



BONNIE J. ADDARIO  
**LUNG CANCER**  
FOUNDATION



SAVING LIVES AND ADVANCING  
RESEARCH BY EMPOWERING  
THOSE LIVING WITH AND  
AT RISK FOR LUNG CANCER.

[LungCancerAlliance.org](http://LungCancerAlliance.org)

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Center for Devices and Radiological Health (FDA-2018-N-3405)  
Food and Drug Administration  
10903 New Hampshire Ave., Bldg. 66, Rm. 5441  
Silver Spring, MD 20993-0002  
(Submitted electronically: [Letise.Williams@fda.hhs.gov](mailto:Letise.Williams@fda.hhs.gov))

**Re: (Docket No.: FDA – 2018-N-3405) U.S. Food and Drug Administration Patient Engagement Advisory Committee; Notice of Meeting on Utilizing Real-World Data from Patient-driven Platforms that Connect and Empower Patients; Comments – Addario Lung Cancer Foundation & Lung Cancer Alliance**

Dear Sir or Madam:

The Bonnie J. Addario Lung Cancer Foundation (ALCF) and Lung Cancer Alliance (LCA) appreciate the opportunity to provide comments on the U.S. Food and Drug Administration (FDA) Patient Engagement Advisory Committee meeting on utilizing real-world data from patient-driven platforms that connect and empower patients. ALCF and LCA are international and national patient advocacy groups, representing lung cancer patients, survivors, caregivers, and medical professionals improving care, education, and research to help save lives and address the leading cause of cancer death - nearly a third of this nation's total cancer mortality burden. Our joint societies applaud the FDA in their patient engagement regarding the utility of real-world data within the context of medical devices, regulation of devices, and their use by patients.

Provided below are the ALCF and LCA comments regarding the areas currently underway and our experiences with e-platforms, social media, registry, and effective digital reach in harnessing real-world data and the development of resources based on qualitative and quantitative analysis. Our joint organizations have seen first-hand the usefulness of real-world data and support the FDA implementation of post-market surveillance (PMS) data/real-world data.

As the FDA may be aware, there are numerous message boards and online communities representing a variety of patient populations whose insight and experiences are being collected and evaluated in a quantitative way. Our organizations continue to utilize social media platforms, including Facebook, Twitter, and apps to reach the specific patient and caregiver populations. We generate survey tools that capture valuable information with both short and longer-term experiences among targeted groups (e.g., targeted therapies and other lung cancer treatments). Using patient and caregiver reported experiences to drive and inform development of educational tools has resulted in better management of symptoms and side effects. In addition, the patient reported information on a variety of targeted treatments, their experiences with side effects, etc.; help address quality of life issues around different treatment modalities.

A specific and robust example of data registry and patient and provider reported outcomes, is currently achieved through the Lung Cancer Patient Registry (LCR) sponsored by the Bonnie J. Addario Lung Cancer Foundation (ALCF), the American Lung Association's LUNG FORCE (ALA) and the International Association for the Study of Lung Cancer (IASLC). Provided below is an overview, background, goals and objectives, target audience, timeline, examples of how the registry is being used, research underway, as well as the current and future registry status.

### **The Lung Cancer Patient Registry**

**Overview:** The Lung Cancer Patient Registry (LCR) sponsored by the Bonnie J. Addario Lung Cancer Foundation (ALCF), the American Lung Association's LUNG FORCE (ALA) and the International Association for the Study of Lung Cancer (IASLC), is an organized system to directly involve patients in the collection of their disease data and engage the medical community in the analysis and use of this data to improve patient care. It is an example of a platform that is a rich source of patient-generated health data, which could be used in some cases as real-world evidence to help protect public health and promote health care innovation.

By creating a centralized registry of robust, on-going data to which patients, healthcare professionals, researchers, industry, policy makers, etc. have access, the LCR provides the platform to:

- support and facilitate improved disease management and standard of care;
- evaluate the clinical and cost effectiveness of different treatments, products, and services;
- measure patient outcomes to assess the quality and efficacy of health care provided;
- use aggregate data as a tool to find patterns to better predict the medical future and lead to improved outcomes and quality of life for patients over time. Trends not previously known will be a door-opener to discuss access and to see if certain populations are receiving sub-optimal care compared to others.

The LCR was created by ALCF to ensure that a high-impact source of information is available to all patients and the medical community. The Registry is a critical tool for efficiently delivering quality care, improving well-being, saving lives, and effectively managing resources.

### **Background:**

Decision makers in research, industry, policy and health care settings are actively seeking robust sources of patient data to inform their practices. The Registry is ideally positioned to capitalize on this growing interest by building a high-impact source of patient information. Registries give patients a direct means to participate in the care continuum, leveraging their input and insights to focus priorities and improve outcomes. Beyond measuring trends in care, The LCR will help improve our ability to communicate with patients by establishing a direct communication network.

