to pretty much echo us and replicate our strategies. But I also recognize that, to some extent, it could adversely affect our agenda."

NCI funding allocations from 1990 – 2012 are shown in Figure 8 for the cancers receiving greater than $100 million in 2012, with funding for all years adjusted for inflation to 2012 dollars. The cancer types are organized by funding level, with breast cancer – which has the most funding, at $603 million – at the bottom, and pancreatic cancer – which has the least funding among the cancers on this list, at $105 million – at the top. Some observations can be made: funding for both breast and prostate cancer increased dramatically in the early 1990s, and prostate cancer funding increased again in the early 2000s. Funding for AIDS has been fairly constant in this period, and AIDS ranks fourth in NCI research funding after breast cancer, lung cancer, and prostate cancer. It is impossible to determine with certainty how advocacy has affected these appropriations. In his memoir, Varmus went on to say more about

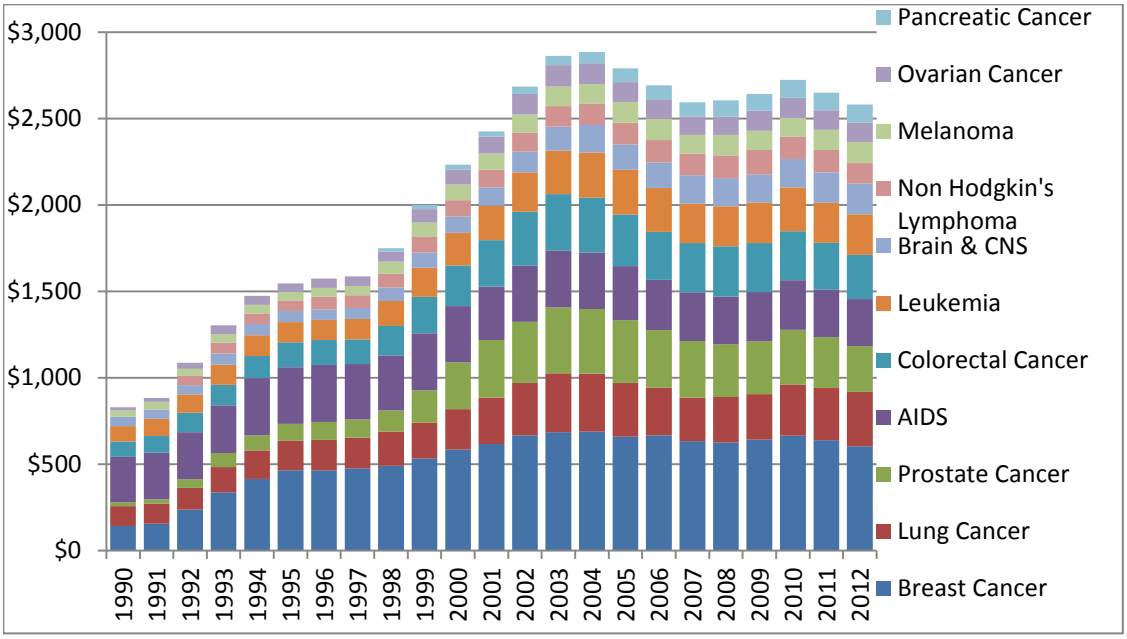

Figure 8. NCI funding by cancer type, adjusted for inflation, in thousands of dollars.

advocacy:

> "These comments are not meant to imply that advocacy for research on specific diseases is necessarily wrong, or that NIH leaders can simply divide up the funds according to the quality of grant applications, regardless of the research objectives. NIH must be (and it is) attentive to subject matter, and it must ensure (and it does) that at least some work is going on in all important areas. … In this regard, the NIH must walk a narrow line: to respond responsibly to public health needs and yet to provide the freedom for investigators to exercise their imaginations as fully as possible" (Varmus, 2009, p. 167).

NCI and AIDS Funding

NCI was a major player in AIDS research from the earliest recognition of the AIDS epidemic, when it was manifesting as Kaposi's sarcoma, a rare skin cancer. NCI researchers were responsible for key advances such as the development and testing of AZT as an antiretroviral therapy (National Cancer Institute, 2013a), and NCI maintained funding for AIDS research even after HIV was identified as an infectious disease (Shilts, 1987). Nearly 9% of the NIH funding for AIDS research is currently administered through NCI, down from more than 40% in 1985. Appropriations for AIDS funding account for just over 5% of the NCI budget, down from more than 10% from 1994 to 1996. Because AIDS research is a significant item within the NCI budget, and because activism for increases in AIDS funding has generally been distinct from activism for increases in cancer funding, it is important to consider the degree to which changes in AIDS appropriations contributed to changes in total NCI appropriations.

Figure 9 shows the change in total NCI appropriations from the previous year levels, as well as the changes in AIDS appropriations within NCI from the previous year levels, for years from 1984 to 2012. (These numbers are not adjusted for inflation.) In some years, such as 1992 and 1998 to 2005, it is clear that the NCI budget increased significantly, but also that

changes in the AIDS budget within NCI were not a major contributor to this increase. However, in other years, including the late 1980s and 1994, changes in AIDS appropriations appear to make up a significant portion of the total increase in NCI appropriations.

These data are demonstrated more clearly in Figure 10, which shows the changes in AIDS appropriations within NCI as a percentage of the total change in NCI appropriations. In 1994, the NCI appropriations increased by $75 million from 1993 funding levels to $2.08 billion. AIDS appropriations within NCI increased by $40 million from 1993 funding levels to $213 million, and made up 10.2% of the NCI budget. The change in AIDS appropriations made up 53% of the increase in total NCI appropriations. Of course, this can also occur in the opposite direction: in 2006, both total NCI appropriations and NCI appropriations for AIDS decreased. Appropriations for AIDS made up 52% of this decrease.

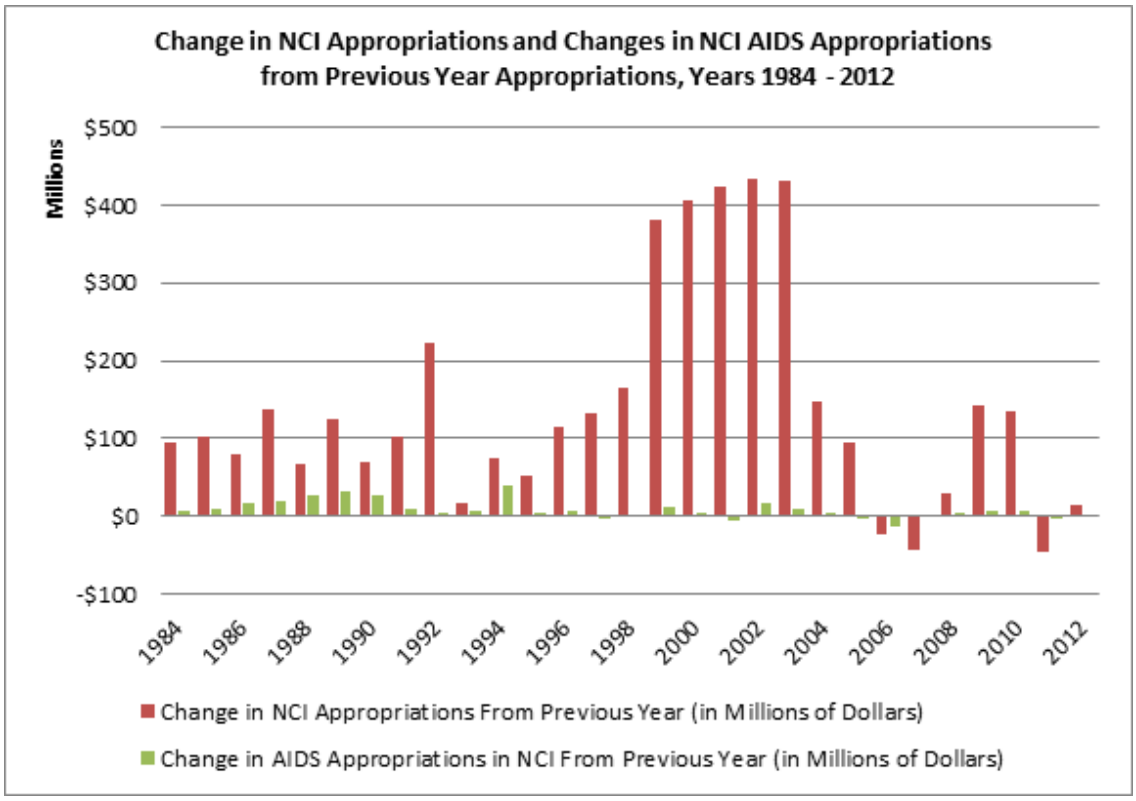

Figure 9. Changes in NCI appropriation and NCI AIDS appropriations in millions of dollars.

The Fight for a Role in Directing National Funding

In 1993, *Science* ran a special report on breast cancer research that included a short report on the politics surrounding research funding. In it, the NBCC is described as "the most visible lobby to stack the National Institutes of Health (NIH) since the AIDS activist group ACT UP began making a loud noise in the streets of New York" (p. 616). The funding was appreciated by scientists, but there was also a fear that advocates would adversely influence the scientific process (Marshall, 1993); Varmus in particular was outspoken about the need for basic science research, which may not have an immediately obvious application to any particular cancer type but can be fundamental to future advances (Stabinger, 1995).

Advocates, on the other hand, did not see a bit of disruption as a bad thing. Furthermore, as advocates were responsible for securing additional research funding, they

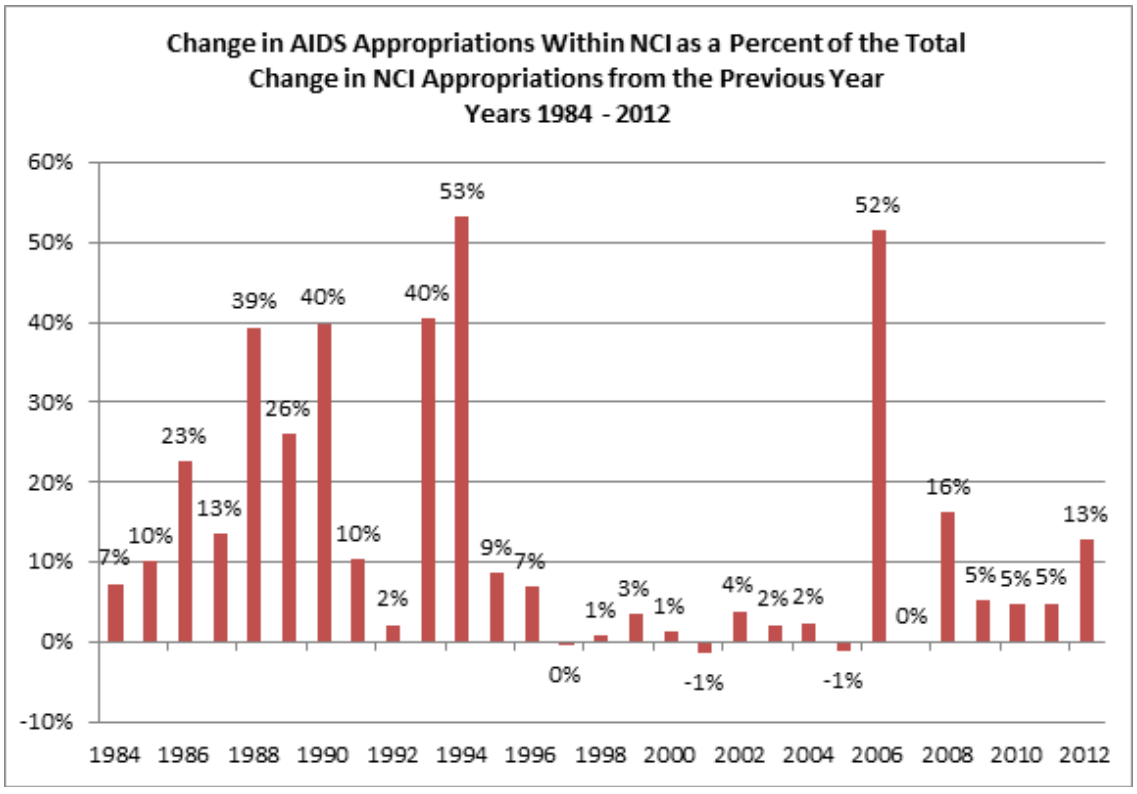

Figure 10. Change in NCI AIDS appropriations as a percent of the total change in NCI appropriations.

also sought increased access to research review panels and advisory boards (Erikson, 1995). One consumer comment particularly encapsulated the role that advocacy had played in making the DOD funding available: "Breast cancer survivors are responsible for the money being there for [breast cancer] research. It makes sense to have input into how the money is spent" (Andejeski, Breslau, et al., 2002, p. 132).

Advocates wanted a chance not only to lobby for increased funding, but also to bring their perspectives on research priorities, often with the hopes of reducing the likelihood that their friends, daughters, and granddaughters would one day face a breast cancer diagnosis. "We have to stop business as usual. We have to change the direction and really put our emphasis on basic science and prevention, and not such a large emphasis on treatment," NBCC member and surgeon Susan Love commented (Marshall, 1993, p. 616). NCI's attitudes and programs were seen as too restrictive: "a huge battleship that you just can't turn on a dime" (Stabinger, 1995).

Before receiving the breast cancer research funding, the DOD did not award external research grants and had no reason to develop a peer-review process to determine research awards. While there were early indications that the Army might work closely with NCI's established peer-review program to allocate the funding, they instead decided to build their own program from scratch (Stabinger, 1995; Watson, 1992b). The Institute of Medicine (IOM) provided initial recommendations for the original $25 million program and then reviewed the continuing program in 1997. The 1997 IOM review notes that, in line with its 1993 recommendations, "The unique aspects of the Army program include the involvement of consumer advocates at both levels of review – scientific merit review and programmatic review leading to funding recommendations – and the ability to quickly change directions and goals ("turn on a dime") on a year-by-year basis" (Committee to Review the Department of Defense's Breast Cancer Research Program, 1997, p. vi).

This was one of the first explicit inclusions of "consumer advocates" as a part of the peer-review process to determine where research funding should go, and breast cancer activists both created this opportunity and took full advantage of it. Since 1992, the CDMRP has used its $2.4 billion in congressional appropriations for breast cancer research to fund 6,314 breast cancer research awards. These awards have been selected from a total of 47,659 applications, and consumer reviewers have played an active role in determining what awards should be funded since 1993 (Department of Defense, 2014). As one researcher who served on a peer-review panel with consumers noted, "they are the reason money is available for research" (Andejeski, Breslau, et al., 2002, p. 126).

