Energy and Commerce Committee
Subcommittee on Health
"21st Century Cures: Incorporating the Patient Perspective"
9:00 am, July 11, 2014
Testimony from
Richard F. Pops
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I would like to thank Chairman Pitts, Ranking Member Pallone and all of the members of this Committee for holding this hearing today, and for inviting me to testify. I also want to thank Chairman Upton and Congresswoman DeGette for spearheading the 21st Century Cures Initiative.

The simple and powerful concept of incorporating insights of patients, individually and collectively, throughout the continuum that begins with the discovery of new drugs and extends all the way to ensuring patients have access to them – is centrally important to the future of the life sciences industry – and one of the great opportunities for us to have a transformative impact on our nation's healthcare system. I appreciate the chance to be with you this morning, and look forward to working together to develop meaningful policy proposals to bring safe and effective new medicines to patients. This is an exciting time in both science and public policy, as the future holds the promise of dramatic advancement to improve the lives of patients and the potential to reduce the overall cost of care. This is due, in large part, to the Committee's efforts in starting the conversation and we must make the most of this opportunity.

I have served as the CEO of Alkermes for more than two decades. In that time, I have been able to participate in the emergence of the biotechnology industry – a dynamic, science and patient focused industry that has transformed the lives of millions. Our company develops innovative new medicines that address the unmet needs and challenges of people living with debilitating chronic diseases such as opioid addiction, schizophrenia and depression. Our growth has been achieved by staying true to our core mission of directing our world class scientific resources to patient-inspired treatment solutions with

practical, real-world benefits. This approach is entirely dependent on considering the patient perspective early and throughout our drug development process. I also serve on the boards of other biotechnology companies and as well as that of both BIO and PhRMA. This has afforded me the opportunity to be deeply engaged in the PDUFA V negotiations as well as the preparations ongoing for PDUFA VI, where consideration of elevating the patient voice has already emerged as a key theme.

Today, I would like to propose a new framework for patient involvement in developing new medicines. In order for this framework to be successful, it will require consistent and meaningful engagement between the *three* major parties involved: innovative biopharmaceutical companies, the Food and Drug Administration (FDA) and the patients who stand to benefit. It is based on three core principles:

i. These interactions, including outcomes and decisions resulting from greater patient inclusion, must be data-driven, based on sound science, separate from other powerful and passionate advocacy messages that patient groups deliver to policy-makers.

ii. The engagement framework, for seeking and incorporating patient input, must be actionable. It must improve the overall efficiency of drug discovery, development, and regulation, rather than adding new steps in, a process that is already highly complex. This is particularly important for the young biotech companies developing their first drugs on limited resources.

iii. This new approach must preserve and enhance FDA's gold standard of safety and efficacy. I believe deeply that increased patient input can co-exist with efficiency and the highest level of scientific rigor in the drug development process.

To date, patient input to industry has been somewhat limited, often simply in response to specific information requests. To my knowledge, there is no comprehensive, transparent and consistent process for integrating patient input broadly into industry decision-making. Many companies recognize the value of the patient perspective. In fact, they are restructuring to enhance their ability to effectively seek and

integrate input from patients and patient organizations. However, from a general industry perspective, there is no standardized way to solicit, receive, or respond to patient-generated input. Information gained through this type of exchange could have a meaningful impact on a range of critical decisions we make, including the selection of specific product candidates and the design of clinical trials. This is an important missing link that can have a profound impact on scientific opportunities.

At the FDA, patient engagement is not a new concept. Historically, the FDA has frequently focused on discrete regulatory questions and decisions, such as participating in an Advisory Committee meeting for a specific product application. However, several provisions included in the PDUFA V Goals Letter and FDASIA, such as a structured benefit-risk framework, patient-focused drug development, and the policies derived from the ExPERT Act, have resulted in meaningful new expansions of patient engagement. FDA has also been open to, and has taken initial steps to include, patient input into their reviews, which now must be translated into actionable, data-driven advice and guidance. These first steps are important, but they are just that – first steps. The proposed framework would build on the momentum from these policies and programs that currently exist.

As I mentioned previously, I believe the future of drug development will require consistent collaboration between the *three* parties: innovator companies, FDA, and the patients who are affected by diseases. The historical paradigm of drug regulation as a bi-lateral process between the FDA and industry has essentially become outdated. Science and society have continued to advance, and the relationship between the biopharmaceutical industry, FDA, and patients has evolved, while the existing regulatory framework lags behind. Patients are organizing in new ways and their critical role in driving innovation is becoming more the rule than the exception. We have a 20th century framework for 21st century drug development.

To tackle the increasingly complicated scientific and regulatory questions that lie ahead (in areas such as genetic disorders, cancer, chronic diseases, and rare disorders), all three parties must be engaged and working together throughout the drug development continuum. Moving forward, this data-driven, collaborative process in industry decision-making will be able to better explore ways to accelerate new drugs to market, by proposing meaningful improvements to the existing regulatory framework. These would include, but are not limited to:

i. Clinical trial design (including issues such as patient-reported outcome measures, biomarker validation, adaptive design, and endpoint selection);

ii. Clinical trial enrollment (including outreach efforts to identify patients and provide opportunities for them to enroll in clinical trials);

iii. FDA's methodology for benefit-risk assessment for new drug applications and disease indications; and

iv. Post-market data collection (including both safety and adverse events data, and identification of promising opportunities for study of new disease indications for approved products and next-generation product development).

