

PATIENTS PILOT PROJECT APPLICATION

PATient-centered Involvement in Evaluating the effectiveNess of TreatmentS

Application Title:

Engaging Patients with Systemic Lupus Erythematosus to Elicit Meaningful Treatment Outcomes

Principal Investigator: Xinyi Ng, BSPHarm

Doctoral Candidate, Department of Pharmaceutical Health Services
Research, University of Maryland School of Pharmacy

Principal Investigator Qualifications: *Please limit to no more than 3 sentences*

Xinyi Ng has experience conducting survey-based studies that assess health-related quality of life in patients with rheumatic diseases, and disease patterns in Rheumatology. Over the past 2.5 years, she has been developing expertise in methods for preferences-elicitation and is the project coordinator on a study funded by the National Institute of Mental Health (NIMH) that uses advanced preference elicitation methods. In this capacity, she has played a key role in the development, pilot-testing, administration, and analysis of survey data collected using a Best-Worst Scaling survey instrument.

Co-investigator(s):

1) *Dr. Michelle A Petri, MD, MPH*

Professor of Medicine, Johns Hopkins University School of Medicine

2) *Dr. Susan dosReis, PhD*

Associate Professor, Department of Pharmaceutical Health Services Research, University of Maryland School of Pharmacy

Co-investigator(s) Qualifications: *Please limit to no more than 3 sentences.*

Dr. Susan dosReis has been successful in building partnerships with community stakeholders and engaging consumers in research that is designed to elicit preferences for treatment and outcomes. Her research projects, which are funded by the NIMH and the Patient-Centered Outcomes Research Institute, has blended qualitative research to identify the healthcare concerns that are most important to patients. This research is building a solid foundation for comparing treatments that are most effective in achieving the outcomes patients most desire, and thus will generate meaningful information to assist patients in choosing treatments that are right for them given their own personal situation.

Dr. Michelle Petri, the Director of the Johns Hopkins Lupus Center, has more than thirty years of clinical experience in treating patients with Systemic Lupus Erythematosus (SLE). She is particularly interested in the long-term outcomes of SLE and its treatment, and has led numerous studies examining the effectiveness and risks of SLE medications. In 1987, she established the Hopkins Lupus Cohort, an ongoing longitudinal study of morbidity and mortality in SLE, which has made important contributions to the understanding of corticosteroid toxicity in SLE, risk factors for adverse outcome, and the value of different clinical measures in SLE management.

Relevance and Future Goals: Using no more than three sentences, describe the relevance of this pilot project to the PATIENTS infrastructure building and/or training activities. In this section, include information on your methods but be succinct and use plain language that can be understood by a general, lay audience.

By engaging the Systemic Lupus Erythematosus (SLE) patients and clinicians at the Johns Hopkins Lupus Center, this pilot project will foster a sustainable and committed partnership that will encourage future collaborations and expand the PATIENTS program capabilities for comparative effectiveness research in SLE. **Under the mentorship of Drs. dosReis and Petri, this pilot project will provide an excellent training opportunity for me to further develop and refine my expertise in patient-centered outcomes research in the field of Rheumatology.** Building upon the PATIENTS program focus on engagement and training, my dissertation will build upon this pilot to evaluate the benefit-risks of SLE treatment from the patients' perspectives so that I can prepare a competitive dissertation grant that will estimate preference weights for the benefits and competing risks of SLE treatment.

Estimated Budget: Using the space below, please detail proposed expenses.

Item	Amount	Brief Justification
Gift cards for 17 - 25 patients participating in in-depth interviews and focus groups	\$█ (17 to 25) x = \$█ to \$█	To compensate patients for the interviews or focus groups sessions, each of which will take approximately two hours to conduct.
Transportation costs for the 26 patients participating in in-depth interviews and focus groups	25 x \$█ = \$█	Patients will travel to UMB for the interviews and focus group sessions. The budget reflects round-trip taxi fares of \$█ each. For those who drive, they will be compensated at \$█ per mile and for parking.
Transportation costs for graduate student assistant to recruit patients	\$█	A subway round-trip ticket from UMB to John Hopkins costs \$█. The PI will be onsite (JHU) twice weekly for an anticipated period of 7-8 months thus, the costs will be around \$█.
Printing of flyers	\$█	Recruitment flyers to be handed out to patients. We budgeted for approximately 350 flyers based on the Fedex price list.
Gift cards for 120 - 200	\$█ x (120 to 200) = \$█ to \$█	To compensate patients for completing the survey instruments, which will take 30 to 45 minutes.