Same Fight, Different Scale: Local Breast Cancer Advocacy

Through similar efforts but on a smaller scale than the NBCC's successful lobbying for research funds that were administered through the DOD, San Francisco-area advocacy groups NORCAL and Breast Cancer Action helped advocate for the establishment of a California Breast Cancer Research Fund. From its start in 1993, this fund included advocates on peer review committees. While scientists and researchers framed the same objections to patient involvement as have been made at a national level, activists responded by pointing to trials where public investigation exposed significant problems, as well as all that patients can offer. One NORCAL newsletter noted, "Translation research can happen much more quickly, clinical trials can be more relevant and filled with willing volunteers, and the projects that are funded can be moving more in the direction of issues critical to breast cancer advocates: true early detection, a cure or cures that are real, and ultimately, prevention of disease" (Anglin, 1997, p. 1410).

BCA also worked to play a role in a new Specialized Programs of Research Excellence (SPORE) grant at UCSF, first awarded in 1993, and one of the first mechanisms through which NCI sought patient involvement. Breast Cancer Action board member Deborah Collyar noted,

"The outcome is up to the scientific community. We can be adversaries or partners in accelerating research. We would prefer to be partners," and, "We are serious about getting into the research process either voluntarily or through legislative actions if necessary" (Collyar, 1993). Collyar went on to become the team leader of the SPORE Advocacy Core at UCSF (Fintor, 1994).

Issues of Background and Legitimacy

Cancer advocates have faced many issues familiar to AIDS treatment activists, and questions of legitimacy are no exception. Before they were able to accomplish what they hoped to, AIDS activists had to work to gain credibility by mastering an understanding of the research issues and clinical trials processes. However, at the same time, some activists feared becoming too like the researchers they sought to upset; one noted, "I've seen a lot of treatment activists get seduced by the power, get seduced by the knowledge, and end up making very conservative arguments." This led Epstein to question, "Is the notion of a 'lay expert' a contradiction in terms?" (Epstein, 2004, p. 342).

Parthasarathy (2010) suggests that activist strategies are necessary to break through the "expertise barrier" that prevents those without specialized knowledge or training from fully participating in science and technology policy. Breast cancer advocacy is one of two examples described across several categories of actions available to activists. In one category, deploying established expertise, he describes how NBCC's formal advocate training program, Project LEAD, gave advocates the tools to bring both their personal experience and a strong scientific understanding to their research work. Furthermore, groups such as NBCC included experts such as Dr. Susan Love, who could work within the established system. In another category, introducing new policy-making logics, Parthasarathy discusses how breast cancer advocacy has worked to change the federal research model to focus on scientific areas that were more likely to improve health; this has been less successful. However, actions attacking

bureaucratic roles have had major effects, such as the inclusion of advocates on scientific peer review panels (Parthasarathy, 2010).

Governmental entities that involve patient perspectives have used various methods to identify "legitimate" advocates. In order to become a CARRA advocate at NCI, participants must first be nominated and then submit an application. Participants are required to be a survivor or caretaker and participate in a training workshop before beginning to review. In a recent study of the program, qualitative interviews were conducted with 19 CARRA advocates between 2010 and 2011. Several themes were identified, particularly around the importance of training advocates and providing support for their active engagement in the review process with expert researchers. Confidence and belief in the importance of representing the advocate perspective are especially important; as one advocate noted, "Our [societal] training has always been 'the scientist knows best.' Well, the scientist may know best about 99 percent [of the proposal], but our 1 percent, we really know best." However, the article also stressed common themes of the importance of enabling advocates to fit in with panels of researchers, and present their perspectives in a "fashion that's acceptable to the panel" (Gilkey, 2014, p. 117).

Eligibility requirements for consumer reviewers in the DOD CDMRP differ by disease area; for breast, lung, ovarian, and prostate cancer, potential consumer reviewers must be a "survivor of or person living with" that specific cancer. Additional eligibility requirements include active advocacy participation and ability to provide a broad patient perspective, rather than solely representing your own experiences. These can be seen in Figure 11, taken from the CDMRP website (Department of Defense, 2014).

In descriptions of the development of PCORI, key challenges are also described, including what the leaders refer to as a "lack of common language and training on the part of patients and resistance on the part of researchers to questions that are not research

generated" (p. 393). The article goes on to state, "To participate effectively, patients and stakeholders will need to acquire a certain level of training to have productive conversations with research partners and to 'translate' their health and health care questions into meaningful research" (p. 397). While acknowledging that researchers, too, will need to play a role, this clearly places the onus on patients and stakeholders to adapt to the research environment, rather than facilitating a research environment more accommodating of patient input (Fleurence et al., 2013).

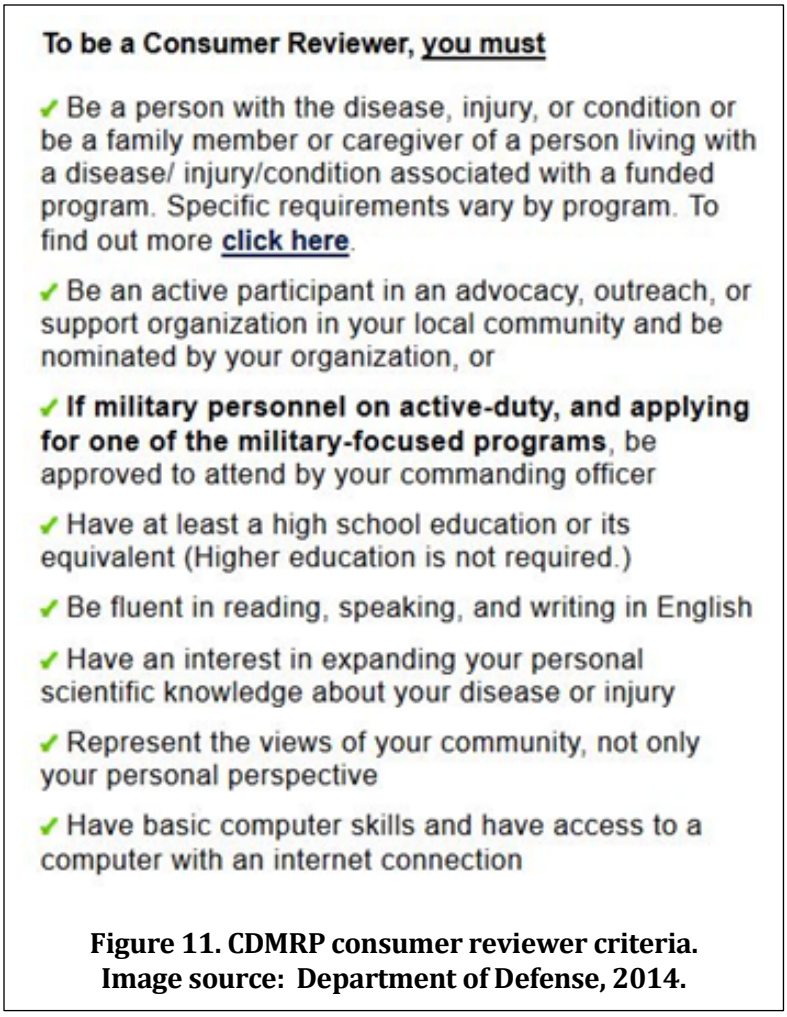

Figure 11. CDMRP consumer reviewer criteria. Image source: Department of Defense, 2014.

Issues of advocate legitimacy and training have also been explored in the UK. One ethnographic study of patient involvement in the National Cancer Research Network in the UK conducted interviews, observation, and document analysis. Particularly of interest were interviews with PPI participants on their views of what they contributed to the research process. The authors emphasize the "experiential expertise" discussed by interviewees, who repeatedly mentioned their experience as a patient or carer as a reference for their discussion of future research, allowing them to bring a different perspective, although that perspective

was not detailed by these interviewees. Another key topic area was the perception of interviewees that their involvement was less legitimate than professional researchers, and that they would avoid overly challenging professional practices. Prior knowledge, skills, and certifications gave them an added sense of credibility, suggesting their roles were more accurately "scientifically engaged lay experts." However, participants also noted that too much knowledge might render them less able to represent alternative perspectives. Training was essential to their ability to communicate with professionals, and participants argued for normative frameworks by which advocates ought to behave (Thompson, Bissell, Cooper, Armitage, & Barber, 2012).

Interviews with UK researchers regarding consumer involvement in health research found that researchers acknowledged the potential importance of consumer involvement, but that this did not necessarily translate to their encouraging consumer involvement, which the authors refer to as the "know-do gap." The authors believe that one reason for this gap was epistemiological dissonance, with researchers fearing that lay knowledge was not appropriate in the research process, and believing lay consumer experiential knowledge was not equivalent to their academic qualifications. While researchers seemed to seek out more "professionalized" consumers who were perceived to be more able to contribute to the research process, they also expressed doubts that consumer representatives were actually representative of consumers as a whole, and thus that their opinions may not be valid (Ward et al., 2010).

Another qualitative study evaluated lay participants in Local Research Ethics Committees, which serve the role of the IRB in the UK. Through 45 interviews with committee members, observations of 20 meetings, and a survey of all 218 LRECs, the author examined the experiences and contributions of lay members to the committees. Interestingly, she found that many of the lay members had previous experience as health professionals, suggesting that they

were not truly "lay" people, and in interviews, participants suggested that there may not be a "true" lay person, since any member would have an interest in research that separated them from most of the population. Similarly, she found that lay members exerted influence by becoming "expert LREC members" and, as such, reasserted the same research paradigms shared by the professional members (Dyer, 2004).

Issues identified in a systematic review of patient involvement in the UK included tensions between stakeholder groups such as researchers and public, public understanding of research concepts such as a randomized controlled trial and need for a control, time and cost of PPI, and jargon used. One important issue that was identified as being raised in two articles was that funding should be allocated for public involvement, both to pay public advocates for the value of their time – thus acknowledging their value – and to reimburse for their out of pocket expenses (Boote et al., 2010).

Considering the Value of Patient Advocacy

Philosophical Arguments

Arguments for public involement in health research have been based on moralistic, epistemological, and consequentialist grounds, because of the moral need to include those for whom research is being done, the benefit of incorporating experiential perspectives, and the resulting improvements in quality, respectively (Boote et al., 2010). The moral perspective is the most philosophical, and least evidence-based, of these arguments. It can be traced to the 1978 Declaration of Alma Ata, which stated in Item IV that "[t]he people have the right and duty to participate individually and collectively in the planning and implementation of their health care" (World Health Organization, 1978).

More recently, in a short 1995 commentary, the director of the NIH Office of Medical Applications Research (OMAR) noted that "it also seems clear that if the NIH Consensus Conference panels are making recommendations on the health care of patients with various

illnesses that these patients or patient group should have their appropriate input to the proceedings" (p. 4). OMAR oversaw consensus conferences on medical and scientific issues since 1977, and included breast cancer patient advocacy groups throughout the conference process beginning with an October 1994 conference on Breast Cancer Follow-up (Ferguson, 1995); the Consensus Development Program was officially retired by NIH in 2013 (National Institutes of Health, 2013).

In the 1990s, one of the founders of the Cochrane Collaboration attempted to take the perspective of a patient in inquiring what health research should aim to provide: reliable evidence, based on properly controlled studies; evidence that is relevant to patient goals rather than surrogate endpoints with little practical meaning; the opportunity to participate in research when there is uncertainty about which option is preferable; research that is applied and evaluates how interventions will actually work in practice; and recognition that basic research provides an incomplete perspective. While this was a laudable exercise, the author acknowledged that he is still constrained by his perspective as a researcher, and that as a result, perhaps the most important patient requirement of health research is lay involvement to provide alternative perspectives on what is important and what may be of concern. This is reinforced by a quotation included from a UK consumer advocate, who noted that "[r]esearchers cannot assume that their own values and priorities apply to others who do not share their world" (Chalmers, 1995, p. 1318).

Recent scientific advances may make public involvement even more of a moral imperative. For instance, The Cancer Genome Atlas has the potential to significantly change the shape of cancer treatment. However, not all cancers will be immediately included, and the decisions around which cancers to solicit will play a large role in what types of cancers accrue benefits from genomics research. As a result, these decisions must be made on both scientific and social grounds (Foster, Mulvihill, & Sharp, 2006).

Face Validity

When the Institute of Medicine compiled recommendations for improving the outcomes of cancer survivors by focusing on the transition of care following active treatment, the effectiveness of survivorship care plans had not been formally evaluated. Their seminal 2006 report, "From Cancer Patient to Cancer Survivor: Lost in Transition," noted that this lack of evidence was not sufficient to not include care plans in their recommendations:

> despite the lack of evidence to support the use of survivorship care plans, the committee concluded that some elements of care simply make sense – that is, they have strong face validity and can reasonably be assumed to improve care unless and until evidence accumulates to the contrary. Having an agreed-upon care plan that outlines goals of care falls into this "common sense" area (Institute of Medicine, 2005).

Philosophical arguments in support of patient advocacy are often bolstered by similar "common sense" conclusions. One perspective is that community involvement can encourage ethical research, contributing to the protection of research subjects by identifying risks that may not be apparent to researchers, particularly non-physical risks that may lie in the social realm (Dickert & Sugarman, 2005).

Other arguments with face validity have more consequentialist approaches, noting that patient advocates can play an important role that facilitates research by setting a relevant research agenda, advising on outcomes that matter to patients, improving the design of studies so that participation is more acceptable to potential research subjects, and assessing the information and consent being provided (Goodare & Smith, 1995). In an article on patient advocates and NCI cooperative groups, which coordinate large clinical trials, advocate Deborah Collyar gave several examples of successful relationships. She describes this as "an essential partnership" between the advocates and the cooperative groups, where advocates bring

attention to areas that may not produce the most profitable innovation but could offer substantial benefit to patients (Collyar, 2008).

Among the successes Collyar describes is the story of Michael Katz, a multiple myeloma patient who brought his patient perspective, honed through his volunteer work with other patients through the International Myeloma Foundation, to the Eastern Cooperative Oncology Group (ECOG). Katz has described being prescribed dexamethasone (dex), a powerful steroid used in conjunction with Lenalidomide, and having to take a lower dose due to the overwhelming side effects. He, and many other patients, did well at the lower dose, and Katz recommended comparing the standard-dose of dex to a lower dose. When this was integrated into ECOG trial E4A03, the trial had to be closed early because the low-dose patients so clearly had a survival advantage (Katz, 2012). When the trial was published in *Lancet Oncology* and discussed in NCI press releases, its origins in patient advocacy were not discussed; however, Katz was listed as one of the paper authors (Rajkumar et al., 2010).

In her early work with Breast Cancer Action in the 1990s, Collyar described why she felt advocacy involvement was so important, noting that advocates raised the profile of quality-of-life issues in research and "ask questions that scientists have never thought of, let alone considered, and it is making a difference in the way they think" (Fintor, 1994, p. 660). One interesting comment from interviews with 15 UK health researchers was that public involvement may be less important for clinician researchers, who have regular contact with patients, than for scientists with less regular interaction with the population for whom their research is intended to benefit, suggesting that laboratory-based basic scientists may particularly benefit from an advocate's alternative perspective, while clinicians are more familiar with patient concerns (Thompson et al., 2009). However, research has shown that researchers may simultaneously be health care consumers, but this does not mean that they prioritize issues in the same way that non-professionals do (Boote et al., 2002).

