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

February 13, 2015

The Honorable Fred Upton
2183 Rayburn House Office Building
Washington, DC 20515

Dear Chairman Upton:

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 draft 21st Century Cures legislation. 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 regulatory and other barriers to research that can accelerate discovery, development, and delivery of medical treatments can greatly benefit people with arthritis. Your legislation goes a long way towards addressing research needs around discovery and development of treatments. However, we would like to see more emphasis on the delivery pillar. 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.

The Arthritis Foundation would like to offer the following comments on your draft legislation.

Title I – Putting Patients First by Incorporating their Perspectives into the Regulatory Process and Addressing Unmet Medical Needs

Subtitle A – Patient Focused Drug Development

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.

We support your effort to strengthen the patient perspective in FDA's regulatory processes, and agree that a more structured framework for incorporating patients will help ensure their needs are addressed at every step of the process. However, we also urge you to identify ways patients can be involved early in the drug development process, and we seek clarification on how drug sponsors may be able to communicate with patients on drugs they are working on without legal ramifications.

Subtitle B – Surrogate Endpoint Qualification and Utilization

Strengthening the surrogate endpoint qualification process and identification of biomarkers is vitally important to people with arthritis. This is 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.

We support the effort to make it easier to qualify endpoints, which can help bring drugs for osteoarthritis to market. However, we urge you to authorize additional funds in this provision to give the FDA the resources to implement this provision.

Subtitle C and F – Approval of Breakthrough Therapies and Devices

Access to breakthrough therapies and devices is important to people with arthritis, particularly persons with rare and/or terminal types of arthritis. The Arthritis Foundation is pleased you are addressing ways to bring breakthrough therapies to patients more quickly, but we seek clarification on the definition for unmet medical need, in addition to how rigorous patient safety standards will be kept in place when considering early stage clinical data.

Subtitle G – Expanded Access

Expanded access programs can help patients to access drugs they would not otherwise have access to. Currently, even if a patient meets the criteria for compassionate use programs, there may still be obstacles, including:

- The physician must determine that the probable risk from the drug is not greater than the probable risk from the disease;
- The company that makes the drug is not required to offer it outside their clinical trials, and they may not be willing or able to do so;
- The company may not have enough of the drug available for all patients requesting expanded access. Some companies establish a lottery system to determine which patients will have treatment access, and others make the decision on a case-by-case basis;
- Investigational drugs are expensive to make. Most insurance companies will not pay for access to an investigational drug and there may be additional cost for administering and monitoring them.

Subtitle H – Facilitating Responsible Communication of Scientific and Medical Developments

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. The Arthritis Foundation believes preservation of the physician-patient relationship is a vital part of patient care. Physicians should be able to prescribe the drugs that will best treat their patients without fear of violating regulatory policies.

Subtitle K – Cures Acceleration Network

The Cures Acceleration Network holds great promise for getting treatments to patients more quickly. We support your effort to strengthen CAN by giving it more flexibility. We particularly support the provision authorizing additional funds for research on repurposing drugs. Many drugs used to treat arthritis were developed for other diseases like cancer. For example, methotrexate is a form of chemo-therapy that many people with auto-immune forms of arthritis use to help manage their symptoms. Our hope is that drugs currently in the pipeline for other auto-immune and inflammatory diseases and cancer will also be relevant to arthritis prevention and treatment.

Subtitle L – Dormant Therapies

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 the 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. This provision could help incentivize manufacturers to re-investigate dormant therapies for arthritis. However, we would seek clarification on the definition of unmet medical need and the process by which this provision will be implemented.

Subtitle M – New Therapeutic Entities

As stated above, many drugs for arthritis, like those for osteoarthritis, fail to meet clinical endpoints. Investigators do not fully understand the pathophysiology of the disease, and therefore do not have the correct patients enrolled in clinical trials and thus cannot demonstrate success. Extending exclusivity for two years could incentivize more research into arthritis drugs and greatly benefit patients waiting for effective treatments for their disease.

Subtitle N – Orphan Product Extensions Now

There are several types of arthritis among both children and adults that are considered rare and have orphan drugs. 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. Providing additional exclusivity for a drug that treats a rare disease can benefit people who suffer from rare forms of arthritis.

Title II – Building the Foundation for 21st Century Medicine, Including Helping Young Scientists

Subtitle A – 21st Century Cures Consortium Act

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 support your effort to increase the level of public-private partnerships through a consortium based on the European Union's Innovation Medicines Initiative.

Subtitle B – Medical Product Innovation Advisory Committee

It is important for Congress to be continually advised by the scientific community on the discovery-development-delivery cycle. We urge you to include a patient representative on this panel, as the patient perspective is an important component of the research enterprise, particularly as it relates to the delivery of new drugs.

Subtitle F – Building a 21st Century Cures Data Sharing Framework

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 improving quality of care. A data sharing framework could help achieve this goal. In terms of using registries, we recommend you include patient advocacy groups in the consultation with clinical experts section, as organizations like the Arthritis Foundation are heavily involved in registries.

Subtitle G – Utilizing Real-World Evidence

Utilizing data on how drugs affect patients and how patients experience symptoms is critical to advancing research. This provision fits in line with the Arthritis Foundation's Scientific Strategy, supporting data collection from registries, and other federal efforts like PCORI's PCORNET.

