

1999-2015

BENCH-TO-BEDSIDE PROGRAM REPORT



www.cc.nih.gov/cc/btb/

Updated October 2016

TABLE OF CONTENTS

Background.....	3
Applications and Funded Projects.....	4
Collaborators.....	5
• Intramural Investigators.....	5
• Extramural Investigators.....	6
Program Funding.....	8
• By the Numbers.....	8
• Funding Structure.....	8
• Funding Process.....	10
• Program Management	11
Review Process.....	13
• Levels of Review.....	13
• Review Criteria.....	13
• Review Team.....	14
Scientific Highlights.....	15
• Data Sources.....	15
• Program Metrics.....	15
• BtB Benchmarks.....	17
• BtB Accomplishments.....	18
• Select Projects.....	19
• Communicating Award Successes.....	24
Alignment with U01 Program.....	25
• Distinctions between the BtB and U01 Award Programs.....	27
Appendix: List of all funded projects, 1999 – 2015.....	29
Contact Us.....	53

BACKGROUND

The Bench-to-Bedside (BtB) awards program was originally established by the Director of the Clinical Center, with the approval of the Director, NIH in 1998. Developed to encourage intramural collaborations between basic and clinical researchers, investigators are also challenged to consider cross-institute projects with the goal of speeding translation of promising laboratory discoveries into new medical treatments. Research teams initially included at least one basic and one clinical intramural researcher. The awards, which were \$100,000/year for 2 years, were supported by Clinical Center “carryover funds” for the first two competitive cycles, after which the institutes agreed to continue the program with their own funds. Beginning in 2003, NIH offices ‘joined’ the program. That year, the Office of AIDS Research (OAR) and the Office of Rare Diseases Research (ORDR) provided funding to support awards in their respective disciplines. In subsequent cycles, several other offices joined OAR and ORDR, and the portfolio of scientific categories was enhanced. In 2008, the awards were increased to \$135,000/year for 2 years. Over time, the program’s purpose has evolved; these small awards are viewed as seeds for new translational research projects reflecting partnerships between basic and clinical investigators, many of which lead to larger awards with expanded scientific goals.

In 2006, the program’s scope further broadened to encourage partnerships between intramural and extramural clinical researchers. This expansion required significant involvement of extramural leadership (i.e., the Extramural Activities Working Group), as well as the NIH Office of the General Counsel (OGC) to create a unique mechanism to fund both intramural and extramural investigators within one award. The efforts were successful and, since that time, 272 extramural investigators at 93 U.S. and international sites have received BtB funds via administrative supplements to existing NIH grants. Since 2006, ~90% of applications each cycle represent partnerships between intramural and extramural investigators.

Since the Bench-to-Bedside Program began over 15 years ago, a total of 238 collaborative projects have received approximately \$56 million in funding, representing partnerships among multiple NIH institutes and centers and extramural institutions. Applications are reviewed by a review team comprising intramural and extramural investigators (many are NIH R01 grantees). Awarded projects receive funding from various NIH offices and institutes and represent the research interest of the donor source. For some awards, recipient ICs either co-fund or fully fund awards.

The BtB Program has been reviewed favorably on multiple occasions beginning in 2004 with *The NIH Director’s Blue Ribbon Panel on the Future of Intramural Clinical Research*, chaired by Edward J. Benz, M.D. and Joseph L. Goldstein, M.D. The final report of the panel states, “The Bench-to-Bedside Awards program serves as a superb example of a highly successful program that fosters collaborations among intramural scientists and clinicians in areas of research that have the potential for improving understanding of an important disease process or for leading to a new therapeutic intervention. NIH should continue to foster and even expand this program.” In 2007 and 2010, two external program reviews by an independent contractor produced positive results with consistent support from all key stakeholders (see: <http://clinicalcenter.nih.gov/cc/btb/evaluations.html>).

The 2009 *FasterCures* Task Force on the NIH IRP Report offered the following comments on the program: “Some efforts have been made to increase use (of the Clinical Center). In 1999, the Clinical Center established the Bench-to-Bedside Awards to integrate the work of basic and clinical scientists on the NIH campus. The program expanded in 2006 to encourage partnerships between intramural and extramural programs. In 2009, the program will expand again so that extramural principal investigators with an existing NIH grant can initiate proposals by seeking an intramural partner at NIH to be the project leader and point of contact. This type of mechanism might be adapted to encourage greater use of the Clinical Center by extramural investigators collaborating with Clinical Center scientific personnel.” (Source: *FasterCures Task Force on NIH IRP Report, 2009*). Most recently, in 2010, the Scientific Management Review Board’s “*Report on the NIH Clinical Center*” included this recommendation for the BtB Program: “The role of the NIH Clinical Center should be to serve as a state-of-the-art national resource, with resources optimally managed to enable both internal and external investigator use.... In addition, the successful Bench-to-Bedside Program, which creates partnerships between intramural and extramural investigators, would benefit from a stable funding model with increased resources” (pg. 14).

APPLICATIONS AND FUNDED PROJECTS

Since the program’s inception, its management has remained within the CC Office of the Director, staffed by three individuals with distinct and complementary qualifications. Applications are accepted once a year. Great enthusiasm for these ‘small’ seed awards exists among intramural and extramural researchers, as depicted by the steady increase in applications until 2012. The most recent cycles highlight the diminishing funds for the program. As shown in Figure 3 below, available program funds decreased in 2011, leading to a decline in applications in subsequent years. While the program continues to thrive, its long term growth will depend on stable funding.

Figure 1. Funding by Cycle, \$ in Millions, 1999 – 2015

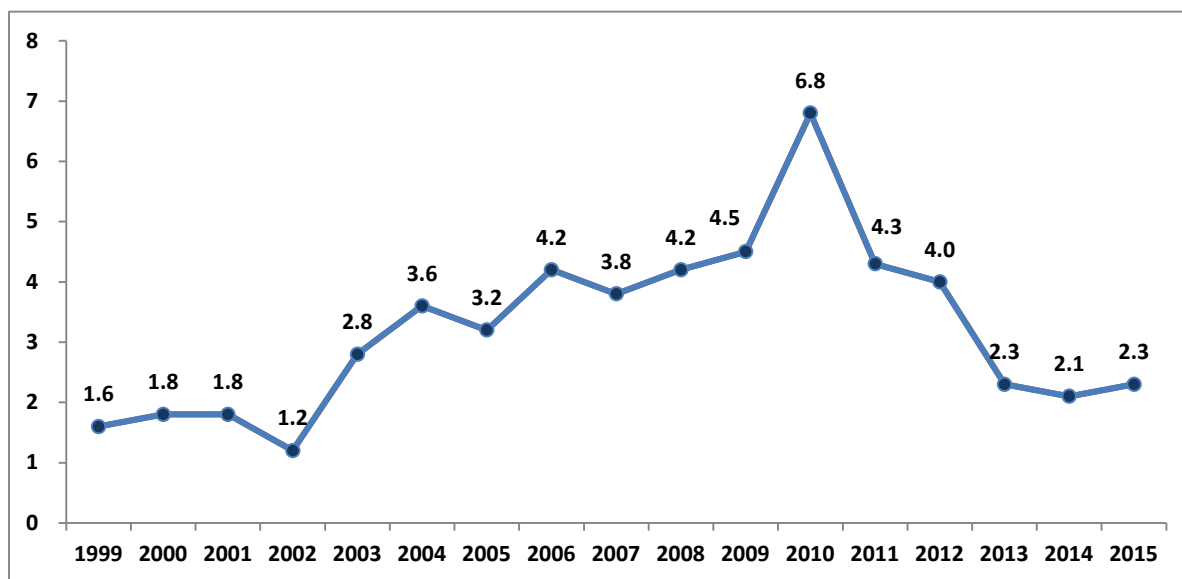
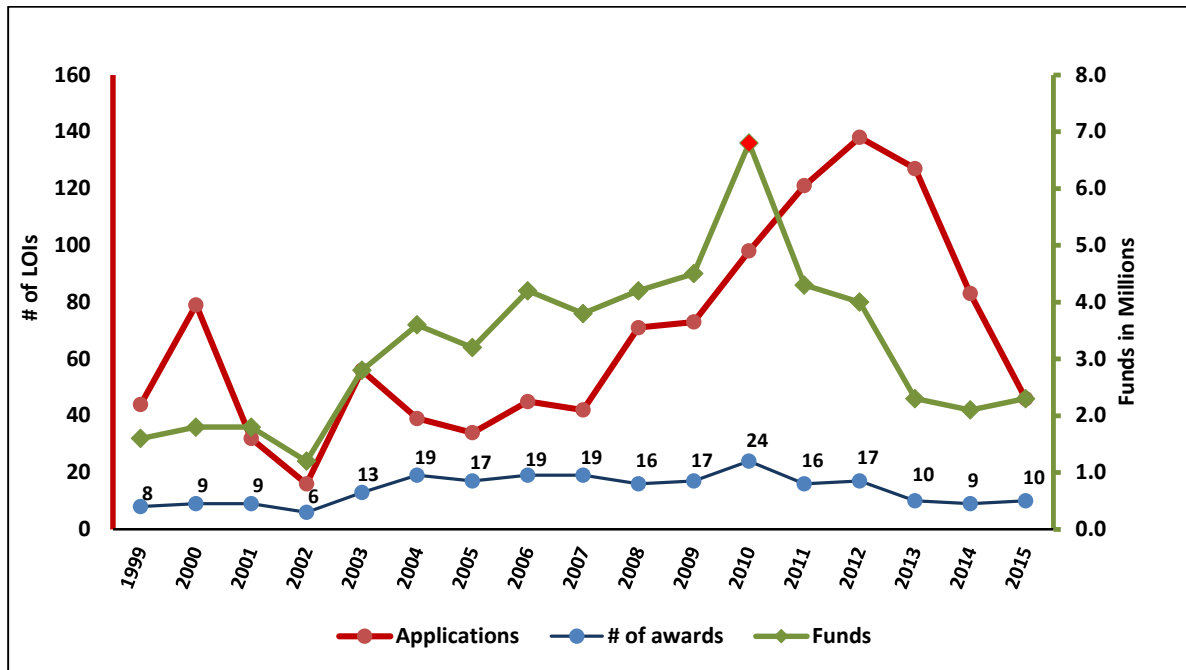


