Patient Perspectives on Disease Impact and Treatment Options: A Stratification Tool

As required under the Prescription Drug User Fee Act V (PDUFA) reauthorization\(^1\), the Food and Drug Administration (FDA) is developing a framework for conducting benefit-risk assessments. 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 each patient subpopulation impacted by the disease based on age and disease progression or severity.

To complete Section II:

1) Describe how the disease is diagnosed and whether there are common delays in diagnosis or misdiagnoses.
2) Describe the characteristics of the disease, such as prevalence, symptoms, and comorbidities associated with the disease.
3) Identify the social factors that are of importance to patients and caregivers and describe the impact of the disease and comorbidities on those social factors.
4) Identify outcome measures (patient identified or patient reported) that would best meet patient/caregiver 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 options, if any, in treating or managing the disease for the specific subpopulation.
3) Describe the safety of current FDA-approved treatment options, 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 time¹  
**Prevalence**: Total number of cases of disease existing in a population²  
**Mortality Rate**: The number of deaths due to a disease divided by the total population³  
**Effectiveness**: The drug or therapeutic treatment has been shown in clinical trials to have clinically significant results⁴,⁵  
**Heterogeneity**: Refers to the phenomenon that people can respond differently to the same treatment⁶  
**Toxicity**: The degree to which a medicine is poisonous; how much of a medicine can be taken before it has a toxic effect⁷  
**Safety**: Therapeutic option is determined to be safe based on clinical trials in that the benefits outweigh risks⁸

Please send any comments to Eric Gascho, Assistant Vice President, Government Affairs, at egasco@nhcouncil.org or 202-973-0545.

### Potential Considerations

<table>
<thead>
<tr>
<th>Potential Considerations</th>
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<tbody>
<tr>
<td><strong>What are the subpopulations that are relevant to the condition?</strong></td>
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<tr>
<td>- Age (Child Adult; Elderly Adult)</td>
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<td>- Severity of disease (Mild, Moderate, Severe, End-of-Life)</td>
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<td>- Other factors or predispositions (gender, race, occupation, etc)</td>
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<td><strong>How is the disease diagnosed?</strong></td>
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<td>- Do diagnostic tests exist?</td>
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<td>- Is misdiagnosis or delayed diagnosis common in the disease?</td>
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<tr>
<td>- What impact does misdiagnosis and delayed diagnosis have on managing and treating the disease?</td>
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<td><strong>What is the incidence of the disease (how many people are diagnosed with the disease annually)?</strong></td>
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<td>- What is the prevalence of the disease (how many people are living with the disease)?</td>
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<td>- What are the mortality rates of the disease?</td>
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<td>- Does the disease impact different social groups within these subpopulations (e.g., racial/ethnic groups)?</td>
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<td>- What are the most significant symptoms that patients experience resulting from the condition?</td>
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<td>- What are the other illnesses or conditions associated with the disease in this specific subpopulation?</td>
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<tr>
<td><strong>What are some of the social factors that may impact decisions regarding treatment, management, and/or outcomes of the disease for the specific subpopulation?</strong> (e.g., does geographical location or financial cost impact access to treatment? Does ability to work or care for family members impact treatment decisions? Does the role of family or caregivers in decision-making impact treatment decisions?)</td>
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<tr>
<td><strong>Are there existing or potential measures that could effectively evaluate the following?</strong></td>
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<tr>
<td>- Symptoms: Function (e.g., ability to complete activities of daily living, including social interactions)</td>
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<td>- Quality-of-life: General health status</td>
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<tr>
<td>- How many FDA-approved treatment and management options are available for the subpopulation?</td>
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<tr>
<td>- Are the available FDA-approved treatment and management options slow/modify the disease, or cure/prevent the disease?</td>
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<tr>
<td>- What are you currently using to help treat your condition or its symptoms?</td>
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<td>- What specific symptoms are addressed?</td>
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<td>- What specific symptoms are not addressed?</td>
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<tr>
<td><strong>Do the treatment or management options relieve symptoms?</strong></td>
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<td>- How well do they work within the subpopulation?</td>
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<td>- How well does the current regimen treat the most significant symptoms of the disease?</td>
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<td>- Is there heterogeneity in treatment effect (in other words, do different patients respond differently to the same treatment)?</td>
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<tr>
<td><strong>Are there any barriers that may impact or impede patients ability to access the necessary treatment and management options?</strong></td>
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### I) Identification of Subpopulations

#### 1) Disease State and/or Stage

- **Description of the disease on relevant specific subpopulations**
  - Describe how the disease is diagnosed.
  - Describe the frequency and impact of misdiagnosis or delayed diagnosis.

#### 2) Impact on Subpopulations

- **Identify the social factors that are of importance to the specific subpopulation.**

#### 3) Impact on Social Factors

- **Identify outcomes measures (patient identified or patient reported) that would best meet patient/caregiver needs and priorities.**

#### 4) Outcome Measures

- **Describe the number of FDA-approved treatment and management options available for the specific subpopulation.**

### II) Description of Disease Diagnosis and Impact

#### 1) Diagnosis

- **Describe the disease and its impact on managing the disease?**

#### 2) Impact on Social Factors

- **Describe the symptoms associated with the disease in this specific subpopulation.**

#### 3) Impact on Social Factors

- **Describe the comorbidities associated with this disease for the specific subpopulation.**

### III) Description of Treatment and Management Options

#### 1) Availability of Treatment and Management Options

- **Describe the safety of current FDA-approved treatment and management options, if any, as it impacts the specific subpopulation.**

#### 2) Effectiveness of Treatment and Management Options

- **Describe the effectiveness of current FDA-approved treatment and management options.**

#### 3) Safety of Treatment and Management Options

- **Describe the safety of current FDA-approved treatment and management options, if any, in addressing the disease for the specific subpopulation.**

#### 4) Use of Treatment and Management Options

- **Describe the range of treatment and management options used by the specific subpopulation for this disease (FDA approved and non-FDA approved).**

#### 5) Access to Treatment and Management Options

- **Describe any barriers that may impact or impede patients’ ability to access the necessary treatment and management options.**

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