Patients' shifting goals for deep brain stimulation and informed consent

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Abstract

Objective

To determine using a repeated-measures, prospective design whether deep brain stimulation (DBS) results in changes in the importance of symptom and behavioral goals individually identified by patients with Parkinson disease (PD) before DBS surgery.

Methods

Fifty-two participants recruited from a consecutive series completed a semistructured interview soliciting their rank-ordered symptom and behavioral goals and corresponding visual analog scales measuring perceived symptom severity and limits to goal attainment. Rank orders were reassessed at 2 times after DBS. Changes in rank order over time were examined with χ^2 analyses. The relationships between change in symptom severity/limits to behavioral goal attainment and change in rank order were examined with mixed-effects linear regression models.

Results

Most participants changed the rank order of their symptom (81%) and behavioral (77%) goals 3 months after DBS surgery. Change in rank order of symptom goals was significantly related to change in severity ratings such that improvements in self-reported symptom severity were associated with reductions in rank. In contrast, no such relationship was evident for the behavioral goals.

Conclusion

These data illustrate how patients' primary goals for DBS shift early in stimulation and highlight the important differences between symptom and behavioral goals. Changes in the rank order of symptom goals were related to changes in symptom severity, whereas subtler shifts in behavioral goals were unrelated to improvements after DBS. This observation suggests that DBS does not affect goals that may be more reflective of core personal values. The findings provide empiric data that can help improve the informed consent process.

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Glossary

ADL = activities of daily living; DBS = deep brain stimulation; VAS = visual analog scale.

Systematic investigation of patients' goals and expectations is particularly important in the context of a neurosurgical intervention such as deep brain stimulation (DBS), in which the primary motivation is to improve the patient's quality of life.¹ We previously demonstrated that the majority of patients' preoperative severity ratings for their individually defined goals for DBS were not significantly correlated with standard research clinical outcome measures.² This observation suggests that the most commonly used measures to assess benefits associated with DBS for the treatment of PD may not fully capture the goals most important to patients. This discrepancy may contribute to miscommunication between the patient and DBS team concerning expectations. Another potential source of miscommunication is that patients' primary goals for DBS change over time.

We hypothesized that patients' rank order of symptom and behavioral goals would shift over the course of DBS such that there would be a devaluation of attained goals and an increased valuation of unattained goals. To test this hypothesis, we investigated the rank-ordered goals that patients with PD articulated as most important in their decision to pursue DBS over time.

Methods

Standard protocol approvals, registrations, and patient consents

The current study was part of the larger study discussed above.² The study was approved by our Institutional Review Board. All participants provided their fully informed written consent. The study was funded by the National Institute of Neurologic Disorders and Stroke (award RC1NS068086).

Participants

The published literature documents large effect sizes associated with DBS in the treatment of motor symptoms of PD. We adopted a conservative approach to calculate power and assumed a moderate effect size (0.50) and a very conservative test-retest reliability coefficient of 0.30 for our severity rating. On the basis of these assumptions, a sample size of 50 will result in power of 0.91 for a repeated-measures design that incorporates 3 assessments.

The majority ($n = 52$, 88%) of a consecutive series of 59 patients scheduled to undergo DBS for the treatment of PD agreed to participate in a study examining patients' goals for DBS. Seven chose not to participate for the following reasons: perceived burden of study demands ($n = 1$), frail health ($n = 1$), scheduling conflicts ($n = 2$), and no response ($n = 3$). Inclusion in the study was limited to patients who were >18

years of age, were native English speakers, and had not undergone a previous neurosurgical procedure for PD. No patients were excluded on the basis of the above inclusion criteria. Per standard clinical practice at our institution, all patients were approved for DBS surgery on the basis of a multidisciplinary team assessment comprising neurologic, neurosurgical, neuropsychological, and psychiatric (if needed) evaluations, as well as explicit discussions about patients' goals for surgery.³ All patients were recruited from a single, large academic medical center from July 2009 through June 2011.

