

Center for Biologics Evaluation and Research
Strategic Plan
FY 2012 – 2016

Innovative Technology Advancing
Public Health



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Message from the Center Director, Karen Midthun, MD

As the Director of the Center for Biologics Evaluation and Research (CBER), I am pleased to issue the CBER Strategic Plan which outlines our plans and strategic direction over the next five fiscal years (2012 – 2016) for achieving our mission and contributing to the overall Food and Drug Administration’s strategic priorities.

CBER’s strategic plan is based on our mission and vision and identifies our strategic goals, and related objectives and strategies. Our plan is aligned with the FDA Strategic Priorities document and the Department of Health and Human Services’ strategic plan. CBER’s plan reflects the contributions of all offices and divisions, and it sets forth the Center’s four overarching program goals and two cross-cutting goals:

- Goal 1: Increase the nation's preparedness to address threats as a result of terrorism, pandemic influenza and emerging infectious diseases
- Goal 2: Improve global public health through international collaboration including research and information sharing
- Goal 3: Enhance the ability of advances in science and technology to facilitate development of safe and effective biological products
- Goal 4: Ensure the safety of biological products
- Goal 5: Advance regulatory science and research
- Goal 6: Manage for organizational excellence and accountability

This plan describes these goals, which will guide Center decision-making and future planning. Our overarching program goals focus on Center programs while the cross-cutting goals span across organizational and program lines to support the achievement of Center program goals.

We will use this plan as a living document, allowing us to modify our direction or approach as significant new information becomes available. We intend to review and update the current plan periodically.

Together, these goals, objectives and strategies form our vision for how our Center can contribute to improved public health in the years to come. We have a clear direction, and look forward to the challenge of building on our successes to serve the American public.

Karen Midthun, MD
Director, Center for Biologics Evaluation and Research

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Introduction

Mission

The mission of the Center for Biologics Evaluation and Research (CBER) is to ensure the safety, purity, potency, and effectiveness of biological products including vaccines, allergenics, blood and blood products, and cells, tissues, and gene therapies for the prevention, diagnosis, and treatment of human diseases, conditions, or injury. Through our mission, we also help to protect the public against the threats of emerging infectious diseases and bioterrorism.

In fulfilling our mission as a Center in the U.S. Food and Drug Administration (FDA), we apply the highest ethical standards and integrity to:

- Develop, maintain and support a high-quality and diverse workforce
- Ensure compliance with laws and regulations through review, education, surveillance and enforcement
- Conduct research as an essential element of science-based decision-making

Organization of CBER

CBER is composed of seven program offices and one staff office serving the Center. Three of the seven program offices are product offices covering vaccines, blood, and cell and gene therapies. The remaining four offices provide cross-cutting support in the areas of compliance; surveillance, epidemiology and biostatistics; communication, outreach and development; and administrative services. The Office of the Center Director provides policy and direction to the program offices. (See Appendix D)

Vision

CBER uses sound science and regulatory expertise to:

- Protect and improve public and individual health in the United States and, where feasible, globally
- Facilitate the development and approval of, and access to safe and effective products and promising new technologies
- Strengthen CBER as a preeminent regulatory organization for biological products

Organization of the Document

Six strategic goals are presented. The goals have an overview followed by a description of each objective with its corresponding strategies. These goals, objectives and strategies are interrelated, and successful achievement of one goal or objective can impact the success of other goals, objectives and strategies.

This interrelatedness is especially shown in cross-cutting goal 5, Advance Regulatory Science and Research, where three of its objective statements mirror the language of three program goals. This was done by design to emphasize the significant role that regulatory science and research contribute to the CBER's regulatory decision-making, policy development and program administration. The strategies employed in the three objectives will achieve the regulatory science and research priorities supporting the CBER program strategic goals.

Strategic Goals, Objectives and Strategies

Goal 1: Increase the nation's preparedness to address threats as a result of terrorism, pandemic influenza and emerging infectious diseases

CBER seeks to ensure the nation and world are prepared to respond quickly to a terrorism incident, pandemic influenza and outbreaks of new emerging infectious diseases (EIDs) by enhancing the availability of safe and effective biologic medical products. These threats could harm many US citizens, overwhelm our medical care infrastructure and introduce risk to the security of the nation.

The safety and availability of the nation's blood and tissue supply are also threatened by terrorism, pandemic influenza and EID. Infectious agents transmitted from donated blood and tissues to patients may spread an infectious disease. A radiological/nuclear attack could render collection and transfusion facilities unusable for a long time.

CBER will continue to collaborate with HHS divisions, other Federal government partners, FDA Centers and the Office of Regulatory Affairs (ORA), and other stakeholders to prepare for health-related emergencies and to address these threats.

Objective A: Increase the nation's preparedness for pandemic influenza

CBER is facilitating the development of pandemic influenza vaccines through regulatory pathways to expedite the availability of vaccines. CBER supports efforts to increase manufacturing capacity using new and existing technologies and to develop more efficient methods for testing the potency of influenza vaccines.

CBER is responsible for the regulatory review of both seasonal and pandemic influenza vaccines, and CBER's laboratory program contributes in various ways to facilitate influenza preparedness. These efforts include the identification and proposal of candidate strains for seasonal and pandemic vaccines; preparation of reference strains to support development by licensed manufacturers of seed stocks for seasonal and pandemic vaccine productions; and development of reference reagents that are used in lot-release testing of seasonal and pandemic influenza vaccines.

CBER will continue to provide regulatory support to the industry developing sustainable influenza vaccine production capacity. CBER will consult outside experts in the Vaccines and Related Biological Products Advisory Committee (VRBPAC) to obtain recommendations and advice related to vaccines, including discussions involving the safety and effectiveness of influenza vaccines and the selection of strains for future seasonal influenza virus vaccines.

Together, these critical activities help ensure the safety, quality, and potency of seasonal influenza vaccines prepared annually, as well as facilitating the development and availability of vaccines.

