

Site Visit Report

Review of Intramural Research

Office of Blood Research and Review

CBER, FDA

Date of Site Visit: July 22, 2005

Date of Report: February 10, 2006

OBRR Site Visit Review Team

Site Review Committee
was selected for a
broad base of
background and
experience, including:

- ❖ BPAC members
- ❖ Basic science research
- ❖ Clinical research
- ❖ Blood collection
- ❖ Industry

Dr. James Allen, Chairman

Dr. Harvey Alter

Dr. Michael Busch

Dr. Donna DiMichele

Dr. Marcos Intaglietta

Dr. Harvey Klein

Dr. Suzette Priola

Dr. George Schreiber

Dr. Michael Strong

Dr. Peter Tomasulo

Dr. Ching Wang

OBRR Intramural Research Review

- ❖ Periodic review of progress and performance of OBRR research program
- ❖ Review intent: Overarching summary of research program's goals and support
 - Not a focused review of individual investigators and their work

OBRR Intramural Research Review

Review included:

- ❖ Evaluation of background information about OBRR and its function within CBER
- ❖ Written research program descriptions
- ❖ Report on Review of Research Programs at CBER (dated October 21, 1998)
- ❖ CVs of research investigators
- ❖ Selected publications
- ❖ Oral presentations, questions, discussion

CBER Review of Research Programs

Summary from 1998 Report:

- ❖ The review committee strongly endorsed:
 - The fundamental need for basic science research at OBRR to support its regulatory mission
 - Adequate funding of the research program to assure its success and its ability to attract first-rate scientists

OBRR Intramural Research Review

Background: OBRR maintains an active laboratory research program that is:

- ❖ Integral to FDA's Critical Path research initiative
- ❖ Mission-focused to enhance its regulatory functions
- ❖ Primarily targeted at current regulatory issues but with flexibility to respond to new regulatory concerns and safety issues

OBRR Intramural Research Review

- ❖ Principle investigators and senior research staff at OBRR are expected to spend about half time on research activities and half time on regulatory activities
 - Balance is rarely achieved
 - Does not account for regulatory time frames and priorities
 - Does not account for other significant and time-consuming activities

OBRR Intramural Research Review

Evidence of research program success:

- ❖ In total, senior scientists in OBRR regularly publish more than 50 articles per year and have abstracts accepted at scientific meetings
- ❖ Progress assessments on external laboratory site visits generally are favorable
- ❖ OBRR staff sponsor and organize workshops on specific topics of importance

OBRR Intramural Research Review

Overall site review summary:

- ❖ OBRR research programs merit high grades for depth and quality of research
- ❖ Research agendas have been diversified and productive
- ❖ Research programs are directly applicable to the FDA's Critical Path of biologics product development

OBRR Intramural Research Review

In comparison with the 1998 CBER review, the OBRR research programs have improved in:

- ❖ Focus and relevance to mission
- ❖ Quality
- ❖ Diversity of funding sources, eg through developing innovative alternative funding sources and establishing collaborations

Conclusions & Recommendations: 1

- ❖ The FDA's emphasis on a strong intramural research program to support its Critical Path program for effective and efficient regulatory activities is important and commendable.
- ❖ Experienced and active research scientists on staff involved in both regulation and research that supports the regulatory effort is essential for the most effective regulatory program that facilitates approval of biological products and protects the health and safety of the American public.

Conclusions & Recommendations: 2

- ❖ OBRR management and scientists are highly commended for the depth and quality of the research program, especially considering the simultaneous heavy regulatory workload.
- ❖ The research program has increased in diversity, productivity, and value over the years despite significant restrictions in both budgets and personnel.
- ❖ The research program contributes directly to the FDA's Critical Path program.

Conclusions & Recommendations: 3

- ❖ The issue of sufficient time and qualified personnel to conduct research remains important.
- ❖ The work environment must be competitive to be able to attract outstanding young scientists and to retain senior scientists as principle investigators and regulators.
- ❖ These issues are critical to the continuation of an effective and productive research program that supports the regulatory mission.

Conclusions & Recommendations: 4

- ❖ Funding to support the OBRR research program is a critical issue.
- ❖ This includes support for basic activities including reagents, supplies, and adequate equipment.
- ❖ The meager budget available to OBRR through Congressional appropriations to support research directly is totally inadequate to conduct even a significant part of the wide range of important program priorities for which OBRR is responsible.

Conclusions & Recommendations: 5

- ❖ Developing options to increase the OBRR research budget through sources outside the FDA are essential, although difficult and time-consuming for OBRR staff.
- ❖ Opportunities for collaboration and to seek acceptable funding sources must be pursued, although this obviously must be accomplished within the confines of the research priorities established by OBRR (and the restrictions that exist for the FDA as a Federal regulatory agency).

Conclusions & Recommendations: 6

- ❖ Adequate laboratory space and equipment are essential components of a strong and productive research program.
- ❖ If these cannot be assured in the future, they could have an impact on future research activities (and the ability to attract and retain quality research investigators).
- ❖ These issues need to be addressed as funding is sought to support the research program.

Conclusions & Recommendations: 7

- ❖ It is imperative that OBRR have the flexibility, capacity and resources to address new scientific and regulatory issues that become apparent at any point in time, perhaps as a crisis.
- ❖ Planning for these is difficult, especially when OBRR is also faced with decisions about trying to develop a more focused research program.
- ❖ These issues must be factored into the decisions about future research program directions and priorities.

Conclusions & Recommendations: 8

- ❖ Given current research funding limits, OBRR must decide whether to try to maintain a broad array of research activities that address the range of its mandate or whether to focus on a smaller number of research topics and priorities.
- ❖ A narrow focus allows staff to develop greater expertise and critical mass in fewer areas.
- ❖ With a narrow focus, OBRR could define a research matrix based on the potential to collaborate with academia or industry through contracts, etc.

Conclusions & Recommendations: 9

- ❖ A related issue to the breadth of the research focus is the need for OBRR to define the best mechanism to identify research priorities to be pursued, either through intramural research or outsourcing.
- ❖ This issue may already be resolved by OBRR and a good mechanism may be in place, but it was not presented to or discussed with the Committee.

Conclusions & Recommendations: 10

- ❖ OBRR needs to give attention to the potential or perception of bias being introduced into the regulatory process by intramural research findings that are portrayed as FDA policy positions.

Conclusions & Recommendations: 11

- ❖ Visibility of the OBRR research program is important for its broader acceptance and support.
- ❖ Despite the meritorious work accomplished, the extent, quality, and importance of the work is not widely recognized or appreciated outside the FDA
- ❖ OBRR/CBER must define and exploit opportunities to expand the visibility of their research programs.
- ❖ The research program activities should be strongly and visibly linked to the regulatory activities that they support.

Conclusions & Recommendations: 12

- ❖ To directly enhance funding to support research activities, OBRR should work with FDA, DHHS, and Congress to identify creative funding mechanisms.
- ❖ Establishing a research endowment fund that could receive contributions from major philanthropic organizations, private donors, and regulated industry might be one example for which precedents already exist.

OBRR Intramural Research Review

Summary:

- ❖ The Committee strongly supports the FDA's emphasis on a strong intramural research program to support its Critical Path Initiative for effective and efficient regulatory activities.
- ❖ Adequate funding and other resources, including outstanding staff, are essential to support OBRR's research program and the FDA's Critical Path Initiative, which in turn facilitate the important licensure and regulatory activities of OBRR.