Figure 2. Trends in Funding Parallel Applicant Interest, 1999 - 2015



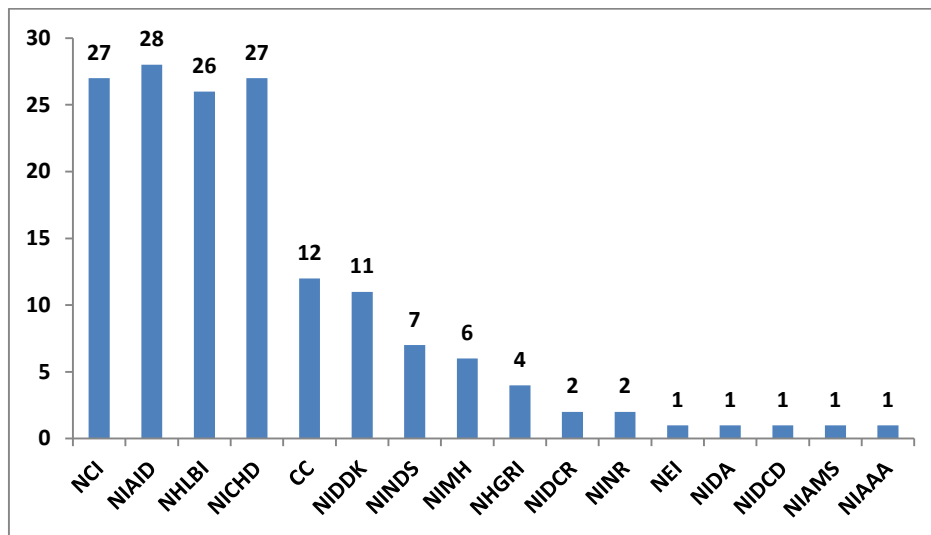
COLLABORATORS

Since its introduction in 1999, the Bench to Bedside program has sought to foster productive collaborations between intramural investigators from different disciplines, and since 2006 the scope has expanded to integrate investigators outside the NIH. Between 2006 and 2015, 427 intramural and 272 extramural investigators partnered on 157 awards. These investigators have represented a wide spectrum of intramural and extramural institutions.

Intramural Investigators

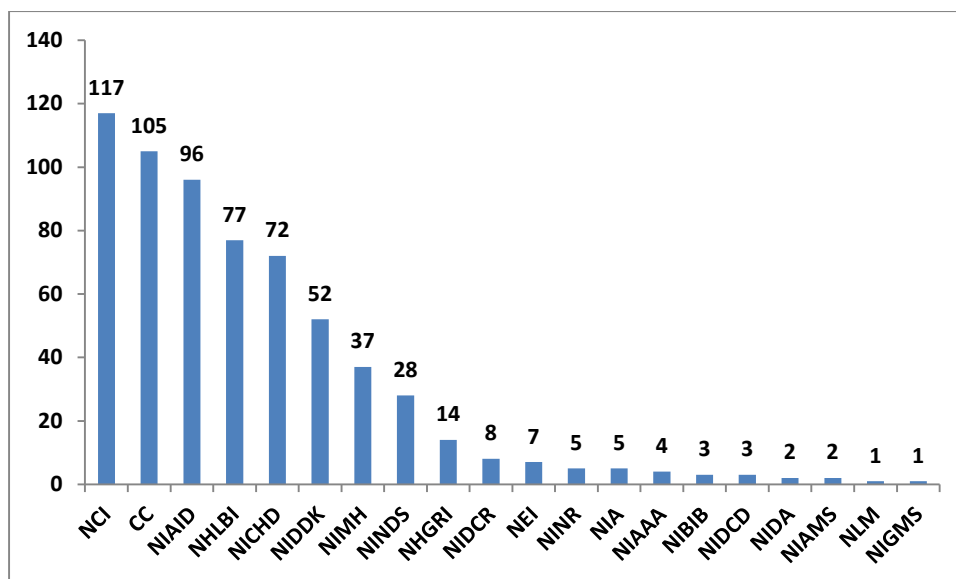
Between 2006 and 2015, intramural investigators from 14 ICs have led 157 BtB awards.

Figure 3. Bench-to-Bedside Awards by Lead Institute, 2006 – 2015



69 percent of these projects have been led by investigators from 4 institutes: National Institute of Allergy & Infectious Diseases (NIAID), National Institute for Child Health & Human Development (NICHD), National Cancer Institute (NCI), and the National Heart, Lung, & Blood Institute (NHLBI). Multiple IC investigators may be involved on a single award. The number of times specific ICs had investigators collaborating on BtB awards is shown as follows.