These are incredibly exciting areas for consideration.

Successfully improving and integrating data-driven patient participation will require all parties – industry, regulators, and patients alike – to be equally committed to evolving the way we work together and enhancing our respective engagement capacities. This will allow us to collaborate consistently, transparently, and in an appropriately action-oriented way. We must all have skin in the game – responsibility to improve the efficiency of drug development to bring new products to patients sooner; and accountability to ensure products are safe and efficacious.

As we proceed, it is important to acknowledge there will be a number of challenges that will need to be addressed in order for this effort to succeed. While not an exhaustive list, these issues would include:

i. Data and Scientific Rigor – Interactions between FDA, industry, and patient groups must be grounded in a common standard for data and scientific rigor oriented toward informing specific process improvements. To do this, patient groups, industry, and the FDA must enhance our respective levels of sophistication and receptivity to meaningfully interact in a way that embraces scientific data. Likewise, data quality standards, best practices, and other operational guidelines will need to be developed to govern enhanced collaboration.

ii. Clinical Trial Regulations – Current rules can limit our ability to solicit and incorporate patient feedback and will need to be reevaluated to accommodate the new framework.

iii. Intellectual Property/Data Protection – Any approach to more extensive collaboration among industry, FDA, and patient groups must include a thoughtful approach to enhancing data-sharing, which is essential to enabling innovation, while providing adequate measures to protect and ensure the responsible use of proprietary data and intellectual property.

iv. Safety and Efficacy – We must constantly remind ourselves, and assure patients and payers, that nothing envisioned in this framework in any way undermines the existing FDA gold standard for safety and efficacy.

There is much to be done, and I cannot claim to have all the solutions. In fact, I believe the answers will be identified only through a collaborative, interactive process involving all parties. However, I am convinced we can accomplish something profoundly important by working together, through initiatives like the 21st Century Cures process, to develop a framework for enhanced patient participation in industry decision-making, based on sound scientific data and focused on actionable process improvements.

To that end, what might be the most appropriate next steps? First, starting immediately and moving forward, Congress, industry, FDA and patient groups should work together to develop and implement this new framework for thoughtful, data-driven interaction throughout the biopharmaceutical discovery, development, and delivery processes. As I mentioned earlier, we are not starting from scratch. This framework should build on existing patient-focused provisions of PDUFA V/FDASIA.

We should also undertake a thorough analysis of existing statutes and regulations to identify appropriate updates to encourage the kind of interaction and collaboration the framework is intended to facilitate. It is important to note, I do not think these changes to law and regulations would necessitate the creation of new programs or significant expansions of existing authority. Rather, I expect we would identify and address instances where existing requirements simply did not envision the possibility of the broad interaction we advocate today.

At its core, this proposal calls for collaboration to better access and apply the unique data and perspectives of the patient community to industry and regulatory decision-making, thereby enhancing our ability to deliver innovative new medicines to patients sooner. The concept of a new and comprehensive patient-inclusive framework is both ambitious and, at the same time, modest. Ambitious as it could result in a dramatic change to the way we discover and develop innovative medicines. Modest as it is not a new regulatory pathway or authority, but rather builds on a foundation already in place.

I stand ready, along with my team at Alkermes and colleagues in the biopharmaceutical industry, to work with you on this important effort. Once again, thank you for the opportunity to appear before you today. I would be happy to take questions from the members of the Committee.

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Summary of Testimony from
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We propose a new framework for patient involvement in developing medicines. In order for this framework to be successful, it will require consistent and meaningful engagement between the three major parties involved in the development of new medicines: innovative biopharmaceutical companies, the Food and Drug Administration (FDA) and the patients who will benefit from new therapeutic options. The new approach we envision is based on three core principles:

i. These interactions, including outcomes and decisions resulting from greater patient inclusion, must be data-driven, based on sound science, separate from other powerful and passionate advocacy messages that patient groups deliver to policy-makers.

ii. The engagement framework, for seeking and incorporating patient input, must be actionable. It must improve the overall efficiency of drug discovery, development, and regulation, rather than adding new steps in a process that is already highly complex. This is particularly important for the young biotech companies developing their first drugs on limited resources.

iii. This new approach must preserve and enhance FDA's gold standard of safety and efficacy. I believe deeply that increased patient input can co-exist with efficiency and the highest level of scientific rigor in the drug development process.

This framework is not without challenges. These include, but are not limited to:

i. An emphasis on data and scientific rigor,

ii. Further flexibility in clinical trial regulation,

iii. Assurances for intellectual property and data protection with any enhanced data-sharing, and;

iv. Evolving the regulatory framework for further collaboration while maintaining the existing safety and efficacy of FDA approvals.

Moving forward, Congress, industry, FDA and patient groups should work together to develop and implement this new framework for thoughtful, data-driven interaction throughout the biopharmaceutical discovery, development, and delivery processes. This framework should build on existing patient-focused provisions of PDUFA V/FDASIA.

We should also undertake a thorough analysis of existing statutes and regulations to identify appropriate updates to encourage the kind of interaction and collaboration the framework is intended to facilitate. These changes to law and regulations would not inevitably necessitate the creation of new programs or significant expansions of existing authority. Instead we would identify and address instances where existing requirements simply did not envision the possibility of the broad interaction we advocate today.

At its core, the proposal is that we work together to better access and apply the unique data, experiences, and perspectives among the patient community in industry and regulatory decision-making, in order to enhance our ability to deliver innovative new medicines to patients sooner.