CBER intends to prepare for pandemic influenza through the following strategies:

- a. Develop and evaluate improved tools and methods to enhance safety, quality and potency testing of influenza vaccines, including more efficient methods to develop reference reagents for potency testing, as well as alternatives to the traditional test used to measure influenza vaccine potency; these efforts could allow for a more rapid response in pandemic outbreaks.
- b. Develop and evaluate new preclinical methods to screen novel adjuvants for their potential for adverse effects prior to starting clinical trials. Availability of new adjuvants may enhance the immune response to the influenza vaccine antigens and may also result in the availability of more doses of vaccine during a pandemic.
- c. Upgrade the Lot Release System to include multiple enhancements to support pandemic preparedness.
- d. Provide guidance to influenza virus vaccine manufacturers.
- e. Continue close monitoring of influenza vaccine manufacturing facilities both domestically and in foreign countries.

Objective B: Facilitate development, evaluation and availability of high priority medical products (including medical countermeasures)

CBER works with the HHS Public Health Emergency Medical Countermeasures Enterprise and industry on a broad array of projects aimed at making our nation better prepared to respond to biological, chemical and radiological or nuclear threats through medical countermeasures, including safe and effective biological products. We collaborate with HHS, Department of Homeland Security (DHS), Department of Defense (DOD) and other Federal government partners to identify and monitor medical countermeasure (MCM) product needs and approaches. These MCM products are used to diagnose, treat or prevent disease from pathogen or agent exposure, such as anthrax and smallpox. In this regard, CBER continues to work toward having vaccines, blood and blood products, tissues and cellular therapies, readily available. For example, cell-based therapies may be needed for wound repair, regeneration of tissues, and reconstitution of bone marrow, and blood and blood components may be essential for treatment.

CBER participates in the FDA Medical Countermeasures Initiative by providing leadership and coordination in the governance structure, continuing to facilitate the review process, advancing regulatory science for biologics-based MCM development and evaluation, and contributing to agency efforts to enhance the legal, regulatory and policy framework for effective public health response. We participate in devising risk communication strategies in public health emergencies, contributing to health

informatics/scientific computing, designing intramural programs for MCM regulatory science, facilitating external science partnerships, and modernizing labs to conduct MCM regulatory science.

The development and approval of vaccines and therapeutics against biological, chemical, radiological, or nuclear substances present unique challenges because human efficacy studies may not be ethical or feasible. Therefore, many of these products may rely on using nonclinical data to support claims of efficacy for licensure.¹ CBER intends to facilitate the development, evaluation, and availability of high priority medical products through the following strategies:

- a. Develop and evaluate nonclinical models to study pathogenesis and identify relevant correlates of immunity.
- b. Create methods and nonclinical models to evaluate the safety of vaccines, including adjuvants.
- c. Determine biomarkers of pathogenicity and develop new methods to evaluate and ensure safety of vaccines.
- d. Study the mechanisms of innate and adaptive immunity against viral and bacterial diseases and mechanisms of immunopathology, including developing new approaches to inducing protective immunity.
- e. Collaborate with NIH on the preclinical safety and activity testing of stem cell products.

Objective C: Develop reagents, evaluate new methods and implement policies to maintain a continued safe and adequate supply of blood and tissues during emergencies

CBER is responsible for ensuring that blood, blood products and tissues remain free of contamination, which may occur naturally or through terrorism, and that these biologic products are available to individuals who need them. These products are at risk for contamination by known or unknown infectious agents. New infectious agents are emerging and could compromise product safety and availability, whether due to the spread of disease vectors (e.g. dengue, babesiosis), travel and immigration (e.g. malaria, Leishmania), terrorism (e.g. anthrax) or previously rare or unknown infectious agents (e.g. vCJD, SARS-CoV, Chikungunya virus, Q-fever agent). Further, due to their labile nature, these products often cannot safely be subjected to currently available sterilizing or removal methods to ensure the absence of bacteria, fungi, parasites, viruses or prions. We are vigilant about minimizing the risks of emerging agents to these critical public health products.

¹ For more details on the requirements for licensure under these conditions, refer to the following document, commonly referred to as “The Animal Rule”: New Drug and Biological Drug Products; Evidence Needed to Demonstrate Effectiveness of New Drugs When Human Efficacy Studies Are Not Ethical or Feasible; Final Rule - 5/31/2002.

CBER supports a proactive, systematic and comprehensive approach towards pathogen detection and response to threats by engaging in the following strategies:

- a. Improve the microbial safety of human tissue products and enhance methods to inactivate pathogens.
- b. Continue to inspect facilities that manufacture human tissue-based products to monitor compliance with all applicable regulations.
- c. Continue to enhance collaborative efforts to identify, monitor, prioritize and act on EID issues and emerging threats to blood and tissues.

Goal 2: Improve global public health through international collaboration including research and information sharing

CBER works in the global community to improve human health in the world's populations. Our international engagements have been informed by the knowledge that protection of global public health against infectious disease threats translates into protection of public health in America.

We recognize the importance of actively playing both leadership and partnership roles in global health and we have a number of significant international activities. The international need for vaccines, blood and blood products, and other biological products to treat or prevent ailments and emerging threats to health is a challenge to international health care agencies. We live in an increasingly global and interdependent environment. Infectious disease threats such as malaria, Leishmania, Chikungunya virus, dengue fever (for which mosquito vectors are present in the U. S.), avian influenza, new hepatitis viruses, HIV, TB and diarrheal diseases not only affect millions around the world, but also threaten our population. Medical innovation, manufacturing and regulation are increasingly global, posing challenges and opportunities. New paradigms are emerging in the form of non-governmental organizations (NGOs) and product development partnerships (PDPs) to address unmet product development needs which have unique regulatory pathways and associated regulatory science challenges.

CBER plays a critical role in contributing to global public health. We conduct a robust International Program, engaging in international activities using a range of mechanisms to realize a global public health goal: regulatory harmonization/convergence, where feasible, regulatory capacity building, information sharing, international standards development and collaborative research. Incorporating these tools in a cross-cutting approach, CBER developed four objectives described below to enhance its role in improving global public health.

Multiple corollary benefits can be realized from our efforts including: streamlining global product development to ensure products reach more patients sooner; safeguarding the public trust in the quality of our oversight and thus in the products we regulate; applying the best scientific and regulatory judgment to our decision-making; leveraging resources; accessing state-of-the-art science; and executing our responsibility as a global leader.

