



4164-01-P

DEPARTMENT OF HEALTH AND HUMAN SERVICES

Food and Drug Administration

[Docket No. FDA-2017-N-0001]

Food and Drug Administration, Center for Drug Evaluation and Research Rare Diseases Public Workshop: Strategies, Tools, and Best Practices for Effective Advocacy in Rare Diseases Drug Development

AGENCY: Food and Drug Administration, HHS.

ACTION: Notice of public workshop.

SUMMARY: The Food and Drug Administration (FDA), Center for Drug Evaluation and Research (CDER), is sponsoring a public workshop entitled “CDER Rare Diseases Public Workshop: Strategies, Tools, and Best Practices for Effective Advocacy in Rare Diseases Drug Development.” This public workshop builds upon previous CDER patient advocacy public workshops and is primarily for the rare disease community to help them effectively understand what FDA needs to enhance drug development. This effort is consistent with FDA’s efforts to support the integration of patient experience in drug development programs, including through implementation of the “Patient-Focused Drug Development” provisions of the 21st Century Cures Act (Cures Act). This public workshop will include case studies demonstrating the beneficial overlap of effective advocacy techniques and FDA regulations in rare disease drug development.

DATES: The public workshop will be held on October 30, 2017, from 8 a.m. to 5 p.m.

ADDRESSES: The public workshop will be held at FDA White Oak Campus, 10903 New Hampshire Ave., Bldg. 31 (the Great Room), Silver Spring, MD 20993. 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 <https://www.fda.gov/AboutFDA/WorkingatFDA/BuildingsandFacilities/WhiteOakCampusInformation/ucm241740.htm>.

FOR FURTHER INFORMATION CONTACT: Francis Kalush, Center for Drug Evaluation and Research, Food and Drug Administration, 10903 New Hampshire Ave., Silver Spring, MD 20993-0002, 301-796-5429, PASE-RARE-DISEASES@fda.hhs.gov

SUPPLEMENTARY INFORMATION: FDA is announcing a public workshop entitled “CDER Rare Diseases Public Workshop: Strategies, Tools, and Best Practices for Effective Advocacy in Rare Diseases Drug Development.” The purpose of the public workshop, consistent with FDA’s broad effort to more comprehensively include patients’ perspectives and experiences with a disease or condition in the drug development process, including through implementation of the “Patient-Focused Drug Development” provisions of the Cures Act, is to aid in bridging the gap between rare disease patients’ stories and data needed to support drug development. This public workshop will include presentations on strategies, tools, and best practices on key aspects of rare diseases drug development and engaging with FDA. There will be an opportunity for questions and answers following each presentation.

Registration: There is no registration fee to attend the public workshop. Early registration is recommended because seating is limited, and registration will be on a first-come, first-served basis. There will be no onsite registration. Persons interested in attending this public workshop must register online at <https://www.fda.gov/Drugs/NewsEvents/ucm565398.htm> before September 30, 2017. For those

without internet access, please contact Francis Kalush (see FOR FURTHER INFORMATION CONTACT) to register.

If you need special accommodations due to a disability, please contact Francis Kalush (see FOR FURTHER INFORMATION CONTACT) no later than October 23, 2017.

Transcripts: A transcript of the public workshop will be available for review at the Dockets Management Staff (HFA-305), Food and Drug Administration, 5630 Fishers Lane, rm. 1061, Rockville, MD 20852, and on the internet at <https://www.regulations.gov> approximately 30 days after the public workshop. Transcripts will also be available in either hard copy or on CD-ROM, after submission of a Freedom of Information request. The Freedom of Information office address is available on the Agency's website at <https://www.fda.gov>.

Dated: August 30, 2017.

Anna K. Abram,

Deputy Commissioner for Policy, Planning, Legislation, and Analysis.

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