Measures

The study used a mixed methodology. All participants completed a semistructured interview before surgery probing their expectations regarding DBS with respect to symptom and behavioral goals. Participants were asked to provide at a minimum their top 3 (with a maximum of 6) symptom and behavioral goals. The participants rank ordered their symptom and behavioral goals separately. Imbedded within the structured interview were visual analog scales (VASs). Participants used the VAS to indicate the current severity of each identified symptom goal, with 10 representing the greatest severity. The behavioral goals were measured in a similar way, with 10 representing the greatest limit to participation. Thus, the VAS provided a means of quantifying the efficacy of DBS in addressing participants' individually defined goals on a standard metric. The research protocol, including the semistructured interview and the patient-rated VAS, was completed before surgery and at 3 and 6 months after DBS surgery. Finally, all participants were asked at the 6-month assessment to indicate if, given their experiences with DBS thus far, they would choose to undergo surgery again. This question provides an indirect measure of satisfaction with DBS. Responses were coded as yes, no, or uncertain. The data were collected by research personnel who were not directly involved in the participants' clinical care. During the postoperative interviews, the participants' original symptom and behavioral goals were presented in alphabetical order, and they were asked to rank their goals. All interviews were audiotaped and transcribed.

A modified grounded theory approach was used to identify symptom and behavioral goal categories. Grounded theory is an inductive methodology in which the raw data are approached with no or minimal preconceived hypotheses. Through a process of careful and repeated review of the data, themes are identified and eventually categories or codes within the raw data are identified. Our approach represents a modified grounded theory in that input from a highly experienced multidisciplinary DBS team was used to propose preliminary symptom and behavioral goal categories. After

data collection, these categories were evaluated and revised on the basis of participants' verbatim responses and naturally occurring sets. Final categories were established that were based on consensus discussion using an inductive, recursive process common in qualitative research.⁴

In addition to the research measures, all participants completed standard clinical research outcome measures, including the Parkinson's Disease Quality of Life scale⁵ and the Unified Parkinson's Disease Rating Scale-II and -III subtests.⁶

Analyses

Changes in the distribution and rank order of participants' goals for DBS were assessed with χ^2 analyses and descriptive statistics. Analyses were limited to the top 3 ranks because all participants were required to rank order a minimum of 3 symptom and behavioral goals. The relationships between changes in rank order and changes in symptom and behavioral goal severity ratings were examined with mixed-effects linear regression models. Change in rank order was coded for every goal with the use of a 4-point ordinal scale with a total range of -3 to +3. The potential ordinal values differed depending on the initial rank. For example, if a participant ranked a specific goal first, there were only 4 changes in ranking possible: no change (value = 0), drop to second rank (value = -1), drop to third rank (value = -2), and drop to less than third rank for those participants who provided >3 goals (value = -3). With the same logic, the corresponding ordinal options to illustrate change in the second-ranked goals were as follows: +1 = move up to first rank, 0 = no change, -1 = drop to third, and -2 = less than the third rank. Finally, the values for the initially third-ranked goals were the following: +2 = move to first rank, +1 = move to second rank, 0 = no change, and -1 = less than the third rank. Change in severity ratings (or limits to participation for the behavioral goals) based on the VAS was computed with a simple change score: severity rating at baseline minus severity rating at postoperative month 3. Consequently, higher scores indicated greater benefit.

Data availability

Anonymized data relevant to this study will be shared by request with any qualified investigator pending appropriate Institutional Review Board approvals.

Results

Participant characteristics

Fifty-two participants completed the baseline assessments. Data were available for 47 of the participants at month 3 and 45 at month 6 (3 participants withdrew for personal reasons, and the remaining 4 did not complete the study because they did not have surgery within the study time frame). Most of our participants were men ($n = 39$, 75%) with an average age of 61.3 years (SD 9.3 years) and average time of PD of 9.1 years (SD 4.1 years). All were white and non-Hispanic/Latino. The subthalamic nucleus was the surgical target in all but one of

the participants. Outcomes after DBS based on the Unified Parkinson's Disease Rating Scale, Parkinson's Disease Quality of Life-39, and participants' individually defined goals were reported in our previous report, and significant benefit was observed on all measures.

Baseline symptom and behavioral goals

Participants' symptom goals sorted into 7 categories: tremor, gait, rigidity, dyskinesias, other medication side effects, non-motor, and other motor. The other medication side effects code included any physical symptom that participants directly attributed to their medications (e.g., drowsiness), recognizing that some of the symptoms that participants attributed to their medications may in fact have been symptoms of PD or other medical disorders. Nonmotor symptoms included cognitive, psychiatric, sleep, fatigue, and pain symptoms not attributed to medication side effects. Other motor symptoms represented a mix of participants' motor symptom concerns not included in the other categories.