Objective A: Promote research and information-sharing globally to address diseases and emerging threats impacting human populations

Promoting regulatory research and sharing of both scientific and regulatory information are critical to advancing the development of products of global need. Not-for-profit NGOs and PDPs are now important contributors to global efforts for developing medical products to address the world's unmet needs. Working with these entities on these needs is critical for the successful launching of new products in the global market. As an example, recently CBER started collaborating on the PATH Malaria Vaccine Initiative

(PATH-MVI) to develop improved laboratory tests for predicting the level of safety and effectiveness of experimental malaria vaccines before they are used in human clinical trials. We will explore new collaborations and creative new paradigms such as the PATH-MVI to enhance the development of new products.

Sharing both scientific and regulatory information with foreign regulatory counterparts and international health agencies contributes to best regulatory practices and product oversight. This exchange also strengthens and better informs our own regulatory decisions. For example, pharmacovigilance data sharing can enhance detection of safety concerns and their evaluation.

CBER intends to promote research and information-sharing globally through the following strategy:

- a. Advance research and information sharing in under-funded global health areas through interactions with Federal government agencies, foreign regulatory counterparts, international health agencies, NGOs and PDPs.

Objective B: Facilitate global access to vaccines and biological products that address critical health needs

Collaborations with international health bodies such as the World Health Organization (WHO) and the Pan American Health Organization (PAHO) and efforts to support regulatory capacity building are key to facilitating global access to needed biological products. CBER collaborates with these organizations and others to help provide assurances of quality, safety and efficacy for products supplied to the global community through such programs as the vaccine prequalification program.

Regulatory capacity building strengthens foreign counterpart organizations to effect science-based policies that are consonant with our own, supporting consistency of a science-based approach to regulation across countries and assuring access to products among developing countries. Moreover, such efforts can lead to adoption of policies by other NRAs and thus provide another layer of protection in the international environment. Regulatory capacity building can also enhance data integrity and human subject protection for clinical trials conducted abroad. Likewise, GMP compliance can be enhanced for foreign manufacturing sites when an NRA's on-site regulatory oversight complements FDA's efforts. Regulatory capacity building takes many forms, including directed training exercises, personnel exchanges and mentoring foreign regulators.

The WHO vaccine prequalification program is a cornerstone of global access to vaccines by providing a mechanism for the assurance of quality, safety and effectiveness for the purchase of vaccines by agencies of the United Nations for distribution to countries in need. CBER serves as the NRA that evaluates the quality, safety, and effectiveness of selected US-licensed vaccines in support of this WHO program. Additionally, CBER provides to PAHO and WHO follow-up information on vaccine safety and quality over

the lifetime of the products. These vaccines are critical to addressing global public health in developing countries by preventing infectious diseases and reducing mortality and morbidity.

CBER intends to facilitate global access to vaccines and other biological products through the following strategies:

- a. Facilitate the development of vaccines to prevent diseases in the developing world.
- b. Improve the information sharing on the efficacy and safety of vaccines and blood and blood products that address critical global health needs.
- c. Support the efforts with WHO, other regulators, manufacturers and NGOs to enhance efforts to control significant infectious diseases.

Objective C: Harmonize existing regulatory standards and work towards prospective harmonization of standards on new biological product areas to promote global public health

Harmonization of existing product standards and prospective harmonization in newly emerging therapies remains an important means to facilitate global access to safe and effective products. Regulatory harmonization is a process by which divergent regulatory guidance is brought into alignment. Models for regulatory harmonization range from the higher level perspective of shared principles to explicit concurrence of technical specifications.

The harmonization effort occurs through various established formal processes such as the International Conference on Harmonization of Technical Requirements for Registration of Pharmaceuticals for Human Use (ICH) and the Global Harmonization Task Force (GHTF); through confidential exchanges with counterpart regulatory agencies during guidance and policy development such as the dedicated cluster discussions with the European Medicines Agency (EMA); and through outreach efforts to engage stakeholders to establish common perspectives in emerging areas.

CBER intends to harmonize existing regulatory standards through the following strategies:

- a. Continue to engage in regulatory dialogue with foreign counterparts, on topics including product manufacturing issues, potential product shortages, post-marketing product safety signals, preapproval issues and regulatory standards harmonization.
- b. Recommend tactical ways to achieve greater harmonization in the interpretation and application of technical guidelines with the ICH.

Objective D: Collaborate in international scientific efforts to establish and maintain reference materials and standards for biologics

Standards, both written and physical, are fundamental to the control of biologic products, with international standards having served as the underpinning for the global control of biologics for more than a century. Collaborative laboratory work and research to establish international physical standards contribute to the streamlining and consistent global development of biological products. Establishing reference materials and standards for biologics in the international community will advance CBER's efforts to facilitate global access to vaccines and other biological products.

CBER works with other standard setting bodies in addition to WHO such as National Institute for Biological Standards and Control (NIBSC) and the European Directorate for the Quality of Medicines and Healthcare (EDQM) along with its associated committees and the Pharmaceutical Inspection Cooperation Scheme (PIC/S).

CBER intends to collaborate in international scientific efforts through the following strategies:

- a. Advance efforts to develop and improve sharing of reference materials and biological standards with international scientific community and foreign regulatory bodies.
- b. Implement relevant international collaborations on global issues such as pandemic influenza and HIV.
- c. Serve as a PAHO/WHO Collaborating Center for Biological Standardization and as an Essential Regulatory Laboratory within WHO's influenza vaccine network.

Goal 3: Enhance the ability of advances in science and technology to facilitate development of safe and effective biological products

CBER is committed to facilitating the development of new biological products for a broad range of complex diseases. We seek to expedite the development of innovative and complex biological products, including those representing the exciting medical promise of personalized medicine, such as gene therapies, cell-based and tissue-engineered products; vaccines against pandemic influenza and other infectious diseases; and new technologies to enhance the safety and availability of blood and blood products.

We will use the advances in science and technology to design better ways to predict the safety of biological products early in their life cycle and conduct mission-related research to facilitate product development.

CBER is participating in an agency task group that is implementing the abbreviated approval pathway for biosimilar and interchangeable biological products established by the Biologics Price Competition and Innovation Act of 2009. This task group envisions developing technical standards and criteria to address specific scientific, legal and policy complexities associated with biosimilars. CBER's scientific expertise and knowledge provides a strong foundation for these efforts.

We will also continue to collaborate with private and public institutions to create new tools for developing and testing new products. Reflecting past input from the FDA Science Board, CBER will use its scientific expertise in developing the new product evaluation science to efficiently review and support the development of biological products.

