



National Health Council Statement for the Record

21st Century Cures Initiative Hearing – July 11, 2014

House Committee on Energy and Commerce

The National Health Council (NHC) is pleased to submit written comments for the July 11, 2014, House Committee on Energy and Commerce, Subcommittee on Health hearing on “21st Century Cures: Incorporating the Patient Perspective.”

The NHC is the only organization that brings together all segments of the health community to provide a united voice for people with chronic diseases and disabilities as well as their family caregivers. Made up of more than 100 national health-related organizations and businesses, its core membership includes the nation’s leading patient advocacy groups, which control its governance. Other members include professional societies and membership associations, nonprofit organizations with an interest in health, and major pharmaceutical, medical device, biotechnology, and insurance companies.

The NHC is deeply committed to promoting the development of new treatments that could enable people with chronic diseases or disabilities to live longer, healthier, and more robust lives. The magnitude of patient need is great.

More than 133 million Americans – over 40% of the U.S. population – live with a chronic disease or disability. But for many people there are no treatments and existing treatments work for only 50-75% of the patients who currently use them. There are limited treatment options for too many diseases and disabilities, including mental health ailments, neurological, autoimmune and many rare diseases, nor for the prevention of diseases and disabilities. Millions of patients struggle daily with Alpha-1, ALS, Alzheimer’s, epilepsy, lupus, mesothelioma, and multiple sclerosis waiting for new, better, or any treatments. The same applies for people living with thousands of other diseases.

Today, there is broad agreement on the need to incorporate patient perspectives into drug development. There is growing acknowledgement that patients can have important roles in the drug development process beyond their traditionally more passive role as research subjects.

The Patient-Centered Outcomes Research Institute (PCORI), which was created in 2010 by the Affordable Care Act, is showing that investing in patient engagement can be a path to generating research that is more useful for decision makers.

The Food and Drug Administration (FDA), too, initiated the Patient-Focused Drug Development (PFDD) program, which aims to collect information on patient and caregiver perspectives and preferences across 20 disease areas. The creation of PCORI and FDA's PFDD program are certainly important first steps toward advancing patient engagement but our work cannot end there.

The NHC is actively engaging with FDA as it looks to refine the agency's framework for assessing benefits and risks in drug approval to ensure it better reflects patient perspectives. We are also urging FDA to work with us and other stakeholders to use its PFDD program as a foundation for developing guidance on how patients can be engaged in the drug development process. The incorporation of the patient perspective related to benefits and risks, as well as the outcomes that are most meaningful to the ultimate end users of medical products will aid in our shared goal of delivering high quality medical to people with chronic diseases and disabilities. To ensure the most desirable outcomes, this engagement should occur throughout the entire continuum of the research and development process.¹

¹ See Exhibit A: National Health Council Diagram on Patient Engagement in the Drug Development Process

FDA can play an important role in encouraging sponsors to incorporate patient input, which we believe will ultimately enrich the FDA regulatory review and approval process and accelerate patient access to new treatments. FDA should work with health care stakeholders such as sponsors, patient organizations, health care providers, and researchers to develop guidance to address issues related to patient engagement. Areas in which guidance is needed is in:

- *Defining the patient community.* The patient voice is represented by a wide range of individuals and organizations. Often, the terminology used to describe the individuals and organizations that comprise what is known broadly as the patient community is inconsistent or fails to capture the distinctions among them. For example, patients, patient advocates, and consumers are sometimes used interchangeably or are grouped together and caregivers are often excluded altogether.
- *Describing meaningful engagement.* Patient engagement can represent a range of activities, from passive engagement (e.g., clinical trial participation) to more active participation (e.g., research development). Across this spectrum of activities, the elements that constitute or can help achieve meaningful engagement have yet to be clearly defined.
- *Developing a framework and methods for engaging patients.* A framework would offer a structure and process for patient engagement, as well as the expected outcomes of that engagement. While there are existing reviews of patient engagement methods, they have not yet been fully examined for their application to drug development. Guidance on appropriate methods is crucial to implementing a process for involving patients.
- *Identifying and removing barriers to meaningful engagement.* While many companies are actively soliciting input from patients, there are many barriers that must be addressed.