Figure 4. IC Involvement on BtB Awards, 2006 – 2015

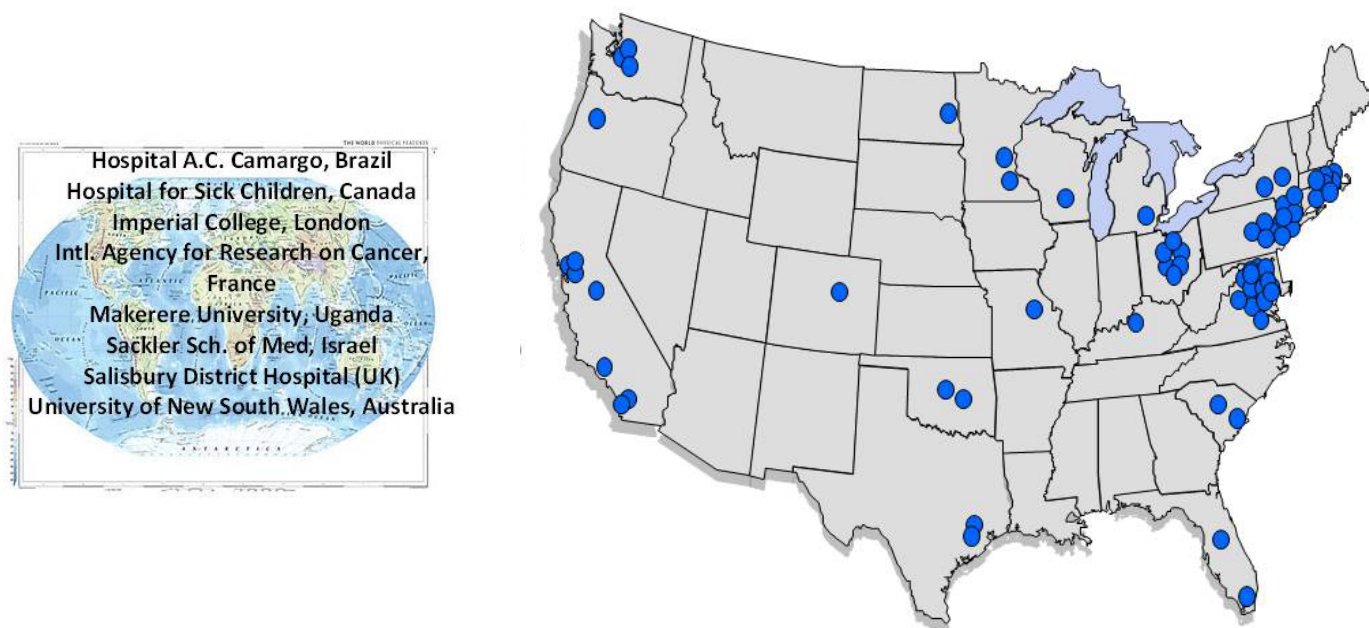


Extramural Investigators

Since extramural investigators were invited to partner on BtB awards in 2006, they have been involved in 143 projects (over 90% of awards during this period). These 272 investigators have represented 90 U.S. and international organizations. The map following illustrates the geographic diversity of the domestic institutions represented.

Originally, only intramural investigators could serve as the lead Principal Investigator (PI) on BtB projects; however, in 2009 NIH leadership endorsed a recommendation to allow extramural investigators to serve as the lead PI.

Figure 5. Domestic Extramural Institutions Partnering on BtB Awards, 2006 - 2015



BtB Extramural Collaborator(s)

Albert Einstein College of Medicine	Ludwig Institute for Cancer Research	University of California, San Francisco
Baylor College of Medicine	Maimonides Medical Center	University of Cincinnati
Benaroya Research Institute	Massachusetts General Hospital	University of Colorado
Beth Israel Medical Center	Mayo Clinic, Rochester, MN	University of Illinois
Boston University	Mayo College of Medicine	University of Louisville
Brigham & Women's	MD Anderson Cancer Center	University of Maryland
Case Western Reserve University	Medical University of South Carolina	University of Massachusetts Amherst
Catholic University of America	Memorial Sloan-Kettering Cancer Center	University of Massachusetts Worcester
Children's Hospital & Clinics of Minnesota	National Naval Medical Center	University of Miami
Children's Hospital of Philadelphia	Nationwide Children's Hospital	University of Michigan
Children's National Medical Center	Novartis	University of Minnesota
Cincinnati Children's Medical Center	Oklahoma University Health Sciences Center	University of Missouri-Columbia
Cleveland Clinic	Onconova Therapeutics, Inc.	University of North Dakota
Columbia University	Oregon State University	University of Oklahoma
Drexel University	Prince George's County Hospital	University of Pennsylvania
Fred Hutchinson Cancer Center	San Francisco General Hospital	University of Pittsburgh
George Washington University	SAIC	University of South Carolina
Georgetown University	St. Michael's Medical Center	University of Southern California
GlaxoSmithKline Pharmaceuticals	Stanford University	University of Virginia
Gynecologic Oncology Group	Temple University	University of Washington
Hackensack University Medical Center	The Rockefeller University	University of Wisconsin
Harvard University	Tufts University	US Food and Drug Administration
Howard University	Uniformed Services University of the Health Sciences	Walter Reed Army Medical Center
Ichan School of Medicine at Mt. Sinai	University of California, Davis	Washington Hospital Center
INOVA Fairfax Hospital	University of California, Los Angeles	Washington University
Johns Hopkins University	University of California, San Diego	Weill Cornell Medical College
Karmanos Cancer Center/Wayne State		Yale University

PROGRAM FUNDING

By the Numbers

BtB has provided over \$55 million in award funds to 238 projects over its 17 year history. Since 2006, when the program expanded to include extramural investigators as partners on BtB awards, BtB has provided over \$38 million. Of that amount, intramural investigators have received \$25 million, and extramural investigators have received \$13 million. For the last 5 award cycles (2011-2015), the average/year for total awards is \$3.14 million.

Between 2006 and 2015, 157 awards have been provided in 11 specific categories, with the most awards provided in the Rare Diseases (55), AIDS (36), and Minority Health (19) categories. It is noted that the Office of Rare Diseases Research restricted funding over the past three cycles such that the entire requested amounts have not always been awarded.

Funding Structure

Originally funded by Clinical Center carryover funds, the donor office model was introduced in 2003. Both the Office of Rare Diseases Research (ORDR) and the Office of AIDS Research (OAR) requested to join the program with funds targeted to high scoring projects within their respective disciplines (i.e., rare diseases and AIDS). These partnerships became models for other NIH donors, and in subsequent years, a total of ten entities provided funds to support BtB projects within the scope of their unique research interests.

Bench-to-Bedside Awards have been funded by the following NIH components:

- National Institute on Minority Health and Health Disparities (NIMHD),
- National Center for Research Resources (NCRR),
- Office of AIDS Research (OAR),
- Office of Behavioral and Social Sciences Research (OBSSR),
- Office of Intramural Research (OIR),
- Office of Dietary Supplements (ODS)
- Office of Rare Diseases Research (ORDR) in the National Center for Advancing Translational Research (NCATS),
- Office of Research on Women's Health (ORWH),
- Therapeutics for Rare and Neglected Diseases (TRND)

Another federal agency, the Food and Drug Administration (FDA), provided funds for two cycles and has indicated interest to participate in future cycles. The Office of Dietary Supplements (ODS) joined the program in 2014 and continues to participate.

Additionally, institutes have provided BtB funds in two situations:

- (1) some categories require funds from the ICs of the intramural investigators;

- (2) high scoring projects that could not be funded by donor funds have been funded entirely by the institute of the intramural PI on six occasions.

In the most recent 2015 cycle, five offices provided funds: OAR, OBSSR, ODS, ORDR/NCATS, and ORWH. While the donor offices' generous support has enabled the growth of a thriving BtB Program, the donor office model presents several challenges. The major challenge is that funds are not stable from year to year, as supporting offices have withdrawn funding due to shrinking office budgets. In recent cycles, as noted in the following table, several funding offices pulled their support, and often, this decision was not made until after the review process for the given cycle was finished. Additionally, the donor office model does not always result in funding for the overall highest scored projects.

