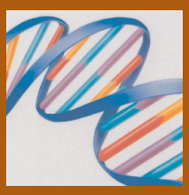
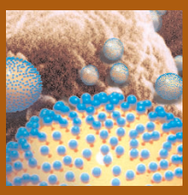
SCIENCE AND THE REGULATION OF BIOLOGICAL PRODUCTS




From a

Rich History

to a

Challenging

Future

In memory of Harry M. Meyer, Jr., MD,

Former Director, Bureau of Biologics,

Food and Drug Administration

1928-2001

Dr. Meyer and Paul D. Parkman, MD,

developed the first licensed

rubella virus vaccine.

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Kathryn C. Zoon, PhD
Director,
Center for Biologics
Evaluation and Research

Message from the Center Director

July 1, 2002, marked the Centennial of the 1902 Biologics Control Act, an event of great significance in the history of public health. This year, the Department of Health and Human Services (HHS), Food and Drug Administration (FDA), and Center for Biologics Evaluation and Research (CBER) commemorate passage of the Act and 100 years of biologics regulation. The Act established the authority to regulate biological products and ensure their safety for the American public. Until that time, biologics were not subject to federal oversight and lacked standards for quality, safety, purity, and potency.

As is sometimes the case, the Biologics Control Act was passed as a result of tragedy. In 1901, 13 children died after receiving diphtheria antitoxin contaminated with tetanus. The Act provided, for the first time, a legislative framework for the regulation of biologics and a means to protect Americans from unsafe products.

Since passage of the Act, CBER has established a proud record of regulatory stewardship and research accomplishments. CBER's history illustrates how science and innovative regulation go hand in hand. As part of HHS, CBER works closely with other Department agencies to achieve the goals set forth by HHS to ensure a strong public health safety net for all Americans. I believe that CBER's tradition of integrating innovative science with innovative regulation has enhanced our ability to protect the public health, and has led to safer and more effective biological products.

This book describes the rich history of biologics regulation, highlights key research contributions made by CBER scientists over the last 100 years, and offers a glimpse into the exciting and challenging future of biomedical discoveries and regulation. I am humbled, yet very proud to be a part of CBER's legacy and invite you to join me on this historic journey. CBER welcomes the future, its challenges and opportunities, and will build upon its record of success by ensuring another 100 years of safe biological products.

Regulation of FDA Products

Based on Sound Science, Law, and Public Health Impact

Review

Research

Surveillance

Policy

Compliance

HISTORY OF BIOLOGICAL

Marine Hospital Service
Original Public Health Agency

Koch isolated
Anthrax Bacillus

Pulblic Health
Service Lab
of Hygiene
J. Kinyoun

Antitoxins

1798

1800

Smallpox
Vaccination

1878

1886

Louis Pasteur
Rabies Vaccine

1887

1888

Roux & Yersin
Diphtheria Toxin

1890

Introduction

The regulation of biologics by CBER is founded on both science and law. Throughout the 20th century, developments in biologics regulation have been made possible by advances in scientific knowledge. As stated by Paul Parkman, MD, a former CBER Director, CBER "is a science-based organization that regulates biological products. It has to have a strong scientific component and a strong regulatory program melded together." Thus, many CBER staff are both researchers and regulatory reviewers. John Finlayson, PhD, Associate Director for Science, Office of Blood Research and Review, believes that "our excellence in CBER is a direct result of the fact that people are expected to wear many hats at the same time," and hopes that "the researcher-reviewer model can be preserved into the future."

This publication chronicles the long scientific history, as well as the 100 years of legislative history, related to biologics. In addition, it emphasizes the importance of the interactive relationship between science and law—a relationship that CBER has used effectively to make life-saving biological products available throughout the 20th century and will continue to use throughout the 21st century.