The researchers Collyar and other advocates were working with in the 1990s agreed that the partnership was beneficial, noting, "The main thing medical professionals were worried about was whether the advocates were there to be confrontational or to be partners … but in our case, the process has been a two-way street. The activists have been an important component of the success of this process" (Fintor, 1994, p. 659).

Although the researchers in this case came to appreciate the advocate participation, their initial concerns echo seemingly "common sense" arguments regularly put forth by scientists opposed to integrating public and patient involvement and advocacy in research without evidence of its benefit (Thompson et al., 2009). One review grouped these various arguments into seven key areas: "representativeness, quality, bias, influence, consumers' expectations, increased cost and length of research, and overlapping roles" (Boote et al., 2002, p. 226). These include issues of whether a single advocate can represent all of a group's interests (Florin & Dixon, 2004), whether lay people can understand and contribute impartially to high quality research, and whether lay perspectives may unduly influence the direction of research (Boote et al., 2002).

Advocate influence was certainly seen in the allocation of research funds in 1992, and researchers such as Varmus were outspoken in their fears that these earmarks would favor research for a particular disease or more applied research (Stabinger, 1995). A related potential issue is that, as funding is increasingly divided, disease areas with less active advocates could pay the price: "if advocates for AIDS, breast cancer, and Alzheimer's disease succeed in garnering larger slices of the resource pie for their groups and the overall health budget remains static, smaller and smaller slices will be left for other groups that support equally deserving causes but that lack activists to argue on their behalf" (McCabe et al., 1995, p. 141).

Regardless of the validity of these arguments, they certainly contribute to tension between researchers and advocates (Boote et al., 2010). While the Institute of Medicine supported survivorship care plans on the basis of their face validity, they also recommended that "research should be undertaken to assess the impact and costs associated with survivorship care plans, and to evaluate their acceptance by both cancer survivors and health care providers" (Institute of Medicine, 2005, p. 5). Similarly, additional evidence should be brought to bear on discussions of the proper roles and investments in advocates in the research system.

Research Evaluating Patient Advocacy in the United States

Unfortunately, there has been almost no evidence of the effects of patient advocacy in research in the United States: just two published evaluations with quantitative data were found in the literature. The first of these was in the context of the peer-review process developed for the DOD's Breast Cancer Research Program (BCRP). The BCRP was thought to be the first program to involve "consumer reviewers" for all scientific topics, including basic research, rather than just on areas more directly applicable to consumers, and was the first to evaluate consumer impact, including how lay involvement was perceive. This evaluation consisted of a cross-sectional survey study of the funding process for the Fiscal Year 1995 awards. In this review cycle, 42 onsite panels included 85 consumers and 638 scientists. Scientists were notified that two consumers would be participating in most panels. Participants were surveyed before and after the review process to compare opinions on consumer involvement. After the review process, scores assigned by consumers and scientists were also compared (Andejeski, Bisceglio, et al., 2002).

Quantitative analysis found that, generally, consumers gave slightly more favorable scores to proposals than the scientists. 76.2% of proposals received the same score as they would have if only scientist scores were considered. 15.2% received higher scores, and 8.6%

received lower scores. In the opinion surveys, consumers had a more positive view of consumer participation than scientists in all pre- and post-panel measures. However, significantly more scientists had a positive view of consumer participation in the post-panel than in the pre-panel on all measures, and in the post-panel, at least 70% of scientists reported a positive view of consumer involvement on all measures. In response to the question, "Was it beneficial to have consumers on peer review panels?" 69.2% gave a positive response in the pre-panel survey, and 83.9% gave a positive response in the post-panel survey ($p \leq 0.001$) (Andejeski, Bisceglio, et al., 2002).

A qualitative analysis of the same survey results reported more specific pre- and post-panel observations and opinions offered by both scientists and consumers. Scientists reported concerns about consumer abilities to understand and contribute to the scientific discussion, and the relevance of consumer input in a decision that "should be 99% scientific merit" (p. 132). However, many comments were more positive. One researcher comment in the post-panel survey particularly encapsulated many of the "common sense" arguments made above: "Consumers, in this case patients, were able to put things about some of the studies in practical perspective for the scientists" (Andejeski, Breslau, et al., 2002, p. 126). This mixed-methods review of the DOD funding process has been the most comprehensive evaluation of patient advocacy in the United States thus far, and one of the only evaluations of patient advocacy in cancer. Both the quantitative and qualitative results suggest that advocates had a positive effect, but they are by no means conclusive.

A second quantitative evaluation of patient involvement in research in the United States described the input of Gulf War veterans in the design of consent documents for a randomized controlled trial of participants with Gulf War veterans' illnesses in the VA. The consumers were asked to provide suggestions for changes to the consent document but did not make major changes. Ultimately, there were few significant differences in acceptance rates or

personal characteristics seen between participants of the clinical trial who were offered the consumer-edited consent form and those who were offered the researcher-developed consent form. The researchers saw this as evidence that consumer involvement did not produce a measurable benefit or harm, but that studies of the impact of consumer involvement could feasibly be embedded in larger trials (Guarino, Elbourne, Carpenter, & Peduzzi, 2006).

Qualitative research in the United States has been similarly scarce. One group used focus groups to evaluate parental perspectives of exceptions from informed consent in pediatric resuscitation research in situations where obtaining consent may not be feasible (Morris, Nadkarni, Ward, & Nelson, 2004); another conducted 29 qualitative interviews with environmental breast cancer advocates and researchers, in conjunction with ethnographic observations, to analyze public involvement in breast cancer research and environmental issues (McCormick, Brody, Brown, & Polk, 2004). In the latter study, prejudices were found to be the greatest barrier to both advocates and researchers, with advocates lacking confidence in their abilities to deal with researchers, and researchers afraid that activists were "hysterical women." The researcher-advocate partnerships resulted in significant attitudinal changes on both sides, with advocates feeling empowered and scientists growing to appreciate the lay perspective (McCormick et al., 2004).

A process evaluation of the California Breast Cancer Research Program (CBCRP) Community Research Collaboration (CRC) program, which was designed to promote Community-Based Participatory Research (CBPR), found several areas in which lay collaborators or advocates had played an important role. Funded academic-community partnerships had enormous success in unexpected areas, such as retention, as one academic researcher noted: "We had a 99% follow up rate. That is unheard of in research. The community partners were apologizing that they lost one woman to follow-up. We were teasing them that it was a good thing that they lost one woman or no one would have believed the

study was for real" (p. 435). At the same time, community partners faced unexpected challenges, including harsh criticism from grant reviewers (Plumb, Price, & Kavanaugh-Lynch, 2004).

Other similar work focuses less on perspectives from a community of patients or consumers and more on standard CBPR (Pinto, 2009; Pinto, Specto, Rahman, & Gastolomendo, 2013; Swartz et al., 2004). Traditionally, CBPR tends to focus on geographic communities rather than communities constructed out of a shared illness experience. Because of the differences between the communities in question, this area of research is less likely to serve as evidence specifically for the benefit of patient advocate participation in medical research, and is not described here.

In 2012, a systematic review was prepared at the request of the PCORI for use in developing future recommendations for methodologies in patient-centered outcomes research. While the resulting report was lengthy and detailed, its findings were unfortunately questionable. Its literature search and expert consultation identified 5,560 possibly relevant articles, of which 194 were included in the final review. Of these, 11 were systematic reviews, 103 were qualitative research, and 7 were identified as randomized controlled trials (RCTs) and described in further detail in a table, "Findings of randomized controlled trials." However, none of these seven reported articles included an RCT evaluating patient involvement. Instead, they each used qualitative, and occasionally quantitative, feedback from community or patient representatives when designing RCTs (Atkinson et al., 2011; Daugherty et al., 1995; Edwards, Wyatt, Logan, & Britten, 2011; Koops & Lindley, 2002; Marsden & Bradburn, 2004; Shagi et al., 2008; Swartz et al., 2004). No data captured during the RCTs was intended to evaluate the impact of patient or community participation. While this research is certainly valuable, it is unclear why this review separates it from other qualitative research (Garces et al., 2012).

One of the few existing studies that incorporated a randomized evaluation of patient involvement, Guarino et al.'s (2006) study of patient revisions to an informed consent document, was not included in the review as an RCT or in any other section. Additionally, the majority of studies included in the review – including four of the seven "RCTs" – were conducted outside of the United States, raising concerns about the broad applicability of their findings, particularly since the country in which the research was conducted was not reported for the cited articles so that it could be considered in future work (Garces et al., 2012).

International Research

Unfortunately, the PCORI report only highlights the relative lack of work on patient advocacy or involvement being done in the United States, a discrepancy that has been noted by other researchers (Tritter & Lutfey, 2009). Slightly more work has been done in other countries, and particularly in the United Kingdom. However, the cultural context for patient work in health research is very different, which is an important limitation when considering these studies.

United Kingdom Health System Context

The United Kingdom National Health Service (NHS) operated under a "consensus management" approach in the 1970s and early 1980s that sought "collective responsibility founded on close association and respect" (Snow, 2013, p. 75). This all changed in 1983 with the publication of the Griffiths Report, led by Sainsbury managing director Roy Griffiths, which advised a "general management" approach that emphasized managerial responsibility and authority. Perhaps the most memorable phrase of the report spoke directly to the perceived need for better management: "if Florence Nightingale were carrying her lamp through the corridors of NHS today she would almost certainly be searching for the people in charge" (Department of Health and Social Security, 1983).

However, the beginnings of patient involvement in the NHS can also be seen in the Griffiths Report: "Businessmen have a keen sense of how well they are looking after their customers. Whether the NHS is meeting the needs of the patient, and the community, and can prove that it is doing so, is open to question" (Department of Health and Social Security, 1983). Thus, in the UK, the focus on patient involvement evolved from an emphasis on accountability to the needs of the "customer," and recognition of the importance of using consumer feedback to improve services (Boote et al., 2002). Perhaps due to Griffiths' retail experience, this was sometimes called the "supermarket model" of consumer involvement in healthcare (Winkler, 1987).

The United Kingdom medical research and development program is an integrated part of NHS, a relatively recent reform arising from fears that NHS was the key group that needed to use the results of medical research, but that most medical research was not being conducted with NHS needs in mind. Many believed that the research focus needed to change so that research could be better put to use, especially in improving NHS outcomes and efficiencies (Black, 1997). As a result, the United Kingdom has since tended to invest more heavily in health services research and less in clinical research.

The importance of consumer feedback across NHS, and specifically in research, was emphasized in a 1999 report on "Patient and public involvement in the NHS." It read, "Research and development (R&D) in the NHS needs to focus on what is important for patients and users. To achieve this patients and service users need to be involved at all stages of the R&D process" (p. 20). These stages are explicitly described as prioritization, commissioning, undertaking, and disseminating research. This report also described the work of "Consumers in NHS Research," a committee organized in 1996 that was designed to help guide the process of involving consumers in research (Department of Health, 1999).

This consumers group was later named "INVOLVE – promoting public involvement in NHS, public health and social care research" and continues to promote effective public involvement in research (INVOLVE, 2003). They advise the National Institute for Health Research, manage the "People in Research" website to connect citizens with research opportunities, support research on the impact of public involvement, and conduct other projects as appropriate (INVOLVE, 2013), generally creating an active interface for those interested in patient or public contributions to research.

Clearly, there are several important differences between patient involvement in research in the United States and in the United Kingdom. In the United Kingdom, patient and public involvement was developed through top-down, consumer-focused directives, rather than through grassroots movements. As a result, as was discussed in the introduction, patient participants may have less power than if they had played a more active role from the beginning. Research in the United Kingdom is also conducted primarily through the NHS, and, as a result, is both more coordinated and more focused on health services research than in the United States. Thus, the type of research that patients are involved in differs as well. Finally, patient and public involvement has been promoted more uniformly, through an active central organization. This has encouraged more research into the impact of patient involvement, but has also created a very different system than is seen in the United States.

Research from the United Kingdom

Keeping these differences in mind, it is interesting to consider some of the evaluation research that has been done in the UK. The work has been primarily qualitative, and many studies utilize methods rarely seen in the United States, particularly the Delphi and nominal group techniques. Even in the UK, little research quantifying the impact has been done; instead, the majority of research is formative and could contribute to later evaluations.

In one such formative study, authors utilized a two-round Delphi study and follow-up interviews with key participants to assess what aspects of public involvement on research were perceived as feasible to evaluate. Participants included university researchers, members of the public, and policy makers. Participants reached consensus (defined as 80% agreement) that 5 of 16 areas would be possible to evaluate: identifying research topics, prioritizing research topics, disseminating research, assessing the effect of involvement on the public participants, and assessing the effect of public involvement on researchers. Issues that participants did not think could feasibly be evaluated included the effect on research design, collecting data, interpreting research findings, determining the usefulness of research findings, and implementing research findings (Barber, Beresford, Boote, Cooper, & Faulkner, 2011).

In this study, 75.2% of Round 1 Delphi participants agreed that public involvement was important because it "is of ethical and moral value in itself, regardless of its impact on research" (p. 232). In a qualitative analysis, one participant noted that "if it is not having an impact it is a pretty pointless waste of time. Involvement must be meaningful. There is no point in going through the motions because it is the right thing to do" (p. 236). Another said that, despite arguments that it was morally valuable, the potential impact of public involvement was the strongest argument for it. Assessment of the effect of public involvement on research quality was seen to be especially difficult, particularly because of issues defining quality (Barber et al., 2011).

Another more formative study consisted of a workshop utilizing nominal group technique and a two-stage Delphi study conducted to measure the degree of consensus among consumers, researchers, and consumer-researchers as to what is required to successfully involve consumers in NHS research. These Delphi rounds reached consensus on 8 principles that were deemed clear and valid, each of which had between one and three indicators. These principles and indicators include:

1) Agreement on roles of the consumers, as indicated by documentation of these roles.
2) Researchers budget for consumer involvement, and funding is available for indirect and travel costs of consumers.
3) Researchers respect the unique and differing skills and perspectives offered by the consumers, as documented in reports and papers.
4) Training and support is available to consumers, as indicated by agreement and access.
5) Researchers are capable of involving consumers successfully, and complete any training necessary to do so.
6) Consumers contribute to plans for recruitment and how to share information with participants.
7) Consumer involvement is described in publications, with details about the involvement.
8) Consumers provide input on how to disseminate research findings to consumers as a whole.

In the "future research" section, authors note that the transferability of these principles and indicators will need to be examined to determine their applicability to non-UK situations (Boote et al., 2006).