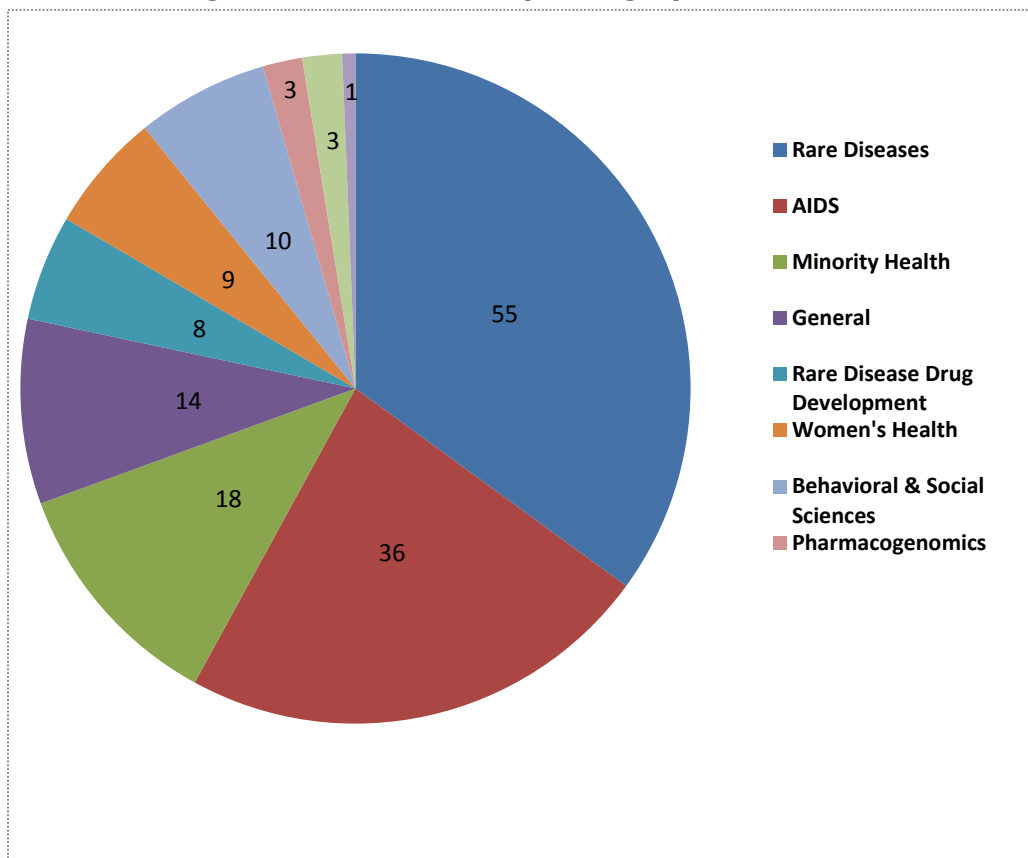
Table 1. Donor Funds 2006-2015

Source of Funds 2006-2015	
Program Donors	# of Awards
Office of Rare Diseases Research (ORDR/NCATS)	61
Office of AIDS Research (OAR)	38
NIH Institutes	20
National Institute on Minority Health & Health Disparities (NIMHD)	19
Office of Research on Women's Health (ORWH)	9
Office of Behavioral & Social Sciences Research (OBSSR)	6
Food and Drug Administration (FDA)	3
Office of Dietary Supplements (ODS)	1

Table 2. Source of Program Funds, 2006 – 2015

Source	2006	2007	2008	2009	2010	2011	2012	2013	2014	2015
OAR	946,765	762,877	995,000	829,200	899,900	919,142	1,022,269	1,066,513	1,032,688	1,039,272
ORDR	870,500	900,000	768,648	898,650	934,683	795,574	1,288,200	444,144	400,000	400,000
NIMHD	493,065	474,792	500,000	414,368	452,700	448,500				270,000
ORWH	161,500	170,000	200,000		270,000	312,000	215,000	270,000	210,000	100,000
NCRR	399,365	383,175	396,366	400,002	429,647	365,642				
FDA				507,650	140,000					
OBSSR				260,340	373,399	277,800		270,000	176,100	270,000
TRND					1,253,663					
DDIR							762,000			
ODS									318,149	
IC	1,308,704	1,144,000	1,372,809	1,175,690	2,023,327	1,217,688	705,000	175,000	269,200	382,000
TOTAL	4,179,899	3,834,844	4,232,823	4,485,900	6,777,319	4,336,346	3,992,469	2,225,657	2,406,137	2,461,272

Figure 6. BtB Awards by Category, 2006 - 2015



Funding Process

Donor office funds are provided to intramural investigators via intra-agency agreements. Funds are provided to extramural investigators as administrative supplements to existing NIH grants*. This funding mechanism has one major advantage for the extramural partner: ease of BtB application versus submission of a new grant. However, the use of administrative supplements limits the pool of extramural investigators who may apply to receive funds on a BtB award to current NIH grantees. Since 2006, 100 grants have been supplemented successfully with BtB funds.

Of note is the tremendous support provided by extramural grants experts from NHLBI and NIDDK. In 2006, senior grants experts from NHLBI partnered with the CC staff administering the program to align funds distribution to extramural grantees within existing NIH grants policies. These efforts are significant, and in 2009, NIDDK senior grants administrators agreed to assume these responsibilities. The CC team meets annually with the NIDDK staff under the leadership of Robert A. Pike, Branch Chief, Grants Management Branch to continually streamline processes and facilitate timely distribution of funds.

*Certain stipulations exist. The grant to receive the supplement must be active during the entire period of the BtB award, and the aims of the grant must be related to the BtB project. These requirements are emphasized in the annual call for Bench-to-Bedside proposals.

PROGRAM MANAGEMENT

The Bench-to-Bedside program is managed by the Office of the Director of the CC. Other NIH offices provide program support, including the Office of Financial Resource Management, CC; Grants Management Branch, NIDDK; and budget offices in the donor ICs. The program management costs to the CC include, in descending order: salary and benefits for BtB program staff, the grants management system proposalCentral, and costs associated with the review session.

Program Staff: The program originally was managed by Pat Piringer, Senior Special Assistant to the Director, Dr. John Gallin. Over time, the program received a higher volume of applications, funded more awards, and aligned its processes with the best practices of NIH grants management and peer review. The staff has increased to meet these requirements. Currently, three CCOD staff members manage all program processes, including the following:

- Call for proposals
- Application receipt
- Review at three separate stages, including feedback to applicants
- Liaison with donor offices
- Awards distribution
- Progress report collection
- Analysis of program management and funding outcomes
- Program reporting including benchmarking metrics of success for awards

Grants Management System: Since 2009, BtB has contracted to use Altum's online grants management system, proposalCentral. This system manages the application, review, and progress reporting processes.

Review Session: BtB conducts an annual one-day review session on the NIH campus. The review process is described on page 13 of this report. Travel, lodging, and per diem costs are covered for all non-federal reviewers. This cost is low, as 71% of reviewers over the past five cycles were either intramural NIH scientists or employees of other federal agencies.

This table outlines the BtB program management costs described above.

Table 3. Program Management Costs to CC, 2009 – 2015

	2009	2010	2011	2012	2013	2014	2015
Program Staff	\$99,200	\$134,392	\$114,825	\$114,825	\$148,390	\$150,171	\$152,219
1 senior special assistant	50%	25%	25%	25%	20%	20%	20%
1 program analyst	---	65%	50%	50%	40%	40%	40%
1 health science administrator	---	---	---	---	40%	40%	40%
Grants Management System	\$43,000	\$33,750	\$28,763	\$26,805	\$26,948	\$26,438	\$20,522
Review Session	\$2,138	\$2,256	\$3,049	\$3,322	\$3,869	\$2,375	\$2,674
Total Costs	\$144,338	\$170,398	\$146,637	\$144,952	\$179,207	\$178,894	\$175,415

REVIEW PROCESS

Levels of Review

The BtB program accepts applications once a year. While extramural investigators may serve as the Principal Investigator for the submission, each project must have a designated lead intramural collaborator who is responsible for all activities involved with the proposal submission.

Proposals are reviewed at three stages:

Letter of Intent: The intramural partner on the project submits a Letter of Intent (LOI) to briefly outline the project's aims, research plan, and the investigators who will be involved. LOIs are submitted via proposalCentral (an online grants management website). Each LOI is reviewed by the Scientific Directors of all involved intramural investigators.

Full Proposal by Scientific & Clinical Directors: If the LOI is approved, the intramural applicant may submit a full proposal via proposalCentral. Each proposal is reviewed by the Scientific and Clinical Directors of all involved intramural investigators.

Full Proposal by Review Team: If the proposal is approved by Scientific and Clinical Directors, the proposal is forwarded for review by the full Bench-to-Bedside Review Team. The review team consists of senior intramural and extramural investigators with expertise in the various research areas funded. The review session is modeled on an NIH study section and reviewers must sign "The National Institutes of Health Conflict of Interest and Confidentiality Certification for Individuals Evaluating All NIH Intramural Programs."