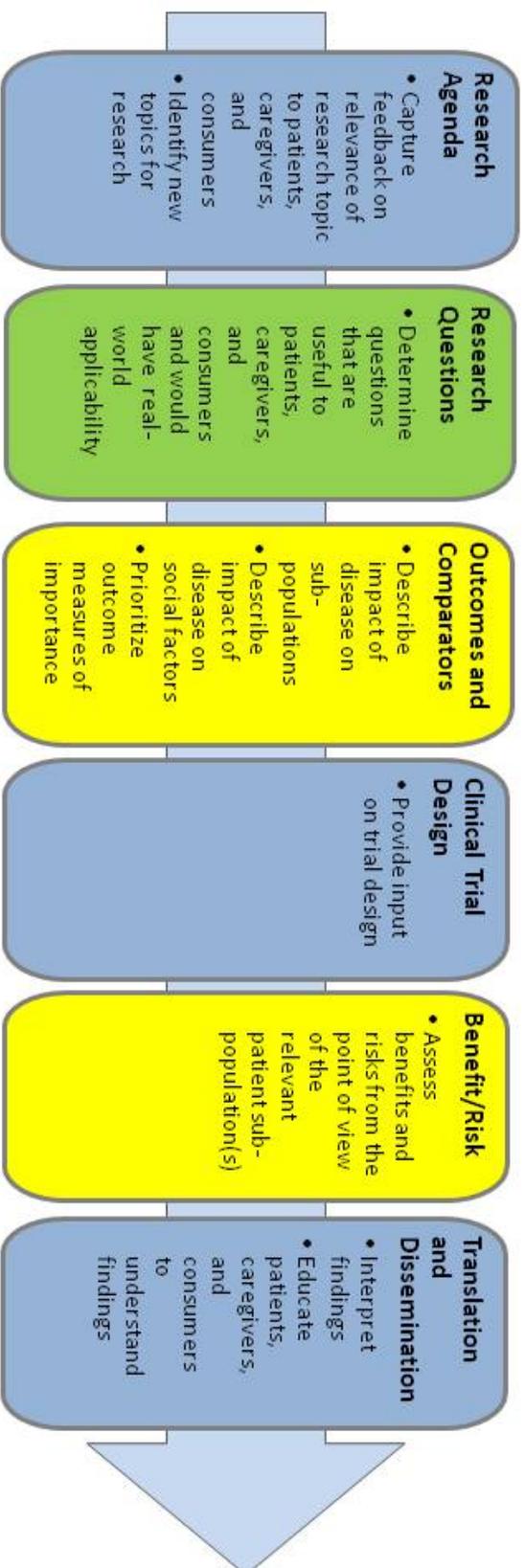
FDA should work with stakeholders to determine which barriers exist and ways to remove them to ensure that sponsors can engage with patients in an appropriate way to better understand the needs of patients.

We also recognize that in order for patient engagement in drug development to move forward on a large scale, the patient community must also be prepared to be active collaborators. To that end, the NHC has created a patient information collection tool to help patient advocacy organizations collect information on the impact of their disease or condition on quality of life, to better understand individual experiences with treatment regimens, and to determine what aspects of treatment or symptom relief are most important to patients. This information tool was originally created to support patient advocacy organizations preparing for FDA's PFDD meetings, but is emerging as an important tool for drug manufacturers in understanding the full scope of the patient experience with a disease.²

We would like to thank you for this opportunity to share our comments. We applaud the committee's efforts to strengthen the patient voice in the development of long awaited treatments and cures to help people with chronic conditions live longer and better lives. We look forward to working with you to find the best ways of ensure that patients are an integral component of the drug discovery, development, and regulatory processes.

