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Concept Elicitation Within Patient-Powered Research Networks: A Feasibility Study in Chronic Lymphocytic Leukemia

Kelly P. McCarrier, PhD, MPH[1],*, Scott Bull, PharmD[2], Sarah Fleming, MPH[2], Kristina Simacek, MA[3], Paul Wicks, PhD[3], David Cella, PhD[4], Renee Pierson, MBA[2]

[1]Health Research Associates, Inc., Seattle, WA; [2]Janssen Global Services, LLC, Raritan, NJ; [3]PatientsLikeMe Inc., Cambridge, MA; [4]Northwestern University, Chicago, IL

ABSTRACT

Objectives: To explore the feasibility of using social media–based patient networks to gather qualitative data on patient-reported outcome (PRO) concepts relevant to chronic lymphocytic leukemia (CLL). **Methods:** Between August and November 2013, US-residing members of the PatientsLikeMe online CLL patient community completed open-ended web-based surveys designed to elicit descriptions of CLL symptoms, impacts, and treatment-related perceptions. Qualitative telephone follow-up interviews were conducted with a subsample of respondents. Survey responses and interview transcripts were coded for qualitative analysis using Atlas.ti. **Results:** Fifty survey responses were included in the analyses. Participants were age 60.5 ± 6.9 years, 54% female, and 96% white. When surveyed, 20% were receiving current treatment, 16% were in remission, and 64% were treatment-naïve. Among respondents, 369 descriptions of CLL symptoms were coded. Fatigue-related symptoms were expressed most frequently, with 54% reporting "fatigue," "tiredness," or both in their responses. These concepts were followed by night sweats (38%), swollen lymph nodes (32%), and frequent infections (28%). Among impacts of CLL, worry and fear (66% of respondents), depressed feelings (52%), and work limitations (50%) were noted most frequently. **Conclusions:** Survey results identified constitutional symptoms of CLL included in existing PRO instruments and the literature. Although the findings suggest that qualitative data obtained through social media applications can be potentially useful in supporting concept identification for newly developed PRO instruments, they also indicate that online approaches alone may not be sufficient to achieve efficient and exhaustive concept elicitation. Further research is needed to identify whether the results can support content validity in the same way as established qualitative research methods.

Keywords: patient-reported outcomes, qualitative methods, social media, content validity.

Introduction

Recent developments have illustrated the great potential for use of online applications to facilitate interactions within patient communities and between patients and care providers [1]. Outside the health care setting, social media such as Facebook or Twitter have demonstrated advantages over traditional media in disseminating and collecting information directly from individuals, and comprise one of the fastest growing digital technologies in the world [2,3]. Patients with long-term conditions are increasingly searching and gathering online stakeholders to find information to quantify their disease progression and better manage their conditions [4].

Within the field of medical outcomes research, patient-powered research networks (PPRNs) are being evaluated as a potentially less burdensome source of biomedical data and may, thus, have a role in emerging methods for qualitative data collection [5,6]. Such communities vary in their strengths as sources of new data and in their potential to be validated against traditional medical data sources, as well as in terms of data quality, level of structure, and ability to support longitudinal data collection [7].

Recognizing the power of direct interaction among patients, companies such as PatientsLikeMe (PLM), and others have developed platforms that enable individuals to take an active role in managing their health by connecting to share experiences, give and receive support from one another, and compare treatment options, disease symptoms, and experiences with people who have similar health conditions [8–11]. This PPRN enables patients to connect and communicate using shared language and experiences that may not be apparent in a clinical encounter [12]. The features of the existing PLM platform and associated patient communities provide a fertile setting in which to explore potential alternative approaches to qualitative data collection to support the development of patient-reported outcome (PRO) instruments.

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* *Address correspondence to:* Kelly P. McCarrier, Health Research Associates, Inc., 6505 216th St. SW – Suite 105, Mountlake Terrace, WA 98043.

E-mail: mccarrier@hrainc.net.

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The use of qualitative data collected directly from an appropriate sample of patients is referred to as a best practice "essential for establishing the content validity of a PRO instrument" [13]. Individual interviews and focus group discussions are the most frequently used qualitative data collection methods for this type of concept elicitation (CE) process. Despite their benefits in terms of the depth of qualitative content, one perceived drawback of these approaches is the labor-intensive nature of qualitative data collection via interview methods [6]. This has led PRO developers to examine alternative methods of ascertaining information directly from patients about the disease-related concepts that are relevant and important to their experiences. Harnessing the potential of PPRNs as a forum for a CE process is one such approach, but its full benefits and challenges have yet to be thoroughly explored by PRO developers [6,14,15].