Review Criteria

The first two levels of review -- LOIs by the Scientific Directors and the full proposals by the Scientific and Clinical Directors -- are not entirely based upon scientific merit. Approval by the Scientific and Clinical Directors signifies that the institute(s) support their investigators' involvement on the project. Review is more rigorous at the third stage. A call for proposals, updated annually, describes the criteria by which full proposals are evaluated by the full Bench-to-Bedside Review Team.

In the 2015 cycle, the criteria for success of a proposal included:

- High quality of science (to be evaluated by the [NIH Enhanced Review Criteria for Research Grants and Cooperative Agreements](#)) with the potential to result in understanding an important disease process or lead to new therapeutic intervention;
- Strong translational science, with the bedside and bench components clearly related; one should lead logically to the next, and both should be strongly developed;
- The proposed translational work has the promise to evolve into an active clinical protocol with patient involvement in the future*.
- Although projects can be exclusively among intramural investigators preferably from more than one IC, collaborations between intramural and extramural investigators will receive priority review;
- The work should be a truly new initiative, not a funding request for work in progress.

****Translational component:** Applicants must explain how the proposed project will involve clinical work with direct patient contact, OR how the proposal results will lead to next steps involving clinical work (ultimately with direct patient contact). Involvement at the Clinical Center is a preference but not a requirement.*

The review team uses a two-tier scoring system for consideration of assigned proposals. Every proposal is initially assigned two separate scores. First, a program score reflects alignment of the

proposal with the Bench-to-Bedside criteria. Second, reviewers follow the NIH grant application scoring system using this 9-point scale to assess overall impact for each project.

Table 4. Overall Impact Score

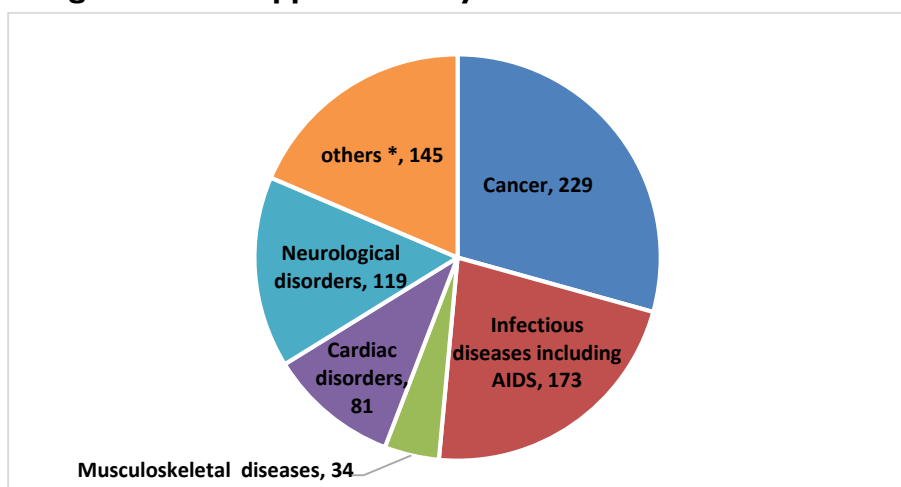
Impact	Score	Descriptor
High Impact	1	Exceptional
	2	Outstanding
	3	Excellent
Moderate Impact	4	Very Good
	5	Good
	6	Satisfactory
Low Impact	7	Fair
	8	Marginal
	9	Poor

Both preliminary scores (i.e., BtB program alignment and overall impact score) for each project are used by reviewers in preparing for projects to be presented for discussion during the formal session. During a full day session on campus, proposals are presented by primary and secondary reviewers, followed by discussions with the entire team. Then, all reviewers score all proposals unless they are recused from discussions and scoring related to conflict of interest.

Review Team

The review team is comprised of both intramural and extramural senior investigators. The reviewers represent diverse scientific/medical disciplines, based upon the research categories funded in a given cycle. Over 90% of the extramural reviewers are current or prior R01 awardees, and many intramural and extramural reviewers have previously held BtB awards. Over 30% of extramural reviewers are also reviewers for the Center for Scientific Review (CSR) or other academic institutions. The expertise of the review team is targeted to meet the volumes anticipated in each of these disease categories as follows.

Figure 7. BtB Applications by Scientific Focus 2009-2015



* Others category includes signaling molecules, etiology, genetics screening and epidemiological studies.

Bench-to-Bedside Review Team (2000 – Present)

Since the program's inception, 37 intramural senior clinical researchers have served as reviewers and since the 2006 cycle, 20 extramural experts have participated (see list). And, since 2000, Neal S. Young, M.D., MACP, Chief of the Hematology Branch, NHLBI has served as the review team chair.

Intramural (37)

Darrell Abernethy, M.D., Ph.D., NIA
Carolyn Bondy, M.D., NICHD
Craig Blackstone, M.D., Ph.D., NINDS
PJ Brooks, Ph.D., ORDR/NIAAA
Fabio Candotti, M.D., NHGRI
Jean Luc Cadet, M.D., NIDA
Dennis Charney, M.D., NIMH
Irene Dankwa-Mullan, M.D., NIMHD
Richard Davey, M.D., NIAID
Joshua Farber, M.D., NIAID
Antonio Tito Fojo, M.D., Ph.D., NCI
Clair Francomano, M.D., NHGRI
William Gahl, M.D., Ph.D., NHGRI
Kevin Gardner, M.D., Ph.D., NIMHD
Lee Helman, M.D., NCI
Daniel Kastner, M.D., Ph.D., NIAMS (now NHGRI)
Alan Kirk, M.D., Ph.D., NIDDK
David Lang, M.D., MPH, CC

Crystal Mackall, M.D., NCI
Harry Malech, M.D., NIAID
Henry Masur, M.D., CC
Henry McFarland, M.D., NINDS
Philip Murphy, M.D., NIAID
Forbes Porter, M.D., Ph.D., NICHD
Jennifer Puck, M.D., NHGRI
Pamela Robey, Ph.D., NIDCR
Merlyn Rodrigues, M.D., Ph.D., NCMHD
Donald Rosenstein, M.D., NIMH
Alan Schechter, M.D., NIDDK
James Shelhamer, M.D., CC
Constantine Stratakis, M.D., D.Sc., NICHD
Warren Strober, M.D., NIAID
Francisco Sy, M.D., Dr.P.H., NCMHD
Daniel Weinberger, M.D., NIMH
Jack Yanovski, M.D., Ph.D., NICHD
Robert Yarchoan, M.D., NCI
Jo Anne Zujewski, M.D., NCI

Extramural (25)

Nilofer Azad, M.D., Johns Hopkins University
Laura Balcer, M.D., M.S.C.E., University of Pennsylvania
Dana Boatman, Ph.D., Johns Hopkins University
Peter Calabresi, M.D., Johns Hopkins University
Irene Dankwa-Mullan, M.D., MPH, NCMHD
Priscilla Furth, M.D., Georgetown University
Richard Horenstein, M.D., J.D., University of Maryland
Jennifer Knight, M.D., Medical College of Wisconsin
Linden Hu, M.D., Tufts University
Justin McArthur, MBBS, MPH, Johns Hopkins University
Kevin McBryde, M.D., Children's National Medical Center
Edward Mitre, M.D., Uniformed Services University for the Health Sciences
Leon Nesti, M.D., Walter Reed National Military Medical Center and NIAMS

Deborah Persaud, M.D., Johns Hopkins University
Dzung Pham, Ph.D., The Henry M. Jackson Foundation for Advancement of Military Medicine and CC
Kyu Rhee, M.D., MPP, NCMHD
Donald Rosenstein, M.D., University of North Carolina
Joan Schiller, M.D., Inova Medical Group
Stacy Shord, Pharm.D., Food & Drug Administration
Marshall Summar, M.D., Children's National Medical Center
Marian Tanofsky-Kraff, Ph.D., Uniformed Services University for the Health Sciences
Ronald Taylor, Ph.D., University of Virginia
Michael Terrin, M.D., University of Maryland Baltimore
Redford Williams, M.D., Duke University
Issam Zineh, Pharm.D., MPH, Food and Drug Administration

SCIENTIFIC HIGHLIGHTS

The BtB program's mission is to foster long-standing collaborations and to spur translational research by providing seed awards to enable researchers to achieve specific and limited aims. The achievement of the BtB program goals are regularly evaluated using metrics described below. The data focuses on projects funded since 2006, when extramural partners were introduced, through 2013, the most recent cycle for which Year 2 reports are available.