² See Exhibit B: National Health Council Information Collection Tool for Patient Organizations

Patient Engagement in Drug Development



- Patient advocacy, caregiver, and consumer organizations
- Individual patients, caregivers, and consumers
- Both

Sources: The National Working Group on Evidence-based Health Care: The Role of the Patient/Consumer in Establishing a Dynamic Clinical Research Continuum: Models of Patient/Consumer Inclusion. August 2008; Mullins CD, Abdulhaini AAl, Lavallee D.C. Continuous Patient Engagement in Comparative Effectiveness Research. *JAMA*. 2012; 307(15):1587-1588. doi:10.1001/jama.2012.442



Exhibit A:



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Exhibit B:

Enhancing Benefit-Risk Assessments by Integrating Patient Perspectives: An Information Collection Tool for Patient Organizations

As required under the Prescription Drug User Fee Act V (PDUFA) reauthorization, the Food and Drug Administration (FDA) is developing a framework for conducting benefit-risk assessments.¹ To inform its work, FDA will be engaging patients, caregivers, and advocates to gather their perspectives and learn more about their specific needs. In particular, FDA will hold 20 public meetings over the next five years, each focusing on a different disease or condition. The aims of the meetings are to gather patient perspectives on the conditions' impact on quality of life, individual experiences with treatment regimens, and potential outcome measures in clinical studies. In addition, the FDA has repeatedly stated that these public meetings are not the only means to obtain patient input. Other methods include formal and informal meetings with FDA staff.

Patient Perspective and Disease Impact Stratification Tool

Goal: To help patient organizations ensure their communications with FDA regarding benefit-risk are comprehensive and, conversely, to help FDA capture the information they need from patients, caregivers, and patient advocates to inform their assessments of benefit-risk

Objective: To provide a way for patient groups to systematically organize issues, stratify their patient population, and identify key topics of focus in preparation for meetings with FDA

Rationale for this Tool: Patient populations affected by certain diseases are often very diverse and can span a wide array of demographics. Further, treatment options and patient needs often vary based on the stage or severity of the disease or condition. Recognizing and communicating these differences across subpopulations will help FDA better understand the varying levels of risk tolerance, from the perspective of both patients and caregivers, as well as where additional focus may be needed within a disease area or condition. This tool was created to help patient organizations collect and collate information that could ensure FDA has a comprehensive and inclusive picture of all affected patients of a disease. Those applying the tool should be mindful of potential variances between patient and caregiver needs and preferences, as well as ensure that information from hard-to-reach populations is captured.

How to Use the Tool: The tool consists of three sections: (I) Identification of Subpopulations; (II) Description of Disease Impact; (III) Description of Treatment and Management Options.

To complete Section I:

- 1) Identify patient subgroups within the broader patient population impacted by the disease

To complete Section II:

- 1) Describe how the disease is diagnosed and whether there are difficulties related to diagnosing the disease, such as delayed diagnosis or misdiagnosis.
- 2) Describe the characteristics of the disease, such as prevalence, symptoms, and comorbidities associated with the disease, and how they impact patient subpopulations.
- 3) Describe the impact of the disease and comorbidities on social factors that are of importance to patients and caregivers and on quality of life.
- 4) Identify outcome measures (clinical, patient identified, or patient reported) that are most relevant to patient/caregivers and would best address their needs and priorities.

¹ Passed as part of the 2012 Food and Drug Administration Safety and Innovation Act (FDASIA)

To complete Section III:

- 1) Describe the number of FDA-approved treatment and management options available for the specific subpopulation.
- 2) Describe the effectiveness of FDA-approved treatment and management options that have been used, if any, in treating or managing the disease for the specific subpopulation.
- 3) Describe the side effect profile and tolerability of current FDA-approved treatment and management options that have been used, if any, as they impact the specific subpopulation.
- 4) Describe the range of both FDA-approved and non-FDA approved treatment and management options used by the specific subpopulation for this disease.
- 5) Describe any barriers that may impact or impede patients' ability to access the necessary treatment and management options.

Definition of Terms

Child: Individuals under 18 years of age

Adult: Individuals 18 to 64 years of age

Elderly Adult: individuals 65 years of age and older

Mild: Disease or condition that does not interfere with daily activities

Moderate: Disease or condition that causes some limitations in daily activities

Severe: Disease or condition that has advanced beyond early stages or significantly impacts daily activities

End-of-Life: The health state of a patient in the end stages of a disease or condition

Treatment Options: Therapeutic options to treat a disease or condition with the goal of curing, slowing, or relieving symptoms of that disease or condition

Management Options: Therapeutic or non-therapeutic options to manage the symptoms and/or progression of a disease but not necessarily with a goal of curing that disease or condition

Glossary

Incidence: The number of newly diagnosed cases of a disease during a given period of time²