Much evaluation research consists of retrospective, reflective case studies, as are described by Carter, Beech, Coxon, Thomas, and Jinks (2013). The case studies discussed by these authors offer examples of how experiential knowledge brought by lay patients and members of the public can improve the research design and funding process. The authors argue that health research generally requires multiple knowledge perspectives: "methodological expertise, practice-based expertise, and the experiential expertise of patients or carers" (p. 307). More specifically, they argue that these cases show how applying patient

experiences and viewpoints can improve the chances of a study being successfully funded, result in more ethical research, and improve the relevance of the research.

In another reflective case study, the authors examined a situation in which researchers were eager to conduct a health services research trial building on research done by Guarino et al. (2006). The proposed trial was intended to be embedded in pre-existing clinical trials, and would involve members of the public designing information and consent forms for patients independent of the researchers. The outcomes would consider whether potential participants were more likely to participate when presented with the information designed by the public, or by the researchers, and whether potential participants felt that they had a good grasp on the information presented in each. However, when this research idea was shared with members of the public, there was not support. They feared that this would polarize members of the public and researchers, rather than demonstrate the importance of public contributions, and that, furthermore, this was not necessarily the best method through which the importance of public involvement could be demonstrated. This study was ultimately abandoned, and the authors argue that such "negative" outcomes are an important indicator of the value of patient involvement, just as much as "positive" outcomes, in that money and time that would otherwise be dedicated to a questionable study are saved (Boote et al., 2012).

Other research has focused on public or patient involvement in priority setting for health research and health policies (Entwistle, Calnan, & Dieppe, 2008). One study developed a prospective study plan based on analysis of reflective discussions throughout the priority-setting process, which included an expert workshop using nominal group and Delphi techniques. For the purpose of this study, reflective discussions were held after advisory board meetings with service users and researchers. Key themes that emerged were "trust and commitment, impact on the wider study, mutual learning, and timing of service user involvement" (Barber et al., 2012, p. 611).

The James Lind Alliance (JLA), an organization in the UK devoted to establishing research priorities in different research areas with the input of clinicians and patients, has supported two literature reviews focusing on the inclusion of patients and clinicians in priority-setting. Non-researcher clinicians and patients were found to be able to engage directly or indirectly with research, and either consult on decisions or collaboratively be involved in decision-making (R. Stewart & Oliver, 2008). The second review also describes an increasing use of formal priority-setting processes such as group interactions and utilization of the Delphi method to involve clinicians and patients. The authors suggest that non-researcher involvement is largely seen as an academic exercise, and thus does not have as wide an impact as it could were these priorities then implemented in research funding decisions (Stewart, Caird, Oliver, & Oliver, 2011).

Another review, including both published and grey literature, focused on the changes to the provision of health services in the UK as a result of incorporating patient input. The focus was not specifically on health research, but they did find that changes were recorded, particularly in case studies. However, there was insufficient evidence to determine whether the changes were positive or impacted quality of care, user involvement, or user health (Crawford et al., 2002).

Research from Other Countries

A broad systematic review of literature concluded that the US, the UK, Canada, and Australia, have all promoted patient involvement in health research. However, authors note that universally, minimal research has been done to measure the impact. Their extensive 2007 review found only three evaluations of public involvement in cancer research and calls for more rigorous assessment of the impact of public involvement. Advocacy, particularly in the research process, was seen as strongest in the United States (Hubbard et al., 2007).

Another review synthesized information on instances where patient and public involvement programs were used to develop and implement clinical practice guidelines. Most relevant articles published on the topic were published after 2002 and involved work in the US, UK, Australia, and Germany, and covered health problems ranging from cancer to mental health. Involvement typically included participation in a working group or other meeting, where participants were encouraged to bring in patient values and perspectives (Légaré et al., 2011).

Other countries in which some research has been reported include the Netherlands and Italy, as well as in international organizations such as the Outcome Measures in Rheumatology (OMERACT) conferences (de Wit, Abma, Koelewijn-van Loon, Collins, & Kirwan, 2013; Mosconi, Colombo, Satolli, & Liberati, 2007; Nahuis & Boon, 2011; Van de Bovenkamp, Trappenburg, & Grit, 2010). For instance, the assessment of the impact of patient involvement in the OMERACT conferences considered a period of over ten years and relied on document review and 32 interviews. The authors found five key categories in which patients had contributed: "widening the research agenda; including patient relevant outcomes in core sets; enhancing patient reported instruments; changing the culture of OMERACT and consequences outside OMERACT" (p.1). Importantly, these authors argue that patient involvement and contribution to research may often go unnoticed because its greatest value lies in the experiential perspective, which is often expressed in non-scientific comments of agreement or disagreement. These comments often validate a research idea, or suggest new directions, but because they are not systematic or rigorous their value may not be properly attributed (de Wit et al., 2013).

CHAPTER THREE: METHODS

This chapter discusses the participants, sampling method, and interview instrument used in this cross-sectional, qualitative interview study. It will also present the data collection, management, and analysis procedures.

Participants

To be eligible for the study, individuals were required to be English speakers, aged 18 or over, who self-identified as patient advocates for cancer research. Individuals who did not identify as patient advocates for cancer research, were under the age of 18, or were non-English speakers were excluded. Recruitment ceased after 13 participants, when the primary investigator determined that data saturation had been achieved.

Sampling

Potential participants were identified and contacted in November and December 2013 through purposive sampling of an online community for patient advocates in research and then snowball sampling from the original sample. This method made it possible to find a relatively homogenous group, reducing the variation in the fairly small sample and simplifying analysis, and allowed individuals to self-identify as eligible for the study, which was important given the lack of a clear definition of a patient advocate in research. The primary investigator distributed invitation emails through this online community in November 2013 and January 2014. The email included the rationale for the study, time frame, eligibility requirements, contact information where people should send questions and get additional details, and a request that people receiving the email send it on to others who might be eligible. Individuals who contacted the primary investigator after receiving this email were asked if they would like to participate in the study, and, if so, the primary investigator arranged a telephone interview at a time convenient for the participants. All individuals who contacted the primary investigator to arrange an interview were assumed to meet the eligibility requirements.

In addition to this purposive sampling, the study also utilized snowball recruiting techniques. At the end of each interview, participants were asked to refer anyone else who they think would be interested in participating in the study by forwarding the initial email that they had received. Those to whom the email was forwarded then had the information necessary to contact the primary investigator to arrange an interview, if interested and eligible. No incentives were provided for study participation or referrals. Ultimately, 11 participants responded directly to the forum emails, while 2 participants were referred by another participant.

Instrument

Interviews were based upon a semi-structured interview guide with twenty questions covering five key content areas (Table 1). These content areas were based on the topics identified in the literature review. Questions assessing demographic information were also asked.

Patient Advocacy Measures

The first content area consisted of two questions on background information, including, "How would you define patient advocacy?" The second content area consisted of three questions on patient advocacy experiences, such as, "What do you do as a patient advocate?" The third area consisted of five questions on patient advocacy impacts, e.g., "How do you think your patient advocacy work has had an impact on cancer research?" The fourth area consisted of two questions on measurement of patient advocacy impacts, such as, "How have the effects of patient advocacy been evaluated?" Lastly, the fifth content area consisted of four questions on other topics of interest, such as, "How do you represent the views and opinions of people who have different experiences or opinions than [you/your loved one]?"

These questions formed the basis for discussion, but the interviewer diverged from the guide order and content as necessary to follow the flow of the conversation and probe into new

Table 1. Patient advocate interview guide – primary interview questions.

Background Information
1. How would you define patient advocacy?
2. How did you get involved in patient advocacy?
Patient Advocacy Experiences
3. What do you do as a patient advocate?
4. What skills or traits do you think a patient advocate should have?
5. How has your patient advocacy work changed over time?
Patient Advocacy Impacts
6. How do you think your patient advocacy work has had an impact on cancer research?
7. How has patient advocacy as a whole had an impact on cancer research?
Measurement of Patient Advocacy Impacts
8. How have the effects of patient advocacy been evaluated?
9. How would you evaluate your work as a patient advocate?
Other Topics of Interest
10. What makes you a good patient advocate?
11. How do you represent the views and opinions of patients who have different experiences or opinions than [you/your loved one]?
12. What training do you believe a patient advocate should have?
13. How would you recommend disseminating the results of these interviews?

topics that arise in the course of an interview. Additionally, questions from the interview guide were not explicitly asked if participants had already addressed the topic in response to a previous question or probe. Both topical and motivational probes were used to elicit more details and stories (Hennink, Hutter, & Bailey, 2010). For example, open-ended topical probes invited participants to provide more information on a specific issue. Motivational probes typically included pauses and comments such as "okay."

Because data collection and analysis took place simultaneously, mid-course adjustments to the interview guide were made throughout the data collection process as additional key themes were identified (Hennink et al., 2010). Questions and topics were

revised and added to the guide throughout this iterative process. For instance, the topic of patient advocate training was raised in several early interviews, and as a result, the primary investigator added a question regarding advocate training to the interview guide for future interviews.

Demographic Information

In addition to open-ended questions covering key topic areas for discussion, seven survey questions were asked to collect demographic and relevant quantitative data. Demographic data included age, gender, ethnicity, and educational background. Other quantitative data included whether the participant received training to be a patient advocate and, if so, what that training entailed; when the participant became a patient advocate; and what organizations the participant had worked with as a patient advocate.

Procedures

Institutional Review Board Approval

Before beginning recruitment and data collection, IRB approval was obtained for this study from the Emory Institutional Review Board. The study was also approved by the Clinical and Translational Research Committee at Emory's Winship Cancer Institute. Although there was no physical risk to participants, discussing their personal cancer experiences may have resulted in some emotional risk. The interviewer was prepared to be empathetic in those circumstances, but it did not prove necessary to pause or stop any interviews when discussing these potentially sensitive subjects. Additionally, while participants were not directly asked questions regarding protected health information, some volunteered this information in the course of discussing how they became involved in patient advocacy.

Study Consenting

After potential participants expressed interest in participating, the primary investigator provided them with the information sheet for the study, which included an

overview of the study purpose, procedures, potential risks and benefits, information about confidentiality, and assurances that study participation is voluntary and that the study participant has the right to withdraw at any time. The primary investigator also encouraged potential participants to ask any questions they had about the study or the details included in the information sheet. Immediately before conducting study interviews, the primary investigator read the consent form to each participant and obtained oral consent. Oral consent was obtained because interviews were conducted over the telephone. In addition to consent for study participation, this consent included permission to digitally record the study and to include any protected health information provided in the course of the interview.

Data Collection

Data was collected through one-on-one telephone interviews. These interviews were conducted between November 2013 and February 2014 and took between 30 and 78 minutes, with an average interview time of 58 minutes (SD=14 minutes). Interviews were scheduled based on the availability of participants and were conducted using the semi-structured interview guide discussed in the **Instrument** section above.

All interviews were recorded using a digital recorder. The three initial interviews were transcribed verbatim by the primary investigator using the TranscribePro online application. The remaining interviews were transcribed by a professional transcription service, and two of these transcriptions were randomly selected and quality checked by the primary investigator. Data for this study consisted of interview recordings, answers to demographic and quantitative survey questions, verbatim transcriptions made of the interview recordings, notes taken by the interviewer during the interviews, and memos taken by the primary investigator throughout the data collection and analysis process.

Data Management

All data were kept by the primary investigator on a password-protected computer and backed up to a secure server. Interview recordings were removed from the recording device when transferred to the computer. The names and contact information for participants were known only to the primary investigator. Each participant was issued an identification number and pseudonym, and all recordings, transcripts, and analyses were labeled with that number or pseudonym. The list matching participants to identification numbers and pseudonyms was memorized by the primary investigator, and all documents linking the numbers and names were deleted. While complete confidentiality can never be assured, all identifying information was removed from the interview transcripts before analysis.

Data Analysis

Interview transcripts were read and annotated by hand to identify potential codes or issues in the data (Hennink et al., 2010). The primary investigator then read and annotated all interviews in MAXQDA 11 data management software (VERBI Software, 2013). These annotations were used to develop preliminary codes via inductive and deductive methods. Memoing was done throughout the process to track the development of inductive and deductive codes.

Preliminary inductive and deductive codes were arranged hierarchically based on topic and perceived relationship to create a coding tree and defined in a codebook. One example of a deductive code was *"advocate training"* because training had been identified as a potential issue before the interviews were conducted, and a question was included asking what, if any, training advocates had received in the quantitative section of the interview to ensure that information about training was captured for all advocates. In contrast, one example of an inductive code was *"rubber-stamping,"* because participant perceptions of

advocates who were willing to rubber-stamp grant applications for researchers came up during the interviews and was not anticipated.

The primary investigator coded each interview in MAXQDA 11 based on the codebook definitions and examples. Code development was saturated, with no new codes emerging in the final interview transcription (Hennink et al., 2010), although additional issues may have arisen in additional interviews. A textual data analysis process described by Hennink et al. (2010) was used to develop a thick description of certain issues. The data were first searched by codes and topics, and all data retrieved from each of these searches were closely read. Cross-case comparisons were made by comparing issues across all interviews. Additional data analysis techniques were used to describe variability in the data by developing construct tables and conceptually clustered matrices (Miles, Huberman, & Saldaña, 2013).

In the following section, the results of this analysis will be presented. Results are divided between the two major research questions. Within each question, major themes and sub-themes are presented with relevant supporting quotations.

CHAPTER FOUR: RESULTS

I think a very important part of having advocates at all these tables is reminding them that you're healthy today, but you don't know what's coming down the road. What do you want waiting for you when you get a diagnosis or someone you care about gets a diagnosis? Is this good enough for you?

- *Participant L*

Participant Characteristics

Demographic Characteristics

All participants self-identified as patient advocates in cancer research by indicating interest in the study. Of the 13 participants, 11 responded directly to the recruitment email sent over the patient advocate email list-serv. Each of the other participants was referred by someone who had already participated. Interviews with the 13 participants took an average of 58 minutes (SD=14 minutes) and included seven demographic survey questions.

As is shown in Table 2, all participants self-identified as White or Caucasian, and 12 of the 13 participants (92.3%) were female. The average age of participants was 62 years old, with a range of ages between 47 and 70 years old. All participants reported receiving some higher education: three had completed some college, four had completed college, and six had received a postgraduate degree.

Advocacy Characteristics

Each participant was a cancer survivor and primarily worked as a patient advocate for research in a specific cancer type, although, as is discussed in more detail below, some also worked on broader issues. Nine of the 13 (99.2%) participants focused primarily on breast cancer research, while two focused on ovarian cancer, one on lung cancer, and one on multiple myeloma. Participants reported working in research patient advocacy for an average of 10 years (SD=6.1), with responses ranging from two to 21 years; participants had begun working as advocates as early as 1991 and as recently as 2012.

Table 2. Participant demographic and advocacy characteristics.

