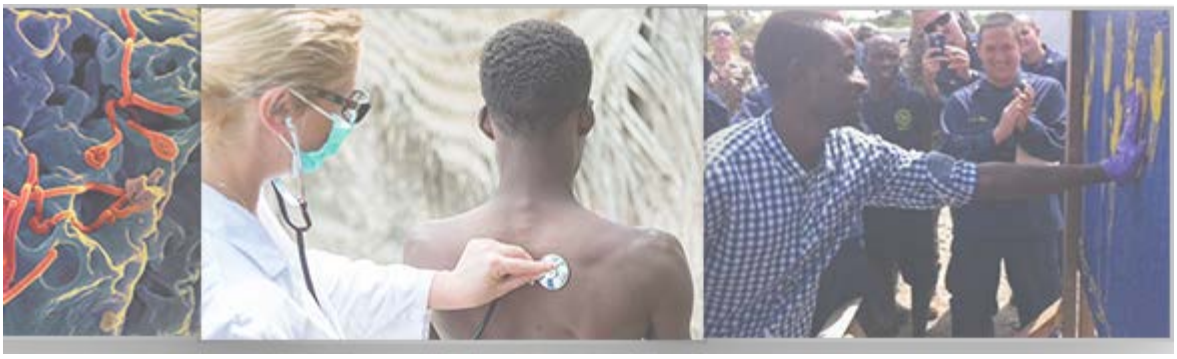
U.S. FOOD & DRUG ADMINISTRATION
Medical Countermeasures Initiative Update

August 30, 2018



A Flexible Clinical Trial Design Suitable for Emerging Disease Outbreaks

FDA CDER statisticians are designing trials with adaptive features to make clinical evaluation of new drug treatments more efficient and informative

In 2014, FDA Center for Drug Evaluation and Research (CDER) statisticians and their collaborators at NIH and in West Africa faced the challenge of designing a trial to assess treatments for Ebola virus that could be conducted in a medical emergency. In their design, they used a Bayesian approach that include options for adaptations of the trial due to rapidly changing events on the ground and newly acquired information about the disease.

Read more

Note: FDA is continuing our work to help expedite the development and availability of medical products – such as treatments, vaccines, diagnostic tests, and personal protective equipment – with the potential to help bring Ebola outbreaks under control as quickly as possible. You can find the latest at Ebola Preparedness and Response Updates from FDA.

Images (left to right): Scanning electron micrograph of Ebola virus (credit: NIAID); doctor checking patient vital signs; U.S. Public Health Service officers celebrate as a Liberian man adds his handprint to a "survivors' wall." Each patient who overcame Ebola after treatment at the USPHS mobile hospital outside Monrovia during the 2014-2015 Ebola epidemic was given a set of clothes and essentials and invited to mark their recovery with a handprint (photo: FDA)

Related links:

- Statistical Considerations for a Trial of Ebola Virus Disease Therapeutics, published in *Clinical Trials*, January 2016
- MCMi Extramural Research, including additional Ebola-related projects funded by FDA

FDA adds four tropical diseases to priority review voucher program to encourage drug development in areas of unmet need

FDA announced the addition of Lassa fever, chikungunya virus disease, rabies, and cryptococcal meningitis to the list of tropical diseases.

Applicants who submit applications for drug or biological products to prevent or treat these diseases may qualify for a tropical disease priority review voucher (PRV). A tropical disease PRV can be used to obtain priority review of a subsequent drug application that does not itself qualify for priority review. *Related: MCM priority review voucher program*

Expiration date extensions

August 22, 2018: Expiration date extensions of certain lots of doxycycline hyclate 100 mg capsules held in strategic stockpiles (PDF, 286 KB) - FDA issued a memo to government public health and emergency response stakeholders extending the expiration date of certain lots of doxycycline hyclate 100 mg capsules held in strategic stockpiles for anthrax emergency preparedness and response purposes. *Also see: Expiration Dating Extension*

August 21, 2018: FDA takes additional action to mitigate shortages of EpiPen by extending expiration date for specific lots of medication - FDA took additional action to mitigate shortages of EpiPen (epinephrine) auto-injector by extending the expiration date of specific lots of 0.3 milligram products marketed by Mylan by four months beyond the labeled expiration date. *(August 21, 2018) Also see: FDA approves first generic version of EpiPen (August 16, 2018)*

Events

- **September 4, 2018:** Facilitating Competition and Innovation in the Biological Products Marketplace (Silver Spring, MD) - FDA is announcing a public hearing on FDA's approach to enhancing competition and innovation in the biological products marketplace, including by facilitating greater availability of biosimilar and interchangeable products. Electronic or written comments will be accepted after the public hearing until **September 21, 2018.**
- **September 5, 2018:** N95 Day 2018 Webinar: Panel Discussion of Trending Topics on Respiratory Protection, 1:00 - 2:00 p.m. ET, hosted by CDC/NIOSH - NIOSH experts will share the science behind the established guidance and recommendations**.**
- **September 5-6, 2018:** Medical Product Shortages during Disasters: Opportunities to Predict, Prevent, and Respond - A Workshop (Washington, DC), hosted by the National Academies of Sciences, Engineering, and Medicine, and sponsored by the HHS Office of the Assistant Secretary of Preparedness and Response. To attend in-person, register in advance.
- **September 12, 2018:** Public hearing on FDA's Predictive Toxicology Roadmap (Silver Spring, MD and webcast) - FDA is seeking comments on how to foster the development and evaluation of emerging toxicological methods and new technologies and incorporate them into regulatory review, as applicable. *Note: registration deadline was August 29, 2018. Also see: FDA's Predictive Toxicology Roadmap (PDF, 2.2 MB)*
- **New! September 13, 2018:** FDA Grand Rounds webcast, 12:00 - 1:00 p.m. ET - Bisphenol A: Toxicology and Pharmacokinetic Data to Inform On-Going Safety Assessments, presented by K. Barry Delclos, PhD, Research Pharmacologist, Division of Biochemical Toxicology, FDA's National Center for Toxicological Research - register in advance
- **September 14, 2018:** Public workshop - Advancing the Development of Pediatric Therapeutics 5: Advancing Pediatric Pharmacovigilance (Silver Spring, MD and webcast) - To provide a forum to

