

# American College of Rheumatology National Research Agenda 2016 - 2020

*This research agenda has been developed and published by the ACR Committee on Research. The research agenda is intended to document important areas for research in rheumatic diseases and reflects priorities as identified by the rheumatology community at large.*



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## I. INTRODUCTION

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Rheumatologic conditions are the most common cause of disability in the United States and have a significant impact on the lives of patients with these diseases. Rheumatic disease is a broad term that encompasses diseases that affect the muscles and joints (including osteoarthritis and rheumatoid arthritis) and autoimmune diseases that affect multiple organ systems (e.g., systemic lupus erythematosus and Sjögren's syndrome).(1) These conditions are most often chronic, requiring long term care, and have wide spread impact including psychological stress, hindering of social function and career development or school performance, reduced work productivity, decreased quality of life, and in some cases, higher mortality rates and comorbidities.(2-10)

Over one quarter of Americans are affected by a rheumatic disease and people of any age may be affected.(11) For example, at least 52.5 million adults in the US have a form of arthritis.(12) Currently, there are no cures for rheumatic diseases, but as science and technology advance, so too should our knowledge of these diseases and the therapies used to treat them. Better methods of identifying and diagnosing rheumatic diseases are needed as diagnosis is difficult for many conditions.(13) Additionally, better treatments and management strategies are needed to improve quality of life and functional ability while minimizing side effects from therapies. A major goal of the American College of Rheumatology (ACR) is to promote research aimed at improving the health of patients with rheumatic disease. Within the ACR, the Committee on Research (COR) is responsible for facilitating the research goals of the organization and providing leadership in research and research training in rheumatology and related professions. In this role, the COR is also charged with maintaining a national research agenda.(14) The purpose of a research agenda is to set the compass for the organization in terms of research initiatives and facilitate the ACR's advocacy for the research goals identified.

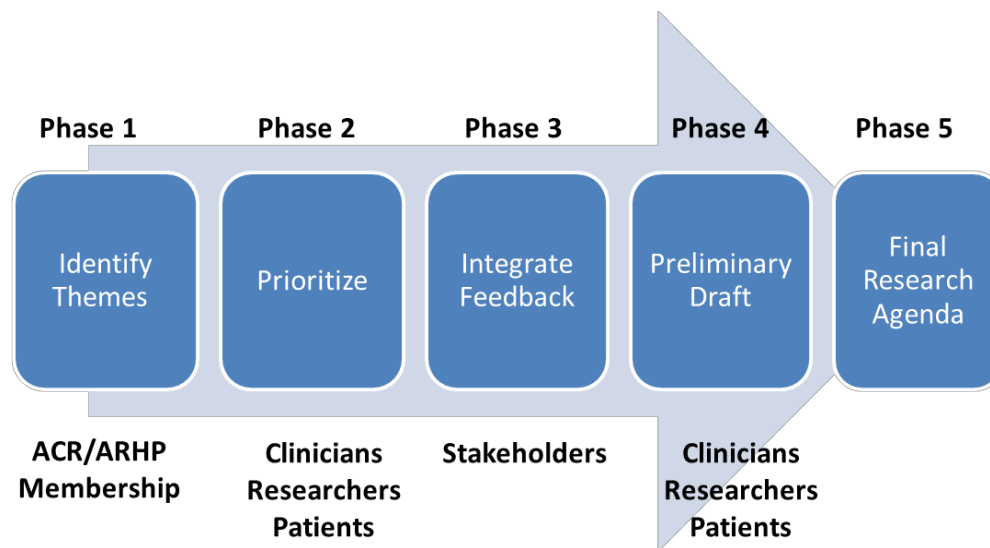
The goal of this Research Agenda is to focus on important questions facing patients with rheumatic diseases, rheumatologists caring for these patients, and the relevant stakeholders. Additionally, the agenda should stimulate promising research that will improve the lives of patients with rheumatic disease. Thus, the COR aimed to 1) identify the most important questions faced by patients with rheumatic disease and their rheumatologists, 2) to define the research goals felt to be of greatest importance by patients, physicians and stakeholders, and to 3) formulate the 2016-2020 ACR Research Agenda based on these findings. While most

research agendas are created by researchers, the COR was committed to obtaining a broader perspective. We used a multistage approach to first identify important questions from the general ACR/AHRP membership and then sought input from a carefully selected sample of patients, clinicians and researchers to prioritize and refine the research goals into a final version of the 2016-2020 ACR Research Agenda. We anticipate that the research agenda will stimulate promising, clinically relevant research, motivate collaborative engagements among researchers to accomplish the stated goals, serve as a foundation for partnerships between the ACR and other organizations, and guide the development of new funding mechanisms.

