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Reliance on Advocacy is the Symptom not the Disease

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Introduction

In their article, “Rare Disease, Advocacy and Justice: Intersecting Disparities in Research and Clinical Care”, Halley and colleagues use three case examples to describe challenges patients with rare diseases face, which may lead to an ethical dilemma - reliance on advocacy to maximize their chances of survival through demanding quality care and seizing opportunity through funding and legislation. Unfortunately, advocacy has an equity problem due to long standing racial and socio-economic health disparities in the U.S. as the authors elucidate. Although Halley et al. are correct that “rare disease advocacy is not inherently problematic,” our reliance on advocacy indicates failures of the health care system. We must shift our gaze from the band-aid (reliance on advocacy) to the wound (health system failures) in order to improve equity for rare disease patients. Advocacy creates networks of power, influence, and capital to help patient communities interact with government funding agencies, industry, and health care system. When a reliance on advocacy has been identified, there is an opportunity to address systemic underutilization of long-standing community partnerships in formalized and equitable ways.

Vulnerability and Rareness

As Halley et al. (2023) note, rare diseases collectively impact nearly 25 million Americans, have suspected genetic etiology, and most emerge during childhood. Rare disease may be a primary cause of disability, death, and lower health-related quality of life. Families affected by rare diseases face compounding difficulties such as limited access to appropriate health care, uncoordinated care, and barriers to adequate insurance coverage. At the same time, challenges at the federal level leave rare disease communities to fend for themselves (self-advocacy) or rely on public advocacy (Halley 2021). An important question then is whether our systems are even designed to generate equity through the inclusion of smaller patient community priorities and needs within the larger population of patients and capital goals of the health care system. I suggest that this discussion of the reliance on advocacy can be reconstructed as a case example of the correlation between vulnerable social status and health outcomes.

Shi and Stevens (2005) define vulnerability as having greater risk for poor health status and health care access. Traditionally, vulnerable groups have been characterized by diseases (e.g., HIV), age groups (e.g., children), and demographics (e.g., rurality). Individuals within

rare disease communities lack adequate access to care and have few federal initiatives to reduce the barriers. The vulnerability of rare disease communities is specific and overlaps with additional aspects of an individual's lived experiences that contribute to their reliance on advocacy to obtain appropriate health care. Halley et al. (2023) argue that a downstream consequence of reliance on advocacy is inequity in research funding, published papers, and new drug approvals as witnessed between cystic fibrosis (CF, which predominantly affects white communities) and sickle cell disease (SCD, which predominantly affects Black communities). However, it is crucial to recognize that this particular ethical dilemma is borne of the compounding effect of the overlap between rare disease and racialization.

Identities Intersect and Disparities Compound

The three case examples in the target article describe specific challenges related to newborn screening, spinal muscular atrophy, and hypermobile Ehlers-Danlos Syndrome including, lack of representation of diverse patient voices within the broad ecosystem of screening, diagnosis, treatment, and research for rare diseases, public/private conflicts over medical costs and coverage, and information asymmetries between patients and providers where rare disease patients have been burdened with acquiring specialized knowledge about their condition and developing the necessary skills to communicate (and sometimes educate) their health care providers. The authors argue that the glaring example of inequity in outcomes from cystic fibrosis advocacy versus sickle cell disease advocacy showcases the problem of reliance on advocacy, which can “parallel well established racial disparities in the US” (Farooq et al. 2020). However, this phenomenon is not simply a parallel. Rather it reflects social hierarchies embedded in health systems and society as a whole. Crenshaw (1991) describes the creation of social location through intersecting identities, leading to experiences that qualitatively differ from those stemming from any singular identity. The negative experiences of racialized rare disease patients are therefore compounded at the intersection of racism and ableism. As a result, these patients are frequently marginalized within both the broader patient communities and society at-large. Viewing reliance on advocacy as a root cause of healthcare inequity can obscure the ways that this intersection of rare disease and race points to the need to account for multiple grounds of identity when considering how to address inequity in health care. If we instead reframe the differences between cystic fibrosis and sickle cell disease advocacy as an example of intersectionality, we find common cause and the possibility of shared strategic actions to address healthcare systems that are fundamentally characterized by a variety of structural inequities.

Democracy

Health care in the United States is not equally available to all individuals. National strategies to reduce health disparities have focused on increasing access to care and resources such as health insurance but vulnerable communities are at greater risk for delays in care or missing care altogether. The authors offer top-down approaches for stakeholder-specific mitigation strategies such as government agency review of rare disease funding allocations, industry duty, and reciprocity to patient communities through philanthropic investment and innovative payment arrangements. These strategies may have short-term benefits, but they run the risk of simply reifying the current vulnerable location of rare disease patients, which is at the behest of larger systems to act on their behalf. What is missing in these approaches

is meaningful partnership or building long-standing relationships between these larger organizations and the rare disease patient communities (Gerido et al. 2023). Such failures of government agencies, industry partners, and academic researchers to meaningfully engage communities—whether these are rare disease communities, minoritized communities, or other sociopolitically marginalized groups—has led to a reliance on advocacy as a necessary response to vulnerability and de-prioritization. Yet, the authors position the problem of inequity as a result of advocacy. Is not advocacy a tool and strategy to support patients to directly address the intersectional challenges of rare disease and equity? Although the United States recognizes no specific right to health care, the first amendment of the Constitution grants individuals the right of association: that is, individuals are allowed to come together to address issues of mutual interest. This right of association has provided communities an avenue to advocate for their healthcare needs, but the advocacy is ultimately not the source of the inequity; rather, it is an inadequate response to fundamental systemic inequities. When vulnerable communities—including rare disease communities—are engaged in ways that build upon their strengths, the outcomes are long lasting partnerships and fewer disparities.

Conclusion

The authors have argued that patient communities have a role to play in mitigating inequities but warn us of the ills of advocacy as the *de facto* system for access to research and clinical care. While they are entirely correct that advocacy reflects inherent system inequities, I argue that it is not the cause of inequity. Synergies and sustainable partnerships must be cultivated between stakeholder groups to successfully mitigate inequity.

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