Demographic Characteristics	**n (%)**
Female	12 (92.31%)
Caucasian	13 (100%)
Age, M (SD)	61.92 (7.30)
Education Level	
Some College	3 (23.08%)
College	4 (30.77%)
Masters or More	6 (46.15%)
Advocacy Characteristics	
Cancer Survivor	13 (100%)
Cancer Type	
Breast Cancer	9 (69.23%)
Ovarian Cancer	2 (15.38%)
Lung Cancer	1 (7.69%)
Multiple Myeloma	1 (7.69%)
Years as Advocate, M (SD)	10.23 (6.10)

Overview of Key Themes

Participants in this study generously shared their time and stories about their perceptions and experiences as patient advocates, and those stories have formed the basis for these results. As much as possible, the results have been conveyed through direct quotations where each participant is identified by letter, from A through M. Unfortunately, due to the unique stories shared and the familiarity of many patient advocates with each other within the relatively small community, some of the described activities were potentially too identifying to include. As a result, only activities mentioned by multiple participants were included in the description of activities and impact.

The participants in this study discussed many aspects of their patient advocacy experiences. Their interviews were analyzed with the goal of answering two primary research questions: first, what the role of patient advocates is, and second, how their work impacts research. Figure 12 offers a proposed model of the results, with each of the elements of the model discussed in greater detail later in this chapter under the relevant research question.

In this proposed model, the first row, labeled ***Inputs,*** addresses the first research question. This row includes elements affecting how an advocate carries out his or her role of advocating for patient issues. These elements can be considered in two primary categories. First are the ways in which advocates establish **credibility**, including their representativeness and their formal training or research knowledge. Second are the **strategies** that advocates use when working with researchers, including emphasizing that patients are real people, using specific communication styles or approaches, and asking questions. Although establishing credibility and using these strategies helps enable advocates to work effectively, they also face significant **barriers** to their work, including issues around time, compensation, and reimbursement; initiation of advocacy activities; advocate organization and infrastructure; and larger systemic problems in the research system.

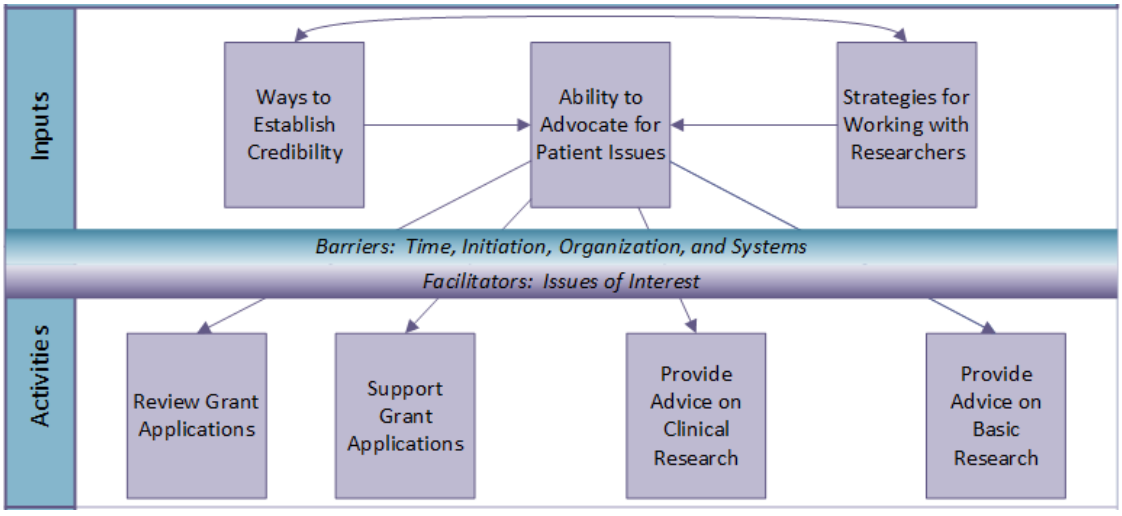

Figure 12. Proposed model of patient advocacy inputs and activities.

The second row, labeled ***Activities***, addresses the second research question. This row includes the broad categories of activities in which study participants described taking part. These activities are driven by the issues of interest to patients and to patient advocates. Activities include reviewing grant applications, supporting grant applications, providing advice on clinical research, and providing advice on basic research. These activities are the ways in which participants impact research; however, the actual impact of these activities is unknown since there has been so little research and evaluation in the area.

Research Question 1:

What is the role of the patient advocate in cancer research?

In every interview, participants said their primary role as patient advocates in research was to provide the *patient perspective to researchers*. As is discussed in the section on the second research question, by bringing a patient perspective to researchers, advocates hope to have an impact by ensuring that research meets the needs of patients as much as possible. This section describes advocate efforts to increase their success in carrying out their role as a patient representative in research. Generally, advocates take various approaches to establish their credibility and use a variety of strategies in their work with researchers to meet their goal of aligning research with patient needs, but their work is hampered by a variety of barriers.

Ways to Establish Credibility

Participants emphasized different ways for advocates to establish the credibility of their perspective as patients, with the understanding that the patient perspective is unique and valuable. Even when medical or research professionals had personal cancer experiences, either having been a cancer patient or having had a close family member with cancer, their viewpoint was described as distinct from patient advocates, because their familiarity with the medical system allowed them to navigate it more easily than a patient coming in with little or no medical knowledge. Participant K spoke to this point, saying that "a doc or nurse, or social

worker who works in the hospital, they're not advocates in the sense that we are, because they don't experience it the way we do." It was particularly important to several participants to have an "outsider" perspective. While a number of participants had some background in health care or science, none were doctors or medical researchers.

Representativeness

A core element of advocate credibility was their representativeness of a larger patient population. All participants said that advocates should be able to speak to multiple patient perspectives. This representativeness could be enhanced by maintaining contact with other cancer patients through non-advocacy activities, as Participant H described:

> "We have to be able to represent the spectrum of patients, at least in that disease area, if not broader than that. So it can't be one or two people that you know who have had an experience and that's it. … I've always encouraged people to, you know, attend support groups or online groups, pay attention to what's happening with patients today, because that's what we're supposed to be helping with, not what happened to us, you know, two, five, ten, even twenty years ago. That's not helpful today."

Even beyond representing different patient situations, representativeness also includes advocating for issues that an advocate may not personally believe in. For instance, Participant H said, "Prophylactic mastectomies, okay? Now personally, I have a very hard time with that. But I have looked at and talked with women who have made the decision to have prophylactic mastectomy and it's clear to me that even though I would not make that decision, it's a valid decision for some people." As a result, she speaks to those views when it is appropriate, even though she does not share them.

Some participants brought up concerns about adequate representation of two specific populations in medical research and patient advocacy: 1) minorities and 2) patients who had not survived or were unable, due to active treatment, to participate. Participant A described

the underrepresentation: "Many, maybe most, of us are White women … it's very rare that you're going to see in this area minority women on these committees, or whatever. And that's probably because they don't have the time, and they're busy working, and, you know, they don't have time to volunteer!" As a result, some participants discussed focusing specifically on alleviating minority underrepresentation by understanding and including perspectives on cultural and racial issues. Participant D described her perspective on representing these and other viewpoints:

> "[L]ike when I was diagnosed, I was a 44-year-old woman who was married, in a good marriage. Well, there are women who have husbands that are not supportive when they're diagnosed. There are women who are single moms, there are women who are young and still want to be moms. There are all sorts of cultural and race issues that come across, and so my job is … to have as broad a perspective as I possibly can, recognizing there's no way I can cover everything, but just trying to do the best I can."

Other participants spoke specifically to their representation of patients who were currently in treatment, or who had not survived, and thus could not participate as patient advocates. This was particularly true for patient advocates in cancers with high mortality rates. Participant M said: "[W]e're there because of the people who are not there. By looking at the research, we're not looking at research that's going to benefit us often. I mean, I'm – I've been in remission, so I'm not in treatment anymore, but I'm speaking for women that are in treatment, or women that are dying right now."

A common concern among participants was that a lack of objectivity would detract from an advocate's credibility and representativeness. There were several examples of advocates who focused on their own cancer experience or a personal agenda instead of representing the broader patient perspective. Participant L noted:

> "A lot of times people come to this with, 'I want my cancer cured.' You know, or this is my experience, and this is what it's all about. And that's just not true, you know? People have a lot of different experiences, so you have to be more open-minded. You have to be willing to represent a broad base, not your own individual case, even if you're just dealing with your own type of cancer."

Representativeness could easily be lost if advocates were not aware of current patient issues, were unwilling to bring up issues even if they did not personally support them, or were unable to put aside their personal experience when necessary to represent the broader patient perspective.

Training and Research Knowledge

Some participants expressed support for *formal training* to help new advocates develop a sense of representativeness. Most participants supported training as an opportunity to establish a fundamental knowledge of research concepts to facilitate discussion with researchers, and all but two participants discussed either receiving or providing training on research advocacy. The most common training discussed was the National Breast Cancer Coalition Project LEAD Institute, which seven participants said they had participated in and spoke positively of. Participant C said of Project LEAD that when she joined a group of patient advocates in research, "They said stop – don't open your mouth until you go to this group. And that's exactly what should be said. I don't think anyone should open their mouth until they go through Project LEAD."

Other options for training outside of breast cancer included sessions at larger research conferences and through the Research Advocacy Network. Additionally, participants said that trainings were generally provided before the grant review process for NCI, DOD, and Komen. In discussing new advocates participating in the DOD review process, Participant K linked training to the concept of representativeness: "The main thing is they have to go there, and

they understand that it's not about you. … It's more, you represent more people than yourself. And that's – you know, that's something that we tell the advocates, and they get a training session to, you know, to understand that more fully."

While training was generally described as helpful, both initially and on a continuing basis to ensure that advocates were aware of the latest issues, Participant C questioned whether it truly helped provide advocates with credibility, noting that "we all talk about Project LEAD. … [I]t isn't like (laughs) oh, did you pass or did you flunk?" She went on to say:

> "I've been in rooms where every time an advocate opened her mouth, I just shrunk in my seat, and she was definitely a squeaky wheel. She wouldn't give up her – you know, she was speaking about her disease … I mean, people are not trained equally… you have issues about how seriously we're taken and so I don't know, ultimately – I feel bad for scientists, you know, it's like they don't know what they're getting themselves into, dealing with us. And if there's – you know, if we can see all the letters behind their name but they can't see any letters behind our name, why would they look at us differently. "

Because advocate training is not required, tracked, or standardized for many activities, it is difficult to use it as an indicator of credibility. Similarly, it cannot ensure that advocates are aware of or following unspoken best practices, such as providing representative patient perspectives. To alleviate this, Participant A supported requiring training as a step toward standardizing the patient advocate process and hopefully giving the field more legitimacy. While training requirements were not discussed by other participants, many emphasized the importance of training, with Participant B saying that "NCI and everybody is saying that they want an advocate on the table … if you have to have an advocate, then you should make sure that it's an educated, trained advocate."

Helping advocates understand their role as patient representatives was described as one important component of training, but participants did give more emphasis to the importance of training in understanding and being able to discuss research concepts. Participant A noted, "[W]e need to be trained. There's no way you can go into this absolutely cold. Unless you've got a PhD in science. … [W]e always want more training, we want to keep up to date, and so it's important that we get to conferences and workshops." This speaks to the perceived importance of both scientific knowledge and continuing education. Similarly, Participant G said, "I think that like anything else, the more you can be involved and the more you're not sitting there listening to a foreign language, the more engaged you feel."

Participant K, however, conveyed a tension between an advocate's continuing to learn about research while still maintaining a patient perspective:

> "[T]he more that I know, the – you know, I think the more that I can really accomplish in making change … but also, I'm aware that I don't want to become part of the research community. … It's important to understand that the people I'm focused on are the people with cancer who don't know what the heck is going on, you know?"

Speaking to this, the two advocates who had not received or provided training ranged from neutral to opposed to the idea of formal advocate training. Participant J had not received training, although she mentioned that she would like to attend sessions eventually. However, she feared that training in research could reduce advocates' abilities to provide an outside perspective focused on the patient issues, instead turning their attention to areas of the research that researchers are better equipped to consider, saying:

> "[I]t seems to me that in breast cancer advocacy, a lot of the groups that are doing a lot of advocate work have this idea that advocates need to really know the science. They really need to understand, you know, about breast cancer, and genetics, and genomics, and all of this treatment, very, on a very detailed level. And I sort of question that.

> … Like, to me, having, training advocates to think the way that the doctors do, or the way the researchers do, to me isn't necessarily adding anything to the process. I'm not against it. But I'm not particularly for it. I'm like, I think advocates ought to be positioned and empowered to say things that are different, that are, you know, to come from a different perspective, because they are a different perspective in the system."

When asked if she had received training, Participant D merely said, "No. Nothing. Thank goodness!"

Strategies for Dealing with Researchers

Participants described using a variety of specific strategies when dealing with researchers to enhance their effectiveness as advocates. One strategy involved emphasizing to researchers that patients are "real people" their research can thus have a real impact. Additional strategies around communication included adopting different styles and approaches as necessary and asking questions rather than making direct statements.

Emphasizing that Patients are "Real People"

Participants noted that many researchers had difficulty understanding the relationship between their research and real patients. Participant K described the transition that has occurred since the early years of advocacy in breast cancer research:

> "A real person has cancer nowadays, more people are aware of that. But really when we started with this whole program in 1993 or whenever it started, '92, I forget, '94, whatever it was, they really didn't see it that way. … [A]nd that's one of the things that's changed, that you know, you really have to think about the person behind it and not just your elegant research."

Despite the changes, basic scientists were generally still seen as less in touch with patient needs, with Participant H saying, "the basic scientists never talk to patients, so they were kind of in this bubble, in a way … and have no idea of whether their work was relevant or not to

people, or how it could actually be more relevant to people." Similarly, Participant D said that "[S]ome of the researchers that I've seen that worked the best with patients do have a clinical side … [I]t's not just clinicians but I think clinicians are more attuned to it and probably have a better skill set."

However, Participant F noted that basic scientists could be more willing than clinicians to acknowledge the disconnect between their experiences and the patients their research would hopefully impact, listen to patient advocates, and alter their perspective:

> "If it's a clinician that's in research as well, they're not as open to it because they're treating patients and they don't like smart patients as a rule. They want their patient to come in, and they want their patient to say, 'Yes sir, no sir and thank you so much and I love you and you know, I'm going to name my first child after you.' But the people in the lab don't feel that way. You know, they're in the lab, they're strictly working on the science. The ones in the lab are actually more open to listening to this."

Participant F went on to describe the change in perspective that an advocate story or message could cause in researchers:

> "And I always tell them [scientists], now, when you go back to your lab and you go back to work, remember, that's not a piece of tissue and it's not just a blood droplet. That's a patient that wants to live and is counting on you to make that happen. … You know, then they start thinking a bit differently. Like, wow, I'm in charge. I actually, I actually could discover something here that increases someone's life."