Among the expected challenges of online clinical research, the reliance on self-reported clinical data, particularly with regard to diagnostic and other clinical characteristics, is an acknowledged limitation of online social network–based approaches to PRO-related CE efforts [6]. When contrasted with the clinician-based patient screening and recruitment approaches typically employed with in-person concept elicitation, the use of self-reported data in online data collection potentially limits the degree to which researchers can accurately describe study samples. Although researchers have developed strategies to attain clinical confirmation of key clinical information gathered directly from patients via self-report, the willingness of patients to authorize such access to their medical information is currently unknown and thus warrants investigation.

Essential to understanding the full promise of these new approaches will be empirical tests of specific CE methods leveraging PPRNs. Although there have been recent examples of PRO development undertaken using online tools and PPRN-based approaches [16,17], we believe our study to be among the first to specifically examine this particular combination of qualitative methods in a way that allows comparisons to the sets of concepts elicited through previous condition-specific PRO development efforts.

In this paper, we describe the process and results of administering an online CE questionnaire coupled with telephone follow-up interviews to participating members of an established digital patient community for the purpose of identifying condition-specific symptoms, impact, and treatment-related concepts expressed as relevant and important to patients with chronic lymphocytic leukemia (CLL).

By conducting data collection in this way, we sought to evaluate whether there is reasonable proof-of-concept for the feasibility of this PPRN approach as a tool for qualitative concept elicitation. Specifically, we intended to examine four research questions:

- How does the set of identified symptom and impact concepts resulting from this CE process compare with those known in the literature and assessed in existing PRO instruments developed to measure the CLL experience?
- Is there evidence of saturation of concept for the data collected through open-ended PPRN questionnaire responses?
- Are additional concepts identifiable through qualitative follow-up interviews to supplement PPRN questionnaire data?
- What are study participants' attitudes toward providing researchers with access to their medical record information to confirm details of the clinical diagnosis of CLL and supplement patient-reported data from a PPRN?

Selection of CLL as a Case Study

Patients with CLL were considered a valuable test case for examining the usefulness of a PPRN to collect patient data for several reasons. First, since CLL is not a highly prevalent disease [18–20], collecting patient data from CLL patients online would illustrate the extent to which digital communities can be useful in recruiting relatively uncommon conditions and rare diseases. Second, the PLM platform is optimized for use by patients with chronic health conditions, as it allows patients to track symptoms, treatments, and side effects over time [8,9]. CLL, unlike tumor-based cancers, tends to resemble a chronic disease, in which patients must manage their condition and treatments over time [21,22]. Last, CLL provides an optimal case study to evaluate this novel CE approach because several disease-specific PRO instruments have already been developed for use in CLL [23,24]. The literature supporting these existing instruments provides evidence of the CLL-specific concepts identified via prior patient research, against which the findings of this study can be compared.

PRO instruments such as the FACT-Leu [23] and the EORTC Quality of Life Module for Chronic Lymphocytic Leukaemia [24] were designed with patient input specifically for use in CLL and have similar content. The importance of the concepts captured in these instruments is further supported by other instruments such as the Life Ingredient Profile for hematologic malignancies, which includes extensive physician input [25]. As such, comparing the breadth of patient-expressed concepts elicited in the current study with the collection of CLL-specific concepts known to prior researchers facilitates assessment of the feasibility of the PPRN approach for effective concept elicitation.

Methods

This mixed methods study employed a nonprobability convenience sample of adults with CLL to participate in a web-based survey and telephone interview, through which both quantitative (via closed-ended survey items) and qualitative (via open-ended survey items and verbal responses to the semistructured interview questions) data were collected. Eligibility criteria were designed to facilitate a sample of patients with CLL, which would be broadly reflective of the variations in patient, disease, and treatment characteristics present in target populations for CLL clinical trials in the United States. Subjects were eligible for inclusion in the study if they: 1) were 18 years of age or above, 2) were residents of the United States, 3) were members of the PLM CLL community, and 4) self-reported a diagnosis of CLL on the online questionnaire.

Prior to study data collection, the study protocol was reviewed and approved by Quorum Review IRB (Seattle, WA), and all participants provided informed consent. During registration with the system, all PLM users provided consent to be invited to participate in research. Following IRB approval of the study protocol, potentially eligible participants were identified by PLM from the CLL member pool. Beginning August 15, 2013, qualified patients were sent an initial contact message via PLM's internal private messaging system and invited to participate in the study. Those who did not respond to the message within 10 days of the initial invitation were sent a reminder message.

In addition to these approaches to existing PLM members, IRB-approved recruitment advertisements were placed on the Google and Facebook webpages. Additional invitations to new PLM members with CLL were sent every 4 to 6 days while the recruitment advertisements brought new members to the site.

All interested patients who responded to their invitations were asked to complete the online consent form via the internal PLM survey system, and once they consented, they were asked to complete the web-based study questionnaire (described below). Participants who completed the survey were compensated with a $45 Amazon gift card.