Prevalence: Total number of cases of disease existing in a population³

Mortality Rate: The number of deaths due to a disease divided by the total population⁴

Effectiveness: The drug or therapeutic treatment has shown therapeutic benefits based on information from laboratory studies, clinical trials, and real-world experience.^{5,6}

Heterogeneity: Refers to the phenomenon that people can respond differently to the same treatment⁷

Toxicity: The degree to which a medicine is poisonous; how much of a medicine can be taken before it has a toxic effect⁸

Safety: Therapeutic option is determined to be safe based on clinical trials in that the benefits outweigh risks⁹

Tolerability: the degree to which overt side effects can be tolerated by the person given the drug or therapeutic treatment¹⁰

Please send any comments to Eric Gascho, National Health Council Assistant Vice President of Government Affairs, at egascho@nhcouncil.org or 202-973-0545.

² <http://www.health.ny.gov/diseases/chronic/basicstat.htm>

³ Ibid.

⁴ Ibid.

⁵ <http://www.fda.gov/downloads/Drugs/.../Guidances/ucm078749.pdf>

⁶ http://www.stanford.edu/group/biodesign/regulatory/materials/safety_slides.pdf

⁷ <http://pcori.org/assets/MethodologyReport-Comment.pdf>

⁸ <http://www.medterms.com/script/main/art.asp?articlekey=34093>

⁹ http://www.stanford.edu/group/biodesign/regulatory/materials/safety_slides.pdf

¹⁰ <http://www.fda.gov/downloads/Drugs/GuidanceComplianceRegulatoryInformation/Guidances/ucm073137.pdf>

Patient Perspectives on Disease Impact and Treatment Options: An Information Collection Tool