When advocates referred to their role of conveying to researchers the potential effect of their research on patients, many described their actions with sensory phrases such as "voice of the patient" and "face of cancer." Emphasizing the uniqueness of the patient advocate's role, Participant G commented that "only we really can speak from that patient's voice." When asked to define patient advocacy, Participant B concluded by saying, "I think that people get

into different parts of advocacy and some people get involved in all of it, but I think to me, I guess it's the voice of the cancer patient."

By providing a visual "face" and an audible "voice" of cancer patients, advocates transform an abstract patient population into real people. When discussing her work with basic scientists, Participant E noted, "They don't have a face – face for cancer. I think, you know, advocates give them a face for cancer. This is what it looks like. These are real people going through real issues." Similarly, Participant C noted, "Other people have no voice. Here – we're here, and we have a voice, that's what – why we're here."

Communication Styles and Approaches

Advocates can have differing attitudes and approaches toward their work, and participants highlighted certain of these as necessary for success. In many ways, this relates back to the question of training, because one key approach was to be able to speak the researcher's language. Although Participant K raised the issue of tension between research knowledge and representativeness, she also noted that throughout her time as an advocate, "I learned a lot more about science. I learned the language. I try to use the appropriate language when I'm speaking to researchers, so I think fortunately or unfortunately, they need to hear it in their language before they can really relate well." This was seen as an advantage to advocates as well. Participant G said, "I think that like anything else, the more you can be involved and the more you're not sitting there listening to a foreign language, the more engaged you feel."

While Participant D felt that understanding the language was important, she also noted that at times it could be useful not to use a scientific vocabulary:

> "[W]hile I think having an understanding of the technical jargon and terms is important and it does kind of catch them off guard when you can rattle it back in their face, I also think trying to lay it down, so to speak, is a good way to help train them not always to

> scientifically speak, because that's when they start intimidating people and that's when they start making science something that people can't relate to."

Thus, while approaching researchers with scientific language can be an asset, it can also be helpful to be able to return to lay language: depending on the situation, either might be an appropriate strategy. Participant J took this concept a step further, saying, "I think advocacy, patient advocacy, and any kind of advocacy is really just the task of translating between different views, different experiences of the world, different kind of languages." In addition to being able to speak both lay and scientific language, advocates may need strong communication skills to translate between the two.

Careful communication is also essential when advocates are working – and often disagreeing – with scientists. Participant H said, "Communication skills are very important for patient advocates to have as well. … Respecting, you know, other people's opinions, being able to disagree with them but not in a negative way. You can be forceful, you can even be strident, but you can't be pushy. I don't know exactly how to say that – assertive but not aggressive, I guess." Similarly, Participant B commented, "[T]here's a way to disagree, and you have to disagree respectfully. … I'm very careful of how I speak to people and how I – you know, and a lot of times, I have to bite my own tongue." She believed it was necessary for advocates to "pick your battles" in order to maintain the respect of researchers.

These participants discussed the need for care in communications from the advocate's perspective. Participant L, on the other hand, noted that these strategies were to some degree a necessary response to the researchers they work with. She said that "you know, that's important, getting people who understand – I don't want to say what their place is, but how to properly interact with scientists, who can sometimes be a little standoffish."

Asking Questions

One more concrete approach that several participants mentioned as a strategy in talking with researchers was the use of questions instead of comments or statements. Participant K described an experience at a scientific conference where she had asked a question about an issue important to patients, saying, "I knew the answer to it, you know, because that's what you're supposed to do. You know, I try to raise – I mean, for me, I try to raise an issue. So I know the answer, I just want them to acknowledge it." Similarly, Participant C said, "[W]hat makes me, I think, a better advocate is asking good questions. I know how to ask questions, I'm not afraid to ask questions." Participant L spoke with more depth about why this strategy might be necessary:

> "You know, instead of saying, you should be doing this, you can say, well, is there a reason you can't do this? You know, that can change the whole conversation … I basically call it feminine wiles. It's something we used to have to have, you know? You need to stroke these people, men and women. I mean, they have egos. Many of them have very big egos, and so you have to sometimes play a little dumb. I remember at one meeting we had, it was an [organization] meeting. I got up and asked a question. But I knew the answer to the question. But I wanted them to talk about it. … I wanted them to talk about it in public. I want them to discuss it. I want them to think about it."

By asking questions that they knew the answer to, advocates can engage researchers in discussion and encourage them to think about specific issues without appearing overly forceful.

Barriers to Action

The strategies and methods used to build credibility discussed above facilitate the work of advocates, but there are also significant barriers. Key barriers identified by participants included issues of *time, compensation, and reimbursement* for advocacy activities,

because advocacy can take significant time and is rarely compensated. Another barrier was the *initiation of advocacy activities,* as many advocacy activities are initiated as the result of pressures from the community or public policy level. More systemic barriers included the *organization of advocates* and *current research systems* that may incentivize research that is not patient-focused.

Time, Compensation, and Reimbursement

From the advocate perspective, key barriers to advocacy can include the time required and the lack of compensation. These barriers are linked, because working as an advocate can take significant time, which may not be possible to balance with a full-time job. Participant D said, "If I were to get back into paid employment, I would have to stop some of – at least some of what I'm doing because there's no way I can continue to do everything that I'm doing."

If advocacy work is not compensated, it may only be possible if the advocate has other means of support. Participant A said, "I'm lucky that I have been able to put time aside, you know, my husband supports me and allows me to do this stuff, I feel really lucky." Participant A went on to suggest that this issue might contribute to the lack of minority representation among advocates, noting that "you know, it's very rare that you're going to see in this area minority women on these committees, or whatever. And that's probably because they don't have the time, and they're busy working, and, you know, they don't have time to volunteer!"

Reimbursement can also be an issue when advocates may have to pay to attend trainings, conferences, and meetings. Participants described a variety of experiences, with some organizations covering their costs and others expecting them to pay out-of-pocket. Participant B worked with one organization that was good at "giving us go-to, you know, education processes and you know, they cover cost for – because for a lot of the advocates, you know, we're not rich. We're not doctors, so we're lay people with jobs and you know, a lot of us are still paying for some of our treatment." Participant C, however, was asked by an

organization to be an advocate for guideline development, and was not able to attend the meeting in-person because her costs would not be covered. While the issue was not discussed in every interview, no participants mentioned being reimbursed or compensated when working directly with researchers, for instance on a grant.

Initiation of Advocate Activities

Another barrier seen across interviews was the current infrastructure in which advocacy takes place. Most advocacy work is not initiated by the researcher or the advocate – instead, *it is mandated* by administrators and funding organizations. Many of these requirements are certainly in response to the work and persistence of advocates. Participant L said, "You know, about, I guess, 15 years ago, there became this push to have an advocate on every project at the NCI. It was sort of, you know, if you're going to do a project, you better have an advocate, because if you don't, they're just going to complain." However, many participants said that their roles were still too limited, and they were frustrated that they could not do more than what had been mandated from above.

As mentioned previously, different funding agencies required different degrees of advocate involvement. The California Breast Cancer Research Program was widely seen as one of the most advocate-friendly funders, because it requires that researchers include an advocate on their grant applications as well as including advocates during the review process. However, inclusion has not necessarily translated to involvement. Participant C said, "You're lucky if you get an email from the scientist saying thank you, and I got the grant." She wished that greater involvement was required, hoping that "we'd be wanted versus needed. Right now, we are needed. We're needed because it's a requirement to have an advocate on – on that grant, on the LOI [letter of interest]. The second our name is there, they don't need us and they don't want us."

The DOD involves advocates in the grant review process, but does not require that researchers include advocates as part of their grant proposals. Participant A had recently contacted a program manager at DOD to ask why advocate involvement was not required, or at least, "why wasn't it even mentioned on any of the mechanisms? And she never answered me directly, she just said, we will fund the best research, you know, with collaborations and partnerships that are going to benefit the patient. … So you see, it's a bit of an uphill battle." Similarly, Participant C said that when she had last been a DOD reviewer:

> "I said you know, in reading all these grants, it was fabulous, I loved this opportunity, but how come there weren't any advocates required to be on the grants? And she said – you're at the table. Like I realize we're at the table but you know … [t]his is a perfect time to get – to start collaboration. I think that – that to me is the issue. There is not a partnership, a real partnership. To a large degree, I think most advocates are seen as, you know – you know, flies on bread."

When it came to involving advocates during funding allocation and grant reviews, Participant G, said "NCI is one of the worst of requiring advocates." Participants reported issues in the other mechanisms through which NCI involved advocates. Participant L noted that "NCI put out this edict, and they started bringing advocates in because they had to. … [T]hey were starting a training, and then unfortunately, this office of liaison sort of got their budget cut, and the whole structure got changed when Varmus came in." Recent changes at NCI similarly affected the opportunities for advocates in cooperative groups. Participant H said that after the cooperative group mergers, "we're still advocates working with [cooperative group], but as far as like, our proactive approach of what we were going to do was sidelined."

Funding and administrative organizations have facilitated advocacy by requiring advocate involvement in certain areas, but this has also created a barriers. Researchers tend, at least initially, to only involve advocates when it is required. Even if researchers may be

interested in working with advocates, Participant G said, "One thing that comes up a lot that researchers tell us is oh, the timelines are so short to apply, so even to contact an advocate, or do the research, there isn't a lot of time." Similarly, Participant J said that "in the places where they're doing research… at a very high level, things go very, very fast, and it's a little bit hard sometimes to kind of, get into the discussion, even when people want it." Especially when there are limited resources, advocate involvement outside of the required roles may be seen as unnecessary. Additionally, because the required roles are externally defined, it is difficult for advocates to redefine them, and they have limited control over changes that may hinder their activities.

Organization of Advocates

Compounding these barriers is the fact that many advocates are not aware of what other advocates are doing. Participant A said, "I think a lot of research advocates work in silos just like the scientists. ... Nobody really knows what's going on. … It would be nice to know nationally, what are everybody doing? Who are they doing it with?" Similarly, when asked what type of training would be most useful to her, Participant J said that "an understanding of how complicated the landscape of advocacy is just within cancer. … Sort of what's the big picture of all the places that advocates are involved in the system, sort of formally and informally. And then you could say, well, on the community level, well, yes, it's informal and there's lots of different models, but here are some different models."

Participant C felt that the lack of organization could be intentional and was a major barrier to advocates being able to promote patient interests as a whole: "I'm sure many other institutions keep their advocates disorganized because with organization, there's power. Whether they do this deliberately or otherwise … if you're organized, then you have power. You can grab the purse strings, you can make really good decisions based on – on the whole." Participant B also felt that better organization could facilitate more effective advocacy, saying,

"[I]f there was a way that advocates could get together, if it was a more organized way, it might help."

Current Research Systems

Many participants also brought up their frustration with aspects of the research system itself. Participant L gave the example of the clinical trials system, saying, "The big mantra when we first got started – when I first got started in all this was we need more people in clinical trials." Since approximately three percent of adult cancer patients were participating, patient advocates were seen as a resource that could reach out to patient populations and help enroll more patients in trials. However, as Participant L then went on to discuss, the low enrollment was less due to patients being unaware or unwilling to participate and more due to the structure of the clinical trials system:

> "[T]hey couldn't be over 65 years old. And they had to live near the place that they had the, you know, treatment, and then they couldn't have any co-morbidities, and the list went on and on. … So you get the younger people and the healthier people in trials, and then when the drugs get approved, they go into the clinic, and who do you give them to? The people with co-morbidities, and who are sicker, and older. You know, and it made no sense."

Advocates have worked to change the eligibility restrictions for clinical trial participation, with some success, but larger programs continue to be an issue.

Broader systemic issues were acknowledged by several participants. Participant D said, "I think the system needs to change and it needs to change sooner rather than later, and sometimes I worry about how people are so caught up into what it is now and are scared about what changes might mean for them." Similarly, Participant A noted that "it's how do you change, you know, how do you change a system that's so entrenched. … [W]e're all very good at

asking questions and telling you what's wrong, [interviewer name], but it's like, how do we make it better?"

Ultimately, participants questioned what impact they could have if the larger system was not working. Participant H said:

> "[N]o matter how much tweaking we do to the system, and there's a whole lot of tweaking going on, but none of it's going to be super successful unless we change some of these fundamental issues. … All of the incentives that are built into the research system are basically thwarting efforts to get results to patients. So we have to realign the incentives."

Because all advocates work in the context of the larger research system, their work is often limited by it. Although participants were frustrated by this, as Participant H indicated, they also saw it as yet another challenging area in which their patient perspective could help bring about meaningful change.

Research Question 2:

How does the work of patient advocates impact cancer research?

Issue-Driven Activities

With few exceptions, participants said that the goal of participating in various advocacy activities is not to improve research simply by being present. Instead, it is to raise the profile of particular issues through their actions and participation in specific activities. As a result, participants tended to interweave descriptions of their activities and the issues they are interested in. However, there is a minor yet significant distinction in their descriptions of issues: issues may be important to a typical patient, and an advocate would represent that *patient's needs and interests*, or an issue may be of interest to patient advocates as a result of their familiarity with and perspective of the research system.

Issues of Interest to Patients

Participants described patients as primarily concerned with the effectiveness and side effects of potential treatments they might receive if they participate in clinical research. Participant D said of her work as an advocate:

> "What I bring to the table is more 'how does this affect patients' and 'what do patients really want,' and besides a home run, you know, everybody knows we want a home run. But how we get there is – sometimes makes as much difference as a home run itself. And if you're being dragged into the home plate and you don't have any way to enjoy it, then you may not have wanted that home run. And it depends on where you are in your life stage, for instance, or what certain side effects mean to you."

While patients, like researchers, are often ultimately seeking for a cure, their interest in a treatment depends both on how well it is going to work and on what side effects it is going to produce.

Unfortunately, doctors and researchers may not be fully aware of the importance of side effects, because patients do not always tell them. Participant K said, "[T]he docs are very well meaning, but you know, they don't understand. I mean, for the most part, we know studies show patients don't tell them when there's a problem. They tell the nurse. They don't tell the doc." Thus, patient advocates play an important role in helping doctors and researchers understand what the side effects are. They also offer guidance on how to balance the impact side effects are likely to have on patients with the possible benefit. For instance, Participant M said that when advocates participate in grant reviews, "We're not going to be in favor of – of funding something that would possibly give somebody two more months of survival but with horrible side effects."

Patients are also concerned with the *practical issues of research participation*, including logistics such as cost and the extra time participation will take, the additional tests required,

and the consent form. Discussing what might keep a patient from participating in a clinical trial, Participant I said, "Patients never want more tests. It really comes down to a burden, and that burden could be time. It could be cost. It could be – nobody likes to do what's called a bone marrow biopsy because of pain. So it could be different things." Participant B talked more specifically about the problems in the informed consent process, saying that "we work a lot on consent, you know, because consent tend to be so legalese that you know… I look at them and I say you know, no one's going to understand this and if they don't understand the consent, they're not going to sign."

