NATIONAL OFFICE
Advocacy and Access Department
1615 L Street, NW #320
Washington, DC 20036

February 23, 2015

The Honorable Lamar Alexander
Dirksen Senate Office Building
Washington, DC 20510

The Honorable Richard Burr
Russell Senate Office Building
Washington, DC 20510

Dear Senators Alexander and Burr:

The Arthritis Foundation, on behalf of the more than 50 million adults and children in the U.S. living with arthritis, welcomes the opportunity to comment on the Innovation for Healthier Americans report. There are over 100 types of arthritis, and there is no cure for the disease. Further, certain types of arthritis like osteoarthritis – which affects over 27 million Americans – don't have any effective pharmaceutical treatments. For others, treatment can be very complex - people with auto-immune forms of arthritis like rheumatoid arthritis often rely on multiple types of drugs, including biologic drugs, to manage their disease.

Reducing barriers to research that will accelerate discovery, development, and delivery of medical treatments can greatly benefit people with arthritis. Your report goes a long way towards addressing research needs around discovery and the development of treatments. However, there is little emphasis on the delivery of those treatments to patients. The costs of drugs are prohibitive for many people with arthritis, even with insurance. Insurance designs like specialty tiers require people to pay up to 50% of the cost of their drugs out-of-pocket. For people who rely on biologic drugs – like those to treat rheumatoid arthritis – this can represent thousands of dollars each month. Any legislation seeking to improve the biomedical research system should also address patient access and cost issues, as development and approval of treatments means little if patients can't access those treatments.

One important way to address affordability of medications is to include the Patients' Access to Treatments Act in your legislation. This bill would protect patients from prohibitive cost-sharing by limiting cost-sharing in specialty tiers to the non-preferred brand drug tier (usually tier III). In 2015, for the first time 100% of Medicare Part D plans are using specialty tiers, and the commercial market is beginning to follow suit - the number of employer plans with specialty tiers has increased from 3% to 23% in less than 10 years. Efforts to reign in proliferating cost-sharing structures will greatly improve the ability of patients to access medically-necessary drugs and lead productive lives.

The Arthritis Foundation would like to make the following comments on strengthening the biomedical research system:

<u>Patient Perspectives in the Drug Development Process</u>

The Arthritis Foundation believes that patient perspectives during the drug development process are vital to developing drugs that will best suit the needs of patients. Many people with arthritis are on multiple medications, and for complex forms of the disease, it is not uncommon for patients to take 40 or more pills a day. It is important that the FDA understands the patient experience, including what types of reactions and side effects they have to medications, and the level of difficulty in accessing and adhering to medications. The Arthritis Foundation made several suggestions to the FDA in their recent

request for comments on patient perspectives in the drug development process, including the creation of a patient portal on the FDA website, the use of tele-town halls, webinars, and forums to educate patients about drug development, inclusion of arthritis in the disease-specific meetings calendar, and more robust use of patient registries to collect data on patient experiences.

A more structured framework for incorporating patients will help ensure their needs are addressed at every step of the process. We urge you to include more robust processes for patients to be involved in the drug development process in your legislation, and to identify ways patients can be involved early in the process.

Biomarkers

Biomarkers are relevant to most, if not all types of arthritis. For example, although osteoarthritis affects 27 million Americans, there is no qualified biomarker for the disease, and no effective pharmaceutical treatment. Strengthening the surrogate endpoint qualification process and identification of biomarkers is vitally important to people with arthritis.

We urge you to include a provision in your legislation to create a pathway for qualifying surrogate endpoints to make it easier to bring drugs to market for diseases like osteoarthritis.

Patient Access

As stated above, discovery and development of treatments have little meaning if patients can't access them. There are several ways legislation can address this issue:

- **Cost-sharing.** Limit cost-sharing requirements for specialty drugs by including legislation like the Patients' Access to Treatment Act.

- **Provider-patient relationship.** The Arthritis Foundation believes preservation of the physician-patient relationship is a vital part of patient care. Many patients rely on off-label drugs to treat their disease. For example, low doses of certain anti-depressants, muscle relaxants, and anti-seizure medications can all help relieve symptoms of fibromyalgia. Legislation should address ways for physicians to prescribe the drugs that will best treat their patients without fear of violating regulatory policies.

- **Medicare coverage and costs.** Legislation should allow Medicare beneficiaries more coverage with regard to drugs from clinical trials in which they participate. It should also reform the local coverage determination process to make it more reliable and effective, which would greatly improve the ability of beneficiaries to access new treatments in a timely manner

- **Telemedicine.** There is a dramatic shortage of pediatric rheumatologists, as many states have only 1 pediatric rheumatologist, and several states have none. We encourage you to find pathways to navigate state licensure issues, so that physicians in one state can provide tele-health services to patients in other states, which would greatly benefit our JA population. We also urge you to find ways to expand tele-health policies beyond Medicare, so that more patients – like our JA patients – can better access the benefits of telemedicine.

