PCORI 90056532

Measuring the Priorities of Patients With Type II Diabetes Using Likert Scale and Best-worst Scaling

NCT02637609

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JHSPH IRB Research Plan for New Data Collection

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Study Title: Advancing stated-preference methods for measuring the preferences of patients with type 2 diabetes.
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I. Aims of the Study: Describe the aims/objectives of the research and/or the project's research questions or hypotheses.

"Advancing stated-preference methods for measuring the preferences of patients with type 2 diabetes" focuses on advancing methods for patient and community engagement in patient-centered outcomes research (PCOR) and has three objectives. First, we will demonstrate good practices for patient and community involvement in PCOR projects by applying principles of community-based participatory research (CBPR) to engage an advisory board, the Diabetes Action Board (DAB), comprised of community- and national-level stakeholders. Second, we will address methodological gaps pertaining to the use of stated-preference methods in PCOR. These include identifying the best methods for identifying patient priorities (Aim 1), the best method for designing a preference study (Aim 2), and comparing stratification versus segmentation strategies for analyzing preference heterogeneity (Aim 3). We also seek to assess the relevance of stated preference methods to patients and stakeholders using both qualitative and quantitative methods (Aim 4). Third, we will demonstrate good practices for applying stated-preference methods by studying the priorities and preferences of patients with type 2 diabetes. This will provide an important case study for future researcher wanting to apply these methods in PCOR.

Research can be segmented into three phases:

Phase 1: Qualitative interviews and focus groups with community members (n=50) to develop the survey, which will be used in part 2 of the project.
Phase 2: Local (n=10) and national (n=50) pilot surveys, implemented in the first year of the project. The pilot surveys will guide the national survey (n=1000), implemented during the second year of the project.
Phase 3: Local pilot of a follow-up survey (n=20) to assess research subjects' attitudes towards the importance and validity of survey results and thereafter a national follow-up survey (n=300). Part 3 will be implemented in the third year of the project.

While the work in parts 2 & 3 will take place on both local and national levels, the general survey design and implementation methods will be similar. The main difference is the mode of survey delivery (local surveys will be primarily paper surveys, while national surveys will be electronic surveys) and appearance (GfK's KnowledgePanel's® online survey will have a slightly different format than the surveys used with the local community).

This application will describe only the work to be done in Phase 1. Amendments will be submitted for Phases 2 and 3 as preparations for them are developed.

II. Background and Rationale: Explain why this study is being done. Summarize briefly what is already known about the issue and reference previously published research, if relevant.

This project aims to address gaps in the understanding and applications of methods to conduct patient-centered outcomes research (PCOR) and is focused on advancing methods for engaging patients and community stakeholders in PCOR. Despite the clear need to engage patients and stakeholders as part of any PCOR research, current PCORI methodology guidelines give little detail on how this should be done.

Based on current international best practices, there are two main strategies that can be applied to identify and measure the values of patients and stakeholders (Facey et al, 2010). First, patients and stakeholders can be actively engaged through consultation or included in decision-making panels (Barham, 2011; Vogt et al, 2006). Second, qualitative and quantitative research methods can be used to scientifically study what factors patients and stakeholders value the most (Rotter et al, 2012; Bridges 2006; Bridges & Jones, 2007; Lee et al, 2008). Such methods are often referred to as stated-preference methods as they aim to document the priorities and preferences of patients and stakeholders (Bridges, 2003).

Stated-preference methods can be qualitative, using interviews, focus groups, direct observation or meta-synthesis methods to document priorities and preferences (Kinter et al, 2009; Bridges, Gallego & Blauvelt 2011; Hansen et al, 2011). Stated-preference methods can also incorporate quantitative survey approaches including rating and ranking (Phillips et al, 2002; Bridges, Lataille et al. 2012), willingness-to-pay (Johnson, Manjunath et al, 2006), conjoint analysis (Casciano et al, 2011), and best-worst scaling (Louviere & Flynn, 2010). Of these, choice-based conjoint analysis (also known as discrete-choice experiment) has become the most common (Ryan & Gerard, 2003; Bridges et al, 2008; Marshall et al 2010), and clear guidelines have emerged for utilizing them (Bridges, Hauber et al, 2011; Viney et al, 2002; Lancsar & Louviere, 2008).