gather information on the latest developments in pediatric pharmacovigilance from the perspective of various stakeholders and to expand the conversation to include the utility and challenges of emerging pharmacovigilance tools, including specific challenges associated with pediatric data tools. To attend in-person or by webcast, register by **September 6, 2018.**

- **New! September 17, 2018:** Science and Regulation of Live Microbiome-Based Products Used to Prevent, Treat, or Cure Diseases in Humans (Rockville, MD) - Public workshop to exchange information with the scientific community about the clinical, manufacturing, and regulatory considerations associated with live microbiome-based products, when administered to prevent, treat, or cure a disease or condition in humans. *Note: registration deadline was August 28, 2018. Also see: Statement from FDA Commissioner Scott Gottlieb, M.D., on advancing the science and regulation of live microbiome-based products used to prevent, treat, or cure diseases in humans*
- **New! October 3, 2018:** Vaccines and Related Biological Products Advisory Committee (VRBPAC) public meeting (Silver Spring, MD and webcast) - The VRBPAC will meet in an open session to discuss and make recommendations on the selection of strains to be included in an influenza virus vaccine for the 2019 southern hemisphere influenza season.
- **October 29-30, 2018:** Save the date for BARDA Industry Day (Washington, DC) - Engage and network with members of BARDA, ASPR and other government and industry stakeholders. Registration coming soon. BARDA invites pharmaceutical companies, biotech and other innovators to present a Lightning Talk. Apply by **September 7, 2018.**

Information for industry

- FDA is extending the proposal period for the Quality Metrics Site Visit Program for Center for Drug Evaluation and Research and Center for Biologics Evaluation and Research Staff. Submissions are now due **December 17, 2018**.
- FDA launches new pilot to advance innovative clinical trial designs as part of agency's broader program to modernize drug development and promote innovation in drugs targeted to unmet needs - Drug and biologic companies who participate in the Complex Innovative Designs Pilot Meeting Program will have additional opportunities to meet with agency staff to discuss the use of novel complex innovative trial designs (CID) for their clinical development programs. Complex innovative trial designs includes the use of seamless trial designs, modeling and simulations to assess trial operating characteristics, the use of biomarker enriched populations, complex adaptive designs, Bayesian models and other benefit-risk determinations, and other novel designs. The new program is aimed at helping to solidify the science used to support these novel approaches, and promote their adoption in drug development programs where these trial constructs can advance innovation. Sponsors may submit meeting requests for the pilot program through June 30, 2022.
- Reminder: Comments are due on the draft guidance for industry, Smallpox (Variola Virus) Infection: Developing Drugs for Treatment or Prevention (PDF, 120 KB) by **September 10, 2018**.

More: MCM-Related Guidance by Date

In case you missed it

- Reminder: Professional and citizen scientists are invited to test their bioinformatics skills and software tools in a challenge to identify pathogens from the FDA-ARGOS database within host samples using NGS short-read data as part of the precisionFDA CDRH Infectious Disease NGS Diagnostics Biothreat Challenge, open now through **October 4, 2018**.
- FDA is requesting nominations for voting members to serve on the Device Good Manufacturing Practice Advisory Committee and device panels of the Medical Devices Advisory Committee in the Center for Devices and Radiological Health (CDRH). Nominations received on or before **October 22, 2018** will be given first consideration.

- From DoD - Breakthrough in Malaria Prevention: USAMMDA Announces FDA Approval of New Antimalarial Drug - The U.S. Army Medical Materiel Development Activity (USAMMDA), Fort Detrick, Maryland, announced approval by FDA for the new antimalarial drug, tafenoquine (Arakoda). The new drug application for Arakoda, submitted by 60° Pharmaceuticals (60P) in partnership with the U.S. Army Medical Research and Materiel Command, received approval for the prevention of malaria, following priority review. This is the first new FDA-approved prophylactic drug for malaria in over 18 years. Tafenoquine (Arakoda) is an antimalarial indicated for the prophylaxis of malaria in patients aged 18 years and older. *Also see: approval letter (PDF, 43 KB) and product label (PDF, 278 KB) (August 10, 2018)*
- From HHS ASPR - BARDA and its partners bring new ammunition to the battle against superbugs - Since 2010, BARDA has worked with 12 private companies on developing 15 new antibiotics, and already three have earned approval by the FDA. These approvals, among the 40 approvals of BARDA-sponsored medical countermeasures, mark critical milestones in our nation's health security preparedness. *(August 27, 2018)*
- From NIH - NIH begins clinical trial of live, attenuated Zika vaccine - Vaccinations have begun in a first-in-human trial of an experimental live, attenuated Zika virus vaccine, known as rZIKV/D4Δ30-713, which was developed by scientists at the National Institute of Allergy and Infectious Diseases (NIAID), part of the National Institutes of Health. *(August 16, 2018)*
- You want to make a difference. FDA wants to hire you. Follow @FDAJobs on Twitter, or visit www.fda.gov/jobs.

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