## **II. METHODS**

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The development of the research agenda was a multi-phase process consisting of the following steps: 1) survey of the general membership of the ACR and AHRP to identify important research questions, 2) Delphi exercise to prioritize among the questions identified, 3) integration of stakeholder comments after a round table discussion, and 4) refinement of the research agenda by the Committee on Research (Figure). Each of the steps is described below.



#### *Phase 1: Item Generation*

- A web-based survey was sent to all members of the ACR and AHRP living in the United States with an available email address in April 2015.
- The survey included basic demographic questions and then asked participants to “list the five most important research questions that need to be addressed over the next five years in order to improve the care for patients with rheumatic disease.”
- The demographics of the participants were summarized descriptively.
- We conducted a modified grounded theory analysis to identify the key themes from the questions submitted.
- We examined the frequency with which each theme was reported; in this analysis, each person could only contribute “once” to a theme
- Using wording from the respondents, we constructed statements phrased as research goals around the themes.
- We removed disease-specific terms in order to generalize these statements for all patients with rheumatic disease.

#### *Phase 2: Prioritization of the Identified Themes*

- We purposefully selected one hundred individuals to participate in a web-based survey in June 2015. The invitees were representative of the following groups: patients, clinicians (academic and community), research (all types with diverse areas/diseases of

- interest), allied health professionals, pediatric and adult rheumatology, men and women, all career stages, and all regions of the country.
- The survey included basic demographics, any perceived conflicts of interest, and 30 statements generated from the data obtained in Phase 1. Respondents were asked to rate each item on a scale from 1 (not important) to 10 (very important) and optionally provide comments for each item.
  - Respondent demographics and item scores were summarized.
  - Wording of the items was changed according to individual suggestions.
  - Additional comments regarding each items importance were summarized and presented with the items.

### *Phase 3: Stakeholder Input*

- The draft research agenda assembled from the results of Phase 2 was presented at a stakeholder meeting on June 27, 2015 at the Rheumatology Research Foundation *Partners in Rheumatology Leadership Summit*. Stakeholders in attendance included representatives from various non-profit foundations associated with rheumatic diseases (e.g. Arthritis Foundation, Lupus Foundation, Sjögren’s Foundation, and Physical Therapy Foundation), representatives of the National Institutes of Health, and the President of the Rheumatology Research Foundation.
- Some of the individual items were discussed as was the broader context and potential uses of the 2016-2020 ACR Research Agenda.
- Following this meeting, the COR met to discuss the results to date and the input received.
- This resulted in collapsing related items, moving three items into a separate category (“supporting the research goals”) and dropping of six items.

### *Phase 4: Solicitation of feedback on the Preliminary Research Agenda*

- Participants in Phase 4 were the same as those in Phase 2.
- One of the concerns from the stakeholders and COR was the underrepresentation of clinicians in Phase 2. In order to increase clinician input in the second round of the Delphi survey, 17 additional clinicians were invited to participate.

- The web-based survey was distributed in August 2015 and included basic demographic questions followed by the individual items in the preliminary research agenda. Similar to Phase 2, respondents were requested to rate each item on a scale from 1 (not important) to 10 (very important) and optionally provide comments for each item.
- Respondent demographics and the median, mean, and standard deviation scores for each item were summarized.
- Changes suggested by respondents were made, including some wording changes and collapsing of items. Comments from this phase were also summarized.