I) Identification of Subpopulations	II) Description of Disease Diagnosis and Impact				III) Description of Treatment and Management Options				
1) Disease State and/or Stage Subpopulations	1) Diagnosis	2) Impact on Subpopulations	3) Impact on Social Factors	4) Outcome Measures	1) Availability of Treatment and Management Options	2) Effectiveness of Treatment and Management Options	3) Safety of Treatment and Management Options	4) Use of Treatment and Management Options	5) Access to Treatment and Management Options
<ul style="list-style-type: none"> Describe the impact of the disease on relevant specific subpopulations 	<ul style="list-style-type: none"> Describe how the disease is diagnosed. Describe the frequency and impact of misdiagnosis or delayed diagnosis. 	<ul style="list-style-type: none"> Describe the impact of the disease on the specific subpopulation. Describe the symptoms of the disease experienced in the specific subpopulation. Describe comorbidities associated with this disease for the specific subpopulation. 	<ul style="list-style-type: none"> Identify the social factors that are of importance to the specific subpopulation and describe the impact of the disease and comorbidities on those social factors. 	<ul style="list-style-type: none"> Identify outcome measures (patient identified or patient reported) that would best meet the patient/caregiver needs and priorities. 	<ul style="list-style-type: none"> Describe the number of FDA-approved treatment and management options available for the specific subpopulation. 	<ul style="list-style-type: none"> Describe the effectiveness of FDA-approved treatment and management options, if any, in addressing the disease for the specific subpopulation. 	<ul style="list-style-type: none"> Describe the safety of current FDA-approved treatment and management options, if any, as it impacts the specific subpopulation. 	<ul style="list-style-type: none"> Describe the range of treatment and management options used by the specific subpopulation for this disease (FDA-approved and non-FDA approved). 	<ul style="list-style-type: none"> Describe any barriers that may impact or impede patients' ability to access the necessary treatment and management options.
<ul style="list-style-type: none"> What are the subpopulations that are relevant to the condition? <ul style="list-style-type: none"> – Ages (Child Adult, Elderly Adult) – Severity of disease (Mild, Moderate, Severe, End-of-Life) – Other factors or predispositions (gender, race, occupation, etc) 	<ul style="list-style-type: none"> How is the disease diagnosed? <ul style="list-style-type: none"> • Do diagnostic tests exist? • Is misdiagnosis or delayed diagnosis common in the disease? • What impact does misdiagnosis or delayed diagnosis have on managing and treating the disease? 	<ul style="list-style-type: none"> What is the incidence of the disease (how many people are diagnosed with the disease annually)? <ul style="list-style-type: none"> • What is the prevalence of the disease (how many people are living with the disease)? • What are the mortality rates of the disease? • Does the disease impact differ across various groups within these subpopulations (e.g. racial/ethnic groups)? 	<ul style="list-style-type: none"> What are some of the social factors that may impact decisions regarding treatment, management, and/or outcomes of the disease for the specific subpopulation? (e.g., does geographical location or financial cost impact access to treatment? Does ability to work or care for family members impact treatment decisions?) <ul style="list-style-type: none"> • Does the role of family or caregivers in decision-making impact treatment decisions? 	<ul style="list-style-type: none"> Are there existing or potential measures that could effectively evaluate the following? <ul style="list-style-type: none"> – Function (e.g., ability to complete activities of daily living, including social interactions) – Quality-of-life – General health status 	<ul style="list-style-type: none"> How many FDA-approved treatment and management options are available for the subpopulation? What are the available FDA-approved treatment and management options? What are you currently using to help treat your condition or its symptoms? What specific symptoms are addressed? What specific symptoms are not addressed? 	<ul style="list-style-type: none"> Do the treatment or management options relieve symptoms, slow/modify the disease, or cure/prevent the disease? <ul style="list-style-type: none"> • How well do they work within the subpopulation? • How well does the current regimen treat the most significant symptoms of the disease? • Is there heterogeneity in treatment effect (in other words, do different patients respond differently to the same treatment)? 	<ul style="list-style-type: none"> Do the available treatment and management options have a lot of side effects? <ul style="list-style-type: none"> • What are the side effects (e.g., are they mild, toxic, etc.)? • What is the impact of these side effects on quality-of-life? • How do the impact of these side effects on functional capacity? • How do the treatment and management options affect daily life on the best days and worst days? 	<ul style="list-style-type: none"> What treatment and management options are currently being used by this subpopulation to help treat the condition or its symptoms (e.g., FDA-approved medicines, over-the-counter therapies, including non-drug therapies such as physical activity, homeopathic remedies)? <ul style="list-style-type: none"> • How has the treatment and management regimen changed over time and why? 	<ul style="list-style-type: none"> Are there any physical, regulatory, economic, or other barriers or potential barriers that could impede access to the treatment? <ul style="list-style-type: none"> • Are the treatment and management options currently placed under FDA's Risk Evaluation and Mitigation Strategy (REMS)? • Are the treatment and management options covered by appropriate public/private insurance? • If so, are any utilization management tools applied?
<ul style="list-style-type: none"> What are the other illnesses or conditions associated with the disease in this specific subpopulation? 	<ul style="list-style-type: none"> What are the most significant symptoms that result from the condition? What are the other illnesses or conditions associated with the disease in this specific subpopulation? 	<ul style="list-style-type: none"> What are the most significant symptoms that result from the condition? What are the other illnesses or conditions associated with the disease in this specific subpopulation? 	<ul style="list-style-type: none"> What are the most significant symptoms that result from the condition? What are the other illnesses or conditions associated with the disease in this specific subpopulation? 	<ul style="list-style-type: none"> What changes have patients had to make in their lives because of their condition? 	<ul style="list-style-type: none"> How does the condition affect daily life on the best days and worst days? How do the comorbidities affect daily life on the best days and worst days? What changes have patients had to make in their lives because of their condition? 	<ul style="list-style-type: none"> How does the condition affect daily life on the best days and worst days? How do the comorbidities affect daily life on the best days and worst days? What changes have patients had to make in their lives because of their condition? 	<ul style="list-style-type: none"> How does the condition affect daily life on the best days and worst days? How do the comorbidities affect daily life on the best days and worst days? What changes have patients had to make in their lives because of their condition? 	<ul style="list-style-type: none"> How does the condition affect daily life on the best days and worst days? How do the comorbidities affect daily life on the best days and worst days? What changes have patients had to make in their lives because of their condition? 	<ul style="list-style-type: none"> How does the condition affect daily life on the best days and worst days? How do the comorbidities affect daily life on the best days and worst days? What changes have patients had to make in their lives because of their condition?

Please send any comments to Eric Gascho, National Health Council Director of Government Affairs, at egascho@nhcouncil.org or 202-973-0645.



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