Data Sources

BtB projects are funded for two years, and since 2009, program staff have sent requests for progress reports to all lead intramural PIs at the end of the projects' first and second years. Project outcomes referenced are based on the second year reports. In addition to the data mentioned above, additional information resulted from two independent reviews by a contract program evaluation consultant in 2007 and 2010. These two reviews are available online: <http://www.cc.nih.gov/ccc/btb/evaluations.html>. In the 2007 review, progress reports from investigators receiving awards between 1999 and 2006 were sought, and interviews were conducted with representatives from the donor offices. In the 2010 review, progress reports for awards between 2006 and 2009 were collected. Finally, some data were gained via staff follow up with PIs whose second year reports showed progress but not completion towards specific outcomes (e.g., patents pending and manuscripts awaiting publication).

Program Metrics

It is difficult to quantify the scientific impact of research, but numbers and quality of published articles and patents are tangible metrics. BtB awards are not expected to immediately result in the development of new interventions or new compounds since these accomplishments require significantly more funds than a BtB award provides as well as many years of work. Nevertheless, BtB awardees have directly impacted public health despite small monetary awards and short award periods. The table below summarizes reported outcomes from the PIs from the 2006-2013 cycles who completed Year 2 Progress Report forms. Additional outcomes may be attributable to BtB awards, but were not yet available at the two-year mark when year two reports are due.

Table 5. Program Metrics, 2006 – 2015

Metric	Number
Projects	147
Publications	222
# articles in high impact journals (rating >3)	169
Clinical Protocols (new and amended)	86
Patents awarded	16
INDs	4

Table 6. Patents Related to BtB Awards, 2006 – 2014

PI (IC)	Title of the related BtB award (year)	Title of patent (Patent #)
Daniel Fowler, MD (NCI)	Novel Suicide Gene-Modified Donor Th2 Cells for GVHD Prevention (2006)	Rapamycin-resistant T cells and therapeutic uses thereof (US 8075921 B2) Thymidylate kinase mutants and uses thereof (US20090074733 A1)
Peter Burbelo, PhD (NIDCR)	Humoral Response Profiling of Viral and Cellular Tumor Antigens for Predicting, Diagnosing, and Monitoring HIV Malignancies (2007)	Serological screening for hhv-8 infection using antigen mixtures (US20110294147)
Mark Gladwin, MD (NHLBI)	Contribution of Stromal Free Hemoglobin, Red Cell Membranes, and Red Cell Lysate on Nitric Oxide Inactivation in the Chronic Hemolytic State (2007)	Nitrite and nitrite-metheme therapy to detoxify stroma-free hemoglobin based blood substitutes (US8551536 B2) Methods of treatment for hemolysis (US20100239692 A1)
Forbes Porter, MD, PhD (NICHD)	Characterization of Glycosphingolipid Accumulation in Smith-Lemli-Opitz Syndrome and Treatment with N-butyldoxynojirimycin (2007)	Substrate reduction therapy (US8557844 B2)
Leonid Margolis, PhD (NICHD)	HIV-1 suppression by Acyclovir in patients co-infected with human herpes viruses from basic mechanism to clinical application (2008)	Attenuated DNA viruses which increases production of chemokines (US 7968298B2)
Peter Burbelo, PhD (NIDCR)	Development of a diagnostic test for latent Tuberculosis infection (2009)	Methods for the detection of analytes in a sample (US 2007/0259336 A1)
J. Silvio Gutkind, PhD (NIDCR)	Targeting mTOR as a novel mechanism-based therapy for head and neck cancer (2009)	Chemoprevention of head and neck squamous cell carcinomas (US 2011/0200556 A1) Methods of preventing the development of mucositis and related disorders (US 2014/0066472 A1)
Forbes Porter, MD, PhD (NICHD)	Combinatorial therapy, lipidomics and protein profiling in NPC (2010)	Substrate reduction therapy (US20140080769 A1)
Forbes Porter, MD, PhD (NICHD)	Biomarkers of neurodevelopment in Smith-Lemli-Opitz Syndrome (2010)	Biomarkers for Niemann-Pick C disease and related disorders (US 20090286272 A1)
Richard Childs, MD (NHLBI)	Exploring the anti-tumor effects of in vitro expanded natural killer (NK) cells against renal cell carcinoma sensitized to NK-TRAIL cytotoxicity with Bortezomib (2010)	Selective access to cryopreserved samples US (2012/0064603 A1)
Bradford Wood, MD (NCI)	Optical guidance for improved prostate cancer surgery (2010)	System, Methods, and Instrumentation for Image Guided Prostate Treatment (US20070232882 A1)
Leonid Margolis (NICHD), PhD	Valacyclovir for treatment of HIV-1 infection (2011)	Compounds and Methods For The Treatment of Viral Infection (US 2011/0184003 A1)
D. Ory (Washington University), & F. D. Porte (NICHD)	Cyclodextrin therapy for Niemann-Pick C1 disease	Cyclodextrin for the treatment of lysosomal storage diseases (WO2014022841 A1)

BtB Benchmarks

The funding amount for BtB awards is a very small fraction of other comparable programs. BtB funding is compared to these three similar NIH grants, all with a duration of one or two years:

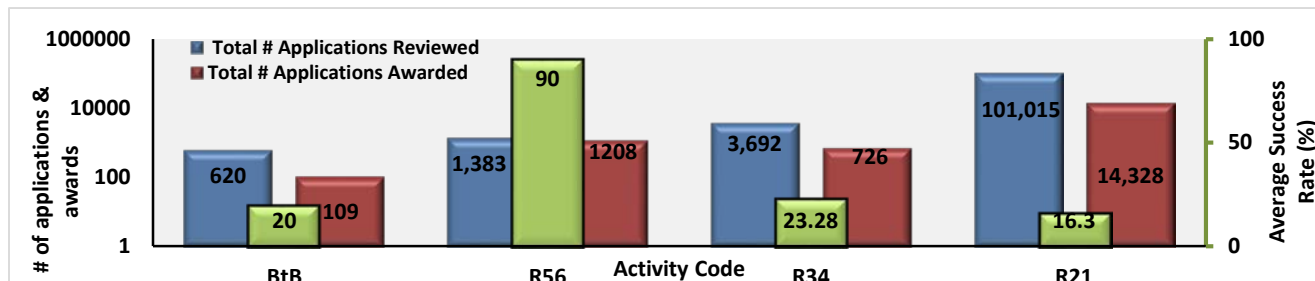
- **R56 Awards** are for high priority, short term projects and are designed to provide limited interim research support based on the merit of a pending R01 application while applicant gathers additional data to revise a new or competing renewal application. This grant underwrites highly meritorious applications that if given the opportunity to revise their application could meet IC recommended standards and would be missed opportunities if not funded. Interim funds end when applicant succeeds in obtaining an R01 or other competing award built on the R56 grant.
- **R34 Awards** are planning grants to provide support for the initial development of a clinical trial or research project, including the establishment of the research team; the development of tools for data management and oversight of the research; the development of a trial design or experimental research designs and other essential elements of the study or project, such as the protocol, recruitment strategies, procedure manuals and collection of feasibility data.
- **R21 Awards** are exploratory developmental grants to encourage the development of new research activities in categorical program areas. Support generally is restricted in levels of support and time.

These three programs are funded at a ratio of at least 5-12 times as much as BtB, yet the BtB average success rate is very similar as depicted in the following data.