Patient advocates represent these patient concerns to help ensure that the burden on patients to participate in research is as low as possible. Participant H noted that when advocates work with researchers on clinical trials, "[W]e try to look at the spectrum and think about what situation those people are in, where their mindset may be, so that we can try to match the trial, make it as easy as possible for them to enroll and be involved in the trial, if possible, while keeping the scientific end points, you know, accurate and all of that."

Issues of Interest to Patient Advocates

The distinction between issues important to the average patient and issues important to patient advocates is clear when considering clinical trials. Patients, as discussed above, are primarily concerned with practical issues around trial participation such as the required tests and potential side effects. Patient advocates, however, are also concerned with addressing more systemic issues such as how to increase clinical trial participation rates so that trials are more likely to succeed. These are issues that a patient unfamiliar with the research system may not be aware of, but that patient advocates are in a position to consider. When working with basic scientists, advocates are concerned with the potential applicability and translation of their work to patients. In addition, individual advocates may also be interested in more

specific issue areas, such as complementary and integrative medicine, environmental concerns, genomics and personalized medicine, data sharing, or health system disparities.

Participant B said that, when she started her work as an advocate, "I think that was the thing that really shocked me, was how few of people are in a clinical trial." As a result, advocates emphasize the importance of maximizing the value of each clinical trial. Participant H discussed how advocates have pushed for additional aspects of trials out of respect to the patients participating:

> "[A] lot of times, we're the ones who have pushed for quality of life or even correlative science endpoints or companion studies to be done with these studies, because the way we look at it is people who are enrolling in the clinical trials are gold, and we should treat them like gold and we should help answer as many questions as we can for that group of patients, because they're contributing a lot and we need to respect them for that."

Similarly, Participant G described how advocates want to encourage collaboration and ensure that results from every trial have an impact, even if the trial is unsuccessful, saying "[O]bviously we've got to use what little money we have now to the best advantage and you know, that's why we're passionate about topics such as making sure that trials that fail are published, and making sure that researchers know what other researchers are doing and that they collaborate with each other." Through this work, advocates hope to reduce duplication so that research can focus on new areas with new potential.

Advocates may also think about issues patients would not know to consider when enrolling in a trial, such as whether the trial therapy will be accessible after the trial ends. Participant A gave an example: "[S]ay the agent works for this particular group of study patients, what happens at the end of the study, you know, will they still have access to this agent. And if so, will it be affordable, will insurance cover it … practical issues that, when

you're a clinician or a researcher you don't, you're not aware of because you may not have been impacted by it." Because advocates have experience across the research spectrum, they are aware of issues that might affect patients from enrollment through the close of the trial.

Participants were supportive of basic research, but said that as advocates they wanted to know that scientists were focusing on research that they thought had the potential to help patients. Participant C described asking questions such as, "[H]ow does it really get us to the clinic?… [I]t's great you're finding this in a little microscope but in five years, where is it going to be? Still in the little microscope?" The translation of successful research to the clinic was also a concern; Participant D said, "[O]ne of the questions I ask as an advocate – okay, if you're successful, how do you take this – what's the plans to take it to the next step? And if you don't have the next steps, then why are we investing here? … [I]t's not like in today's environment, you build it and they will come."

Overview of Selected Activities

As can be seen in Figure 13 and in the discussion of issues above, advocates contribute to a variety of research activities. The participants in this research study emphasized four key activities: reviewing grant applications, supporting grant applications, supporting basic research, and supporting clinical research, including trial accrual and design. Nine participants had reviewed grants, seven participants had supported grant applications, six had supported basic research, and eight had supported clinical research. In each of these activities, advocates utilized their different strategies and approaches to impact research by bringing the patient perspective to researchers, with the goal of enhancing the ultimate effect of research on patient outcomes.

Reviewing Grant Applications

In reviewing grants, the primary issue is which of the applications being considered has the greatest potential to impact patients. Participant K said, "I have reviewed many grants,

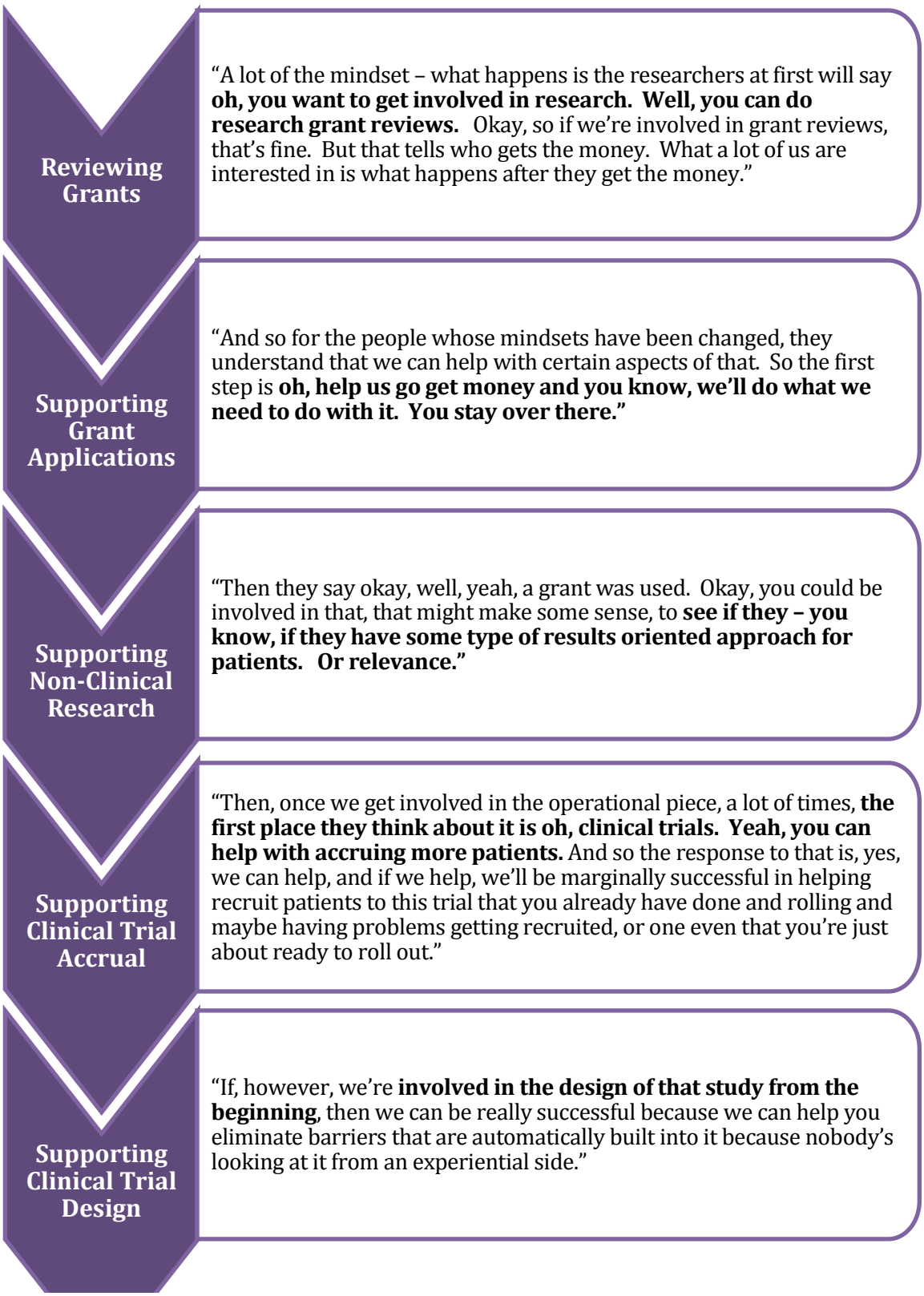

Figure 13. Participant H's description of increasing levels of patient advocate engagement in research.

and I always review them with an eye towards making sure that they're – the outcome will be meaningful to people with cancer, and that it will be used appropriately." Similarly, Participant M said, "We're looking for something to get into the clinic sooner rather than later. It doesn't always happen, but that's what we're looking for." Participants had been involved in grant reviews for a variety of organizations, including the DOD, Komen, NCI SPORE awards, and the California Breast Cancer Research Program. While Komen and the California Breast Cancer Research Program were specific to breast cancer advocates, DOD and SPORE review panels included advocates from the cancer areas being funded.

Supporting Grant Applications

Breast cancer patient advocates were more likely to have had experience supporting grant applications, because the California Breast Cancer Research Program is the only funding mechanism that consistently requires that grant applications include advocate involvement and a letter of advocate support, although other funders occasionally do, too. Yet even with the requirement, participants said that advocates were typically seen as an afterthought. Participant A described her frustration at researchers waiting until the last minute, saying of a recent request for a letter of support that she had received a week before the application was due, saying "I declined, because I was already swamped anyway, but there's always an advocate who'll say yes, I'll help, so they never learn. … [T]hey can just pull somebody up at the last minute, knowing that there'll be an advocate who'll say yes."

Participant C described how researchers often seek an advocate willing to sign off in support of their grant shortly before the grants are due: "all of a sudden we get these emails and they go out to all of us and it says you know, this is from, you know, whoever at whatever lab and we're looking for an advocate to help us with this grant and it's about X, Y, and Z. If you have an interest, contact, you know, this person." Participant C went on to say, "[B]ecause some people are trained and other people aren't, there's a real discrepancy in what the

advocate does. Sometimes an advocate will just rubber stamp, oh, this looks great. Yeah, use my name. Oh, what do you want me to do? Oh, I'll write a letter that you're the best thing in the world, done." Because researchers could easily find an advocate willing to "rubber stamp" their grants, participants found it difficult to leverage the fact that the researchers needed advocate support to get them to agree to more meaningful advocate involvement.

Supporting Basic Research

As was discussed above, patient advocates typically focus on the applicability and potential for translation of basic research. Participant H described the importance of this:

> "[B]y having us ask questions about their work, understanding conceptually what it is they're trying to do, and then asking what issues they face in moving this forward, and asking the questions frankly about okay, well that's great, if your X, Y, Z works, then what? Or how does that get closer to people? What are you going to do? … [T]hey needed to think about that and many of them still don't, but those that do work with patient advocates are at least thinking about what the next step is."

Participants generally said that work with basic researchers began with explanations of what the research involved, followed by probing questions about the relevant issues.

Supporting Clinical Research

Participants described the need for advocate involvement in clinical research so that all of the issues described above surrounding patient participation in research could be raised and adequately addressed. However, their involvement was often divided between helping with trial accrual after a study had already been designed and was not enrolling enough patients, and more rarely helping during the initial study design phases. Participant I said they could provide a variety of feedback in that scenario: "if a trial has been approved for a while and it isn't accruing, how do you fix those things? Or how do you make people more aware of them?"

However, participants said it was much more useful to be involved earlier, when the trial was first being designed, so that they could help minimize the issues that would cause patients not to enroll. Participant B said, "they're always asking advocates after a trial was not accrued, you know, and it was like at that stage, we really can't help at that stage." Instead, she said they were most useful during trial development: "if something like's a glaring not going to work, you know, at least we have a say on the trial development and I think that helps." Glaring issues could include anything that would present too great a burden for patients. As was mentioned in the Issues section, advocates could also help ensure the trial was designed to answer relevant questions.

Comments Regarding Evaluation

Participants were often frustrated by the lack of clear measures that could help capture their impact, assess best practices in patient advocacy, and improve their work in the future. Participant A noted that "even within the PI community there isn't a consensus on our value and our worth. So, and I don't know what my value or my worth is, either. So, it's horrible, because how do you measure that? How do you measure what you're giving?" She went on to say of more research and evaluation, "I think it would be good to know, because if we knew, then we'd know where to best put our energies."

Overall, however, participants described their work as productive and positive. They shared many anecdotal successes in promoting the patient perspective when it might not otherwise have been considered. Participant H commented on the lasting effect advocacy participation could have, describing how "when you're with a group where patient advocates are actively engaged in the scientific discussions, the tenor of those discussions changes, so that in some meetings, the advocate doesn't have to ask any of those questions. The scientists are doing it themselves, which is great."

Participants perceived many researchers as respectful and willing to listen. Participant B said of the relationship between advocates and researchers, "I mean, sure, it could get better, but I really feel like they are now taking us seriously and you know, it's important that they are but again, it's our job to make sure it stays like that." However, there was also discussion of researchers who were less pleasant to deal with. For instance, Participant G said of working with researchers, particularly on grants, that "it really depends on the researcher. We have several that we know we are valued but a lot of them, it's still last minute." Similarly, Participant C described the wide range of researcher perspectives she encountered:

> "I had an experience where one scientist kept referring to only … two – two reviewers when the third reviewer was an advocate. … [A]nd then I had a very amazing – you know, I had another amazing experience where a scientist came up to me and said, you know, you changed my mind about something and I, you know, just want you to know that. So you have – you never know where you're going to make a difference."

Other participants emphasized that advocate experiences with researchers were constantly evolving; Participant D said, "And so when you're participating in reviews, that process starts, and when you're able to then participate in panels and discussion groups, it continues."

CHAPTER FIVE: DISCUSSION

Research in the United States has increasingly involved and even mandated the inclusion of patient advocates. However, the roles and effects of patient advocates have not been thoroughly studied in the past, making it an important topic for research. As government and funding agencies continue to require additional advocate involvement, it is essential to understand how patient advocates participate in and contribute to the research system so that their positive impacts can be maximized. The purpose of this study was to begin to fill this gap in understanding by exploring patient advocate roles in and impact on cancer research and laying the groundwork for future evaluations.

Proposed Logic Model for Future Evaluations of Patient Advocacy

Figure 14 expands upon the model proposed in Figure 12 in the Results chapter to propose a formal logic model for patient advocacy. Logic models are used in program evaluation to visually depict the underlying theory of change (Frechtling, 2007). By describing the inputs, activities, outputs, and outcomes of a program in a single page, logic models concisely summarize what a program hopes to accomplish and how the program hopes to accomplish it. Although patient advocacy is by no means a single cohesive program, a logic model can be a useful tool in considering what is being done, what outcomes are hopefully being produced, and what elements could be evaluated.

