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Patient-Reported Outcomes

Development of a Symptom Index for Patients with Primary Brain Tumors

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ABSTRACT

Objectives: This study's primary goals included identifying the highest priority symptoms of patients with advanced brain tumors on treatment, comparing patient priority ratings with those of oncology experts, and constructing a brief symptom index using combined input to assess these symptoms and concerns. **Methods:** Fifty patients with advanced primary brain tumors and 10 physician experts were recruited from the National Comprehensive Cancer Network institutions and community support agencies. By using a 40-item symptom checklist, patients first selected up to 10 of the most important symptoms/concerns to monitor when assessing the value of drug treatment for brain tumors, then nominated up to 5 of the very most important concerns, and finally generated additional symptoms/concerns. By using the same checklist as patients, physicians rated each symptom/concern as disease- or treatment-related. **Results:** By using the combined input, a 24-item National Comprehensive Cancer Network/Functional Assessment of Cancer Therapy-Brain Symptom Index (NFBrSI-24) was developed. The NFBrSI-24 showed good internal consistency ($\alpha = 0.84$), significantly

differentiated patients with different levels of functional status ($F_{2,47} = 8.21$; $P < .001$), and demonstrated good convergent validity with the Functional Assessment of Cancer Therapy-General functional, physical, social, emotional, and brain tumor-specific concerns ($\rho = 0.59, 0.57, 0.40, 0.35, \text{ and } 0.50$, respectively; $P_s < 0.05$). **Conclusions:** The NFBrSI-24, an index of the symptoms in advanced brain tumors perceived as most important by both patients and clinicians, improves upon existing measures of brain tumor symptoms through better satisfaction of regulatory requirements for measure development. The findings suggest good reliability and validity, indicating that the NFBrSI-24 is a promising brief assessment of high-priority advanced brain tumor symptoms for research and clinical settings.

Keywords: health-related quality of life, NFBrSI-24, primary brain tumor, symptom index.

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Introduction

The incidence rate of primary malignant brain and central nervous system tumors for the years 2005 to 2009 was 6.5 cases per 100,000 and that for primary nonmalignant brain and central nervous system tumors was 13.5 cases per 100,000 [1]. Patients with brain tumors exhibit a wide range of symptoms depending on various factors such as tumor location, size, degree of edema, and histology. They can present with either acute or subacute neurologic symptoms or a more protracted course of worsening neurologic and cognitive symptoms [2]. The importance of symptom control in cancer, including brain tumors, has been widely recognized because of the extraordinarily high prevalence of physical and psychological symptoms as well as the effect of these symptoms on patients' health-related quality of life (HRQOL) [3–6]. For patients with advanced disease, in which life

expectancy is reduced and there is no cure, relief from symptoms and maintenance of function become primary objectives of medical intervention [7,8]. In evaluating the efficacy of new chemotherapeutic agents, symptom reduction or relief from treatment may be considered a meaningful outcome [9]. Even beyond their association with HRQOL, symptoms can predict disease progression [2,10]. Quinten et al. [10] reviewed 30 randomized controlled trials, including brain tumor trials, from the European Organization for Research and Treatment of Cancer between 1986 and 2004, which included symptom and function assessment. They found that these assessments provided significant prognostic value in addition to the sociodemographic and clinical variables typically examined in relation to survival.

The quality of symptom reports is highly dependent on the instruments and methods (e.g., clinician- vs. patient-report) used to acquire the data. Most recently validated measures of cancer-

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<http://dx.doi.org/10.1016/j.jval.2013.11.006>

specific HRQOL incorporate an assessment of certain prevalent symptoms, such as pain and fatigue, within a broader multidimensional HRQOL assessment [4,11–13]. Disease-specific measures offer the advantages of being more likely to be sensitive to the effect of specific tumor types and associated treatments [14], underscoring the importance of developing tumor-specific symptom lists, as opposed to generic symptom lists, to assess drug efficacy across the broad spectrum of tumors. Only a few existing symptom instruments are specific to brain tumors (e.g., Functional Assessment of Cancer Therapy–Brain Tumor [FACT-Br] [15], M.D. Anderson Symptom Inventory for Brain Tumor [16], and European Organization for Research and Treatment of Cancer Brain Cancer Module [17]), and the item stems and rating scales vary widely even between these measures, highlighting the need for improved measures of symptoms specific to advanced brain tumors.

