CANCER SUPPORT COMMUNITY
A Global Network of Education and Hope

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United States House of Representatives The Energy and Commerce Committee
Hearing: Examining Medical Product Manufacturer Communications
July 12, 2017
Written Remarks Submitted by: Linda House, MSM, BSN, RN,
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Good morning, my name is Linda House, and I am the President of the Cancer Support Community (CSC) global headquarters. Thank you to Chairman Walden, Vice Chairman Barton, Ranking Member Pallone, and all of the members of the Energy and Commerce Committee for allowing me to join you today and offer this testimony.

The Cancer Support Community is an international nonprofit organization whose mission is to ensure that all people impacted by cancer are empowered by knowledge, strengthened by action and sustained by community. CSC serves over 100,000 patients and families annually through a network of 150 affiliate sites and satellite locations across the country as well as through the *Cancer Support Helpline* where patients and their families receive evidence-based programming and social and emotional support. The CSC network delivers close to $50 million in services, free of charge, each year. CSC is also home to the only *Research and Training Institute* focused on collecting and analyzing data to understand and elevate the patient and caregiver voices about the cancer experience.

Thank you for the opportunity to speak with you this morning regarding medical product manufacturer communication.

The Patient Perspective

I am here today to bring you the most important voice in this discussion regarding medical product manufacturer communication—that of the patient. As you may have seen, CSC joined seven other national organizations to submit a response to the Food and Drug Administration's (FDA) recent guidance on this very important issue. We appreciate the FDA's efforts to provide additional guidance in this area and look forward to working with the Agency in the coming months and years to ensure its regulations reflect the best interests of patients.

The last twenty years have delivered unprecedented growth in innovation across all aspects of health care. Never before has the patient had so many options for diagnosis, treatment, and follow-up care. Patients are more engaged, educated, and empowered consumers of their health care than ever before. Yet, despite the emergence of patients as important players—and even leaders—of their care teams, accessibility to comprehensive information continues to be elusive.

The Cancer Support Community will release new survey data next week that outlines the patient experience with treatment decision-making among other factors associated with the cancer

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experience. In our research, we learned that nearly half of all the patients we surveyed made treatment decisions together with their care teams while only 8% leave their treatment decisions fully to their care team. Yet, the data also underscores that patients feel unprepared to make these choices with 1 in 4 reporting that they did not at all feel prepared to discuss treatment options with their physicians.

The data also reflects a growing concern about inadequate collection, reporting, and label updating of endpoints that are meaningful to patients. In our research, 93% of respondents considered quality of life as "very important" when weighing treatment options. Quality of life measured higher than length of life (79%), yet product labels continue to focus very little on fully measuring comprehensive quality of life metrics. Further, product labels almost never reflect updates when there are findings beyond the clinical trial setting including findings about long-term effects that would be meaningful to patients (e.g., neuropathy with chemotherapy administration). A system that does not proactively collect, publish, and share data poses a significant risk to patient care.

Current Limitations
As a starting point, I would like to ground us in a set of long-standing institutional as well as regulatory limitations that restrict the flow of information to patients.

At the core of the FDA's mission is the responsibility to protect the public health. We wholeheartedly support the FDA in this core mission and are eager to work with the agency to meaningfully meet this goal. However, current regulatory requirements create barriers for patients to secure a full complement of relevant and meaningful information on treatment options for their conditions.

"Pre-approval" information is when clinical data is available on a product prior to the product having an FDA label. According to the Pharmaceutical Research and Manufacturers of America (PhRMA), it currently takes an average of 10 to 15 years to bring a drug to market. During that time, much is learned about the way in which the drug works in the body, how the body responds to the drug, the accurate dose, the toxic dose, and the associated side effects. Yet, this treasure trove of information remains out of reach from individuals other than the sponsor and trial investigators. The proactive sharing of information learned during the development stage is subject to pre-approval promotion regulations of the FDA. This is problematic for many of the patients who are being asked to participate in the clinical development process of the 7,000 drugs in clinical development today. A patient's ability to learn about earlier phase information regarding safety or efficacy signals is severely limited. For example, just last week, I was approached on behalf of a patient who was asked to go on a phase II trial for a new drug for a certain type of lung cancer (alk +). Having my own questions about safety and efficacy in the earlier phases of development, including the therapeutic dose, why phase II instead of the phase III trial, I spent time searching publicly available documents. After spending well over 90

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minutes searching, I, an educated consumer, could find only 2 pieces of information that referenced safety and efficacy. The reality is that the early phase results are very promising and relevant to this patient, but they were not proactively shared with him at the time of the request to join the trial and finding them were not intuitive. I would also add that only one of the 2 pieces of information I found was published in a peer-reviewed forum.

Limiting communication of information to only that which is included in the product label poses significant challenges to patients. Product labels reflect information collected in the controlled clinical trials setting with specified patient populations. It is widely observed that the performance of a drug may be different once it is introduced into general use, which will likely be a broader, less-controlled population. CSC appreciates the work of the FDA and sponsors of phase IV studies, but also recognizes that these studies do not capture comprehensive data for the use of a product in the real world. Also, it is a rare occurrence for the label to be updated in a manner that would allow for proactive communication about findings.