Objective A: Integrate genomics, proteomics, high sensitivity gene sequencing and other cutting-edge scientific technologies into regulatory oversight to expedite product development and review

CBER aims to continue to integrate novel, applicable scientific technologies into its regulatory oversight to facilitate review of innovative and novel products in a timely manner. Advances in science and technology show great promise for the development of new safe and effective biological products. FDA is working to expedite the use of advanced technologies and methods and scientific discoveries - such as newly identified clinical biomarkers, adaptive clinical trial designs, and genomics - in regulatory policy and guidance for industry. In addition, CBER is identifying opportunities to expand the use of new scientific technologies in genomics, proteomics and metabolomics in order to strengthen science-based regulatory review.

CBER uses the best science available to develop new standards for testing products; provide advice to product sponsors, professional societies, researchers and other government agencies; and facilitate the development of new methods and technologies for enhancing the safety, efficacy and quality of biological products. We evaluate state-

of-the-art tools and approaches, such as nuclear magnetic resonance (NMR), mass spectroscopy, microarray and high throughput sequencing, and other analytical technologies to assess the purity, potency, and quality of the biological products and relevant manufacturing intermediates, such as cell substrates used for biologics production.

CBER continues to integrate genomics and related sciences with the goal of enhancing biologic product development and safety. To this end, CBER launched a new multidisciplinary Genomics Evaluation Team for Safety (GETS) to focus on identifying possible human genetic contributions to adverse reactions. This team applies genomics approaches to improve biologics safety. The overall strategy is to work collaboratively with CBER product offices to leverage “omics” resources at FDA, NIH, CDC, academia and industry so that we influence and shape optimal policy, education, and research.

CBER intends to integrate genomics, proteomics, high sensitivity gene sequencing and other cutting-edge scientific technologies into regulatory oversight through the following strategies:

- a. Demonstrate the feasibility of novel technologies such as genomic and proteomic approaches to assess the consistency and purity of biologic products.
- b. Develop and evaluate new methods to assess the safety of novel cell-substrates to facilitate development and availability of new cell-substrates for vaccines.
- c. Explore the mechanisms of vaccine-related adverse effects and provide ways to mitigate these mechanisms, and biomarkers of predisposition to adverse effects.
- d. Develop highly sensitive, high throughput, multiplex assays and standards for detecting emerging infectious agents.
- e. Develop preclinical models, including identification of product quality attributes, for plasma derivatives and for blood components that have undergone pathogen reduction treatments to assist in the evaluation of the safety and effectiveness of these products.

Objective B: Improve the evaluation of product efficacy in clinical trials through the use of biomarkers² and adaptive designs

CBER will enhance the evaluation of biologic products during clinical trials through the use of new clinical biomarkers and adaptive design approaches. CBER will identify and evaluate new clinical biomarkers that make it easier to assess early in the course of development any adverse reactions to novel biological products and improve the overall evaluation of data gathered. In addition, new clinical biomarkers will provide CBER scientists the opportunity to enhance the overall evaluation of data gathered during clinical trials of treatments for patients with complex medical problems. This work will be part of CBER’s overall effort to design new ways to conduct clinical trials.

² Biomarkers are characteristics that can be scientifically measured and evaluated as indicators of normal biologic processes, disease, or response to drug therapy, and can include specific types of molecules as well as such routine measurements as blood pressure.

CBER will work with industry to implement novel approaches to clinical trial design and analysis, such as Bayesian and adaptive clinical trial designs. New approaches could result in improved evaluation of efficacy of products.

CBER intends to improve the evaluation of efficacy of products in clinical trials through the following strategies:

- a. Continue to participate through the Critical Path Initiative and the Clinical Trials Transformation Initiative.
- b. Draft guidance on initiation and conduct of early phase clinical trials using cellular and gene therapies.
- c. Continue to collaborate with other FDA Centers, NIH and industry through the Biomarkers Consortium to develop biomarkers.
- d. Develop general principles for utilizing genomic biomarkers in clinical trials through International Conference on Harmonization (ICH).
- e. Facilitate the application of advanced technologies and methods and relevant scientific discoveries — such as newly identified clinical biomarkers, adaptive clinical trial designs, and genomics — to regulated products.
- f. Incorporate relevant new knowledge from studies of advanced technologies and methods into recommendations embodied in new regulatory guidance for industry.

Objective C: Advance regulatory science research and update regulatory policy to facilitate product review, including development of relevant animal models

CBER advances regulatory science research and updates regulatory policy that supports product review to reflect the new generation of product evaluation tools and the innovative products we expect to see. To sustain our efforts in regulatory science research, we will develop a long range plan to enhance infrastructure (laboratory facilities and scientific computing capability); to leverage resources in partnering research and regulatory science collaborations; to enhance the scientific expertise of our scientific and technical workforce; and to regularly apply our evaluation processes for regulatory research projects.

In addition, reference materials provide an important research tool. For example, they may be used to characterize the safety and efficacy of many biological products, such as plasma-derived biologics and to develop screening assays for infectious agents in blood. Availability of reference materials for use in lot release testing provides important manufacturing controls for quality and ensures accuracy of dosing.

CBER uses a formal structure to update regulatory policy on a consistent basis. Through the use of expert working groups for policy development and review, CBER created a recognizable structure where policy issues can be surfaced and developed using standard criteria and practices/procedures designed to assure consistency and cross-office

involvement. New and refined policies can then be applied by the Center in the form of internal procedures and industry guidance to obtain high quality and consistent reviews and site inspections.

CBER intends to advance regulatory science research to facilitate product review through the following strategies:

- a. Test and validate methods for rapid detection of microbial contaminants in biologics.
- b. Maintain and expand International Standards Organization (ISO) accreditation for CBER lot release testing and processing.
- c. Develop collaborative scientific programs to evaluate stem cells and other technologically advanced therapeutic approaches to facilitate development, evaluation and availability of novel therapies.
- d. Draft guidance on the preclinical safety assessment of cell and gene therapy products.
- e. Develop a compliance program for inspection of cord blood products and implement a training program for conducting reviews and inspections.
- f. Facilitate the development of new and improved animal models and assays to measure activity and potential drug-induced toxicity at an early stage in product development.
- g. Develop and evaluate animal models that can support the basis for efficacy for certain medical counter-measures that are used to treat or prevent serious conditions caused by biological, chemical, radiological or nuclear substances when human efficacy studies are not ethical or feasible.

