Supporting a Strategic Research Agenda in Transfusion Medicine

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Working Group on Strategies to Optimize Blood Products
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A strategic plan for blood safety necessitates*:

- A proactive, prioritized, and goal-oriented research agenda to improve the safety and availability of the Nation’s blood supply.
- Timely funding.
- Intensifying efforts to influence clinical practices related to blood transfusion and alternative therapies, based on scientific evidence.
- Pursuing opportunities to enhance public health in the management of blood donors.

*ACBSA recommendations, September 2005
Establishing a Strategic Research Agenda

- Need to continuously monitor and identify scientific priorities

- Know what research and resources are currently supported
  - Investigator-initiated research
  - Institute-initiated programs

- Identify and rectify gaps in research support and funding mechanisms, and provide funding opportunities
  - Is the type of research mix i.e., basic, translational, clinical adequate to address scientific priorities?
  - Are resource programs easily accessible?
  - Encourage investigator-initiated research in gap areas (e.g., R01, R21, SBIR)
  - Develop Institute-initiated initiatives that complement investigator-initiated research (RFA, RFP, PAR, and PA)

- Monitor progress using established metrics
Know what Research is Supported

- Grant portfolio in Transfusion Medicine (2003-2009)
  - Average of 10 grant applications per fiscal year (excludes specific RFA or RFP announcements)
  - 0 - 1 grant funded per fiscal year
  - Lack of an external review panel with expertise in transfusion medicine
  - Lack of support for clinical trial pilot studies
  - Lack of support for ancillary studies to clinical studies
  - Lack of support for secondary dataset analyses

- Institute-initiated Programs in Transfusion Medicine
  - SCCOR program in Transfusion (Two U01s) – now completed
  - REDS-II and REDS-III Programs
  - Transfusion Medicine and Hemostasis Clinical Trials Network
  - NHLBI Biorepository: a resource
Encourage investigator-initiated research

- Two NHLBI Program Announcements with review (PARs) are now available to investigators interested in submitting R01 or R21 applications, respectively, that address research topics in transfusion medicine:
  - Special Review Panel with expertise in transfusion medicine and particular areas under review
  - One more due date (October 2012) but PARs to be renewed for 3 more years

Since the issuance of these PARs

- Average of 53 applications received per fiscal year
- Average of 6 grants funded per fiscal year.
Support of Clinical Trial Pilot Studies

- NHLBI Clinical Trial Pilot Studies (R34) – PAR-10-005
  (At http://grants.nih.gov/grants/guide/pa-files/PAR-10-005.html)
  - Budget and Project Period: Up to three years. Direct costs are limited to $450,000 over a three-year period, with no more than $225,000 in direct costs allowed in any single year
  - Standard dates apply
  - Expiration Date: January 8, 2013 but PAR to be renewed for 3 years

- The Clinical Trials Development Resource for Hematologic Disorders (U24)
  - Provides consultative services for the development of multi-site clinical trials to grantees of the Clinical Trials Planning Studies for Rare Thrombotic and Hemostatic Disorders (U34) program and to grantees of the NHLBI Clinical Trial Pilot Studies (R34) (PAR-10-005) program, in the area of blood diseases and resources.
Support of Ancillary Studies and Secondary Dataset Analyses for Clinical Studies

- NHLBI Ancillary Studies in Clinical Trials (R01) – RFA-HL-13-003
  - Applications need to propose time-sensitive ancillary studies related to NHLBI’s mission in conjunction with ongoing clinical trials and other large clinical studies supported by NIH or non-NIH entities.
  - Applications must focus on areas that will not already be studied in the parent trial.
  - Special Application due dates (Sept 24, 2012; Jan 24, 2013)
  - Expiration Date: January 25, 2013 but PAR to be renewed
  - Excellent opportunity for junior investigators
  - Can request $250,000 in direct costs per year

- Upcoming: NHLBI PAR (R21) for secondary dataset analyses
Support of NHLBI Multicenter Clinical Trials

- PAR 10-096 NHLBI Investigator-Initiated Multisite Clinical Trials (Collaborative R01)
  - Trials may be Phase II or Phase III
  - The trials may involve clinical or behavioral interventions.
  - The trials may randomize at the individual (patient) level or at a group level (e.g., randomization of clinics, schools, worksites).
  - Data Coordinating Center must submit a separate application.
  - Can have separate applications for core functions e.g. reading centers, quality of life/economic analyses, imaging centers--but must be heavily justified.
  - NHLBI review (expertise in classical as well as other clinical trial study designs)
Philosophy:
Creative team engineering involves diverse kinds of innovation across basic, applied, translational, behavioral, and clinical research and practice. These innovations include discovery and development of the scientifically new in basic research and translation, but also novel applications or combinations of proven ways to achieve performance specifications, reproducibility, reliability, cost control, quality, manufacturability, ease of use, or validation.