While the active engagement of patients and stakeholders and the application of stated-preference methods to measure the values of patients and stakeholders are both important (Facey et al, 2010) and complementary (Bridges, 2003), stated-preference methods have several important advantages. First, stated-preference methods can incorporate both qualitative and quantitative data on priorities and preferences. Second, stated-preference methods can include large, diverse, and often hard to reach patients and stakeholders. Third, the validity, reliability and generalizability of the priorities and preferences of patients and stakeholders can be evaluated statistically. Fourth, the preferences of different sub-groups of patients and stakeholders can be identified and compared using stratified analysis. Fifth, stated-preference methods can be used to identify groups/clusters of individuals with similar preferences and descriptive statistics can be used to describe the membership of these groups.

III. Study Design

A. Provide an overview of your study design and methods.

Phase 1 of the study will be guided by the Diabetes Action Board ("DAB") and will follow community based participatory research principles. The DAB is comprised of 22 individuals that are experts in type 2 diabetes research, community outreach and stated-preference methods. The objective is to solicit patient priorities and

preferences topics from these focus groups. Information gathered will guide the development of the pretest instruments. Phase 1 will involve focus groups held with people with diabetes 2. These sessions will be audio-recorded and the recording will be destroyed after transcription. If a participant does not wish to be recorded they will not participate in the focus group.

Pilot surveys in *phase 2* of the study will involve a preliminary administration of the survey instrument to participants with type 2 diabetes. With permission of the participants, these preliminary sessions will be audio-recorded (after transcription, the recording will be destroyed). The objective is to solicit feedback on the level of understanding of the purpose of the instrument and its structure as well as the accessibility of the language used. This feedback will then be used to revise and finalize the survey instrument, which will be administered by GfK's KnowledgePanel®. Four types of conjoint analysis surveys will be utilized.

Pilot surveys in *phase 3* of the study will again solicit feedback on the level of understanding of the purpose of the follow-up study and it's structure and language. The results from these pilot surveys will inform the surveys distributed by GfK's KnowledgePanel®, which aim to engage participants to evaluate the relevance of stated-preference methods.

This application will describe only the work to be done in Phase 1. Amendments will be submitted for Phases 2 and 3 as preparations for them are developed.

B. Provide a sample size and a justification as to how you arrived at that number. If you use screening procedures to arrive at a final sample a table may be helpful.

	Local		National		Total
Sample type	Qualitative work (focus groups)	Pilot Survey	Pilot Survey	Survey	
Phase 1	50	0	0	0	50
Phase 2	0	10	50*	1000**	1060
Phase 3	0	20	0	300	320
					1430

*The national pilot survey includes subsets of African American participants (n=10) and Latino participants (n=10).
**The national survey includes subsets of African American participants (n=200) and Latino participants (n=200).

In phase 1, we will seek to develop the instruments with 50 participants. Given the qualitative nature of the study, the sample size will be guided by the expected number of persons needed to reach saturation of feedback necessary to develop survey instruments that measure patient priorities and preferences.

IV. Participants

A. Inclusion criteria: Adults (at least 18 years old) with type 2 diabetes.

B. Exclusion criteria: Individuals younger than 18 years old and individuals without a type 2 diabetes diagnosis.

V. Study Procedures

In this section, provide details of your procedures, particularly as they relate to human subjects. If this is a multi-center study, make the role of JHSPH clear. If the JHSPH will serve as **data coordinating center**, indicate in the sections below which procedures JHSPH will not be performing. Additional information regarding data coordinating centers is requested in a later section. If your study will develop in phases, address each item below by phase.