Table 7. BtB Funding and Similar NIH Programs, 2008-2015

Applications/Awards/Success Rate 2008-2015					
Activity Code	Total # Applications Reviewed	Total # Applications Awarded	Average Success Rate (%)	Direct Costs	Award Length (years)
BtB	620	109	20	Not to exceed \$135K/year, to be shared to by intramural and extramural investigators	2
R56	1,383	1208	90	Direct award amounts can range up to Integrated Review Groups (IRG) recommend level	1 or 2
R34	3,692	726	23.28	Direct costs of up to four \$25K modules or \$100K/ year	1
R21	101,015	14,328	16.3	Not to exceed \$275K/2 years, and maximum \$200K in single year	2

Figure 8. Applications/Awards/Success Rate 2008-2015



Of the three comparable programs described above, R2I and BtB are most similar as both are two year awards intended to foster new research in specific categories. Support is non-renewable, and the awards often lead to larger funded projects; thus, both are viewed as seed awards for expanding collaborative research.

In addition to the similarities in success rate and program structure, it is important to note that while these larger programs benefit from myriad NIH resources, BtB is managed entirely by a staff of three. While each R2I application and award is administered by CSR, IC Council, Program Officers, Budget Officers, Grants Management Specialists, IT staff, Program Analysts, Program Evaluation Officers, and more, a BtB application/award is managed exclusively by three BtB staff members, as described on page 12 of this report. R2I applications are assigned to various divisions within institutes based upon scientific discipline, and the administrative burden of each R2I is shared by multiple IT systems. BtB applications encompass all scientific disciplines, but are reviewed and managed using the diverse expertise of the small BtB team. Further, BtB relies on one electronic award system for all administrative components. Finally, while program analysis for R2I awards is conducted by dedicated program evaluators within each IC, BtB evaluation and analysis is managed entirely by the program's three person staff.

BtB Accomplishments

One BtB program goal is to enable scientists to establish strong collaborations among intramural and extramural colleagues leading to new discoveries including biologics, diagnostic methods, and vaccines. A review of the literature and feedback from BtB awardees on annual project reports offers the following examples of scientific and clinical accomplishments associated with funded BtB projects:

Cancer treatments:

- *Treatment Options for Epithelial Cancers* by developing strategies for therapeutic immune radiotherapy delivering radioactivity directed specifically to tumors and tumor cells
- *Small lymphocytic lymphoma (SLL)/Chronic lymphocytic leukemia (CLL) therapy* by developing approaches of anti-CD20 mAbs that preserve CD20 cell-surface expression on CLL cells which have led to new Rituximab-based combinations, novel schedules, and the design of the next generation of antibodies

AIDS-related treatments and scientific advances in disease development:

- *Eliminating HIV-1 reservoirs* using combination of intense treatment and immune intervention to eliminate activated latently infected cells
- *Uncovering drug resistance and HIV recombination* by establishing a new method with greater sensitivity that permits detection of linked mutations which confer high-level drug resistance

Signaling molecules and genetic disorders and their involvement in disease development:

- *Studies of STAT 3 and its role in cardiovascular disease* resulted in treatment strategies using Angiotensin II receptor antagonists (ATI)-receptor antagonist in Hyper-IgE Syndrome (HIES) patients with aneurysms

- *Lysosomal Storage Disorder and Infantile Neuronal Ceroid Lipofuscinoses studies (INCLs)* resulted in identifying Cysteamine Bitartate (Cystagon) as a potential target to stop the progression of retinal and brain damage in infants with INCL
- *Hutchinson-Gilford Progeria Syndrome (HGPS) and the use of Farnesyl Transferase inhibitors* demonstrated a potential approach to tackle this disorder's effects on body weight, fat tissue, bone mineralization, and fracture rate
- *Discoveries of blood – based biochemical markers for Niemann-Pick type C I* which are transformative for diagnosis and treatment of this disorder as well as an outcome measure to monitor response to therapy

Sickle Cell Disease:

- *Sickle Cell Disease (SCD) and pulmonary hypertension* were linked to sudden death with patients with SCD. These findings led to trials for remodeling the treatment medications used (anticoagulant, oxygen, pulmonary vasodilators and others) for population of patients with SCD.
- *Understanding the interactions between hemolysis and infectious vasculopathy* including AIDS/HIV which modified the prevalence, age of onset, and severity of pulmonary hypertension among sickle cell patients.

Select Projects

The following BtB awards provide examples of scientific projects chosen to represent a primary mission of the BtB program, which is to stimulate collaborations on cutting edge research projects. These accomplishments characterize the broad success of BtB not only in scientific achievements but also in sustaining long lasting collaborations.

Proposing new therapy for ongoing collaborative research

Forbes D. Porter, NICHD, with Daniel Ory, Washington University in St. Louis

Forbes D. Porter, NICHD, and Daniel Ory, Washington University in St. Louis, have collaborated on the following two BtB awards:

- “Development of Combination Therapy for Niemann-Pick Disease, Type C” (2010)
- “Cyclodextrin Therapy for Niemann-Pick C I Disease” (2011).

A third award was granted to Dr. Porter in 2011 with other collaborators titled “Biomarkers of neurodevelopment in Smith-Lemli-Opitz Syndrome”.

The first BtB award laid the groundwork for the second BtB award and the launching of a Phase I clinical trial to evaluate the safety and effectiveness of cyclodextrin as a potential therapy for Niemann-Pick type C I (NPC) (Source: <http://www.ncats.nih.gov/news-and-events/features/npci-trial.html>). The two awards contributed to preclinical studies that led to administration of 2-hydroxypropyl- β -cyclodextrin (HP- β -CD) as a treatment for NPC at the NIH Clinical Center. However, this treatment necessitates neurosurgical implantation of a permanent device and

repeated access to the device to deliver the drug, as the drug is excreted rapidly and crosses the human blood brain barrier very poorly. Data from the first two BtB awards provided the foundation for a project supported by NCATS Division of Pre-Clinical Innovation. For this initiative, NCATS is collaborating with government agencies, academic scientists, the pharmaceutical industry and patient support groups to find potential treatments for this rare disease, Niemann-Pick disease type C1. The team published numerous papers, which have been cited >100 times, and received a patent award “Biomarkers for Niemann-Pick C Disease and Related Disorders” (US 20090286272 A1) for new method to screen and monitor the clinical status of NPC disease.

In the third BtB award, Dr. Porter and colleagues conducted proteomic and biochemical analysis as well as neuroimaging to further understand the pathoetiology and natural history of Smith-Lemli-Opitz (SLOS) syndrome. SLOS is similar to Niemann-Pick in terms of cholesterol synthesis and regulation malfunction. Findings from this award provided valuable information for families of individuals with SLOS, and may serve as outcome measures for therapeutic studies of neurodevelopmental disorders related to dysfunctional cholesterol metabolism. In fact, in other studies, Dr. Porter and other collaborators presented data indicating that SLOS disorder of cholesterol synthesis, caused by mutations in the 3β -hydroxysterol $\Delta 7$ -reductase gene (DHCR7). This primary biochemical defect could lead to a bona fide induction of a Niemann Pick Type C (NPC) disease phenotype, characterized by storage of that is not biochemically processed.

The combination of these findings led Dr. Porter to collaborate again with Dr. Ory, and they received a U01 award through the “Opportunities for Collaborative Research at the Clinical Center (U01)” grant mechanism in 2014. In this award titled “A Phase I Dose Escalation Study of Vorinostat in Niemann-Pick C1 Disease,” the investigators are conducting a Phase I, first-in-human, open-label, single-center, dose escalation study of Vorinostat in adults with NPC1. The study will characterize the feasibility, safety, tolerability, pharmacokinetics, and activity of oral Vorinostat. The investigators are actively enrolling individuals through the posting at the National Niemann-Pick Disease Foundation (NNPDF) (<http://www.nnpdf.org/HDACi.html>) web site.