Goal 4: Ensure the safety of biological products

This goal will strengthen CBER's core mission to ensure the safety of biological products. Under the Food and Drug Administration's Amendments Act of 2007 (FDAAA), FDA gained additional authorities to enhance product safety through required postmarket studies, safety labeling changes, and risk evaluation and mitigation strategies. Coupled with these tools and working collaboratively with the ORA, CBER carries out compliance and surveillance activities to focus on safety throughout the product life cycle. This goal will be accomplished through four objectives: addressing product quality and manufacturing; use of healthcare databases; improved risk assessments; and safe product use.

Objective A: Facilitate increased biologics manufacturing capacity and improved product quality

CBER helps to increase manufacturing capacity by interacting with researchers, developers and manufacturers through outreach efforts and through global coordination to facilitate the development of new manufacturing methods, technologies and best practices.

CBER intends to facilitate increased biologics manufacturing capacity and improved product quality through the following strategies:

- a. Modernize regulations and guidance to provide flexibility which in turn could foster manufacturing innovation and adaptation in the increasingly global regulatory environment.
- b. Foster improved manufacturing technologies and product characterization techniques through interactions with stakeholders including sponsors.
- c. Optimize oversight of manufacturing process by applying risk and science based principles that will help to determine efficient approaches to manufacturing.
- d. Enhance methods to track source materials, raw materials, ingredients, and components used for manufacturing CBER-regulated products.
- e. Lead advanced methods development and validation programs for improved methodologies that monitor product quality.

Objective B: Improve the use of healthcare data to enhance monitoring the safety and quality of licensed biological products

CBER's vision for post-market safety monitoring entails having access to patients' biological product exposures and health outcomes in automated databases, enabling optimal detection and analysis of potential problems in biologics safety. To this end, we will combine large databases from healthcare providers using specific biological products to identify safety problems connected to their use, continue to study the feasibility of tracking potential problems with vaccines in real time, and participate in the Sentinel

Initiative. The initiative is an agency-wide effort to foster rapid electronic exchange of information between private healthcare databases and FDA surveillance systems that will enable CBER to collect, analyze and communicate information more efficiently.

CBER intends to improve the use of healthcare data to enhance monitoring the safety and quality of licensed biological products through the following strategies:

- a. Continue to expand access to and utilize population databases for biologic product safety.
- b. Enhance the active electronic safety monitoring system to strengthen our ability to monitor post-market performance of medical products and augment existing safety monitoring systems.
- c. Develop and assess methods to improve safety signal detection, refinement and validation.

Objective C: Enhance statistical data analysis and mathematical models for improved epidemiological and risk assessments of regulated products

CBER is facing increasing challenges and opportunities as we work to ensure the safety of biological products. The success of our efforts requires careful and effective integration of a variety of safety-relevant activities throughout a biological product's lifecycle. Our efforts include developing new methodologies that enhance product safety surveillance through the use of large datasets (e.g., Medicare and public and private health systems) that reflect real-life experiences of consumers treated with the product. In this comprehensive approach to product safety surveillance, CBER will incorporate enhanced scientific tools such as genomics, advanced statistics and mathematical modeling. We will continue to use the powerful tools of bioinformatics and new approaches to statistics and modeling, to develop new scientific data or methods and to evaluate safety signals.

CBER intends to enhance statistical data analysis and mathematical models through the following strategies:

- a. Evaluate new statistical methods that have the potential to improve the efficiency, consistency and rigor of our review of safety issues in clinical trials, surveillance systems and epidemiological studies.
- b. Participate in agency and Center initiatives to develop the information technology and computational science infrastructure and data standards to enhance evaluation of clinical trial and other sources of safety data.
- c. Develop, evaluate, and apply novel data-mining methods to assess safety concerns throughout the product lifecycle.
- d. Develop standard approaches for data mining Adverse Event Reporting System (AERS) and Vaccine Adverse Event Reporting System (VAERS) including developing standardized queries for extracting data from the AERS and VAERS databases.

- e. Improve process and guidelines for deciding when risk assessment and modeling methods can inform the assessment of safety issues.

Objective D: Promote safe product use through effective risk management and risk communication

CBER's regulatory oversight for biological products requires focus on all facets of managing risks and several ways in which risks are effectively communicated internally within CBER and to industry, healthcare professionals and consumers.

CBER's efforts to keep products safe contribute to patient safety. We will continue to refine our processes and procedures for implementing the new oversight authorities provided by the FDAAA.

After approval, CBER monitors postmarketing reports for risks when signals from healthcare databases suggest a concern as outlined in objective B. As warranted, CBER will institute risk management strategies and employ suitable risk communications to protect the public health. CBER Safety Teams for blood, tissues and vaccines provide coordinated assistance for communication and follow-up on complex, safety issues within CBER. Safety Team activities include coordinating investigations with other agencies and regulatory partners, formulating appropriate responses and communicating with stakeholders.

CBER intends to promote safe product use through the following strategies:

- a. Continue to apply principles of risk management to improve our regulation of products throughout their lifecycle.
- b. Leverage the Managed Review Process (MRP) designed to ensure compliance with regulatory standards and internal practices.
- c. Continue to issue new guidance, rules and standards that provide important direction to manufacturers of biological products.

Goal 5: Advance regulatory science and research

This cross-cutting goal describes how we will advance the Center's regulatory science and research program and contribute to the achievement of the four program strategic goals. The Center not only evaluates new products but also conducts research to support its science-based, regulatory decision-making and policy development.

The Center's regulatory science and research program is coordinated from the Office of the Center Director and through each program office that has a research component. The CBER's research management strategy is based on a strategic planning and prioritization process. The strategy supports the researcher-reviewer model that has served the needs of regulating the complexities of biological products. CBER ensures accountability to its stakeholders through a combination of internal annual and cyclic evaluations, external scientific peer reviews and input from external sources. The goal of this oversight is to ensure that CBER's programs support CBER's priorities.

Regulatory science and research play a significant role in CBER's regulatory decision-making, policy development and program administration. The strategies employed in these objectives will achieve the regulatory science and research priorities supporting the CBER program strategic goals.