In more general terms, a patient registry is a valuable tool for:

- effectively managing resources, identifying new approaches, promoting innovation, and recognizing barriers
- Improving patient well-being and saving lives
- Saving money by improving processes
- Efficiently delivering quality patient care

**Goals/Objectives:** Most discussions of disease registries portray them primarily as repositories of data useful for outcome research. This description is accurate as far as it goes, but we need to take a broader view. We see The LCR not only as a system for the collection and analysis of data but also as an important catalyst for efforts to improve patient outcomes over time.

Our goals are to:

- Identify variations in care and outcomes to benchmark and assess comparative performance at a particular clinic or health system, as well as at regional, national, or even international levels;
- Identify best practices based on in-depth analysis of causes behind differences in performance and actively disseminate best practices to enable their adoption and reduce differences in care over time;
- Measure outcomes based on patient characteristics, such as stage at diagnosis, histology, etc.; Identify unmet medical needs, quality of life, access, and health status issues.
- Use comparative data to lower total health care costs and improve efficiency and efficacy.
- As personalized medicine rapidly becomes a reality in lung cancer, we seek to understand and measure survivorship as lung cancer becomes a chronically managed disease.

The Registry is based on the idea that to improve patient outcomes and quality of life, no silos can exist, collaboration is critical, and there must be open and shared access to all information. By putting this information in the hands of researchers, clinicians, industry, health IT companies, The Registry will organize and engage the medical community around the common goal of better care for lung cancer patients.

**Target Audience:** Patients, health care centers, industry, cooperative groups, CROs, medical professionals, other advocacy organizations, etc. worldwide; ALCF Lung Cancer *Centers of Excellence* (COEs) at community hospitals nationwide. By end of year 2020, our goal is to have 3,000 patients registered worldwide.

**Timeline/Important Dates:** The LCR was launched in Nov. 2016

**Health of the LCR:** through 9/2018 1104 participants and over 850 completed “Lung Cancer Patient Survey’s” and 156 completed “ALCF Immunotherapy Survey’s.”

**Examples of how the Registry is being used:**

At the highest level, the registry lung cancer survey is used to capture information on demographics, symptoms, diagnosis, testing, current treatment, prior treatment, and other areas. This includes information on a macro level about medical devices used to diagnose and treat lung cancer such as types of diagnostic tests (e.g. MRI, CT, Pet/CT, x-ray etc.), treatments with radiation therapy (e.g. IMRT, IGRT, SBRT etc.) and types of molecular testing (e.g. lung cancer specific mutation panel, next generation sequencing).

**Research conducted in the LCR:** The LCR and its partners are joining Adam Dicker, M.D., Ph.D. at Sidney Kimmel Cancer Center, Thomas Jefferson University and Heather Jim, Ph.D. at H. Lee Moffitt Cancer Center and Research Institute, Inc., in a study to learn about side effects of immunotherapy from patients with non-small cell lung cancer (NSCLC) who have undergone therapy with immune checkpoint inhibitors. In addition, the Society for Immunotherapy of Cancer (SITC) has joined the collaboration as a

project partner to maximize awareness of the registry and this research study to both researchers and patients.

Immune checkpoint inhibitors, a form of immunotherapy, have been shown to bring about durable remissions and prolonged survival for patients with NSCLC, but at the cost of toxicity that causes a range of side effects. The goal of this study is to gather information directly from patients and caregivers to better understand what side effects patients have experienced when side effect symptoms began and how side effects have impacted the patients' quality of life. Results from the study will give doctors a greater understanding of how immune checkpoint inhibitor toxicities affect patients and allow them to better inform patients considering immunotherapy treatment for NSCLC.

Nearly a quarter million Americans will be diagnosed with lung cancer this year. Immunotherapy, along with targeted therapy, has helped transform the treatment of lung cancer over the past decade. Gathering information from patients and passing that knowledge on to other lung cancer patients accurately and quickly can help patients live longer, which is our goal.

The study represents the first collaborative effort since the ALCF and the American Lung Association joined forces with the IASLC in December 2017 to expand the Lung Cancer Patient Registry. This study opportunity puts the LCR to innovative use. The patient-provided data used in this patient-driven research will allow us to gain knowledge from patients directly and better inform treatment. Patient-reported outcomes (PRO's) can help show clinical benefit in reducing disease related symptoms, provide more accurate estimates of toxicity, help model treatment costs and improve symptom management. These toxicities really do have costs, and it's important for patients to know how much out-of-pocket costs they might incur.