Two prerequisites to constructing symptom lists for evaluating response to chemotherapy in advanced cancer are as follows: 1) information from patients on their perceptions of the most important symptoms related to treatment and 2) input from clinicians on the prevalence, relative importance, and likely attribution (disease-related or treatment-related) of the array of symptoms and concerns associated with brain tumors. We have completed important groundwork toward fulfilling these prerequisites. Before the work reported here, we completed semi-structured interviews with patients and providers to gather input about brain tumor–specific concerns, resulting in the development of the 40-item FACT-Br, a measure of brain tumor–specific HRQOL [15]. Next, we completed a comprehensive survey of physician and nurse experts at 17 National Comprehensive Cancer Network (NCCN) member institutions, which provided detailed information on the priority symptoms endorsed by oncology experts for nine tumor sites, including brain tumors [18]. In this previous work, we demonstrated that experts in the management of brain tumors hold similar views about the symptoms that are most important to monitor when treating patients and that are applicable to the assessment of drug efficacy. Furthermore, we have shown that almost all the symptoms identified by experts as the most important to assess in treating patients with brain tumors can be derived from a well-established multidimensional HRQOL questionnaire, Functional Assessment of Chronic Illness Therapy (FACIT) measurement system [13]. Patient ratings of symptom relevance and severity of symptoms and concerns, however, especially in the psychological realm, may differ from those of providers [19–22]. The Food and Drug Administration (FDA) presents a similar observation and guidance [23], that definitions of meaningful symptom outcomes should include patient-derived definitions including cognitive interviewing and carefully designed patient-reported outcome measures. When designed in accordance to the FDA guidance, such measures are more likely to be compelling end points eligible for use in registration trials presented to the FDA, and studies seeking to satisfy regulatory requirements for a standardized tool to evaluate drug efficacy with respect to symptomatology [24]. In response, the primary goals of this study were to identify the highest priority symptoms of patients with advanced brain tumors, to compare patients' priority ratings with those of oncology experts, and to construct a brief symptom index using the combined input (i.e., physicians, nurses, and patients) to assess these symptoms and concerns.

Methods

Sample

Patient eligibility and recruitment

Patients were eligible for the study if they were at least 18 years old and had a diagnosis of a grade III or IV brain tumor.

Additional inclusion criteria were experience with chemotherapy for at least two cycles (1 month for noncyclical chemotherapy); no other primary malignancy diagnosed and/or treated within the previous 5 years except nonmelanoma skin cancer; able to understand and provide signed informed consent; sufficient cognitive ability to complete questionnaires without assistance as judged by clinicians and research staff; and fluency (reading and speaking) in English. Patients were recruited from a subset of NCCN member institutions and community support agencies. The NCCN is a not-for-profit tax-exempt corporation that is an alliance of National Cancer Institute–designated comprehensive cancer centers. Patients were also recruited through members of the Cancer Health Alliance of Metropolitan Chicago, a coalition of four community support agencies serving the Chicago metropolitan area. These organizations were selected to provide patient input from community-based practices to balance the preferences of patients from tertiary referral centers, such as NCCN sites.

Physician eligibility and recruitment

Physicians were recruited by e-mail sent to all NCCN member institutions by NCCN headquarters staff. Physicians were eligible to complete the disease-related/treatment-related survey if they were in practice at any of the NCCN institutions and had at least 3 years' experience treating a minimum of 100 patients with advanced brain tumors. Physician input was solicited to determine which symptoms they considered to be disease related versus treatment related.

Procedures

Patient survey

To avoid influencing patients' thinking with any existing questionnaire content, they were first asked in an open-ended interview to "Think of the full range of your experience receiving drug treatment for your illness. Please tell me what you think are the most important symptoms or concerns to monitor when assessing the value of drug treatment for your illness." Patients were then asked, "Please tell me on a scale of 0–10 (with 0 being not important and 10 being extremely important) how important each symptom or concern is to you."

Patients were then asked to complete a 40-item symptom/concern checklist, which included items from FACT-Br [15,25], NCCN Symptom Index for Brain Tumor [18], and FACT-General [12,26]. Patients were first asked to select up to 10 symptoms or concerns on the checklist that they felt were "the most important symptoms or concerns to monitor when assessing the value of drug treatment for brain tumor." Of those symptoms/concerns nominated as most important, patients were then asked to select up to five as "the very most important." Space was provided for respondents to write in symptoms or concerns that were not already listed. Four versions of each checklist were created to control for response bias due to order effect. This symptom checklist was identical to that administered to NCCN physicians and nurses in a previous study [18].

Patients then completed FACT-Br, which consists of 27 items from FACT-General, assessing physical well-being, functional well-being, social/family well-being, and emotional well-being, plus the brain tumor–specific subscale. These responses were used in conducting the preliminary analyses of reliability and validity described in the subsequent sections.

Physician survey

Physicians completed an online survey administered by Survey Monkey. This survey, consisting of symptoms/concerns included in the patient Checklist Coversheet, asked respondents to rate each brain tumor symptom/concern on a five-point scale as to

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