



DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2012-N-0408]

Risk Evaluation and Mitigation Strategy Assessments: Social Science Methodologies to Assess Goals Related to Knowledge; Public Workshop; Issue Paper

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public workshop; request for comments.

SUMMARY: The Food and Drug Administration (FDA) is announcing a public workshop entitled “Risk Evaluation and Mitigation Strategy Assessments: Social Science Methodologies to Assess Goals Related to Knowledge.” The purpose of the public workshop is to initiate constructive dialogue and information-sharing among regulators, researchers, the pharmaceutical industry, health care organizations, health care providers, and others from the general public about survey methodologies and instruments that can be used to evaluate patients’ and health care providers’ knowledge about the risks of drugs marketed with an approved Risk Evaluation and Mitigation Strategy (REMS). The input from this workshop will be used to develop guidance for industry describing the best practices for conducting an assessment of a REMS goal regarding patient and/or health care provider knowledge about a drug’s risk(s). To assist in the workshop discussion and the ultimate development of the guidance, FDA is making available an issue paper that discusses our experience with knowledge assessments for REMS and contains specific questions we hope to receive input on. FDA is also opening a public docket to receive written comments.

Date and Time: The public workshop will be held on June 7, 2012, from 8 a.m. to 5 p.m.

Location: The public workshop will be held at FDA's White Oak Campus, 10903 New Hampshire Ave., Building 31 Conference Center, the Great Room (rm. 1503), Silver Spring, MD 20993-0002. Entrance for the public workshop participants (non-FDA employees) is through Building 1, where routine security check procedures will be performed. For parking and security information, please refer to <http://www.fda.gov/AboutFDA/WorkingatFDA/BuildingsandFacilities/WhiteOakCampusInformation/ucm241740.htm>. Participants are encouraged to arrive early to ensure time for parking and security screening before the workshop.

Contact Person: Colleen O'Malley, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Bldg. 22, rm. 4305, Silver Spring, MD 20993-0002, 301-796-1786, FAX: 301-796-9832, email: colleen.omalley@fda.hhs.gov.

Registration and Requests for Oral Presentations: There is no fee to attend the workshop, and attendees who do not wish to make a formal presentation do not need to register. Seating will be on a first-come, first-served basis. Individuals who wish to make a presentation at the public workshop must register and provide an abstract of your presentation by 5 p.m. on May 21, 2012.

Submit electronic registration requests to make a presentation to KnowledgeAssessmentWorkshop@fda.hhs.gov. Submit written registration requests to make a presentation to Colleen O'Malley (see Contact Person). Please provide your name, title, business affiliation (if applicable), address, telephone, FAX number, and email address. Identify the Panel number(s) for the question(s) you will discuss in your presentation (see section IV of this document).

FDA will do its best to accommodate requests to speak. Individuals and organizations with common interests are urged to consolidate or coordinate their presentations and request time for a joint presentation. FDA will determine the amount of time allotted to each presenter and the approximate time that each oral presentation is scheduled to begin. Persons registered to make a formal presentation should check in before the workshop. Time will be allowed during the scheduled agenda for attendees to ask questions of the panelists. In addition, we strongly encourage electronic or written comments to the docket.

FDA has developed an issue paper entitled “Risk Evaluation and Mitigation Strategy Assessments: Social Science Methodologies to Assess Goals Related to Knowledge” that discusses our experience with knowledge assessments for REMS. The issue paper also contains a number of specific questions that we hope to receive input on. The issue paper can be found on the Internet at <http://www.fda.gov/Drugs/NewsEvents/ucm292337.htm>.

Background information on the public workshop, registration information, the agenda, and other relevant information will be posted on the Internet at <http://www.fda.gov/Drugs/NewsEvents/ucm132703.htm> as it becomes available.

If you need special accommodations due to a disability, please contact Colleen O’Malley (see Contact Person) at least 7 days before the workshop.

Comments: FDA is opening a docket to allow for public comments to be submitted to the Agency on the issues and questions presented in the issue paper or at the workshop. Regardless of attendance at the public workshop, interested persons may submit to the Division of Dockets Management either electronic or written comments by July 7, 2012, to receive consideration. Submit electronic comments to <http://www.regulations.gov>. Submit written comments to the Division of Dockets Management (HFA-305), Food and Drug Administration,

5630 Fishers Lane, rm. 1061, Rockville, MD 20852. It is only necessary to send one set of comments. Identify comments with the docket number found in brackets in the heading of this document. Received comments may be seen in the Division of Dockets Management between 9 a.m. and 4 p.m., Monday through Friday.