Objective A: Increase the nation's preparedness to address threats as a result of terrorism, pandemic influenza and emerging infectious diseases

CBER intends to increase the nation's preparedness through the following strategies:

- a. Develop a comprehensive approach to threats posed by currently recognized transmissible infectious microorganisms in biological products and newly emerging infectious agents.
- b. Develop and explore feasibility of new models that would facilitate use of the "Animal Rule," in which animal studies provide the basis for effectiveness for the approval of new drugs and biologics in specified circumstances.
- c. Contribute to the development of novel regenerative medicine products (e.g., stem cell-based products and tissue engineering medical products) by assessing animal models to identify biomarkers for cell differentiation state and function, localization and controlled proliferation.
- d. Develop a core scientific team to facilitate development and availability of reagents, samples and methods to enable the development and validation of cost-effective screening tests for new pathogens through public-private collaboration.
- e. Assess and encourage development of new technologies to enable rapid, sensitive, specific, high throughput (i.e., multi-plex) testing.

Objective B: Improve global public health through international collaboration including research and information sharing

CBER intends to improve global public health through international collaboration with the following strategies:

- a. Develop and evaluate pre-clinical models to study pathogenesis and protective immunity and to identify correlates of immunity that facilitate development of vaccines to address unmet needs for the developing world.
- b. Conduct international collaborations for surveillance of new forms of HIV that evade blood screening assays, by evaluating HIV strains in Africa and elsewhere around the world.
- c. Transfer FDA advances to the public domain to facilitate development of vaccines and blood and blood products that promote global public health.
- d. Encourage research that supports the development of new products to treat infectious diseases affecting millions globally.

Objective C: Enhance the ability of advances in science and technology to facilitate development of safe and effective biological products

CBER will evaluate and implement novel methods that facilitate development of innovative products for unmet medical needs such as cancer and rare diseases. As we regulate products that rely on living cells, two emerging product areas that hold great promise for regenerative medicine include stem cell-derived products and tissue-engineered medical products (TEMPs), which often are combination products comprising two or more regulated components.

CBER intends to enhance the ability of advanced science and technology through the following strategies:

- a. Evaluate and implement novel methods that enhance product safety or quality and implement novel approaches to clinical trial design and analysis.
- b. Facilitate development of products by developing better preclinical models for assessing new treatments, and evaluating new gene transfer techniques.

Objective D: Improve research excellence and accountability

CBER will improve research excellence and accountability through enhancing facilities, providing training, facilitating opportunities for leveraging resources, and periodically evaluating research programs and the works of our scientists.

CBER will provide state-of-the-art research facilities for research programs through new laboratory and office buildings on the FDA White Oak campus. CBER will use regulatory science to address issues that impact development of complex biologics

products by implementing Cooperative Research & Development Agreements (CRADAs), Research Collaboration Agreements, and Material Transfer Agreements that facilitate scientific collaborations with scientists in other agencies, academia, and industry. The Center will also augment its access to and utilization of high performance computing resources through improved availability of application engineering expertise and talent support for advanced computing.

CBER will also protect intellectual property arising from its research, seek funding from private foundations and government agencies with shared goals, and use fellowship programs to train and develop scientists early in their career and attract and recruit scientists who have skills and knowledge needed at CBER. CBER will conduct research that is critical to public health and regulatory priorities in an efficient and scientifically rigorous manner.

CBER intends to enhance research excellence and accountability through the following strategies:

- a. Strengthen an infrastructure that supports high-quality state-of-the-art scientific investigations.
- b. Provide training opportunities and leverage resources for collaborative science to address novel regulatory issues through science and research expertise.
- c. Evaluate research programs to ensure quality, productivity, and FDA and CBER mission relevance.

Goal 6: Manage for organizational excellence and accountability

CBER has established this goal to ensure its human and capital resources are effectively allocated and managed in order to support the mission of the Center. This goal will also support the Center's efforts to improve program administration, strategic communications and business process modernization.

This cross-cutting goal supports the achievement of the four overarching program goals and the other cross-cutting goal on regulatory science and research. Providing the framework and means to achieve these strategic goals will put CBER in a position to fulfill the regulatory mission of promoting and protecting public health. The promise of 21st Century medicine includes exciting therapeutic strategies that are based on dramatic breakthroughs in medical research. CBER is committed to maintaining knowledgeable and skilled staff that is able to provide timely regulatory oversight to help products reach the market and monitor their safety. This goal will be achieved through seven objectives described below.

Objective A: Recruit, develop, retain, and strategically manage a world-class workforce

Employees at CBER are its most valuable resource. CBER is committed to having the expertise needed to accomplish its mission and to meet the anticipated demands of the future. Recruitment, development, retention and management are more than just human resource issues; they have the potential to impact the public's health and safety. To this end, CBER is committed to attracting the best scientists and administrative talent and supporting and developing these employees.

The broad public health mission of CBER requires staff to constantly hone and update their skills and expertise to keep pace with scientific and technical advances. The Center supports these training needs, starting at orientation of new employees and continuing throughout their careers. This support enables staff to be well prepared to perform regulatory, research, supervisory and other duties.

CBER intends to manage and develop a skilled workforce through the following strategies:

- a. Develop and implement a CBER Strategic Human Capital Plan to include workforce planning and succession planning.
- b. Consider recruitment and retention incentives that will effectively allow CBER to compete in the marketplace for skilled talent.
- c. Evaluate, update and implement a CBER training program that further develops skills and knowledge of our employees.

Objective B: Ensure program integrity and responsible stewardship through effective administration of fiduciary responsibilities

CBER will continue to make improvements to ensure programs are adequately resourced and have the capability to respond to future needs. CBER will strengthen its fiscal oversight, internal controls, and program accountability by integrating and aligning requirements, as well as ensuring compliance with financial reporting.

CBER intends to ensure program integrity and responsible stewardship through the following strategies:

- a. Implement processes to promote timely financial planning for allocating resources, ensure oversight of financial accountability, and strengthen internal controls for identifying and correcting program deficiencies.
- b. Ensure compliance with financial and program requirements and reporting.
- c. Ensure program needs are adequately resourced to build the capability to respond to future needs.

