Frequently Asked Questions

What is the Center for Biologics Evaluation and Research (CBER)?

CBER is the Center within FDA that regulates biological products for human use under applicable federal laws, including the Public Health Service Act and the Federal Food, Drug and Cosmetic Act. CBER protects and advances the public health by ensuring that biological products are safe and effective and available to those who need them. CBER also provides the public with information to promote the safe and appropriate use of biological products.

What products does CBER regulate?

CBER regulates an array of diverse and complex biological products, both investigational and licensed, including:

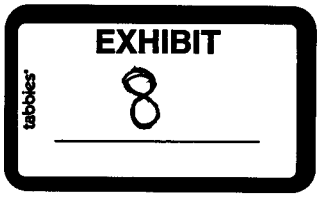
Additional FAQs

- **Allergenics**
  Patch tests used to diagnose the causes of contact dermatitis. Extracts used to diagnose and treat rhinitis ("hay fever"), allergic sinusitis and conjunctivitis, and bee stings.

- **Blood**
  Blood and blood components used for transfusion, such as red blood cells, plasma, and platelets. Pharmaceutical products made from blood, such as clotting factors and immunoglobuilns.

- **Devices**
  Medical devices and tests used to safeguard blood, blood components, and cellular products from HIV, hepatitis, syphilis, and other infectious agents. Reagents used to type blood. Machines and related software used to collect blood and blood components.

- **Gene Therapy**



Gene therapy products that replace a person's faulty or missing genetic material. Gene therapy research could lead to new treatments for cancer cystic fibrosis, heart disease, hemophilia, diabetes, and infectious diseases such as AIDS.

- **Human Tissues and Cellular Products**
  Human tissues for transplantation, such as skin, tendons, ligaments, and cartilage. Cellular products, such as human stem cells and pancreatic islets. Tissue and cellular products have the potential to treat cancer, Parkinson's disease, hemophilia, anemia, diabetes, and other serious conditions.

- **Vaccines**
  Vaccines used for the prevention of infectious diseases, such as mumps, measles, chicken pox, diphtheria, tetanus, influenza, hepatitis, smallpox, and anthrax. Vaccines under development to treat HIV, and to treat or prevent non-infectious conditions, including various cancers.

- **Xenotransplantation Products**
  Xenotransplantation products use live animal cells, tissues, or organs to treat human diseases such as liver failure and diabetes, where human materials are not always available.

What is a biological product?

Biological products include a wide range of products such as vaccines, blood and blood components, allergenics, somatic cells, gene therapy, tissues, and recombinant therapeutic proteins. Biologics can be composed of sugars, proteins, or nucleic acids or complex combinations of these substances, or may be living entities such as cells and tissues. Biologics are isolated from a variety of natural sources - human, animal, or microorganism - and may be produced by biotechnology methods and other cutting-edge technologies. Gene-based and cellular biologics, for example, often are at the forefront of biomedical research, and may be used to treat a variety of medical conditions for which no other treatments are available.

How do biological products differ from conventional drugs?

In contrast to most drugs that are chemically synthesized and their structure is known, most biologics are complex mixtures that are not easily identified or characterized. Biological products, including those manufactured by biotechnology, tend to be heat sensitive and susceptible to microbial contamination. Therefore, it is necessary to use aseptic principles from initial manufacturing steps, which is also in contrast to most conventional drugs.

Biological products often represent the cutting-edge of biomedical research and, in time, may offer the most effective means to treat a variety of medical illnesses and conditions that presently have no other treatments available.

What is the legal authority for regulating biological products?

Biological products are approved for marketing under provisions of the Public Health Service Act (PHS Act). However, because most biological products also meet the definition of "drugs" under the Federal Food, Drug, and Cosmetic Act (FD&C Act), they are also subject to regulation under FD&C Act provisions.

CBER has also been delegated authority to regulate certain drugs closely related to biologics, such as anticoagulants packaged in plastic blood collection containers. CBER regulates these as drugs under the FD&C Act. Similarly, some medical devices used in blood banks to produce biologics are regulated by CBER under the FD&C Act's Medical Device Amendments of 1976. Examples of such devices are automated cell separators, empty plastic containers and transfer sets, and blood storage refrigerators and freezers.

What are the advantages to the public for regulation of biologics under the PHS Act?

The PHS Act emphasizes the importance of manufacturing control for products that cannot be defined. This is extremely important for the safety of our blood supply, our vaccines, especially our live viral vaccines, e.g. varicella and polio. The consistency, safety, efficacy and stability of these products, especially vaccines, are dependent on clearly defining and adhering to the processes described in an application, because the important structural features of the final product cannot be defined and there is an increased risk of the introduction of adventitious agents.

