

July 22, 2014

The Honorable Joe Pitts
Chairman

The Honorable Frank Pallone, Jr.
Ranking Member

Committee on Energy and Commerce
Subcommittee on Health
2125 Rayburn House Office Building
Washington, DC 20515



RE: July 22nd Hearing on the 21st Century Cures Initiative: Examining Barriers to Ongoing Evidence Development and Communication

Dear Chairman Pitts and Representative Pallone:

Thank you for the opportunity to continue the important dialogue about the Committee's 21st *Century Cures* Initiative. The Lupus Foundation of America is the only national force dedicated to improving the quality of life for all people affected by lupus through programs of research, education, awareness, and advocacy. Lupus is an unpredictable and misunderstood autoimmune disease that ravages different parts of the body. It is difficult to diagnose, hard to live with, and a challenge to treat. Lupus is a cruel mystery because it is hidden from view and undefined, has a range of symptoms, hits out of nowhere, and has no known cause and no known cure.

The Subcommittee's July 22nd hearing memo discussed that "different uses for drugs and devices are being discovered constantly, many times for treatment of new conditions and diseases or for populations of patients other than for which they were initially approved." In the case of lupus, this statement is on target. The most commonly prescribed medications for treating lupus are prescribed "off-label," and it is critically important for all stakeholders to have the ability to freely share peer-reviewed information about the safety and efficacy of medications used to treat lupus.

Currently, there are only four FDA-approved medications to treat lupus – Benlysta[®], hydroxychloroquine (an antimalarial), prednisone (a steroid), and aspirin. Benlysta[®], approved by the FDA in March 2011, is the first and the *only* drug designed specifically to treat lupus. All other medications used to treat lupus are drugs approved for other indications such as chemotherapies, used to treat cancer, and immunosuppressants, used post organ transplantation.

The above treatments, other than Benlysta®, have never been specifically tested in lupus. For example, Rituximab was developed and approved for use in rheumatoid arthritis, but it has shown efficacy in some lupus patients and is prescribed “off-label” as a treatment for lupus.

Another example is related to the treatment of lupus nephritis or lupus kidney disease, one of the most serious forms of lupus. Lupus nephritis is most often treated by two different therapies - CellCept and Cyclophosphamide. Neither have been fully tested in lupus nor approved for use in lupus by the FDA; yet, both are considered the standard of care in lupus nephritis. Interestingly, Cellcept was tested against the chemotherapy, and Cellcept was found to be equivalent but not superior; thus, never approved by the FDA.

The real-world provides the perfect opportunity to learn more about the richness of medications and their other potential uses beyond their FDA designation. People with lupus must be treated effectively and in a timely manner, with the best resources available—regardless of whether it is indicated for lupus or not. Otherwise the consequences can be costly and life threatening --including lupus flares, organ failure and long-term hospitalizations. It could be considered unethical not to communicate vital off-label information to physicians treating diseases like lupus.

It is the Foundation’s position that the prescription of off-label medications to people with lupus may already be the standard of care and the communication of these uses may advance public health. If you have any questions or wish to discuss further, please contact Kim Cantor, Vice President of Advocacy and Government Relations at (202) 349-1150 or at cantor@lupus.org.

Sincerely,



Sandra C. Raymond
President & CEO
Lupus Foundation of America