PAR-10-234: Bioengineering Research Partnerships (BRP)[R01]

Purpose. In the context of this program, a partnership is a multi-disciplinary research team, that applies an integrative, systems approach to develop knowledge and/or methods to prevent, detect, diagnose, or treat disease or to understand health and behavior. The partnership must operate according to a clear leadership plan and include appropriate bioengineering or allied quantitative sciences in combination with biomedical and/or clinical components.

- Standard Application Due Dates
- Expiration Date: September 8, 2013
PA-10-009: Bioengineering Research Grants (BRG) [R01]

**Purpose.** The BRGs support multi-disciplinary research performed in a single laboratory or by a small number of investigators that applies an integrative, systems approach to develop knowledge and/or methods to prevent, detect, diagnose, or treat disease or to understand health and behavior. A BRG application may propose hypothesis-driven, discovery-driven, developmental, or design-directed research.

PA-10-010: Exploratory/Developmental Bioengineering Research Grants (EBRG) [R21]

**Purpose.** An EBRG application may propose hypothesis-driven, discovery-driven, developmental, or design-directed research. The research proposed under this program can explore approaches and concepts new to a particular substantive area; research and development of new technologies, techniques or methods; or initial research and development of data upon which significant future research may be built.

- Standard Application Due Dates
- Expiration Date: January 8, 2013
SBIR/STTR: 3-Phase Program

**PHASE I**
- Feasibility Study
- $150K and 6-month (SBIR) or 12-month (STTR) Award

**PHASE II**
- Full Research/R&D
- $1 Million for 2-year Award (SBIR/STTR)

**PHASE III**
- Commercialization Stage
- Use of non-SBIR/STTR Funds
SBIR R43/R44 Omnibus Grant Solicitation

- Phase I: Funding for proof-of-concept or feasibility studies. Generally, up to $150,000 for six months.
- Phase II: Funding for continued R&D effort: Generally, up to $1,000,000 for two years.
- Phase IIB Bridge Awards: Funding for continued R&D effort: up to $1 million in total costs per year for three years.

STTR R41/R42 Omnibus Grant Solicitation

- Phase I: Funding for proof-of-concept or feasibility studies. Generally, up to $100,000 for one year.
- Phase II: Funding for continued R&D effort: Generally, up to $750,000 for two years.
SBIR Omnibus Contract Solicitation

- RFP Topics supposed to be released in August 2012
NIH AIDS Funds are distributed to various NIH Institutes based on priorities established each year

HIV-related applications are funded from an Institute’s AIDS dollar line. HIV-related applications have different application dates

NHLBI HIV/AIDS Program Evaluation

- Ongoing (started in 2012)
- Establishment of a formal NHLBI HIV/AIDS Committee (DBDR Representatives: Shimian and Simone)

Working Group to be held September 6/7, 2012

- Identify Key Scientific Opportunities to Advance Knowledge for both the HIV/AIDS Population and the General Population
- Recommendations from the Scientific Community to Develop the NHLBI HIV/AIDS Program
- Dialogue among Different Scientific Communities about HIV/AIDS Research in Heart, Lung, and Blood Diseases/Resources
- Strategies to Successfully Implement HIV/AIDS Research in Heart, Lung, and Blood
Encourage New Investigators to Enter the Field

- **Research training programs:**
  - Individual Postdoctoral National Research Service Award (F32)
  - Institutional National Research Service Award (T32)
  - Minority Institutional Research Training Program (T32)

- **Career Awards: Mentored research training programs for junior investigators that provide both part-time and full-time support for 2 to 5 years**
  - Mentored Clinical Scientist Development Award (K08)
  - Mentored Patient-Oriented Research Career Development Award (K23)
  - Mentored Quantitative Research Career Development Award (K25)
  - Pathway to Independence Award (K99/R00)
  - NHLBI has six K12 programs for training in benign hematology and transfusion
  - Individual K awards including KL2 from your CTSA

- **PAR-10-034 and PAR-10-033. PAR 10-033 is one of the few opportunities for an R21 application to NHLBI.**
Fostering Research through Collaborations with Other Offices, Agencies, and NIH Institutes

- DHHS, Office of the Assistant Secretary for Health, Office of HIV/AIDS and Infectious Disease Policy
- FDA
- DoD
- HHS Office of the Secretary for Preparedness and Response (ASPR), Biomedical Advanced Research Development Authority (BARDA)
- NIH Institutes – NIAID, NICHD, NIDCR, NCI
BARDA CBRN BAA-12-100-SOL-00011: A Broad Agency Announcement (BAA) for advanced development of therapies for hematopoietic syndrome, bone marrow stromal cell loss, and vascular injury resulting from acute exposure to ionizing radiation

- Area of Interest #4 includes blood products
- Funding by BARDA is through a contract with a heavy emphasis on commercialization; therefore a business plan is necessary as well as dedicated staff with regulatory knowledge
- These contracts have many milestones with specific deliverables at each point. Failure to deliver can result in the termination of the contract.