Objective C: Ensure facilities infrastructure provides a high quality work environment

CBER is committed to maintaining a high-quality work environment, and is now in the preparatory stages for relocation planning and related activities associated with the eventual move to the White Oak Campus (White Oak) in Silver Spring, Maryland in 2014. The new work location will provide state-of-the-art facilities and greater potential for collaboration both within CBER and across FDA in product review, policy, compliance and research. The General Services Administration (GSA) began construction of the new CBER facility in September 2010.

Prior to the actual relocation of staff and equipment to White Oak, CBER will assess ongoing laboratory capabilities, make adjustments to plans, provide technical assistance to FDA and GSA on construction plans, develop plans to inventory laboratory equipment, prepare decommissioning plans for disposal of laboratories at other Federal facilities, finalize acquisition plans for new equipment, and prepare detailed relocation plans.

CBER intends to ensure a high quality work environment through the following strategies:

- a. Ensure that technical assistance concerning CBER requirements is provided to FDA/GSA in the construction of laboratory and office buildings at White Oak.
- b. Develop and implement a decommissioning plan for laboratories that will be closed when the move is completed at White Oak.
- c. Develop and implement a relocation plan for CBER's move to White Oak.
- d. Create strategies to address ongoing research during the move process.

Objective D: Ensure effective strategic communications to address information needs and concerns of both internal and external audiences

CBER will strengthen its practices regarding communications to all audiences. CBER will improve its strategy for providing clear and concise messages about work and product safety, and will ensure those messages reach the right audiences using the most effective channels. CBER supports the implementation of the FDA Strategic Plan for Risk Communication which is located at:

<http://www.fda.gov/AboutFDA/ReportsManualsForms/Reports/ucm183673.htm>

CBER intends to improve communication effectiveness through the following strategies:

- a. Evaluate and improve key CBER communication channels including CBER intranet and internet web sites.
- b. More effectively coordinate the development and execution of internal and external CBER communications.
- c. Assist in the implementation of the FDA Strategic Plan for Risk Communication.
- d. Use social media (i.e., Twitter, etc) to disseminate public health messages about CBER-regulated products.
- e. Standardize and implement posting of safety alerts and labeling changes to coincide with agency process changes (MedWatch).

Objective E: Implement an IT modernization program to provide state-of-the-art integrated information and shared data resources

CBER is committed to ensuring the modernization of its information technology systems to handle the increased complexity concerning the regulation of biological products. Modern integrated systems provide cutting edge analytic tools allowing CBER scientists access to high quality data and effective communication vehicles necessary to achieve its mission of protecting and promoting the public health. Working with the agency's Office of Information Management and the FDA Informatics Governance Board, CBER will advance its implementation of the agency's Informatics and Enterprise Information Technology Initiatives.

CBER intends to refine its IT strategy to strengthen information integration and shared data resources through the following approaches:

- a. Evaluate, recommend and coordinate CBER IT projects to the FDA Informatics Governance Board for consideration.
- b. Leverage and collaborate on IT projects that impact CBER and cross agency business processes.
- c. Ensure CBER concerns are addressed in standards development and Data Standards Committee activities.

Objective F: Foster a culture of continual business process improvement to enhance the overall operation and effectiveness of the FDA

CBER will continue to improve its business processes so that mission critical work is performed in an effective and efficient manner. CBER seeks to make improvements in quality and overall program performance management. We strive to implement quality standards into mission critical programs.

CBER intends to strive toward continual business process improvement through the following strategies:

- a. Develop and implement improved strategic-operational planning processes to execute strategies and support resource decisions.
- b. Conduct analyses of business processes and procedures to ensure effectiveness and improve efficiency.
- c. Implement improvements to address deficiencies where needed.
- d. Implement quality assurance initiatives throughout the Center.

Objective G: Improve transparency, collaboration and participation

In June 2009, FDA introduced its Transparency Initiative which is designed to make useful and understandable information about FDA activities and decision-making more readily available to the public. CBER is committed to assisting in this important endeavor of accountability and open government. CBER will improve transparency to the public and stakeholders to ensure regulatory decision-making and programs are explained in a timely and user friendly manner.

CBER intends to become more transparent by employing the following strategies:

- a. Continue to seek feedback from customers/stakeholders through mechanisms such as customer satisfaction surveys and other mechanisms to achieve a level of excellence in both performance and customer service.
- b. Implement the recommendations of the Commissioner's Transparency Task Force impacting CBER.
- c. Continue to engage all CBER offices in the FDA-TRACK Initiative for managing performance by tracking and evaluating the results of the measures. Share our progress regularly with the agency and the public on this initiative.
- d. Implement the FDA's responsibilities covering CBER under the HHS Open Government Plan.

Appendix A: Alignment of DHHS Strategic Goals / Objectives with FDA Strategic Priorities and CBER Strategic Goals

DHHS Strategic Goals	DHHS Objectives	FDA Strategic Priorities/ Goals/ L-T Objectives	CBER Strategic Goals
Goal 1 – Transform Health Care	B – Improve healthcare quality and patient safety	<p>2.3- Strengthen Compliance and Enforcement Activities to Support Public Health (<i>cross-cutting priority</i>)</p> <p>3.2- Promote Public Health by Advancing the Safety and Effectiveness of Medical Products (<i>goal</i>)</p> <p>3.2.2- Advance Biologics Safety and Effectiveness (<i>long-term objective</i>)</p>	<p>Goal 2- Improve global public health through international collaboration including research and information sharing</p> <p>Goal 3- Enhance the ability of advances in science and technology to facilitate development of safe and effective biological products</p> <p>Goal 4- Ensure the safety of biological products</p>
Goal 2 – Advance Scientific Knowledge and Innovation	C – Invest in the regulatory sciences to improve food and medical product safety	<p>2.1- Advance Regulatory Science and Innovation (<i>cross-cutting priority</i>)</p> <p>3.2- Promote Public Health by Advancing the Safety and Effectiveness of Medical Products (<i>goal</i>)</p> <p>3.2.2- Advance Biologics Safety and Effectiveness (<i>long-term objective</i>)</p>	<p>Goal 2- Improve global public health through international collaboration including research and information sharing</p> <p>Goal 3- Enhance the ability of advances in science and technology to facilitate development of safe and effective biological products</p> <p>Goal 5- Advance regulatory science and research</p>
Goal 3 – Advance the Health, Safety, and Well-Being of the American People	E – Reduce the occurrence of infectious diseases		<p>Goal 1- Increase the nation’s preparedness to address threats as a result of terrorism, pandemic influenza and emerging infectious diseases</p> <p>Goal 2- Improve global public health through international collaboration including research and information sharing</p>
	F – Protect Americans’ health and safety during	<p>2.5- Advance Medical Countermeasures (<i>cross-cutting priority</i>)</p> <p>3.2- Promote Public Health</p>	<p>Goal 1- Increase the nation’s preparedness to address threats as a result of terrorism, pandemic influenza and emerging infectious diseases</p>