A. Recruitment Process:

1. Describe how you will identify, approach, and inform potential participants about your study. Include details about who will perform these activities and what their qualifications are.

   Participants for phase 1 will be drawn from individuals known to the investigators and the DAB and, using a snowball approach, persons they nominate as knowledgeable about type 2 diabetes. Recruitment will be conducted by e-mail or telephone, depending on the information available. DAB members and their associates who contact focus group participants have experience in community outreach efforts. Further, they also have knowledge of type 2 diabetes, connections with type 2 diabetes support systems and knowledge of this project. Recruitment flyers will be posted in appropriate community settings (no clinical settings will be used) (A recruitment flyer and phone/email script is attached.)

2. Address any privacy issues associated with recruitment. If recruitment itself may put potential participants at risk (if study topic is sensitive, or study population may be stigmatized), explain how you will minimize these risks.

   We do not anticipate any privacy issues during any phases of the study. During phase 1, focus group participants will by identified only by their first names or by special identifiers (e.g. numbers).

B. Consent Process:

1. Describe the following details about obtaining informed consent from study participants. If a screening process precedes study enrollment, also describe the consent for screening.
   - Who will obtain informed consent, and their qualifications
     - During phase 1 of the study, study investigators, serving as interviewers, will obtain informed consent. These interviewers will have completed the Human Research Curriculum offered by the Collaborative Institutional Training Initiative (CITI).
   - How, where, and when the consent discussion(s) will occur
     - Consent discussions will occur at the beginning of focus group meetings. The consent discussion will occur in person at the site of the focus group.
   - The process you will use to determine whether a potential participant meets eligibility criteria
     - Participants will self identify as adults with type 2 diabetes.
   - Whether you will obtain a signature from the participant or will use an oral consent process
     - This study, and more specifically phase 1, will use an oral consent process. We request a waiver of signature as that would be the only identifier linking individuals with the research.
   - Whether you will obtain a legally authorized representative's signature for adults lacking capacity
     - We do not anticipate research with any adult participants lacking capacity.

- If children are included in the study, if and how you will obtain assent from them
    - No children will be included in the study.
- If children are included in the study, how you will obtain permission for them to participate from their parent, legal guardian, or other legal authority (if child is in foster care or under government supervision.)
    - No children will be included in the study.
- If you are seeking a waiver of informed consent or assent, the justification for this request
    - NA
- Whether you will include a witness to the consent process and why
    - The moderator will ask participants for oral consent. The note taker will be present to witness this oral consent as a safeguard that oral consent was obtained.
- If the language is unwritten, explain how you will communicate accurate information to potential participants and whether you will use props or audio materials.
    - No props or audio materials will be used. The qualitative protocol (focus group guiding material) will communicate accurate information to participants.

2. Identify the countries where the research will take place, and the languages that will be used for the consent process.

Country	Consent Document(s) (adult consent, parental permission, youth assent, etc.)	Languages
USA	Adult oral consent, qualitative protocol, contact information for principal investigator.	English

C. Study Implementation:

Answer the following:

1. Describe the procedures that participants will undergo. If complex, insert a table below to help the reviewer navigate.

   During phase 1, semi-structured interviews in the form of focus groups will be conducted in person. Focus groups will take place at JHSPH and appropriate locations in the community as identified by stakeholders. The interview will be audio recorded and will be attended by 2 study staff, the interviewer and a note-taker. When they arrive, each participant will be handed an information sheet that they can read before the start of the focus group and can take home. The interviewer will begin the session by explaining the study and by obtaining oral consent. They will then proceed by asking about the informants' backgrounds and experiences in the treatment of type 2 diabetes and the priorities of participants with type 2 diabetes (see Qualitative Protocol and Information sheet).

2. Describe the number and type of study visits and/or contacts between the study team and the participant, how long they will last, and where/how they will take place.

