ISPOR
The professional society for health economics and outcomes research

Improving healthcare decisions

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Stephen Hahn, MD
Food and Drug Administration
10903 New Hampshire Ave
Silver Spring, MD 20993-0002

Dear Dr. Hahn,

ISPOR – the professional society for health economics and outcomes research – is pleased to respond on behalf of its membership to the U.S. Department of Health and Human Services Food and Drug Administration's call for comments on "Patient-Focused Drug Development: Methods to Identify What Is Important to Patients." We strongly agree that these are important issues to address with input from a wide variety of stakeholders and thank the Department for this opportunity to provide our comments.

ISPOR is a scientific and educational society with many of its members engaged in some aspect of health economics and outcomes research (HEOR) related to evaluation of pharmaceuticals. Our membership includes over 20,000 individuals across a range of disciplines, including health economics, epidemiology, public health, pharmaceutical administration, psychology, statistics, medicine, and more, from a variety of stakeholder perspectives, such as the life sciences industry, academia, research organizations, payers, patient groups, government (including some HHS/FDA employees), and health technology assessment bodies. The research and educational offerings presented at our conferences and in our journals are relevant to many of the issues and questions raised in this request for information.

This response was formulated with the assistance of ISPOR's most representative scientific membership groups: the Clinical Outcomes Assessment, Patient Centered, and Health Preference Research Special Interest Groups, as well as our Patient Representatives Roundtable and Institutional Council. We also polled our full membership for comments. It was reviewed by and approved by our current President and myself.

We include a few general comments on the following page. A full set of more specific comments is attached separately. We do encourage the FDA to review the additional comments, both general and specific, attached separately. We believe they make some important points for your consideration.

ISPOR appreciates the opportunity to comment on this guidance on behalf of its members and would be pleased to respond to any questions the FDA may have.

Sincerely yours,

Nancy S. Berg
CEO & Executive Director
ISPOR

1. The guidance acknowledges the role of both patient-reported outcomes (PRO) and patient preference information (PPI) data in identifying what is important to patients. However, after a brief reference to the CDRH guidance on methods for obtaining PPI data on benefit-risk tradeoff preferences there is no further mention of preferences in the document. While much of the content is relevant for both PRO and PPI approaches, the guidance would benefit from much more detail about relevant aspects of PRO and PPI assessments that are unique to each type of information. These unique aspects stem largely from the differences in the nature and objectives of PRO and PPI instruments. For more detail on this point, please see our specific comments for l. 62.

2. We urge you to state, early in the guidance, that "collecting" and "using" patient-generated health data should be undertaken with guidance or leadership from the patient community. The guidance should specify that, from the genesis of product development patient partners (leaders from patient advocacy organizations, individuals representing the condition in question, etc.) should be involved, helping to identify appropriate domains and methods for data collection, then assisting with interpretation and analysis of collected data, then with use and application of the data, and identifying methods for thanking and recognizing the patient communities who provided data. This may be one of the most important points to convey to the expert audience of this document.

3. While we recognize that this guidance is primarily intended to be descriptive about good methods in this area, many of your stakeholders would welcome more detail about specific metrics or criteria that will be applied to the methods used and data collected used when the FDA assesses the evidence presented to you. Any case examples that outline how you expect the methods to contribute to sound clinical outcomes assessment, endpoint development, or other information relevant to regulatory considerations that leverages patient-provided information and preferences would be most useful.

4. As it stands, this guidance, like the first guidance in this series, is an excellent introduction to these methods and establishes a framework providing an important common reference for these aspects of PFDD methods for both lay and expert audiences. However, it provides limited detailed guidance for experts in this area. If providing expert technical guidance is not the intent here, it may be useful to clarify the purpose of this document in that respect.