Drug Development and Approval

Many drugs for arthritis, including those for osteoarthritis, have been abandoned because of their failure to meet a clinical endpoint. We are concerned that over time, manufacturers may choose not to re-investigate a drug because the patent has expired or will expire before the research is completed and the time it takes to go through the federal approval process.

Further, there are several types of arthritis among both children and adults that are considered rare and rely on orphan drugs or have no treatment at all. For example, myositis is an inflammatory disease that

affects the muscles, and can cause flares, muscle weakness, lung and breathing problems, systemic symptoms like fever and weight loss, and joint pain. There are only 50,000-75,000 people in the country with the disease in the US; there is no cure and for some forms of the disease, there is no effective treatment.

We urge you to address these problems by:

- Finding ways to incentivize manufacturers to re-investigate dormant therapies for arthritis.
- Extending exclusivity for new therapeutic entities for two years to incentivize more research into arthritis drugs.
- Providing additional exclusivity for a drug that treats a rare disease.

Research Workforce

Fostering the next generation of researchers is critical to maintaining America's status as a leader in biomedical research. This is a major focus in the Arthritis Foundation's Scientific Strategy to cultivate young and future scientists in pursuing careers in research. We urge you to provide additional funding to NIH to fund more emerging scientists.

Public-private partnerships have great potential for accelerating the discovery and development of drugs, and increasing collaboration and innovation across sectors of the research enterprise. We would support public-private partnerships through a consortium based on the European Union's Innovation Medicines Initiative.

Data Sharing

Data sharing is an important component to improving biomedical research. Patients and physicians need better ways of becoming informed of clinical trials, and researchers need access to data that will help direct them towards the most pressing research needs and ultimately improve quality of care. We urge you to include data-sharing reforms in your legislation that address the following:

NIH data-sharing. Having access to NIH data would help private foundations like the Arthritis Foundation that support research best direct their resources to find a cure. Legislation should facilitate ways that private foundations can access this important pool of data.

Registries. Registries are a critical tool for collecting data on patient outcomes. Participation in a registry is likely to increase what we know about a specific condition, help health care professionals improve treatment, and allow researchers to design better studies on a particular condition, including development and testing of new treatments. Arthritis registries can help discover how arthritis affects daily activities, understand the impact of various treatments, find out which treatments are most beneficial, and uncover identifiable risks for arthritis that can be limited – so arthritis can be prevented.

There are many arthritis registries, including an EHR-enabled registry (RISE), the Arthritis Internet Registry (AIR) and the Childhood Arthritis and Rheumatology Research Alliance (CARRA) network. The CARRA network enables data collection about the major pediatric rheumatic diseases (juvenile arthritis, systemic lupus, dermatomyositis, scleroderma, vasculitis and pain syndromes). The unique and variable features of populations and registry designs provide valuable and complementary data on comparative effectiveness and safety of treatments such as biologic agents. If the arthritis registries in the United States agree to collecting standardized core metrics, then the data can be pooled together to have statistical power to answer questions of interest to everyone. Registries provide overall acceleration of the research process and also provide data to design more effective clinical trials. We encourage you to include provisions in your legislation to increase the availability and use of registries.

Use of health data. The Arthritis Foundation also supports increased use of health data for research purposes. Using health data can benefit patients by better informing research needs and improving outcomes. Specific benefits include:

- Guiding immediate action for cases of public health importance;
- Measuring the burden of a disease (or other health-related event), including changes in related factors, the identification of populations at high risk, and the identification of new or emerging health concerns;
- Monitoring trends in the burden of a disease (or other health-related event);
- Guiding the planning, implementation, and evaluation of programs to prevent and control disease, injury, or adverse exposure;
- Evaluating public policy;
- Detecting changes in health practices and the effects of these changes;
- Prioritizing the allocation of health resources;
- Describing the clinical course of disease; and
- Providing a basis for epidemiologic research.

Medical Device Reforms

Early discussions about comparative effectiveness research (CER) in the United States focused on defining "comparative" and determining the implications for research. Today, the focus has shifted from looking simply at what should be compared to how comparative studies should be designed to answer the practical questions about "effectiveness," particularly in real world settings. The question now becomes does a treatment, service, or method of delivering care work when applied in real world, clinical practice environments?

To answer that question, health care stakeholders need to think beyond traditional randomized controlled trials (RCTs). One way to consider this is to make use of real-world databases that record the processes of care patients receive, such as electronic medical record (EMR) and medical claims databases. We also might work with patient registries that focus on patients with a specific disease or who receive a particular treatment or device and record the medical outcome for those patients; or these registries could be based on a specific population so that researchers can see what happens within that population.

Again, thank you for the opportunity to comment on the Innovation for Healthier Americans report. We applaud you for taking on these important health policy issues and look forward to future opportunities to work with you on this important topic. Should you have any questions or if we can be of assistance in any way, please contact Sandie Preiss, Vice President of Advocacy and Access, at 202-887-2910 or spreiss@arthritis.org.

Sincerely,

Sandie Preiss
Vice President, Advocacy and Access
Arthritis Foundation