During phase 1, participants will only be contacted once and these contacts will occur in person. We expect focus group meetings to last between 1 – 1.5 hours.

3. Describe the expected duration of the study from the perspective of the individual participant and duration overall.

   The study duration is 3 years. Phase 1 will be completed between 9/1/2014 and 12/31/2014. From the perspective of individual participants the duration of phase 1, in the form of focus group meetings and individual interviews, will last between 1 – 1.5 hours.

4. Provide a brief data analysis plan and a description of variables to be derived.

   Phase 1 will identify the priorities of patients with type 2 diabetes. This component will gather basic baseline information about demographic (age, gender) and disease characteristics (diagnosis of T2D given by a physician, time since diagnosis, severity of disease symptoms). Information gathered in phase 1 of the study will guide the formation of a national survey regarding the preferences and priorities of patients with type 2 diabetes.

5. Describe whether you are collecting or storing personal identifiers, and if yes, why you need them, and when and how you plan to dispose of them. Signatures on consent forms are considered to be identifiers.

   Personal identifiers (in the form of first name and contact information) will be stored in order to schedule focus group meetings. These identifiers will not be used during the focus group, will not be used in any data collection, and will be destroyed immediately after the focus group.

6. Answer the following **if they are relevant to your study design**:

   a. If the study has different arms, explain the process for assigning participants (intervention/control, case/control), including the sequence and timing of the assignment.
   b. If human biospecimens (blood, urine, saliva, etc.) will be collected, provide details about who will collect the specimen, the volume (ml) and frequency of collection, how the specimen will be used, stored, identified, and disposed of when the study is over. If specimens will be collected for use in future research (beyond this study), complete the Biospecimen Repository section below.
   c. If genetic/genomic analyses are planned, address whether the data will be contributed to a GWAS or other large dataset. Address returning unanticipated incidental genetic findings to study participants.
   d. If clinical or laboratory work will be performed at JHU/JHH, provide the JH Biosafety Registration Number.
   e. If you will perform investigational or standard diagnostic laboratory tests using human samples or data, clarify whether the tests are validated and/or the lab is certified (for example is CLIA certified in the U.S.). Explain the failure rate and under what circumstances you will repeat a test. For all human testing (biomedical, psychological, educational, etc.), clarify your plans for reporting test results to participants and/or to their families or clinicians. Address returning unanticipated incidental findings to study participants.
   f. If your study involves medical, pharmaceutical or other therapeutic intervention, provide the following information:
      - Will the study staff be blind to participant intervention status?
      - Will participants receive standard care or have current therapy stopped?
      - Will you use a placebo or non-treatment group, and is that justifiable?

- Explain when you may remove a participant from the study.
- What happens to participants on study intervention when the study ends?
- Describe the process for referring participants to care outside the study, if needed.

VI. Data Custody, Security, and Confidentiality Protections

The sections below describe types of data sources and how they will be protected. For the type(s) of data you will have, put an "X" in the appropriate box to the left of the section that best describes how you will minimize the risk of a breach of confidentiality for your study. Note, as appropriate, how you will record/store data. These descriptions represent MINIMAL measures; you may add more stringent protections and other relevant information in B.

Confidentiality: The *LOSS OR THEFT* of 1) original/duplicate version of physical data collection instruments (forms, tapes, etc) or 2) physical devices containing electronic data (i.e. laptop/mobile device, external flash drive(s), is a threat to subject confidentiality. Risk of such a loss/theft is increased during movement/transport of data (in any format), such as in a vehicle or other move. Be sure to train anyone (co-investigators, staff, students, etc.) who might be engaged in the oversight of data handling/storage about this problem. Some typical risk-mitigation strategies would include:

- **minimizing the physical movement of data and/or devices containing data**
- **encrypting electronic data (especially when stored on any mobile device, including flash memory tools, phones, tablets, etc, or when transferring across networks)**
- **making use of reliable courier services (FedEx, DHL, etc) when physical transport of bulk data forms is necessary**
- **minimizing the transfer of identifiable data in physical or electronic form (i.e. removing/separating/destroying identifiable data, when physical transfer of data is necessary)**

