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Willingness of older adults to participate in a randomized trial of conservative therapies for knee pain: A prospective preference assessment

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ABSTRACT

Background: In preparation for a trial of physical therapy (PT) for patients with degenerative meniscal tear and knee osteoarthritis, we conducted a prospective preference assessment – a methodology for estimating the proportion of eligible subjects who would participate in a hypothetical randomized trial.

Methods: We identified patients seeking care from the practices of five orthopedic surgeons. Patients completed a survey asking about their willingness to participate in a hypothetical trial, their treatment preferences, their knee pain, and demographic variables.

Results: We approached 201 eligible patients, of whom 67% (95% confidence interval [CI] 60%, 73%) completed questionnaires. Of these, 24% (95% CI 17%, 31%) were definitely and 39% (95% CI 31%, 47%) were probably willing to participate in the trial. Thirty-three percent (95% CI 23%, 43%) of subjects with no treatment preference were definitely willing to participate as compared to 9% (95% CI 1%, 17%) with treatment preference (p = .001). Patients with higher educational attainment also stated a greater willingness to participate than those with less education (p = .06). In multivariable logistic regression analysis, those with no treatment preferences had greater adjusted odds of stating they would definitely participate than those with a defined treatment preference (OR 5.2, 95% CI 1.7, 16.2), while subjects with an associate's degree or greater were more likely to state they would definitely participate than those with less education (OR 3.9, 95% CI 1.1, 14.1).

Conclusion: In this prospective preference assessment, 63% (95% CI 55%, 71%) of subjects with degenerative meniscal tear expressed willingness to participate in a trial of PT modalities. Individuals with no treatment preferences were more likely to state they would participate than were those with higher education. This methodology can help investigators estimate recruitment rates, anticipate generalizability of the trial sample and create strategies to facilitate enrollment.

1. Introduction

Clinical trials are essential for determining treatment efficacy, and the randomized controlled trial (RCT) is generally considered the gold standard for trial design. While RCTs provide high quality evidence [1], appropriate planning of enrollment strategies can be challenging, and slow enrollment can compromise a trial's timely completion [2,3]. Halpern (2002) suggests that researchers employ a pre-enrollment "prospective preference assessment" (PPA) to predict the number of patients that will need to be approached to reach enrollment goals and understand the characteristics of subjects interested in participating in the study [4]. With greater understanding of factors that impel patients to participate, investigators can devise strategies for timely enrollment of a sufficient and representative sample.

RCTs are especially useful when the results have the potential to influence clinical practice. Degenerative meniscal tear in the presence of knee osteoarthritis (OA) is a widespread and painful condition. Symptomatic knee OA affects over 15 million Americans [5]. Damage

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to the menisci is present in 70–90% of persons with symptomatic and radiographic OA, and clinicians frequently attribute symptoms of knee pain to degenerative meniscal damage [6]. In the past decade, five RCTs have compared physical therapy (PT) alone to arthroscopic partial meniscectomy followed by PT for participants with degenerative meniscal tear. Four of the trials found similar levels of pain relief and functional improvement in both arms [7–11]. These findings suggest that PT should be the first line of treatment for patients with degenerative meniscal tear and/or knee OA [12,13]. However, no current trial has rigorously studied whether outpatient PT is superior to exercises performed at home or whether the effect of PT is due, in part, to a placebo effect.

In preparation for a randomized control trial (RCT) comparing a home exercise program, in-person physical therapy (PT), and in-person topical treatments (that offer a placebo effect) for persons with degenerative meniscal tear, we conducted a PPA study. Our goals were to estimate the proportion of potentially eligible subjects who would be willing to participate in the trial and to identify factors associated with willingness to participate. Identification of demographic features of those who are and are not interested in participating would help investigators understand the generalizability of their sample and might be helpful in developing recruitment strategies to attract persons who are more reluctant to participate. Below, we describe the results of a PPA conducted in anticipation of this RCT comparing home exercise, PT, and a placebo PT intervention for degenerative meniscal tear in the presence of OA.

2. Methods

2.1. Sample

We recruited study participants from the outpatient practices of five orthopedic surgeons at a tertiary academic center in Boston, MA. Inclusion criteria consisted of knee pain for at least two weeks, age greater than 40 years, and English-speaking. Exclusion criteria consisted of evidence of bone-on-bone knee OA, inflammatory arthritis, locked knee, prior surgery on their index knee, attending more than four sessions of PT in the preceding year, dementia, residing in a nursing home, and current pregnancy.