Six behavioral goal categories were established that were based on analysis of the participants' verbatim responses: social, avocational pursuits, activities of daily living (ADL), work, driving, and other. The social category consisted of goals with a clear social component. Avocational pursuits included all hobbies, exercise, and sports-related activities. Specific behavioral activities important for basic daily activities such as drinking, dressing, and eating made up the ADL category. Paid employment, volunteer work, and household chores were included in the work category. Finally, the other category included a combination of activities most often related to medication use (e.g., costs, frequent dosing). Relatively vague responses such as "improve my quality of life" were also included in the other category.

The relative proportions of participants who ranked various symptom and behavioral goal categories were examined with χ^2 analyses. There were significant differences for all 3 of the symptom goals, indicating that not all symptom goal categories were equally ranked before surgery (symptom 1 $\chi^2 [6, n = 52] = 48.96, p < 0.01$; symptom 2 $\chi^2 [6, n = 52] = 19.07, p < 0.01$; symptom 3, $\chi^2 [6, n = 51] = 17.22, p < 0.01$) (figure 1). Control of tremor or improved fine motor control was the top-ranked symptom goal and the most frequently cited goal overall (27.4%). Improvements in gait (20.4%) and nonmotor symptoms (20.4%) were the next most often cited symptom goals. There were no significant differences in the number of participants who chose the various behavioral goals for activity 1 ($\chi^2 [5, n = 52] = 8.23, p = 0.14$) or activity 2 ($\chi^2 [5, n = 52] = 8.69, p = 0.12$) before DBS, whereas significant differences were evident for activity 3 ($\chi^2 [5, n = 51] = 22.06, p < 0.01$) (figure 2). Involvement in social activities was the top-ranked behavioral goal for our participants, followed by avocational pursuits and ADLs before DBS. In the aggregate across all 3 behavioral goals, avocational pursuits are a primary factor in participants' decisions to seek out DBS, followed by work-

Figure 1 Percentage of symptom goals before and after DBS by rank

related factors and the importance of interpersonal relationships and social activities (i.e., social).

Effect of DBS on participants' goals and willingness to have DBS

As we reported previously,⁷ detailed examination of the effect of DBS surgery on different participant-identified symptom goal categories revealed evidence of greater improvements in tremor, gait, dyskinesias, medication side effects, nonmotor, and other motor symptoms after DBS surgery vs the much smaller improvements evident in rigidity from the participants' perspective (time $\times$ symptom category interaction $F_{12,41} = 2.78$, $p = 0.007$). There were no significant differences in the magnitude of improvements in participants' behavioral goal severity ratings across specific activity categories (time $\times$ behavioral category interaction $F_{10,41} = 0.33$, $p = 0.966$), suggesting that similar improvements were evident in all behavioral goal categories over time. Absolute mean changes on the VAS for the different symptom and behavioral goal

categories from baseline to postoperative month 3 did not differ for the symptom (mean = 3.33, range 0.8–5) and behavioral (mean = 3.68, range = 1.3–5.4) goals.

Forty of the 42 participants (95%) who were asked if they would undergo DBS again, in light of their experiences with DBS at the 6-month postoperative point, indicated “yes.” Two responded “uncertain.” None responded “no.”

Changes in rank order of goals after DBS surgery

The majority of participants changed the rank order of their top 3 symptom (81%) and behavioral (77%) goals at 3 months after surgery. In contrast, relatively few participants changed their rank order of symptom and behavioral goals from month 3 to 6 (9% and 16% changed their rank order, respectively). Given the relative stability of participants' rank orders from month 3 to 6, analyses focused on comparisons between the baseline and month 3 rank orders.