Specific Aims: *Using the space below, please insert your specific aims page. This section should include information on the proposed study methods and partnering organization.*

The 320,000 persons in the US afflicted with Systemic Lupus Erythematosus (SLE) have limited options other than corticosteroids to manage the flares that manifest with the relapsing-remitting nature of the disease. Unfortunately, chronic use of corticosteroids is associated with organ damage, cardiovascular and metabolic adverse effects and thus patients must constantly weigh the benefit-risk tradeoff of using corticosteroids. While some patients fear symptom relapse when medication is tapered, others worry about the medication side effects. Current comparative effectiveness research (CER) in SLE is complicated by the multiple clinical endpoints involved in the assessment of treatment effectiveness. These endpoints are not necessarily correlated and include reducing disease activity, preventing organ damage, minimizing adverse treatment effects, and improving patients' quality of life. Therefore, to advance CER of SLE treatment strategies, it will be critical to engage patients in identifying the treatment benefits and outcomes that are most important to them. Using the 10-step model for patient engagement as the conceptual framework, this pilot project will engage SLE patients in identifying the key research questions that will address the concerns and treatment outcomes that matter most to them. Our proposed study entails collaboration between the University Of Maryland Baltimore, a patient living with SLE, and the Johns Hopkins Lupus Center (JHLC). The latter, led by Dr. Michelle Petri, is one of the largest and top-ranked SLE specialty clinics in the United States providing care for a diverse population of SLE patients. Since its inception in 1987, data gathered on the 2,000 patients participating in the Hopkins lupus cohort have led to numerous outcomes-oriented studies. By leveraging the clinical expertise of the Lupus Center and the University of Maryland's infrastructure for patient-centered outcomes research, we will foster a sustainable partnership where SLE patients are empowered to prioritize treatment concerns that will inform future CER studies. The specific aims of this pilot study are to:

Aim 1: To engage SLE patients in developing an instrument for selecting and prioritizing meaningful treatment outcomes.

Challenge: SLE management involves the assessment of multiple clinical endpoints, which are not necessarily correlated and patients may not value each endpoint equally. This highlights the need for early engagement of SLE patients in identifying what matters most to them in managing their SLE. **Approach:** Based on the first 5 steps of the 10-step model, our experience in prior studies indicates this will be completed in 3 months. To identify key concerns and outcomes (step 1), five in-depth, individual interviews will be conducted with SLE patients (i.e., key informants) recruited from the JHLC. Information elicited from these interviews, together with clinical input and input from our patient advisor, will be used to develop a field guide for 3-4 focus groups of 4-5 individuals each. The focus groups will solicit feedback on the relevance, importance and real-world applicability of the themes that emerge from the in-depth interviews (step 2-3). The focus groups will be conducted iteratively, where new issues emerging from a prior session will be presented to subsequent focus groups for thematic validation or refinement. The research team will debrief after each session to determine if saturation of the themes has been reached. The key themes will be matched with treatment alternatives to create a conceptual framework of treatment concerns representing the voice of SLE patients (Step 4-5). This framework will be used to create unique profiles for a choice-based conjoint survey, where patients must select and prioritize among 2-3 profiles of treatment outcomes that are most meaningful to them. **Impact:** *This survey is the first of its kind to be developed by, and for patients with SLE.*

Aim 2: To determine the concordance between SLE patients' treatment outcomes priorities and the outcomes recommended for use in CER of SLE treatments.

Approach: A total of 120-200 SLE patients will complete a choice-based conjoint survey. The JHLC sees 80 patients a week, and assuming a very conservative 25% response rate and a research assistant onsite twice a week, it will take 7 months to attain the sample. However, we anticipate recruitment will be completed in less time. Interim results will be presented to patients, whereby they can verify if the results address the main points, and comment on the ease of completing the survey. This feedback will be used to refine the instrument, validate the profile items (step 7), and ensure that results are believable and meaningful (step 8-9) from the patients' perspectives. The top-ranked treatment outcomes priorities will be compared with outcomes measured in existing CER literature to assess where research is needed to enhance PCOR. **Impact:** *A better understanding of SLE patients' priorities for treatment outcomes will advance patient-centered CER so that future CER are based on outcomes that are most valued by SLE patients, and help SLE patients select the treatment that best align with their priorities.*