Additionally, data may be accumulated through Investigator Initiated Trials (IITs) on diseases that would never reach the investment potential for registration and a label, yet the data may be extremely relevant for clinical care, both positive and negative. This information may never be communicated to clinicians and will almost certainly not be made available to patients who may benefit from the findings. One example of this is lupus treatments. According to the Lupus Foundation of America, there are 1.5 million Americans living with the disease. Yet according to the FDA's website, there are only 4 drugs approved to treat lupus—Aspirin in 1948 followed by corticosteroids (year not listed), a drug originally used as an anti-malarial (Plaquenil) in 1955 and, most recently, Benlysta in 2011. The reality for patients with lupus is that there are many treatments being used to treat their disease in an off-label manner. The lack of proactive communication on the safe and effective use of these "off-label" indications is arguably a patient safety and well-being issue. This example only begins to illustrate the challenges for any patient facing a rare condition.

Information learned outside of the clinical trial setting and not captured in the label can also have a true impact on the patient experience. I saw this a number of times in my role as a medical information administrator for a large pharmaceutical company. In that role, I was charged with answering unsolicited medical questions about a product approved for certain cancers. There are two situations that I will always carry with me to illustrate this. First, I received several reports each week from the sales representatives stating that their customers were repeatedly saying that patients were complaining about burning at the injection site when the intravenous medication was started. Upon investigating the potential issue, I found that when reconstituted, the pH of the product was acidic (around 2.7 to 3.3) which caused burning when coming in contact with the vein wall. A simple solution to this was to place a warm compress at the injection site prior to turning on the infusion. Yet, because this solution was not a part of the product label, it could not be proactively shared with patients or clinicians. The second example dealt with fatigue

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experienced by patients in the immediate 24 to 48 hours post administration. There was a small, published study that demonstrated the administration of low-dose steroid at 8, 16, and 24 hours post administration significantly reduced the patient experience with fatigue. Again, this was not a part of the label and could never be shared proactively with patients and clinicians. Both had a significant impact on patient quality of life.

There are several elements in general clinical practice that contribute to the limitations of patients having access to comprehensive information through their health care team. CSC acknowledges that these may be collateral effects versus the withholding of information, but the outcomes for patients are real nonetheless. The active evolution of the care delivery system from volume to value has brought with it efficiency and cost-containment strategies that focus treatment decisions on a limited selection. One example is institution or system level formularies where hospitals or large systems (e.g., the Veterans Administration) have a limited list of treatment options available for their health care providers to consider as they make treatment decisions for their patients. A second example is the implementation of clinical pathways which are based on scientific information but essentially limit treatment options for patient use. Even more concerning is that a clinical pathway used in one practice may be different than the pathway for the same disease used in another practice. The lack of transparency regarding the data used to make formulary and pathway decisions coupled with the limited patient access to comprehensive information sets is simply unacceptable as we move towards patient centricity as the gold standard.

Another limitation is the inconsistent practice and reinforcement of publishing clinical trial results in scientific journals and other databases (e.g., clinical trials.gov). Findings secured through the clinical trial process, whether positive or negative, may never become a part of a product label, but certainly offer meaningful contributions to the overall body of scientific knowledge. A study by Riveros and colleagues (2013) analyzed 600 trials with results posted on clinicaltrials.gov. They found that 50% of the trials did not have a corresponding published article. Even more alarming are the results found by Anderson and colleagues (2015) which looked at 13,327 trials that had terminated or completed between January 1, 2008 and August 31, 2012. Of the trials, 13.4% posted summary results within 12 months after trial completion, and only 38.3% reported results as of September 27, 2013. The findings from these trials offer meaningful information for patients and providers in both clinical practice and also as foundational knowledge for further drug development and clinical trial participation. There must not only be requirements but also enforcement of requirements to ensure that all results of all trials be posted and/or published within a reasonable period of time.

Finally, industry interpretation of the current regulations are applied inconsistently across companies. This may impact the way in which industry communicates with all stakeholders, but it almost universally results in industry choosing not to speak with the patient or family with the exception of direct-to-consumer advertising tactics. Guidance should be issued and enforced to

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allow patients and families to, at a minimum, secure answers to questions they may have about products they are taking. One very recent example was a call that I made to the medical information line of a pharmaceutical company to inquire about transferring a topical anesthetic from a very large and non-travel compliant tube to a smaller, travel compliant container. The question was whether the transfer from the original container would impact the integrity of the product. The response from the pharmaceutical company was that I would have to speak with my physician to secure the answer.

Recommendations

While these comments have simply scratched the surface on a much broader and deeper issue, it is my hope that I have highlighted in your mind the perspective of patients living with chronic and life-threatening illnesses across the United States. To summarize, the specific areas where we would like to partner and continue to advance the work of this committee, the FDA, and trial sponsors include:

1. Patients and health care providers must have access to medical research findings in a comprehensive and real-time manner.
2. Product labels should be updated in a timely manner and include data from endpoints that matter most to patients and/or there must be another mechanism by which to capture and proactively communicate findings that are clinically meaningful and relevant.
3. Scientifically sound communications about safe and effective uses of a product are essential and should be made available to all stakeholders.
4. Clinical trial data results, positive and negative, should be published by the trial sponsor in a period of time that is reasonable to allow full and meaningful data review while ensuring timely access to information.
5. Data, positive and negative, collected outside of the clinical trial process, inclusive of real-world evidence that is collected and analyzed with appropriate scientific rigor should be published and made available to stakeholders.
6. Proactive medical communication should be tailored to meet the needs and literacy levels of specific stakeholders and should not, for any stakeholder, be limited to only the product label which may not yet exist or be outdated. This includes physicians who are generalists and also specialists, allied health care providers (e.g., nurses and pharmacists), payers, and patients.

Thank you again for the opportunity to bring the patient voice to this important discussion. The Cancer Support Community along with many of our partners in the patient advocacy community stand ready to help improve patient and provider access to information that is vital to planning care for and improving outcomes for patients.

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