Inputs and Activities

As discussed in the Results chapter, the top two rows of this logic model were developed though analysis of the qualitative interview data from this study and help answer the primary research questions for this study: what are the roles of patient advocates and what impact do they have. The ***Inputs*** row shows how ways to establish credibility and strategies when working with researchers influence an advocate's ability to represent the patient perspective and advocate for important issues. This work may be hampered by various

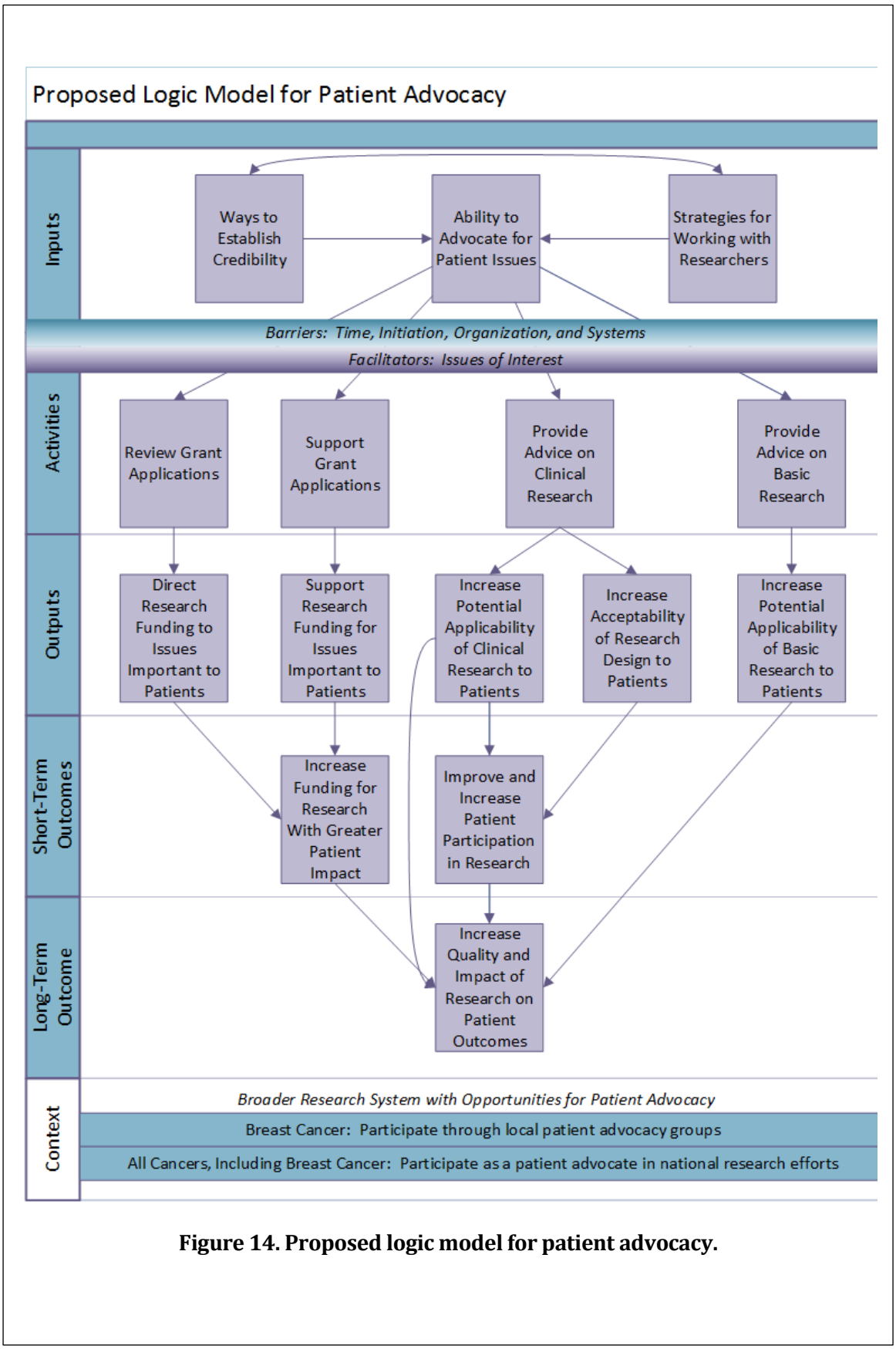

Figure 14. Proposed logic model for patient advocacy.

barriers, but is facilitated by the issues that patient advocates are passionate about and that drive their work.

Many of these findings align well with the limited research currently available. In this study, participants emphasized the importance of representing a broad range of patient perspectives to establish their credibility. Efforts by programs in the US to define "legitimate" advocates based on significant cancer experience, but also the ability to raise issues outside of that personal cancer experience, reflect comparable concerns. Interestingly, research in the UK has focused more on the unique "experiential expertise" that advocates have developed as a result of their individual experiences, rather than whether advocates could – or should – represent a larger patient population (Thompson, Bissell, Cooper, Armitage, & Barber, 2012).

Participants also described using training as a way to build credibility. Similarly, Parthasarathy (2010) argued that training such as Project LEAD is necessary to break through the "expertise barrier" separating scientists and the lay public. However, just as some participants in this study feared that training would reduce their ability to provide a patient perspective, PPI participants in the UK and AIDS activists feared that too much training could be counterproductive (Epstein, 2004; Thompson, Bissell, Cooper, Armitage, & Barber, 2012). While UK researchers were more eager to work with trained advocates, they also were more likely to doubt the representativeness of those advocates (Ward et al., 2010).

The research included minimal discussion of strategies used by advocates when working with researchers. Certain communication styles and techniques were described as necessary for communication with researchers, rather than a strategic decision. For instance, in the qualitative study of NCI advocates, a common theme was that advocates fit in with researchers and share their views in a "fashion that's acceptable to the panel" (Gilkey, 2014, p. 117). Similarly, key figures from PCORI wrote, "To participate effectively, patients and stakeholders will need to acquire a certain level of training to have productive conversations

with research partners" (Fleurence et al., 2013, p. 397). More specific strategies, such as emphasizing that patients are real people or asking questions, have not previously been described.

There has also been scant discussion of the ***barriers*** to advocate actions. A review noted that two articles in the UK argued that funding should be allocated for the compensation and reimbursement of public representatives in research (Boote et al., 2010). Little else was said by researchers about other barriers raised by participants. In the case of systemic issues in the research system, it is clear that this barrier is significant for more than its impact on patient advocacy. However, barriers of initiation and of time, compensation, and reimbursement are important themes emphasized in this study, and more research should be done in the future to examine their effects on patient advocacy.

The sample in this study was clearly not representative of the population in the US with cancer. The participants were overwhelming White, female, and highly educated, but many mentioned in interviews that most advocates shared these characteristics. This suggests a larger concern in the patient advocacy process: whether, potentially as a result of barriers of time, compensation, reimbursement, or larger systemic issues, certain types of patient voices are not being heard. The time requirements and lack of compensation or reimbursement may make it difficult for patients who work full-time to be involved. Similarly, systemic issues such as historic distrust of medical research that are thought to lead to relatively low participation rates in clinical research among minority populations (BeLue, Taylor-Richardson, Rivera, & Grandison, 2006) may also contribute to low levels of minority participation in patient advocacy. The potential causes of these skewed participation rates should be studied more fully so that actions can be taken to address them, particularly as the arenas and activities of patient advocacy continue to expand.

External initiation will clearly continue to be an issue. As described in the literature review, advocates were responsible for significant changes in cancer research funding at NCI, DOD, and within the state of California. They were able to leverage this position to require the initiation of advocacy involvement from governmental agencies. While external, top-down initiation has been a barrier for participants, it also has been one of the most reliable ways in which advocate involvement has been promoted across the research system. Recent patient involvement initiatives established by the federal government such as PCORI show that external initiation is, if anything, increasing. Many participants described the time they had spent lobbying the federal government for research funding and patient involvement. These activities were outside of the scope of this study, but they could be an avenue through which advocates can continue to expand advocacy opportunities through external initiation while also, in the future, paying more attention to the shaping of those advocacy opportunities through lobbying.

The ***Activities*** row highlights four main categories of advocate work: reviewing grant applications, supporting grant applications, providing advice on clinical research, and providing advice on basic research. As discussed in the Introduction, previous researchers have proposed models depicting patient advocacy activities across the research spectrum, from making funding decisions to supporting study design and execution. However, the broader body of work from the UK did not describe patient advocates as involved in reviewing or supporting specific grants; instead, their involvement in funding was at a larger priority-setting level (Entwistle, Calnan, & Dieppe, 2008). Instead, these roles were only mentioned in the US; for instance, the study of the initial DOD review process (Andejeski, Bisceglio, et al., 2002). The frequent involvement of study participants on grant review boards and supporting grants suggests that the common activities of patient advocates in the US research process significantly differ from the activities of advocates in other countries.

Outputs and Outcomes

In contrast to the top two rows, the four bottom rows are not based on participant interviews. Instead, they capture the current uncertainty about the ***Outputs*** of the activities and suggest proposed short-term outcomes, medium-term outcomes, and long-term outcomes. Typically in a logic model, each activity corresponds to at least one unique and measurable output. The lack of clarity around what outputs are valid and measurable indicators of the impact of advocate activity has contributed to the lack of evaluation research in the area.

Two suggested ***Short-term outcomes*** for advocate activities include directing and supporting research funding in issue areas that are important to patients, with the ***Medium-term outcome*** of increasing funding for research with greater patient impact. Other proposed ***short-term outcomes*** include the potential applicability of clinical research to patients and increasing the acceptability of research design to patients, both of which would contribute to the ***medium-term outcome*** of improving and increasing patient participation in research. The final proposed outcome, increasing potential applicability of basic research to patients, along with both medium-term outcomes, contributes to the ***Long-term outcome*** of increasing the quality and impact of research on patient outcomes. All of this takes place in the ***Context*** of the broader research system, which includes opportunities for patient advocacy at the local and national level for advocates representing various cancer populations.

Several participants also mentioned the larger systemic issues hindering research and believed advocates could play an important role in helping to address those issues. However, there were less clarity around what activities this might involve. As a result, this area of potential impact was not included in the proposed logic model, but future discussions with advocates and other stakeholders could certainly inform edits to include this and other activities or issues in later versions of this model.

Strengths and Limitations

It is important to consider possible limitations of this study. Because of the small sample size and distinctive activities and interest areas of some of the participants, it was impossible to fully describe their work without including potentially identifying information. As a result, the sections of this thesis on advocate activities primarily discuss the activity areas described by multiple participants.

Use of a purposive and snowball sampling introduces the possibility of selection bias, potentially raising concerns about the internal validity of the study. Because all information was self-reported, there is also the possibility of recall bias. This sample also included a higher percentage of participants who were White (100%), female (92%), and had a postgraduate degree (46%) than the general population. This raises concerns about lack of representativeness. However, there is no demographic information available for the patient advocate population as a whole with which this sample can be compared. Participants mentioned there were few minorities among the advocate community. Additionally, data analysis was limited by having only one coder.

Most participants were involved in a wide range of patient support and advocacy activities, including lobbying for funding and key legislation on the Hill, leading support groups, participating in IRBs, and volunteering on helplines and hotlines to speak with patients. Due to time and space constraints, these were not explored during the interviews and mentions were not included in the results section. However, these activities are likely to provide important context for the work of patient advocates in research.

In addition to these limitations, this study had several important strengths. By taking advantage of her previously experience with the patient advocate community, the primary investigator was able to ask questions addressing issues that the advocates thought were relevant and important to their work. The participants were voluble, and the data gathered

was rich and insightful. Generally, participants were well-established in their roles and had many stories to share. Patient advocates proved to be interesting individuals to interview because they were so willing to share their stories, perhaps because they described often discussing their experiences and perspectives at scientific meetings.

Implications

This thesis has provided a thorough overview of the origins and context of patient advocacy that previously has been unavailable in the published research. This historical information is essential to understanding the roles patient advocates currently play. As discussed in the literature review, advocate lobbying led to the top-down initiation of many advocate opportunities. As seen in the results, however, because these opportunities were initiated from the top down, advocates had limited flexibility and responsiveness to new situations when defining their roles. This has important implications for our understanding of new and emerging opportunities for patient advocates, such as PCORI.

This study has highlighted the role and impact of patient advocates in bringing a patient perspective to the research system. In this study, the roles that advocates performed had the potential to impact research from all levels of the social ecological model of research. Advocates worked at the intrapersonal level directly with researchers to support grant applications and suggest ways in which basic and clinical research could be more patient-focused. They worked at the interpersonal level as part of research collaborations giving input on research, in addition to working with patients to identify relevant patient needs. At the community level, a significant amount of advocate involvement was seen working with funders to review grants, and at the public policy level, many participants reported lobbying the legislative branch to allocate more research funding. By developing an awareness of the roles that patient advocates currently play, this and further confirmatory research could be used to inform future evaluations, establish standards or best practices for patient advocates, and help

researchers be aware of the issues they should be paying attention to throughout the process. Considering these roles in the context of the proposed social ecological model for research, in addition to the proposed logic model, provides a roadmap for future work.

More immediate implications for advocates relate to training and organization. The results highlight the need for more clarity around what training should involve, whether it should be required for advocacy activities, and how much training might be too much. However, a better understanding of this landscape will be hard to develop without more formal research or a more formal organizational approach by advocates. Enhanced organization and communication could also aid advocates in better defining the various roles they play, as well as identifying common practices and ways in which their work could improve.

From a purely public health perspective, this thesis has implications for future efforts in community-based participatory research (CBPR). With CBPR, public health researchers have attempted to involve communities in directing and assisting with research. However, patient advocates demonstrate that in situations where community involvement is not feasible, involving individual representatives from communities can still help direct research in ways that are important to the community.

Recommendations for Future Research

This exploratory qualitative study has set the stage for several directions in future research. The historical overview in the literature review will aid in developing research questions tailored to the unique situation of the advocates. For instance, it seems essential to consider whether breast cancer advocacy differs from other areas of cancer advocacy in important ways that could impact advocate roles and impact. From this study, it was clear that breast cancer advocacy was more widespread and had more opportunities for advocates to engage from various patient positions.

One important direction for future research is a more comprehensive inventory of patient advocates and their roles. Due to the qualitative nature of this research and the small size of this sample, some roles and activities may have been missed. Furthermore, it was not possible to generate a more generalizable description of what patient advocacy might look like. An important element of this inventory would be the demographics of the patient advocate population. This sample suggested that patient advocates are not necessarily demographically representative of the larger cancer patient population. It is important to evaluate whether this is the case, and, if so, consider potential causes and remedies for this discrepancy so that all patient voices and experiences could more equitably be represented.

Additional area for research could be more exploratory qualitative research into the perspectives of researchers and funders on the impact of patient advocacy. Their thoughts could have important implications for future evaluation efforts. Ultimately, the goal for future research should be a thorough evaluation describing the scope and impact of patient advocacy generally, and more specifically the scope and impact of individual patient advocate activities. This will allow advocates to focus their efforts on the most effective activities.

Conclusions

The lack of research around patient advocacy is troubling. Advocate involvement in cancer research is already widespread. Recent governmental initiation of new programs in cancer and other disease areas suggests that advocacy efforts will only expand in the near future. This is particularly true in cancer, as an aging population will likely result in more cancer diagnoses, a larger patient population, and more patient concerns about the outcomes of cancer research. As advocates, researchers, and the research system invest time and resources in involving advocate perspectives, it is important to have a clearer understanding of the effects of that involvement. Furthermore, it is essential to better define what activities are most likely to have positive impacts so that advocate involvement can be as efficient and

effective as possible. While this study takes an important step forward by proposing a potential logic model for patient advocacy, additional work is necessary to define the outputs in this model, evaluate the outputs and outcomes, and identify best practices among advocates.

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