A. Data Storage

1. Hard Copies of Data Collection Forms.	
X	This activity will not involve receiving and/or accessing hard copies of data
	Data collection forms RECORD NO PERSONAL IDENTIFIERS connecting study participants, and there are no codes providing a link. Data are anonymous.
	Data collection forms INCLUDE IDENTIFIERS. The forms are locked in a secure cabinet or room with limited access by authorized individuals. Forms will be kept in study team's possession during transport and will not be left unattended in a vehicle. When possible, de-identified copies will be used for coding and analysis.
	Data collection forms ARE CODED with study participants' random study ID numbers. Codes/links between study IDs and identifiers are stored securely in a separate place (locked storage cabinet or secure electronic database.)
	Other:

2. Electronic Data	
X	The data do not contain personally identifiable information
	These data are stored on a secure server protected by limited access and strong password systems. Data are coded when possible. Portable electronic devices will not contain identifiable information unless encrypted.
	Other:
3. Other Identifiable Data Storage, Retention, and Destruction (Audiotapes, videotapes, photographs, etc.) will be retained and stored securely (locked in cabinet or room) until:	
X	Transcription is complete, then will be destroyed.
	Analysis is complete, then will be destroyed.
	Study is complete and file is closed.
	Indefinitely. Provide justification for indefinite retention:
4. Existing Biospecimens to be used in this study:	
	HAVE NO PERSONAL IDENTIFIERS.
	INCLUDE IDENTIFIERS AND ARE CODED; the PI will not have access to the link or code connecting the identifiers to the specimens.
	INCLUDE IDENTIFIERS, and the PI has access to those identifiers or to the link/code connecting specimens to individuals. The identifiers and/or code will be stored securely until the study is complete.

B. Certificate of Confidentiality

Will the study data stored in the United States be protected by a Certificate of Confidentiality? If yes, explain who will apply for and maintain the Certificate. (http://grants.nih.gov/grants/policy/coc/appl_extramural.htm)

We will not apply for a Certificate of Confidentiality.

C. Data Security and Sharing

PIs have the responsibility for responsible stewardship of data and protecting data confidentiality. This responsibility includes protecting physical custody of the data, storage and sharing with appropriate data use agreements that contain the appropriate security provisions. Describe any additional plans beyond those identified in the table that you have for storing and sharing the study data and/or materials, and how responsibility for the data will be managed. Include the following details:

1. Where will the study data be stored?

   In the office of the principal investigator, Dr. John F.P. Bridges.

2. Who controls access to the data?

   The principal investigator, Dr. John F.P. Bridges.

3. Will data be shared only if de-identified?

   Yes.

4. What additional (if any) security controls will be in place?

   All data will be anonymous. Additionally, data will be stored on password protected computers with access restricted to study investigators.

VII. Risks of the Study

A. Describe the risks, discomforts, and inconveniences associated with the study and its procedures, including physical, psychological, emotional, social, legal, or economic risks, and the risk of a breach of confidentiality. These risks should be described in the consent documents.

   Risks are exceedingly minimal. Focus group participants could possibly find some questions about challenges in the self-management of their diabetes a bit distressing. There will be no questions about sensitive, personal or private topics during any part of the project.

B. Describe the anticipated frequency and severity of the harms associated with the risks identified above; for example, if you are performing "x" test/assessment, or dispensing "y" drug, how often do you expect an "anticipated" adverse reaction to occur in a study participant, and how severe do you expect that reaction to be?

   Distress about the topics of discussion is likely to be quite rare.

C. Describe steps to be taken to minimize risks. Include a description of your efforts to arrange for care or referral for participants who may need it.