Subtitle H – Coverage with Evidence Development

Out-of-pocket costs are a huge concern for many people with arthritis. The Arthritis Foundation supports the effort to give Medicare beneficiaries more coverage with regard to drugs from clinical trials in which they participate. This provision should include a process to help CMS develop a better coverage decision process.

Subtitle J – Combination Products

There is a number of combination products used to administer arthritis treatments. As you continue your work on regulatory guidance for combination products, the Arthritis Foundation believes that consistency and appropriateness of post-market regulation of combination products is needed, in order to preserve patient safeguards.

Subtitle L – NIH-Federal Data Sharing

The Arthritis Foundation believes that data-sharing can help everyone in the research community to better understand and by extension support discovery and development of treatments for disease. Having access to NIH data would help private foundations like the Arthritis Foundation that support research best direct their resources to find a cure.

Subtitle M – Accessing, Sharing, and Using 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.

Subtitle N – 21st Century Chronic Disease Initiative Act

People with arthritis often live with their disease for years and even decades. In addition, more than half of people with other chronic diseases like diabetes (52%), and heart disease (57%) also have arthritis. For people with juvenile arthritis who are on methotrexate and/or biologics, there is a lot of information that is unknown about the long-term impacts of those drugs. A longitudinal study measuring the long-term impact of disease and treatment can help answer many questions about the effects of disease on quality of life and of treatments on long-term health outcomes.

Subtitle O – Helping Young Emerging Scientists

Fostering the next generation of researchers is critical to maintaining America's status as a leader in biomedical research. This provision fits in line with 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.

Subtitle P – Fostering High-Risk, High-Reward Science

High-risk, high-reward science can yield major breakthroughs, yet the current federal research structure often does not incentivize this type of research. The Arthritis Foundation supports efforts like the Accelerating Medicines Partnership which emphasizes high-risk, high-reward research, and we support efforts to increase this type of research.

Subtitle A – Clinical Research Modernization Act

The current IRB process is often cumbersome and can act as a barrier to accelerating research. The Arthritis Foundation supports your effort to streamline this process.

Any changes to the IRB process and the effects on human research protections must stay true to and reaffirm the highest values of protecting the integrity of research, the well-being of human subjects who participate in research, and the trust of the public.

Title IV – Accelerating the Discovery, Development, and Delivery Cycle and Continuing 21st Century Innovation at NIH, FDA, CDC, and CMS

Subtitle H – Local and National Coverage Decision Reforms

The ability of patients to access and afford their treatments is a vital component of the research cycle. Medicare local coverage determination processes are often cumbersome and difficult to navigate. Reforming this system to make it more reliable and effective could greatly improve the ability of beneficiaries to access new treatments in a timely manner. Opportunities for public comment and disclosure of information about determinations will also benefit beneficiaries.

Subtitle I – Telemedicine

We appreciate your efforts to address telemedicine. Reducing or containing the cost of healthcare is one of the most important reasons for funding and adopting tele-health technologies. Telemedicine has been shown to reduce the cost of healthcare and increase efficiency through better management of chronic diseases, shared health professional staffing, reduced travel times, and fewer or shorter hospital stays.

Studies have consistently shown that the quality of healthcare services delivered via telemedicine is as good those given in traditional in-person consultations. In some specialties, particularly in mental health and ICU care, telemedicine delivers a superior product, with greater outcomes and patient satisfaction.

There is a dramatic shortage in pediatric rheumatologists, as many states have only 1 pediatric rheumatologist, and several states have none. We encourage you to continue 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 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. We also seek clarification on the budget neutrality requirement, and any unintended consequences or other effects it might have on the Medicare program and patient access to care.

Subtitle K – Lowering Medicare Patients OOP Costs

As stated previously, out-of-pocket costs are a huge barrier to many people with arthritis, in terms of accessing and adhering to their medications. We support the goal of this provision in giving beneficiaries more control to choose services that best suit their financial needs. However, we urge you to expand the required list to include information on specialty tier trends and other utilization management tools, and to develop cost-sharing reduction programs as part of this effort.

Out-of-pocket cost issues exist outside the Medicare population as well, and we urge you to expand on this provision for people in the commercial insurance market as well. Including the Patients' Access to Treatment Act, limiting specialty tier cost-sharing, has the potential to dramatically benefit people who currently pay hundreds and even thousands of dollars out-of-pocket every month for drugs that are on specialty tiers.

Subtitle S – Continuing Medical Education Sunshine Exemption

The current Sunshine rules have created myriad unintended consequences, such as preventing physicians from being able to participate in certain patient advocacy efforts, and hindering physicians from participating in clinical trials, resulting in many fewer referrals of patients for clinical trials. We support efforts to address some of these unintended consequences, and encourage the Committee to further investigate the possible need of further exemptions from the Sunshine rules.

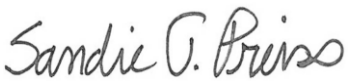
Subtitle D – Medical Device Reforms

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 draft 21st Century Cures legislation. We look forward to future opportunities to work with you on this legislation. 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,



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