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DHHS Strategic Goals	DHHS Objectives	FDA Strategic Priorities/ Goals/ L-T Objectives	CBER Strategic Goals
	emergencies, and foster resilience in response to emergencies		Goal 2- Improve global public health through international collaboration including research and information sharing
Goal 4 – Increase Efficiency, Transparency, and Accountability of HHS Programs	A – Ensure program integrity and responsible stewardship of resources	3.4- Manage for Organizational Excellence and Accountability (<i>goal</i>)	Goal 6- Manage for organizational excellence and accountability
Goal 5 – Strengthen the Nation’s Health and Human Service Infrastructure and Workforce	A – Invest in the HHS workforce to help meet America’s health and human service needs today and tomorrow	3.4- Manage for Organizational Excellence and Accountability (<i>goal</i>)	Goal 6- Manage for organizational excellence and accountability
	C – Enhance the ability of the public health workforce to improve public health at home and abroad	3.2- Promote Public Health by Advancing the Safety and Effectiveness of Medical Products (<i>goal</i>) 3.2.2- Advance Biologics Safety and Effectiveness (<i>long-term objective</i>)	Goal 1- Increase the nation’s preparedness to address threats as a result of terrorism, pandemic influenza and emerging infectious diseases Goal 2- Improve global public health through international collaboration including research and information sharing Goal 6- Manage for organizational excellence and accountability

CBER Strategic Plan FY 2012-2016

DHHS Strategic Goals	DHHS Objectives	FDA Strategic Priorities/ Goals/ L-T Objectives	CBER Strategic Goals
	E – Improve national, state, local, and tribal surveillance and epidemiology capacity	<p>3.2- Promote Public Health by Advancing the Safety and Effectiveness of Medical Products (<i>goal</i>)</p> <p>3.2.2- Advance Biologics Safety and Effectiveness (<i>long-term objective</i>)</p>	<p>Goal 1- Increase the nation’s preparedness to address threats as a result of terrorism, pandemic influenza and emerging infectious diseases</p> <p>Goal 3- Enhance the ability of advances in science and technology to facilitate development of safe and effective biological products</p> <p>Goal 4- Ensure the safety of biological products</p>

Note: In the above table, DHHS Strategic Goals and Objectives are taken from *U.S. Department of Health and Human Services Strategic Plan for Fiscal Years 2010-2015*: <http://www.hhs.gov/secretary/about/priorities/priorities.html>.
 FDA Strategic Priorities/Goals/L-T Objectives are taken from *FDA Strategic Priorities 2011-2015*: <http://www.fda.gov/AboutFDA/ReportsManualsForms/Reports/ucm227527.htm>.
 CBER Strategic Goals are taken from *CBER Strategic Plan FY 2012-2016*: <http://www.fda.gov/downloads/AboutFDA/CentersOffices/OfficeofMedicalProductsandTobacco/UCM266867.pdf>

Appendix B: Glossary of Acronyms

AERS – Adverse Event Reporting System

CBER – Center for Biologics Evaluation and Research (at FDA)

CDC - Centers for Disease Control and Prevention

CRADA – Cooperative Research and Development Agreement

DHHS or HHS – Department of Health and Human Services

DHS – Department of Homeland Security

DoD or DOD – Department of Defense

EDQM – European Directorate for the Quality of Medicines and Healthcare

EID – Emerging infectious diseases

EMA - European Medicines Agency

FDA – Food and Drug Administration

FDAAA – Food and Drug Administration Amendments Act of 2007

GETS - Genomics Evaluation Team for Safety

GHTF - Global Harmonization Task Force

GMP - Good manufacturing practices

GSA - General Services Administration

HIV – Human immunodeficiency virus

ISO – International Standards Organization

IT – Information technology

MCM – Medical Countermeasure

MedWatch – FDA Medical Products Reporting Program

NGOs – Non-government organizations

NIH - National Institutes of Health

NIBSC - National Institute for Biological Standards and Control

NMR - Nuclear magnetic resonance

ORA - Office of Regulatory Affairs (at FDA)

PAHO – Pan American Health Organization

PATH-MVI – PATH Malaria Vaccine Initiative

PDPs – Product development partnerships

SARS-CoV – Severe Acute Respiratory Syndrome coronavirus

TB – Tuberculosis or TB (short for *tubercles bacillus*)

TEMP - Tissue-engineered medical products

VAERS – Vaccine Adverse Event Reporting System

vCJD - variant Creutzfeldt-Jakob Disease

VRBPAC - Vaccines and Related Biological Products Advisory Committee

WHO – World Health Organization

Appendix C: Major Laws affecting CBER

CBER operates under the following legal authorities:

1. Public Health Service Act
2. Federal Food, Drug, and Cosmetic Act* (21 U.S.C. 321-399)
3. Medical Device Amendments of 1976*
4. Clinical Laboratory Improvement Amendments of 1988 (42 U.S.C. 201)
5. Safe Medical Devices Act of 1990*
6. Medical Device Amendments of 1992*
7. Food and Drug Administration Modernization Act*
8. Medical Device User Fee and Modernization Act of 2002*
9. Public Health Security and Bioterrorism Preparedness Response Act of 2002*
10. Project BioShield Act of 2004 (21 U.S.C. 360bbb-3)
11. Medical Device User Fee Stabilization Act of 2005*
12. Food and Drug Administration Amendments Act of 2007*
13. Biologics Price Competition and Innovation Act of 2009*
14. Patient Protection and Affordable Care Act, 2010*

* Authorities under this act do not appear in sequence in the U.S. Code. The authorities are codified as amended in scattered sections of 21 U.S.C.

Appendix D:

