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Lessons learned from founding a rare disease patient organization for hereditary angioedema in Japan: moving from a paternalistic to a power-sharing model of the physician-patient relationship

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Abstract

Objective: To describe our personal experience of the potential of a rare disease patient organization for fostering patient autonomy and creating a space for the practice of power sharing between physicians and patients.

Methods: Using self-reflection and personal autobiographical experience while drawing on the conceptual framework of patient-centredness, we critically reflect on and formulate lessons from our experiences as a patient/researcher and physician/researcher active in a rare disease patient organization for hereditary angioedema established in Japan in 2013.

Results: We identified multiple ways in which patient advocacy meetings shifted the patient-physician relationship to one of sharing power and responsibility. Appearing without his or her symbolic white coat, the physician is transformed into a person. In the context of shared group activities, the patient emerges as a person and one who is increasingly an informed and informing actor.

Conclusion: A dedicated rare disease patient organization has the potential to function as a catalyst for moving from a paternalistic to a power-sharing model of the patient-physician relationship. It can act as a transformative resource for all key actors.

Practice implications: The patient organization potentially reduces formal barriers and allows for the practice of effective patient-physician interactions, even in a cultural setting where paternalism generally shapes relationships. This can build social capital for both patient and physician.

Keywords

Empowerment, individualised treatment plans, patient advocacy organizations, patient-physician relationship, person-centered healthcare, rare diseases, shared decision-making

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Introduction

In advanced care settings, there has been a shift from a paternalistic model of patient-physician communication toward a pluralistic one which, according to Taylor (2009), 'attempts to democratize decision-making, share understanding and empower individuals' [1-3]. This shift is supported by evidence that sharing power and responsibility leads to increased satisfaction with the consultation process, improved disease outcomes and greater treatment adherence [4-7].

The value of a patient-centered approach has been recognized in Japan [8-14]. Nevertheless, in practice, the patient-physician relationship continues to be characterized by paternalistic and diminished patient autonomy [9]. Patients may resist attempts to establish a pluralistic model, expecting 'doctors not only to have professional competence, but also to accept responsibility for the patient's welfare' [10]. This places 'an unfair burden on doctors' [7]. Barriers to implementing a pluralistic model arise from the cultural context and specific institutional settings [8,9].

A paternalistic approach may be particularly problematic in the rare disease setting, limiting the capacity

of patient and physician to create the optimum environment to maximize healthcare outcomes [15,16]. Richards *et al.* have used a 'co-creating health' framework [17], indicating that patients and their organizations need clear views about their role in this process.

Rare disease patient organizations are active partners in some settings. They have worked with healthcare and medical research professionals to shape research *agenda* to develop new treatments and to enhance understanding of different conditions. The involvement of rare disease organizations is 'considered to be critical because patients and their carers possess different kinds of knowledge of their conditions from that of professionals' [18].

Hereditary Angioedema (HAE) is a rare and potentially life-threatening condition that affects approximately 1 person in 10,000 to 50,000 [19]. It is caused by C1-inhibitor deficiency or poor functioning. Diagnostic delay is a major issue for rare disease patients [15,19]. HAE patients in Japan experience 'an unacceptably long delay between onset of symptoms and accurate diagnosis and in the meantime patients suffer many inappropriate treatments' [21]. Current treatment practices in Japan do not sufficiently reflect international guideline consensus regarding on-demand treatment and home therapy, prophylactic care nor the creation of individualized management plans for patients [19,21,22]. Patients who lack reliable and speedy access to treatment are exposed to an elevated risk of life-threatening episodes [23,24]. To address some of these issues, we established a dedicated HAE patient organization in November 2013, which is now active nationally and internationally. We are two of ten founding members [25].

In this paper, we share our initial vision of the need to create a patient organization for HAE, our experiences in establishing and promoting this organization and our observations of the organization's transformative potential. We report the organization's practical achievements, such as the fostering of patient autonomy and the creation of spaces for the practice of power sharing between physicians and patients [19]. This has implications for relationships between patients and other actors involved in healthcare delivery [20]. Additionally, we present our perceptions of the need for this kind of patient organization in the Japanese rare disease environment and conceptualize how the organization was established and the key actors identified. Moreover, we share our observations of how involvement in the organization affects the roles and understanding of patients and physicians and distils basic principles from our experience.

Methods

Drawing on the conceptual framework of patient-centeredness, we critically reflect on and formulate lessons from our experiences as a patient/researcher and physician/researcher active in the rare disease patient organization for HAE. Taking a case study approach, one of us (Yamamoto) reflects on the organization from the position of patient/researcher. Yamamoto initiated the movement to found the organization and worked with

international partners in other national organizations and the umbrella international organization to understand the processes involved. Yamamoto has been the elected president since the organization was formed and has overseen its transition to a registered non-profit organization. She is also on the executive committee of the international umbrella organization. Kitano adds her insights from the position of physician/researcher. She is a practicing paediatrician working in the public health field. Her ongoing research relationship with Yamamoto brought her into the HAE patient organization as a founding member. Kitano has experience in a patient organization in another disease area.

We use this experiential basis to formulate lessons about the dynamics of patient-physician relationships in a Japanese patient advocacy space. The organization has achieved a degree of success in working on the basis of a pluralistic model. To clarify our observations, we reviewed the international scientific literature on patient empowerment, autonomy, patient education, rare disease patient organizations and patient-physician relationships.

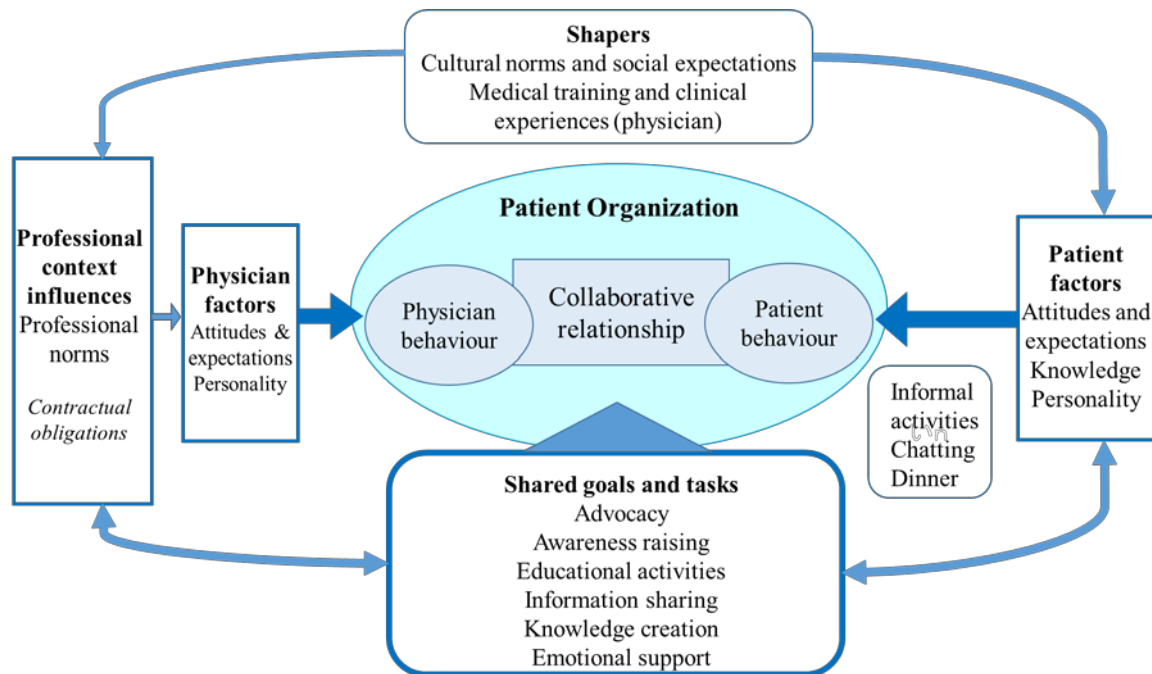
From these activities, we were able to identify the process by which patients and physicians in the organization came to work together on the basis of equal partnership. In our analysis, we use the conceptual framework of patient-centeredness developed by Mead and Bower [1]. No ethical approval was necessary for the current study.

Results

The organization and its achievements

Our case study rare disease patient organization comprises patients, caregivers and physicians as the core team working together to address challenges in the current context. Pharmaceutical companies involved in the development and delivery of HAE treatments are key supporters. Activities include awareness raising through media campaigns and YouTube video content [26]; inviting speakers (patients and physicians from Japan and overseas) to talk about HAE and its treatment; less formal information sharing (social networking tools enhance this role); knowledge creation (formal and informal) through Q&A sessions, newsletters, web content and advocacy to improve the treatment environment and emotional support. With product neutrality a non-negotiable condition, industry representatives have not only provided financial but also in-kind support to enhance the organization's reach through well-targeted media campaigns to raise awareness of the condition (particularly among general physicians) and to conduct research on patient-reported outcomes. Linking up with the international umbrella organization, we have also invited leading overseas physician/researchers and patient advocates to speak at patient-focused meetings and at research meetings aimed at treating physicians. For many physicians, it was novel to attend a research meeting convened by a patient organization.

Figure 1 The conceptual framework of patient-centredness based on Mead and Bower (2000). A power shift occurs in the patient-physician relationship through activities in a patient advocacy organization



(Based on Mead and Bower, 2000:1104)

The socializing impact of the organization

Our observations suggest that regular meetings provide an inclusive and welcoming space for patients, family members and friends, physicians (experts and local providers) and other healthcare providers (HCPs). Through planned activities, learning takes place between HCPs with different expertise levels, between patients with different expertise levels and between patients and HCPs. For patients, attending a meeting for the first time involves a risk of public disclosure of their condition. Many patients had never spoken to anyone else with the same condition outside their immediate family. Within the safe space created by the organization, patients and their families gain confidence and find new ways of framing their experiences. Many have shifted from hiding their condition to being willing to disclose to others that they have a hereditary disease, especially if this is to help improve the treatment environment or raise awareness to ensure early diagnosis. The YouTube video noted above evidences this very well [26].

Practice in the construction of equal partnership

Through the shared identification of priorities, agenda- and goal-setting and mutual decision-making concerning strategy implementation, key actors are provided with a

space to practice power and responsibility sharing. The patient organization provides a space for the development of relatively equal patient-physician and patient-patient relationships, something harder to do in a clinical setting.

Most importantly, we note that physicians are able to interact with patients *who are not their patients* in a setting where power dynamics are less rigid and more open to negotiation. The patient is able to interact with a *physician who is a person* unencumbered by his/her symbolic white coat [1]. At the same time, the patient interacts with peers living with the same condition, whether as a patient or caregiver. Meetings occur outside the consultation room and at a time when acute attack treatment is not needed and contractual obligations are muted. (Figure 1).

Participant-centric knowledge generation and accumulation

Our discussions evidence that HAE, as a broad spectrum disease, varies greatly in its symptoms, signs and treatment effectiveness, both between patients and across each patient's life-course. Therefore, to create optimal individualized management plans, both patients and physicians need to be considered experts in a relationship that effectively implements a benevolent and reflective practice of trial and error (see Figure 1).

As a rare disease, most treating physicians have contact with very few HAE patients in the hospital setting. These

encounters are often constrained, especially by time (see Figure 1). In the group meetings physicians have an opportunity to meet patients with a variety of symptoms and signs who may be responding to treatments in different ways. Physicians listen to patients talking about their understanding of HAE, their experience of living with it and their concerns and desires. At the same time, the patient hears the opinions not only of *a doctor who is not his or her treating doctor*, but also the opinions of other patients and caregivers. Through regular talks by physicians and patients, the shared body of knowledge grows.

Discussion

We have observed multiple benefits for key actors in the patient organization and the transformative potential of its activities. Feedback has come from both informal conversations and post-event evaluations. Here we discuss the benefits of the organization.

In terms of capacity building and advocacy, the organization provides a space in which participants increase their capacity as individuals and as a group working in the rare disease advocacy environment. There is shared identification of priorities, agenda- and goal-setting and implementation strategies that draw on actors' respective strengths [27]. Collaborative partnerships help to support goals that not only benefit the group, but also have an impact in the wider rare disease environment. Examples of collaborative activities include holding shared scientific meetings for the main actors, facilitating dialogue about current treatment guidelines to reflect a patient-centered approach and working toward the establishment of a patient-oriented registry to generate data [28,29].

When considering the rare disease patient organization as a catalyst for key actor interactions, we have illustrated the potential of one rare disease patient organization to create an enabling space in which shared power and responsibility among the main actors is both imagined and practiced. We have suggested that a patient organization can act as a catalyst for moving from a paternalistic to a pluralistic model of the patient-physician relationship. In such a setting, the patient gains autonomy.

There are several practical implications of our work. Although this case-oriented study has limitations (e.g., small sample size, narrative methodology), we suggest that the patient organization can provide a space for patients to interact with physicians in a less constrained way, especially important in a relatively paternalistic setting. This offers the possibility of creative interactions from which new insights are gained and taken-for-granted understandings can be questioned [27,30,31]. With a patient-centered structure, the organization becomes a hub for information for the main actors. It acts as a catalyst for key stakeholder interactions and brings about transformation.

Conclusion

A patient-centered organization can reduce formal barriers and permit effective patient-physician interactions, even in a paternalistic cultural setting. It can be viewed as a resource for generating new knowledge and social capital, which may translate directly or indirectly into improved patient quality of life and lower disease burden [31,32].

Acknowledgements and Conflicts of Interest

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