P R O D U C T S R E G U L A T I O N

Thirteen children die of tetanus due to contaminated diphtheria antitoxin

The Food and Drugs Act

National Institute of Health

Food, Drug and Cosmetic Act

1894 Public Health Labs produce Diphtheria Antitoxin

1901

1902 Biologics Control Act

1906

1912 Public Health Service Created

1930

1937 Division of Biologics Control

1938

HISTORY OF BIOLOGICAL

1940 Rh Blood Group System

1941 **Cohn Fractionation** of Blood

1944 The Public Health Service Act (Lab of Biologics Control)

1948 National Microbiological Institute

1950 1st Live Polio Vaccine in Humans

1955 **Cutter Polio** Vaccine Incident Division of Biologics Standards created

1962 **Kefauver-Harris** Drug Amendments

1972 **BoB** To FDA

Science and Peace will triumph

over ignorance and war,

nations will unite, not to destroy,

but to build, and the future

will belong to those

who will have done most

for suffering humanity.

Louis Pasteur

PRODUCTS REGULATION

Biotechnology Era

CBER

FDAMA

1980's **1988** **1997**

1973 **1982** **1992** **2002**

New Provisions of PHS Act

National Center for Drugs and Biologics

PDUFA

C B E R

The Center for Biologics Evaluation and Research

Division of Biologics Standards staff photo

The Center for Biologics Evaluation and Research (CBER) within the Food and Drug Administration (FDA) is responsible for ensuring the safety, purity, potency, and efficacy of biological and related products (biologics) intended for use in the diagnosis, prevention, treatment, or cure of diseases in humans, and for ensuring the safety of the nation's supply of blood and blood products. Biologics are substances either derived from living organisms—including humans, animals, plants, and microorganisms—or produced by biotechnology. In addition, they can be combinations of these substances. Biologics include vaccines, blood and blood products, antitoxins, allergenic products such as patch tests and extracts, certain tissues, diagnostic devices for HIV and hepatitis, and biotechnology-derived therapeutic products for cancer, arthritis, and other conditions. The number of biologics regulated by CBER is expanding rapidly because of the remarkable growth of research in biotechnology and scientific advances such as completion of the Human Genome Project.

CBER scientists directly support regulatory decisions by carrying out a wide range of activities that begin with premarket product review, continue throughout all aspects of product production, and extend to postmarket review and follow-up.

To fulfill its regulatory responsibilities, CBER:

- Conducts premarket review of new products, as well as review of new indications for already approved products, to ensure that they are safe and effective;

- Facilitates establishment of industry-wide standards and methods, and encourages industry-wide adoption of new technologies—activities that contribute to the improvement of existing products and the development of new products;

- Conducts establishment inspections and product surveillance to ensure that licensed products are in full compliance with appropriate laws and regulations;

- Formulates policy through open communication, public dialogue, scientific and regulatory workshops, and participation in scientific, regulatory, and ethics fora;

- Anticipates public needs and supports informed decision-making in prevention of, and response to, public health crises; and

- Demonstrates international leadership in regulation through development of innovative regulatory strategies and standards, coordinated research, and the use of partnerships.

The Mission of the Center for Biologics Evaluation and Research is to protect and enhance the public health through the regulation of biological products, including blood, vaccines, therapeutics, and related drugs and devices, according to statutory authorities. The regulation of these products is founded on science and law to ensure their purity, potency, safety, efficacy, and availability.

CBER Leadership

Between 1887 and today, biologics regulation has been led by the following directors:

Joseph J. Kinyoun, 1887-1899

Milton J. Rosenau, 1899-1909

John F. Anderson, 1909-1915

George W. McCoy, 1915-1937

Walter T. Harrison, 1937-1940

Milton V. Veldee, 1940-1949

William G. Workman, 1949-1955

Roderick Murray, 1955-1972

Harry M. Meyer, Jr., 1972-1987

Paul D. Parkman, 1987-1991

Gerald V. Quinnan, Jr., 1991-1992 (Acting)

Kathryn C. Zoon, 1992-present