The PHS Act provides the agency with a licensing mechanism to confer approval of biological products and provides additional assurance to people receiving blood products and the millions of children being immunized each year to protect from childhood diseases.

The PHS Act also provides authority to immediately suspend licenses in situations where there exists a danger to public health. This statute also allows us to prepare or procure products in the event of shortages and critical public health needs, and authorizes the creation and enforcement of regulations to prevent the introduction or spread of communicable diseases in the US and/or between states. This law also provides important flexibility in regulation of biotechnology products, which facilitates the introduction and development of new medicines.

How are biological products approved?

The PHS Act requires individuals or companies who manufacture biologics for introduction into interstate commerce to hold a license for the products. These licenses are issued by CBER. Biological products intended for veterinary use are regulated under a separate law, the Virus, Serum, and Toxin Act, which is administered by the U.S. Department of Agriculture.

Licensing of biologic products under the PHS Act is very similar to the new drug approval process for human drugs. Following initial laboratory and animal testing, a biological product is studied in clinical trials in humans under an investigational new drug application (IND). If the data generated by the studies demonstrate that the product is safe and effective for its intended use, the data are submitted to CBER as part of a biologics license application for review and approval for marketing.

After a license application is approved for a biological product, the product may also be subject to official lot release. As part of the manufacturing process, the manufacturer is required to perform certain tests on each lot of the product before it is released for distribution. If the product is subject to official release by CBER, the manufacturer submits samples of each lot of product to CBER together with a release protocol showing a summary of the history of manufacture of the lot and the results of all of the manufacturer's tests performed on the lot. CBER may also perform certain confirmatory tests on lots of some products, such as viral vaccines, before releasing the lots for distribution by the manufacturer. In addition, CBER conducts laboratory research related to the regulatory standards on the safety, purity, potency, and effectiveness of biological products.

What is CBER's role after a biological product is approved and in use?

CBER continues to monitor the safety and stability of biological products that have been approved. Manufacturers must report certain problems to FDA's Biological Product Deviation Reporting System. Manufacturers must also report and correct product problems within established timeframes. If a significant problem is detected, a manufacturer may need to recall a product or even stop manufacturing it.

CBER encourages health professionals and members of the public to report problems with biological products to FDA's MedWatch and, for vaccines, to the Vaccine Adverse Event Reporting System (VAERS).

Does CBER conduct inspections?

CBER inspects manufacturing plants before it approves products, and thereafter, on a regular basis. The purpose of these inspections is to assess whether biological products are made in compliance with

appropriate laws and regulations, and to assist in identifying any changes needed to help ensure product quality.

CBER also may inspect clinical study sites to determine whether the studies are being carried out properly, and to ensure that accurate information is being submitted to the agency. CBER has a number of enforcement tools that may be used when a manufacturer or clinical researcher is in violation of FDA laws and regulations.

What is CBER's response to public health threats and bioterrorism?

CBER has a long history of responding quickly to public health threats. In the past, CBER's role in regulating vaccines against smallpox and polio contributed to the eradication of these dread diseases. Now, in response to the potential threat of a biological attack, CBER is facilitating the development of investigational vaccines against anthrax and smallpox. CBER also is overseeing clinical studies for new vaccines against HIV, hepatitis viruses, and West Nile Virus. In response to the new threats of West Nile virus, "Mad Cow Disease," and Severe Acute Respiratory Syndrome (SARS), CBER has taken strong measures to ensure that blood and tissue products continue to be safe.

How do I find out about ongoing clinical trials to treat AIDS or Cancer?

The National Cancer Institute, part of the National Institutes of Health (NIH), operates a toll free number with information about treatments for cancer. That number is 800-4-CANCER. The National Library of Medicine at NIH has developed a Clinical Trials database to provide patients, family members and members of the public current information about clinical research studies.

NIH also operates a toll free number with information on clinical trials of drugs used to treat AIDS. That number is 800-TRIALS-A.

How do I get a copy of the Code of Federal Regulations that pertain to regulation of biological products?

The National Archives and Records Administration web site includes copies of the Code of Federal Regulations.

Regulations specifically related to biological products can be found on our "Information on Submitting an Investigational New Drug Application For a Biological Product" page.

How do I or my doctor report an adverse event to FDA?

Adverse events and product problems are reported to the FDA Medical Products Reporting Program (MedWatch).

Adverse events (side effects) that occur after the administration of US licensed vaccines are reported to the Vaccine Adverse Event Reporting System (VAERS).

Notification Process for Transfusion Related Fatalities and Donation Related Deaths

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