Figure 2 Percentage of activity goals before and after DBS by rank

A series of χ^2 analyses were conducted to see whether the proportions of symptom and behavioral goal categories ranked first, second, and third changed significantly over time. All χ^2 analyses were significant (χ^2 range 43.25–101.29, $p \leq 0.013$) for the first- and second-ranked symptom and behavioral goals categories when rank orders at baseline and postoperative 3 months were compared. The proportions of various goal categories for the third rank did not differ significantly for either symptom ($\chi^2 [36, n = 46] = 47.19, p = 0.10$) or behavioral ($\chi^2 [25, n = 46] = 30.43, p = 0.208$) goals when pre-DBS and post-DBS rankings (figures 1 and 2) were compared.

To facilitate comparisons across time, the following summary statistics are based on the 47 participants who completed the baseline and month 3 assessments. Before DBS, tremor reduction (28.6%) was the most common symptom goal across all 3 ranks, followed by improvements in nonmotor (20.7%) and gait (19.3%) symptoms. Three months after DBS surgery, the most common symptom goals were nonmotor (27%) symptoms, followed by gait (24.8%) and tremor (22%) symptoms. Figure 1 illustrates the number of participants who ranked the various symptom goals first, second, and third at baseline and month 3. Almost half (46.8%) of the 47 participants indicated that tremor control was their top-ranked symptom at baseline vs 27.7% at month 3. In contrast, the percentage of participants who ranked nonmotor symptoms as their first goal quadrupled over time (baseline 6.4%, 3 months 25.5%). The corresponding data for the second-ranked goal indicate a decline in other motor symptom goals (baseline 12.8%, 3 months 6.4%) and an increase in symptom goals related to medication side effects (baseline 2.1%, 3 months 10.6%) over time.

Less dramatic shifts were evident in the behavioral goal categories. Participation in hobbies/leisure pursuits continued to be the most common behavioral goal across all 3 ranks after DBS (before DBS 29.8%, 3 months 32.6%), with mild drops over time in the proportion of participants who cited social activities (before DBS 16.3%, 3 months 12.7%) among their top 3 goals. Figure 2 compares the number of participants who ranked the various behavioral goals first, second, and third at baseline and month 3. Review of the top-ranked behavioral goal indicates that social pursuits (25.5%) at baseline were most desired and that all categories were relatively equally represented at month 3. More subtle shifts were evident in a comparison of the participants' responses for the second- and third-ranked behavioral goals over time (figure 2).

The results of the mixed model linear regression across all 3 rank orders revealed that changes in symptom severity ratings per the VAS were significantly related to changes in symptom rank ($F_{1,138} = 4.97, p = 0.027$) such that as the symptom severity rating improved, the ranking declined. In contrast, there were no significant relationships between change in behavioral goal VAS ratings and change in rank order ($F_{1,138} = 0.0, p > 0.05$).

Discussion

Our findings document changes in the rank order of patient's symptom and behavioral goals early in DBS. For example, the percentage of patients who indicated that treatment of tremor was the most important symptom goal before surgery dropped from 46.8% to 27.7% at postoperative month 3. Conversely, the number of patients who indicated that nonmotor symptoms were among their top-ranked goals increased. Subtler shifts also were evident in the behavioral goal categories, with greater priority placed on avocational over social goals over time. It is possible that changes in rank order may reflect relative satisfaction with DBS such that those who were less satisfied were more likely to change their rank order. Unfortunately, we did not directly assess overall satisfaction with stimulation, although we indirectly measured participants' interest in having DBS again, given the benefit of hindsight. The overwhelming percentage of participants (95%) indicated that they would choose to undergo DBS again. The lack of variability in participants' responses limits our ability to directly assess the relationship between satisfaction with DBS and changes in the rank order of goals. Future studies should include measures of satisfaction that have a greater range of potential responses to address this question.

The findings indicate that patients' goals for DBS are more complex than reduction in tremor and incorporate subjective motivations related to interpersonal relationships, work, mastery, and living a meaningful life (cf., Maslow⁸). We and others argue that it is the consequences of poor motor function such as limitations on daily activities, negative effects on personal relationships, interference with hobbies, or loss of independence, rather than motor impairment per se, that are the primary drivers of patients' motivation to seek out DBS.^{7,9}

Consistent with our hypothesis, improvement in self-perceived symptom severity was related to shifts in symptom goal rank order after DBS. Once the patient's primary goal for DBS was addressed, other symptom goals reflecting ongoing disease burden rose to the top. The initially lower-ranked symptom goals may be harder to address with DBS, or it may be that the treatment team is unaware that the goals have shifted after DBS surgery, resulting in miscommunication between the team and the patient concerning treatment expectations.

Our hypothesis was not supported when we examined the behavioral goal data: patients' self-perceived improvements in their ability to engage in valued activities were unrelated to changes in their rank order of behavioral goals. This suggests that the behavioral goals may reflect more enduring, core personal values and goals that persist regardless of improvements in the patients' ability to participate. Furthermore, these findings support the distinction between symptom and behavioral goals.

These findings raise interesting ethical questions with respect to the informed consent process and the extent to which, or if,

the DBS team should assume responsibility for addressing patients' changing goals or ongoing disease burden over the course of DBS. This question is particularly relevant given the shift in health care from solely addressing disease symptoms to maximizing an individual's health and well-being.¹⁰ DBS entails a long-term commitment to caring for patients due to the need for regular programming and maintenance of implanted devices. The ongoing reliance on the team (and device), coupled with media portrayals of DBS as "miraculous,"^{11,12} may contribute to ambiguity regarding what the team and DBS can accomplish over time. This is complicated even further in the context of a neurodegenerative disease. This backdrop highlights the need during the informed consent process not only to address the primary symptoms present at the time of surgery but also to extend the consent process to include discussion about the potential benefits and limits of DBS in addressing symptoms that may persist or arise over time.

These findings emphasize the need for ongoing dialogs about goals and motivations for DBS treatment beyond the initial informed consent discussion. Our findings support expanding the informed consent discussion from the traditional risks and benefits associated with the surgical procedure to an explicit discussion with the patient and family about symptom and higher level behavioral goals.⁷ The process of this dialog may lead to a better understanding of patients' expectations and provide opportunities to clarify potential benefits and limits of DBS therapy at the time of surgery and over the course of the disease. Our data suggest that the consent process should include language stating that as DBS successfully addresses a patient's primary symptom goal, the patient's focus may shift to other symptoms reflecting ongoing disease burden for which DBS may or may not be as effective. Conversely, DBS is unlikely to change what matters in their life, or their core values, as reflected in their behavioral goals (i.e., personal relationships, hobbies, work). We argue that such an ongoing dialog with careful attention to symptom and behavioral goals (or patients' values) is important in the context of a treatment such as DBS that relies on a team effort over the course of years.

It is important to note that our findings may not generalize to other DBS teams. In rare cases, patients at our center may not move forward with surgery if their goals are unrealistic¹³; consequently, it is possible that, because of our presurgical evaluation process, our patients may differ in meaningful ways from patients at other centers. In addition, this was an open study. It is possible that participation in a study designed to address patients' goals for DBS (which implies the team's appreciation of and commitment to patients' goals and values) may have resulted in a positive response bias. These are empiric questions and require further study. Finally, data collection should ideally extend beyond 6 months after surgery in future studies to more fully understand and appreciate whether and how patients' goals continue to evolve over the course of their illness and to

incorporate measures of patient satisfaction that provide a range of responses.

Despite these limitations, this is the first study specifically designed to qualitatively and quantitatively assess changes in patients' goals over the course of DBS. Our findings illustrate the important and materially significant differences between patients' symptom goals and more complex behavioral goals for DBS. Although many patients' rank-ordered goals for DBS change early in treatment, changes in self-perceived symptom severity affected only rankings in symptom goals. The subtler shifts in behavioral goal rank orders were unrelated to perceived efficacy of DBS, suggesting that DBS does not alter goals that may be more reflective of core personal values. These empiric data provide insight into patients' values and evolving expectations that can improve the consent process and contribute to ongoing patient care in a manner that is respectful of the patient and the treatment team.

Author contributions

Cynthia S. Kubu: literature search, hypothesis generation, study design, data collection, data analyses, data interpretation, statistical analyses, writing, figures. Thomas Frazier: data analyses, data interpretation, statistical analysis, writing, figures. Scott E. Cooper: hypothesis generation, study design, data interpretation, writing, figures. Andre Machado and Jerrold Vitek: hypothesis generation, study design, data interpretation, writing. Paul Ford: literature search, hypothesis generation, study design, data collection, data interpretation, writing.

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