

April 19, 2017

Division of Dockets Management
Food and Drug Administration
5630 Fishers Lane, Room 1061
Rockville, MD 20852
Submitted electronically

RE: Request for Comments: Docket No FDA-2016-N-1149 Manufacturer Communications Regarding Unapproved Uses of Approved or Cleared Medical Products

On behalf of the undersigned organizations, and the millions of patients we collectively represent, we appreciate the opportunity to submit these comments in response to the notice of public hearing concerning manufacturer communications regarding unapproved uses of approved or cleared medical products as originally published in the Federal Register on Thursday, September 1, 2016 and reopened on Thursday, January 19, 2017.

At the core of the FDA's mission is the responsibility to protect the public health through the safety, effectiveness, quality, and security of drugs, vaccines, and other biological products. As such, the co-signers of this letter uniformly agree that scientifically-sound communications about safe and effective uses of a product are essential. Yet, we also believe that the FDA can maintain the gold standard of patient safety, while also giving patients access to appropriate, evidence-based information necessary for them to make personalized choices through a shared decision making process with their health care provider. Without access to such vetted information, patients often seek out information from sources that are not validated or reliable. Likewise, unless a patient knows to ask their provider about off-label use of a product, there is no recourse other than simple internet searches and word-of-mouth, which can result in misinformation and confusion for patients.

The FDA has worked diligently to anchor their work around the patient, meaningfully incorporating patient perspectives into the drug approval process and creating a network to educate and involve patients more effectively in regulatory decisions. As the FDA reviews its guidances and policies around scientific communication, it is critical to understand that in accordance with this patient-centered approach, consumers deserve access to information gleaned from diverse data sources that both inform the scientific literature and reflect real-world evidence.

Under existing regulations, product labels do not accurately reflect the current body of knowledge on any particular product or device. While we agree that information garnered from controlled, clinical research should be foundational to all communications, it is also vital to take into consideration that for a variety of scientifically sound reasons approximately 40% of prescribing decisions are currently off-label.ⁱ These decisions include the use of an approved drug for an off-label clinical indication, use at a dosage outside of the label's indication, or use as first-line therapy in narrowly defined clinical populations with limited options.ⁱⁱ

In this era of increasingly personalized medicine, it is vital that health care providers and patients have the autonomy to incorporate rigorous scientific evidence from a multitude of sources to best inform treatment decisions that are unique to the individual. This evidence should include peer-reviewed literature, professional guidelines, real-world information gleaned from trusted datasets, broad patient-reported outcomes, and additional clinical effectiveness data. Further, these data sources should be flexible and evolve to reflect our constantly advancing healthcare ecosystem.

Finally, information must be communicated proactively and tailored to meet the needs of different audiences. Health care professionals should receive detailed, accurate information for each different diagnosis and indication to help guide decision making outside of a specific specialty. Payers need frequently updated messaging so that drugs and devices can be appropriately added or removed from formularies. Data shared with payers should include changes in efficacy and population scope in addition to data that supports new indications. Additionally, providing payers with economic data that has been published in a peer-reviewed journal will allow payers to develop evidence-based programs to best meet the needs of their constituents. Finally, patients and caregivers need access to scientifically-sound, yet understandable information that is tailored to their unique needs.

We commend the FDA for taking this initial step in addressing the detrimental impacts that out-of-date labels, and the inability to communicate new discoveries outside of that label, have on patient care. As members of the patient advocacy community, we are committed to ensuring patient safety. However, we are also dedicated to supporting the needs of patients diagnosed with chronic, life-threatening, and/or rare diseases, for which treatment options may be limited. The current restrictions on communications of off-label information may be intended to protect patient safety but, in certain cases, it limits the ability of many patients to learn about, understand, and access vital treatments and therapies. There must be more flexibility and opportunities to proactively share clinical and research findings from diverse data sources beyond the label.

Again, we appreciate the opportunity to submit these comments. The undersigned organizations stand ready to serve as a resource to the FDA as we collectively seek to protect patients while also elevating their voices to inform evolving policy decisions.

Sincerely,

Arthritis Foundation
Cancer Support Community
Leukemia & Lymphoma Society
Lupus Foundation of America
Musella Foundation for Brain Tumor Research & Information, Inc.
National Alliance on Mental Illness
National Organization for Rare Disorders
Oncology Nursing Society

Contact: Kristen Santiago, Cancer Support Community, kristen@cancersupportcommunity.org

ⁱ Riley, J.B., Basilius, P.A. (2007). Physicians' liability for off-label prescriptions. *Hematology and Oncology News and Issues*, 6(5), 24-37.

ⁱⁱ Duke-Margolis Center for Health Policy. (2016). *Policy options for off-label communication: Supporting better information, better evidence, and better care*. Retrieved from https://healthpolicy.duke.edu/sites/default/files/atoms/files/off-label_comms_white_paper.pdf.