Each week, a research assistant (RA) reviewed the clinic schedules of participating surgeons and assessed consecutive patient records using clinical schedule information and data from the electronic medical record. For every patient who met inclusion criteria and had no exclusions, the RA completed a screening form in a secure online database. In the clinic, the RA approached all study subjects deemed eligible after the screening process, reviewed their eligibility, and asked if they would be willing to complete a survey indicating their interest in a hypothetical trial. The RA described the trial as a hypothetical randomized study investigating different non-operative treatments for patients with knee pain, including: (1) PT with a trained physical therapist, (2) topical creams applied to the knee, and (3) education/instruction for therapeutic exercises to perform at home. Study materials the RA presented to patients were written in a manner that was thought to be appropriate for a participant with an 8th grade reading level. Training on how to present these materials and explain complex health topics with patients was minimal. Participants completing the survey were provided with a $10 gift card. We also asked each subject's treating physician to complete a form noting the participant's suspected diagnosis. We describe participants who completed a survey on their willingness to participate in the lager, hypothetical trial as “enrolled” in this pilot study report.

2.2. Data elements

Participants rated their willingness to participate in a hypothetical trial of non-operative physical therapy modalities using a five point Likert scale: definitely yes, probably yes, not sure, probably no, definitely no. Participants completed questionnaire items including information on whether or not they had a preferred intervention amongst the three intervention options offered:1) PT in clinic with a professional therapist, 2) topical creams applied to the knee, and 3) a home exercise program. We also evaluated patient willingness to undergo an MRI scan in Boston if it were required to participate in the trial. Other assessments included the Western Ontario and McMaster Universities Osteoarthritis Index (WOMAC) Pain scale [14] (0 best, 100 worst), Knee injury and Osteoarthritis Outcomes Score (KOOS) Symptoms scale (0 worst, 100 best), and questions on demographics [15]. For this analysis, we specified race as white vs. non-white; ethnicity as Hispanic vs. non-Hispanic; and educational attainment as associate's degree or greater vs. some college, technical school training, or high school graduation.

2.3. Statistical analysis

We evaluated the association between participant willingness to enroll and demographics, clinical characteristics, and treatment preference. Willingness was categorized as “Willing” (definitely yes), “Probably” (probably yes) and “Unwilling” (not sure, probably no, definitely no). We summarized categorical variables as proportions and compared them across groups using a chi-squared test or the Freeman-Halton test, an extension of Fisher's exact test, where appropriate [16]. Continuous variables were presented as means or medians based on normality and compared using the Kruskal-Wallis test. Given that willingness to participate is ordinal, it was also assessed using the Cochran-Mantel-Haenszel statistic for categorical variables and the Jonckheere-Terpstra test for continuous variables [17]. We used logistic regression to evaluate the independent association between definite willingness to participate and baseline demographic and clinical characteristics. We considered in the regression model variables from the univariate analysis that were associated with willingness at a p-value criterion of ≤ 0.15. Candidate variables were eliminated individually using the Akaike information criterion (AIC). The Tables and Figures include the number of responses for each question. Notably, not every participant who completed a survey answered every survey query. Therefore, the total number of responses for each question varies for each variable. Missing data was minimal (less than 4%). We conducted all analysis using SAS version 9.4 (SAS Institute, Cary, NC).

3. Results

3.1. Enrollment and features of sample

Over seven months, we screened 705 patients with knee pain, of whom 134 (19%) ultimately enrolled in the pilot study (Fig. 1). We found 345 of the 705 to be ineligible during initial screening, most commonly due to previous knee surgery or prior attendance at more than four sessions of PT in the past year. Of the remaining 360, 159 were not approached in clinic due to scheduling conflict for the RA, observable exclusion (e.g. translator present, visibly pregnant), or failure to come to clinic. The RA spoke with the remaining 201 individuals; of these, 36 were found to be ineligible and 20 were not interested in completing the questionnaire. Of the 145 patients who agreed to be surveyed, 134 (92%) people completed the survey. Of the 134 responders, physician diagnoses were obtained for 114 (85%) participants (see Fig. 2).

Overall, respondents were predominantly female (66%) and white (82%), and most (77%) had at least an associate's degree. The average age was 63 years (standard deviation [SD] 11) and median body mass index (BMI) was 27.3 kg/m^2 (25th, 75th percentile 27.3, 31.8). The median KOOS Symptoms score was 50 (25th, 75th percentiles 40, 60), and the median WOMAC Pain was 40 (25th, 75th percentile 25, 60). Approximately 65% of subjects reported they had no preference