**Current Status:** ALCF Immunotherapy survey – 155 completed surveys through 10/15/2018; The abstract "Patient-reported toxicities in lung cancer patients receiving immune checkpoint blockade" has been accepted for poster presentation at the Society for Immunotherapy of Cancer (SITC) upcoming 33<sup>rd</sup> Annual Meeting which will be held November 7-11, 2018 in Washington, DC.

P630	Patient-reported toxicities in lung cancer patients receiving immune checkpoint blockade	Heather Jim, PhD; Sandra S. Shaw, BS, MBA; Sarah Eisel, PhD; Aasha I. Hoogland, PhD; David LeDuc, CFRE; Adam P. Dicker, MD, PhD	Immune toxicity; Solid tumors
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**Future State:** Currently the LCR has built a foundation that enables the collection of survey data, demographic data, information on medical providers, and uploading of medical reports and health care records. We imagine a future state would enable integration of EHR data, and additional layers of data sets provided by wearables, mobile devices, and other mobile technology. Innovation will be key to evolving patient reported data sources and the LCR into even more powerful and relevant source of data provided by patients and consumers to all interested stakeholders.

## Patient Reported Outcomes

On August 22, 2018, the Centers for Medicare and Medicaid Services (CMS) convened a Medicare Evidence Development Coverage Advisory Committee (MedCAC) meeting, and panelists heard from CAR T therapy drug makers, health researchers, and policymakers. MedCAC was charged to make recommendations to CMS CAG on how CMS should incorporate patient reported outcomes in future decisions about clinical studies and coverage of CAR T–cell therapy for the Medicare population (those with advanced leukemia and lymphoma).

The majority of MedCAC votes were in favor (i.e., degree of confidence) on 23 questions regarding the requirement of patient reported outcomes (PRO) data tied to national coverage decisions (similar to Coverage with Evidence Development and requirement of registry) specific to the CAR T therapy National Coverage Analysis (NCA). These MedCAC questions and votes were also made within the broader context of oncology, as instructed by Tamara Syrek-Jensen (CAG Director) who clarified that CMS is interested in using required PRO data more generally to other technologies.

MedCAC panelists were concerned that more context and disease-specific questions would need to be present in order to answer the requirement of PRO data with other technologies appropriately. However, the MedCAC Chair instructed panelists to vote on the requirement of PRO data for both CAR T therapy NCA and generalizable to oncology studies.

Novartis used FACT PRO in the registration Phase II JULIET trial of Kymriah to treat diffuse large B cell lymphoma (DLBCL). Kite is using the EORTC tool in its Phase III Zuma-7 trial of Yescarta in second-line DLBCL. Yescarta is approved in patients with aggressive non-Hodgkin lymphoma who have failed at least two prior lines of treatment, and the company indicated it is working with the FDA to identify PROs that can be collected from its patient registry of Yescarta.

We bring this to your attention, as patient reported outcomes (PRO) data are under review by other government agencies (CMS) in addition to the FDA and shared public comments may be helpful. While we see this as an innovative step to help evaluate real-world data in the form of health information etc., stakeholders also cautioned CMS. While PRO data are important, standardization and other work may need to be determined regarding what data should be refined and best to capture as well as duration and a defined/standard system, etc. Bulleted below are areas highlighted by the public comments for thoughtful MedCAC consideration:

- refinement and standardization;
- funding and costs associated with data collection;
- tracking of patient outcomes more easily and accurately;
- patient preference dialogue;
- addressing a population with unbiased education;
- implementation with consideration of burden and survey fatigue;
- meaningful durations (durability and longer-term outcomes);
- iterative and comparative cross product consideration;
- a framework to reduce barriers with enrollment and uptake;
- heterogeneity, generalizability; and
- careful attention to unintended consequences with a transparent and iterative process for refinement

## Summary

In summary and with careful and thoughtful implementation, real-world data and leveraging e-platforms can help fill gaps in an evolving and advancing health information field to inform and move new technologies and medical devices forward in a safe and effective way. ALCF and the LCA applaud the FDA for using PMS data and developing a patient centered committee to consider emerging e-platforms for better patient and consumer engagement as empowered partners in the work of protecting public health and promoting responsible innovation. Our joint organizations support the use of real-world data and strongly believe it improves the field and helps make incremental gains for the future, as it brings texture and color to patient care and management. We also recommend thoughtful consideration of the public comment areas highlighted above in an effort to reduce burdens and avoid unintended consequences.

ALCF and LCA thank you for this opportunity to comment on this important FDA meeting. If you have any questions, please contact Anita McGlothlin at [amcglathlin@lungcanceralliance.org](mailto:amcglathlin@lungcanceralliance.org).

Sincerely,



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