### **III. RESEARCH AGENDA**

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The overarching goal of the research agenda is to inform the types of questions that must be answered in order to improve the health of patients with rheumatic disease and assist rheumatologists in caring for them. This set of research goals can serve as a compass for research initiatives supported by the organization and we hope that it will stimulate partnerships with other organizations as well. The following, depicts in rank order, the finalized goals for the ACR Research Agenda 2016 – 2020.

- A. Define new therapeutic targets and develop new therapies for the rheumatic diseases
- B. Define predictors of response or non-response to therapy and predictors of adverse events
  - a. Define biomarkers for and genetic predictors of therapy response
  - b. Determine the impact of patient characteristics on treatment response including race and ethnicity
  - c. Determine ways in which physicians can incorporate personalized medicine into routine clinical care
  - d. Understand disease subsets and the role of disease phenotype in therapy response and prognosis
- C. Improve our understanding of existing therapies for the rheumatic diseases
  - a. Compare the effectiveness of existing medications in both observational studies and clinical trials
  - b. Conduct pragmatic clinical trials of existing therapies in real world populations

- c. Define optimal methods of reducing or stopping therapy for patients in remission
  - d. Understand the risk of adverse events for new therapies and older therapies including glucocorticoids
  - e. Understand the risks and benefits of biologic medications in subpopulations: e.g. during pregnancy, in patients with concurrent or previous cancer, and in patients with comorbidities
- D. Understand the etiology, pathogenesis, and genetic basis of rheumatic diseases
- a. Determine environmental and genetic risk factors for disease
- E. Understand early disease states
- a. Improve early diagnosis
  - b. Develop biomarkers for early detection
  - c. Identify and understand preclinical disease states
  - d. Determine how earlier treatment changes outcomes
- F. Improve our understanding of the immune system and autoimmunity
- a. Define triggers of autoimmunity
  - b. Determine how epigenetics affect disease susceptibility and inflammation
- G. Develop improved outcome measures including patient-reported outcome measures, imaging modalities, and measures of clinical response and disease activity
- H. Determine how the changing health care landscape in the United States affects patients with rheumatic disease, rheumatologists and rheumatology health professionals
- a. Examine cost-effectiveness of therapies and interventions
  - b. Determine the effects of the 'Choosing Wisely' campaign on rheumatologic care
  - c. Examine disparities in access to healthcare and medications
  - d. Improve access to rheumatologists, diagnostic testing and medications
  - e. Improve timely care delivery and collaborative care for patients with rheumatic disease
  - f. Define how rheumatologists can best adapt to the changing health care delivery models
- I. Examine the role of non-pharmacologic therapy in the management of rheumatic disease



- a. Develop methods to promote and improve adherence to physical activity in patients with rheumatic disease
  - b. Determine the optimal exercise prescriptions for patients with rheumatic disease
  - c. Determine the impact of diet on disease activity in patients with rheumatic diseases
- J. Determine adult outcomes of pediatric rheumatic diseases
- K. Optimize the engagement of patients in their care
- a. Develop patient-centered approaches to care
  - b. Define methods for assisting patients in self-management their disease
  - c. Improve adherence to treatment
  - d. Use mobile health apps and telehealth be used to improve quality of life and patient outcomes
  - e. Optimize the use of the electronic health records to make rheumatology practice safer, more efficient, and higher quality
  - f. Determine methods of reducing disparities in rheumatic disease health outcomes
- L. Understand the burden of comorbidities with rheumatic diseases and the underlying mechanisms for these relationships
- a. Improve identification and management of cardiovascular disease in patients with rheumatic disease
  - b. Define the impact of obesity on outcomes in rheumatic diseases
  - c. Improve bone health
- M. Understand the mechanisms of pain and fatigue in the rheumatic diseases and improve their management
- N. Evaluate the role of regenerative medicine in rheumatic diseases
- O. Determine the effect of aging on the development, progression and management of rheumatic diseases

The following have been identified as supplementary goals which will help to support the scientific goals outlined above.

- P. Advocate for increased support for rheumatology research and rheumatology investigators
- Q. Harmonize data from existing cohorts and registries to optimize research capabilities
- R. Improve patient research partner involvement in research protocols

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