   Focus group participants will be informed they needn't answer any questions they don't want to. Additionally, every effort will be made to protect the confidentiality of participants during phase 1, including assigning identification numbers and the use of nicknames and first names in lieu of full names of participants.

D. Describe the research burden for participants, including time, inconvenience, out-of pocket costs, etc.

   The focus groups in part 1 will last between 1-1.5 hours. Every attempt will be made to accommodate the schedules and meeting locations that best suit local participants. We do not anticipate any other out of pocket costs.

E. Describe how participant privacy will be protected during data collection if sensitive questions are included in interviews.

During the focus group session, participants will be identified by first name only, and will be asked not to discuss what they hear about other participants with people outside the group
Personal identifiers will not be retained when collecting the priorities of type 2 diabetics in phase 1 of the study.

VIII. Direct Personal and Social Benefits

A. Describe any potential direct benefits the study offers to participants ("payment" for participation is not a direct personal benefit).

There will be no direct benefits to focus group participants

B. Describe potential societal benefits likely to derive from the research, including value of knowledge learned.

The societal benefit from the study overall will be an analysis of which stated-preference methods can best assess patient preferences and priorities for the treatment of type 2 diabetes.

IX. Payment:

A. Describe the form, amount, and schedule of payment to participants. Reimbursement for travel or other expenses is not "payment," and if the study will reimburse, explain.

During phase 1, participants who attend a focus group meeting or conduct an individual interview with researchers will be paid $25.00 in the form of a Visa gift card. Parking vouchers will also be offered.

B. Include the possible total remuneration and any consequences for not completing all phases of the research.

Total remuneration for focus group participants will be a $25 gift card.

X. Study Management

A. Oversight Plan:

1. Describe how the study will be managed.

The study will be managed by the principal investigator (PI), Dr. John F.P. Bridges.

2. What are the qualifications of study personnel managing the project?

Tanjala Purnell, PhD and Ellen Janssen will serve as interviewers/note takers during the focus group.
Dr. Tanjala Purnell is an assistant professor of Surgery, at the Johns Hopkins School of Medicine and has experience with community engagement and qualitative research.
Ellen Janssen is a third year PhD students studying under Dr. Bridges. She focuses on patient preference research.

3. How will personnel involved with the data collection and analysis be trained in human subjects research protections? (Use the JHSPH Ethics Field Training Guide on our website.)

All researchers, including the moderators, note takers, and transcribers, involved with the project will have completed the Human Research Curriculum offered by the Collaborative Institutional Training Initiative (CITI). They will also be familiar with the JHSPH Ethics Field Training Guide.

4. If the PI will not personally be on-site throughout the data collection process, provide details about PI site visits, the supervision over consent and data collection, and the communication plan between the PI and study team.

   Not applicable

B. Recordkeeping:
Describe how you plan to ensure that the study team follows the protocol and properly records and stores study data collection forms, IRB regulatory correspondence, and other study documentation. For assistance, contact housecall@jhsph.edu.

   The study team will be closely monitored by the principle investigator, Dr. Bridges. He will make sure team members are aware of the appropriate manner in which to record and store study documentation.

C. Safety Monitoring

   Not applicable

D. Reporting unanticipated problems/adverse events (AE's) to the IRB (***all studies must complete this section***):
Describe your plan for reporting to the IRB and (if applicable) to the sponsor. Include your plan for government-mandated reporting of abuse or illegal activity.

   NOTE: The IRB does not require submission for all AEs, only those that are **unanticipated, pose risk of harm to participants or others, and are related to the study**.

   Any unanticipated problems will be reported to the IRB as soon as possible.

E. Other IRBs/Ethics Review Boards:
If other IRBs will review the research, provide the name and contact information for each IRB/ethics review board and its Federal Wide Assurance, if it has one (available on OHRP's website at http://www.hhs.gov/ohrp/assurances).

   Not applicable

F. Collaborations with non-JHSPH Institutions:

   Not applicable.