PUBLIC HEALTH PREPAREDNESS

Medical Countermeasure Development for Certain Serious or Life-threatening Conditions

What GAO Found

The Department of Health and Human Services’ (HHS) Food and Drug Administration (FDA) established the Animal Rule in 2002 to allow for the approval of medical countermeasures based on animal efficacy studies when human clinical trials are not ethical or feasible. Medical countermeasures are medical products that may be used to prevent, treat, or mitigate potential health effects of exposure to chemical, biological, radiological, and nuclear (CBRN) agents. GAO found that FDA has undertaken efforts to provide information and feedback to developers to support medical countermeasure development under the Animal Rule. For example, in 2015 FDA issued guidance clarifying the types of studies and data needed to demonstrate product efficacy. FDA has approved 16 medical countermeasures under the Animal Rule, 14 of which were approved over the past decade.

Research of Biological Agents in a Clinical Laboratory

Source: Centers for Disease Control and Prevention. | GAO-22-105248

FDA established the Animal Model Qualification Program in 2011 to provide publicly available animal models to support efficacy testing under the Animal Rule for multiple medical countermeasures for a given disease or condition. Researchers and developers can submit models to the program for qualification, and, once qualified, a model can be used by other developers when appropriate. For example, an animal model for inhalation anthrax would include protocols, such as exposure timing and dosage, to produce disease manifestations that adequately reflect inhalation anthrax manifestations in humans. As of April 2022, FDA has qualified one animal model under the program. FDA officials and developers reported that the limited number of qualified models has not impeded product development, citing other ways to identify animal models that can be used for product development. FDA officials and others that GAO spoke with, including some developers and contract research organizations, said the program may still be beneficial. For example, FDA officials said the program could help further future development of medical countermeasures, particularly for CBRN agents that currently do not have approved medical countermeasures.