SUPPLEMENTARY INFORMATION:

I. Background

Title IX, Subtitle A, section 901 of the Food and Drug Administration Amendments Act (FDAAA) (Public Law 110-85)¹ created new section 505-1 of the Federal Food, Drug, and Cosmetic Act (FD&C Act) (21 U.S.C. 355-1), which authorizes FDA to require persons submitting new drug applications (NDAs) or abbreviated new drug applications (ANDAs) for prescription products, or biologics license applications (BLAs), to submit and implement a REMS if FDA determines that a REMS is necessary to ensure the benefits of a drug outweigh the risks of the drug. To require a REMS for an already approved drug, FDA must have become aware of new safety information as defined in the statute. Elements for REMS approved for NDAs and BLAs may include a Medication Guide, a communication plan, and/or elements to assure safe use (ETASU), and an implementation system, if specific statutory criteria are met. All approved REMS for products approved under an NDA or BLA must include a timetable for submission of assessments of the REMS. FDAAA contains provisions that are specifically directed to REMS for ANDAs, and these REMS may include only a Medication Guide and/or ETASU and an implementation system.

Because most REMS include a goal related to knowledge, such as to inform or educate patients and/or health care providers about the serious risks associated with and safe use of a

¹ See <http://www.gpo.gov/fdsys/pkg/PLAW-110publ85/pdf/PLAW-110publ85.pdf>.

drug, assessments for a drug subject to a REMS frequently include assessments of patients' and providers' knowledge. To conduct this assessment, most applicants have undertaken cross-sectional surveys of patients who have taken the drug and health care providers who have prescribed or dispensed the drug.

As a result of FDA's review of the surveys that are included as components of a REMS assessment, the Agency has identified certain challenges to conducting these types of studies. FDA has specific questions about the methodology for obtaining survey data and presenting the results, including about appropriate sample size; methods to ensure representativeness; how to determine endpoints; questionnaire design and analyses; and presentation of survey results.

To date, FDA has worked with individual applicants to attempt to introduce and sustain some measure of consistency in methods and expectations. Although absolute uniformity is not possible, the Agency seeks to solicit information and feedback about valid survey methods that can improve the quality and consistency of REMS assessment surveys. In addition, FDA seeks feedback on whether methodologies other than surveys could be used to obtain this information. Finally, FDA seeks to solicit information about using surveys other than knowledge assessment surveys as a tool to assess whether the elements of a REMS are meeting its goals including: (1) Changes in behavior for both patients and prescribers such as whether a drug is used to a large degree in patients at higher risk of an adverse reaction; (2) burden on the health care system, which could include the time required to accomplish REMS-related activities; and (3) adverse effects on patient access to the drug, such as substantial delays between the time of presentation of a prescription and the time of drug dispensing or prescribers choosing not to prescribe the drug anymore.

II. Why Are We Holding This Public Workshop?

FDA is soliciting information and feedback to optimize the assessment of REMS goals related to knowledge. Because we have received only surveys that assess knowledge, the workshop will invest considerable time in identifying best methodological practices for conducting REMS assessment surveys. However, FDA is also encouraging discussion of alternatives to surveys, given the issues we have observed, as discussed in the issue paper. Feedback received in the docket and resulting from this workshop will assist the Agency in developing guidance for industry.

The workshop objectives are as follows: (1) Initiate constructive dialogue and information-sharing about survey methodologies and instruments used to evaluate patients' and healthcare providers' knowledge about drugs' risks; (2) share current FDA experience regarding social science assessments of surveys as a component of REMS Assessment Plans; (3) obtain information that will be used to develop standardized survey methodologies for evaluating patient and health care provider knowledge under a REMS; (4) discuss alternative methodologies to surveys to assess knowledge; and (5) discuss the use of surveys as a tool to assess patient and prescriber behavior changes, burden on the health care system, and patient access to the drug under a REMS.

III. Who Is the Target Audience and Who Should Attend This Public Workshop?

Although the workshop is open to all interested parties, the target audience includes social science professionals; statisticians; regulators; researchers; and representatives from academia, the pharmaceutical industry, and the scientific community who may be interested in improving the quality and consistency of methodology for evaluating REMS goals related to knowledge.

IV. What Are the Topics We Intend to Discuss at the Public Workshop?

The workshop will include panel discussions and individual and/or joint presentations. The key issues to be addressed are: (1) How should assessments of knowledge be structured to achieve valid, reliable, and informative results; (2) how can surveys be used to assess changes in patient and prescriber behavior, burden to the health care system, and patient access to the drug; and (3) what are appropriate alternatives to surveys to assess educational components of REMS? Two panel discussions will focus on areas in which the Agency requests specific input.

- Panel 1 will focus on using surveys to assess knowledge. Topics will include, but are not limited to, recruiting a representative sample, sample size, question design, process, and endpoints.
- Panel 2 will focus on alternatives to surveys and the use of surveys to assess patient and prescriber behavior changes, burden on the health care system, and patient access to the drug. Topics will include, but are not limited to, recruiting a representative sample, question design, interpretation of results, and specific pros and cons of the alternatives.

V. Transcripts

Please be advised that as soon as a transcript of the workshop is available, it will be accessible at <http://www.regulations.gov>. It may be viewed at the Division of Dockets Management (see Comments). A transcript will also be available in either hardcopy or on CD-ROM, after submission of a Freedom of Information request. Written requests are to be sent to the Division of Freedom of Information (ELEM-1029), Food and Drug Administration, 12420 Parklawn Dr., Element Bldg., Rockville, MD 20857.

Dated: April 27, 2012.

Leslie Kux,

Assistant Commissioner for Policy.

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