SCIENCE BRIEF

The importance of patient-reported outcome data in oncology

Clinical benefit and patient preference

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In recent years, regulatory agencies such as the FDA and EMA have increasingly promoted the use of patient-reported outcomes (PRO) data in the development and approval of cancer products. While the standard primary endpoint for oncology trials is focused on survival and overall response, more recently, regulatory agencies are also placing weight on the importance of PRO-based secondary endpoints that are indicative of clinical benefit in terms of patient symptoms and overall quality of life. In addition, elevating PRO data within the endpoint hierarchy and including PRO-based label claims is a mechanism to help differentiate a cancer product. This review focuses on the new findings on patient preferences in oncology trials, and the clinical benefits observed for the use of PROs in patients with cancer.

Clinical benefit

Clinical research by Basch et al. has found that health-related quality of life (HRQL) and overall survival are improved in oncology patients completing electronic PRO (ePRO).[1] In one study, 766 cancer patients initiating chemotherapy were randomized to usual care or web-based symptom reporting (12 common symptoms from the NCI-CTCAE). A computer-inexperienced group completed the ePRO at clinic visits, whereas the computer experienced group completed the ePRO at home with weekly email reminders. An email alert was triggered to a nurse when symptoms worsened ≥ 2 or reached absolute level ≥ 3. A report tracking symptoms was provided to clinicians at each visit. HRQL was measured via the EQ-5D at 6 months and overall survival was assessed in June 2016 (median follow-up period of 7 years). HRQL was improved more in the ePRO group than the usual care group (34% vs. 18%) and worsened in fewer (38% vs. 53%) (P <0.001). Fewer ePRO subjects visited ER vs. usual care at 1 yr. (34% vs. 41%; P=0.02). Overall survival was improved in the ePRO group (31.2 months in the ePRO group vs. 26.0 months in the usual care group) (difference, 5 months; P=0.03). A similar study by Denis and Basch et al. was published in 2019 and found similar findings in lung cancer patients with overall survival of 22.5 months in the ePRO group vs. 14.9 months in the usual care group.[2] The authors concluded that symptom self-reporting engages patients and is associated with clinical benefit.

Patient preference

Several studies have found that oncology patients find electronic handheld diaries and tablets easy to use, helpful, and preferable over paper, thus alleviating any concern as to whether patient burden, health status, or age impact the feasibility of using ePRO with oncology patients.

- Oncology patients think that electronic symptom reporting positively impacts their clinical care. **87%** of patients think that symptom assessments are important to complete because they help the clinical team know their symptom severity. **79%** agreed that the clinical team takes the symptom scores into account in deciding treatment.[3]
- Metastatic breast cancer patients reported that tablets were easy to read **(94%)** and navigate **(99%)**, helped them to remember and report symptoms **(74%)** and that they would recommend to other patients **(94%)**.[4]
- Non-small cell lung cancer patients preferred electronic versions **(60%)** of the EQ-5D and Functional Assessment of Cancer Therapy-Lung Cancer (FACT-L) over paper formats **(12%)**.[5]

- Another study with cancer patients found that **52%** preferred touch screen to paper, **24%** had no preference, and **24%** preferred paper. There were no missed responses with the electronic version, whereas paper contained over 1000 missing or unusable responses.[6]

Furthermore, Clario clinical research finds that cancer patients believe it is important to report on their symptoms and prefer to report them electronically. To identify preferences for the use of PROs in cancer patients, 185 people with cancer responded to an online survey. The respondents were asked about their preferences on methods and frequency of reporting daily symptoms. Respondents were also asked about their perspectives regarding the importance of reporting symptoms.

More than 90% of the cancer patient respondents thought that it is at least "Somewhat" important to report daily changes in their cancer-related symptoms and 74% reported that it is "Quite a bit" and "Very much" important to report such daily symptoms changes (Figure 1).

If you were participating in a clinical trial for oncology, would you feel there is a benefit to reporting changes in your oncology related symptoms, such as pain and nausea, to the study doctor on a daily basis?

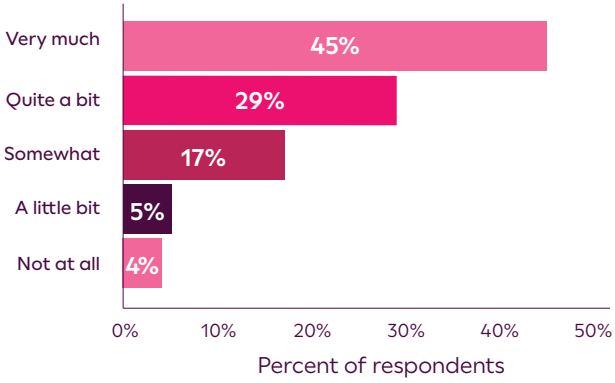

Figure 1. More than 90% of cancer patients feel that it is at least "Somewhat" important to report daily changes in their cancer-related symptoms

Of the respondents, 63% indicated they would prefer to report changes in their daily symptoms in an electronic diary, 18% preferred to report directly to their doctor at study visits, while 14% felt they would prefer a paper diary and 5% would not want to report their symptoms at all (Figure 2).

If you were participating in a clinical trial for oncology, how would you prefer to report your daily changes in symptoms?

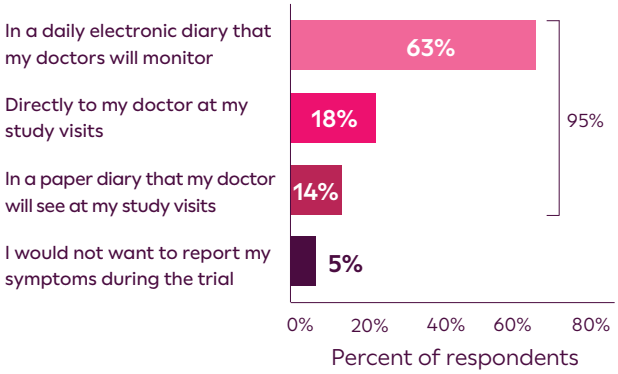

Figure 2: 95% of cancer patients want to report daily changes in symptoms while participating in a clinical trial

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Overall, 95% of patients want to report daily changes in symptoms while participating in a clinical trial (Figure 2). Most (88%) would prefer to complete their study questionnaires at home vs. at a clinic (Figure 3).

If you were participating in a clinical trial for cancer, where would you prefer to complete the study specific questionnaires?

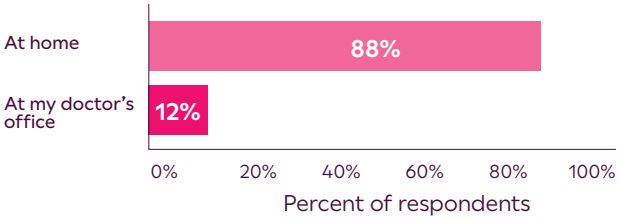

Figure 3: Almost 90% of cancer patients would prefer completing study-specific questionnaires at home, while participating in a clinical trial

Taken together, regulatory agencies are placing more focus on secondary outcome PRO data in addition to survival and overall response when evaluating the clinical benefit of oncology products.[7-12] Missing data and infrequent collection should be avoided when collecting symptom data, and electronic data capture can help achieve this goal. The use of ePRO has even been shown to improve HRQL and overall survival in oncology patients. Oncology patients prefer electronic data capture and demonstrate a willingness and ability to consistently complete questionnaires for the duration of their participation in a clinical trial.

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