Multi-Institute team monitors cardiovascular health of young HIV patients **Leonid Margolis, NICHD, with Michael Lederman, Case Western Reserve University**

The team received a 2008 BtB award, “*HIV-1 Suppression by Acyclovir in Patients Coinfected with Human Herpesviruses: From Basic Mechanism to Clinical Application.*” The award led to a clinical trial testing an innovative approach of using a common anti-herpetic drug, valacyclovir, for treatment of HIV-1 infection. The project was near completion at the end of the team’s first award. The second BtB award, which they received in 2011, allowed them to complete the trial. Through the clinical trial funded by these two awards, the team successfully translated ex vivo findings that ACV inhibits HIV-1 replication leading to two patents in July 2011 related to the trial (US 7,968,298B2 and US 20110184003A1). Their 3 publications have been cited 277 times. Recently Dr. Margolis completed the clinical trials. Experimental data and trial results demonstrated that in HIV-infected patients negative for HSV-2, acyclovir (valACV) decreases HIV viral load.

The significance of this finding is important because it indicates that suppression of HSV-2 with ACV seemed to be a meaningful strategy for both reducing HIV-I transmission and delaying HIV-I disease progression by reducing HIV-I levels.

Vanpouille, C., Lisco, A., Grivel, J. C., Bassit, L. C., Kauffman, R. C., Sanchez, J., ... & Margolis, L. (2015). Valacyclovir decreases plasma HIV-I RNA in HSV-2 seronegative individuals: a randomized placebo-controlled crossover trial. *Clinical Infectious Diseases*, 60(11), 1708-1714.

Long Lasting Collaborations

Bradford Wood, Clinical Center with Umar Mahmood, Massachusetts General Hospital, Marston Linehan, NCI and Peter Pinto, NCI

Dr. Wood and his collaborators received a BtB award in 2010 for their project titled “*Optical guidance for improved prostate cancer surgery.*” This translational research proposal included the development and testing of imaging devices that can provide real-time quantitative guidance. The goal of such innovation was to enhance visualization of the neurovascular bundle using near infrared (NIR) sensing during open, laparoscopic, or robotic assisted surgery, to decrease the likelihood of positive surgical margins and of iatrogenic concomitant nerve injury during radical prostatectomy. The team proposed to perform preclinical testing and optimization in canine models of prostatic resection. Ultimately, they plan to perform a pilot first-in-human feasibility study at the NIH Clinical Center to assess the potential advantages of using NIR guidance as an adjunct to current standard of care imaging. It was important to bring the combined skills of intramural and extramural investigators together in this proposal to successfully achieve the proposed goals and advance prostatic resection techniques. The preclinical developmental work from this project has led to translational plans for needle based optical spectroscopy for tissue analysis during liver biopsy in patients with liver disease and liver neoplasms, which is a planned collaboration with the same team and NCI Molecular Imaging Program. The translational expertise at the NIH Clinical Center was combined with the basic engineering extramural expertise, which resulted in a miniaturized fiber-optic sensor to characterize tissue in real-time on a laparoscope or a needle tip.

Their study resulted in several publications associated with this project including articles in “*Radiology*,” “*Cardiovascular and interventional radiology*,” “*Percutaneous Image-Guided Biopsy*,” and the “*International Journal of Hyperthermia*,” This work is associated with a patent invention as described by the US Patent Office, “*This invention relates to a system, methods, and instrumentation for image guided prostate treatment using a modified catheter: Patent # US20070232882A1.*”

The team includes investigators of multiple disciplines and is comprised of Drs. Linehan, Pinto, Choyke and Wood as well as several extramural collaborators. The team has taken full advantage of the fertile multidisciplinary translational environment existing within the intramural research program of the NIH and academia. These investigators have developed a track record of prior and ongoing collaboration by first earning a BtB award, which led to many advances in structural imaging modalities in cancer studies.

Long Lasting Collaborations

Dima Hammoud, CC with Martin Pomper, Johns Hopkins University

Drs. Hammoud and Pomper received an award in 2011 titled *"Imaging bacterial infection in immunocompromised patients"* in collaboration with several investigators from NIAID and NIDDK in addition to Dr. Steve Cho from John Hopkins. The ultimate goal of the project was to determine the accuracy of a PET reagent 1-(2-deoxy-2-fluoro-1-D-arabinofuranosyl)-5-iodouracil (FIAU) for the detection of viable bacterial burden and potential occult bacterial reservoirs. The investigators proposed to examine FIAU in a cohort of patients with chronic granulomatous diseases (CGD), Autosomal Dominant Hyper-IgE syndrome (AD-HIES). The authors hypothesized that FIAU-PET would be useful to diagnose bacterial infections and potentially differentiate bacterial from fungal infections without invasive cultures as well as establishing a non-invasive technique for follow-up and monitor the response to treatment. They based their hypothesis on findings by Dr. Pomper that the bacterial thymidine kinase (TK) enzyme is capable of phosphorylating (cellular *modification of a protein*) the nucleoside analogue FIAU, while the human and mouse TK enzymes cannot. This finding makes FIAU a great candidate reagent to distinguish bacterial proteins from mammalian protein. When the investigators submitted their project to the scientific review committee seeking approval for their clinical protocol, the committee requested that the applicants must provide additional preclinical studies data, both from both in vitro (cell lines) and mouse model experiments. In vitro experiments were conducted in cell cultures derived from patients with CGD and AD-HIES. Results from those experiments indicated that FIAU was not a substrate for *P. aeruginosa* bacterial strain, which is of major importance for potential use of radiolabeled FIAU in vivo for detection of infectious foci in this patient population. The negative results in this case could be misinterpreted as sterile inflammation or non-bacterial infection if one relies solely on non-invasive PET diagnosis. These findings did not invalidate the huge potential of radiolabeled FIAU as a specific bacterial imaging agent; the results may preclude using it to visualize infections in patient populations where *Pseudomonas* infection is likely. Thus, based on this data the awardees decided that performing the human study in the immunocompromised population at NIH would not be scientifically warranted. The investigators went on to assist other groups to determine the presence or absence of TK in the pathogens of interest. In addition, they proceeded toward performing animal PET studies.

BtB collaborations find common threads across years and partnerships: spotlight on continued obesity research

Jack Yanovski, NICHD, with partners from multiple extramural institutions

Between the 2006 and 2013 B2B cycles, Jack Yanovski, NICHD, received six BtB awards, and his BtB partners have included investigators from six extramural institutions, as well as intramural collaborators:

- Mayo Clinic
- Sackler School of Medicine, Tel Aviv
- Uniformed Services University of the Health Sciences
- University of Minnesota
- University of North Dakota School of Medicine

- University of Wisconsin

In the progress reports for the six projects funded in cycles 2006 through 2013, Dr. Yanovski reported the initiation of five Clinical Center and one animal study protocols. Several of these projects examine genetic predispositions for weight gain and attempt to relate specific genetic variations to behavior as part of their efforts to understand why some people have more difficulty maintaining a normal body weight. For his 2008 project, *"Histaminergic pathways and energy intake in obese women,"* the team studied whether genes that affect histaminergic pathways are involved in human body weight regulation. The project involved a proof-of-concept, randomized, double-blinded, placebo-controlled, dose-ranging study performed to examine the effects of betahistine in women with class I or II obesity. The data indicated that betahistine did not produce an effect on food intake or appetite, and the results point to the need for more potent histaminergic modulators to elucidate the possible role of histaminergic pathways in human obesity. Dr. Yanovski's 2009 project, *"FTO and Eating in the Absence of Hunger,"* examined adolescent eating habits without hunger. The data suggest that overweight and obese adolescents may be especially susceptible to intake in the absence of hunger when stimulated by external food cues. The findings indicated the need for further research to investigate eating without hunger, and in fact his 2013 award, *"Testing Neurobehavioral Endophenotypes for Loss of Control Eating,"* builds on this prior work. In this project, Dr. Yanovski and colleagues proposed to characterize the neurobehavioral mechanisms of Affect Regulation (NAR) of Loss of Control (LOC) eating model in young adolescent girls. Using the NAR model the investigators have planned to examine the social threat stimuli and food cue triggers among vulnerable individuals with LOC and compare them to those without LOC. Currently patient recruitment for this important study is underway. It is important to mention that there is a marked rise on LOC eating during female puberty which makes this award a critical study to understand the neurobehavioral mechanisms of the NAR model. In addition, with obesity recognized as a major emerging public health crisis nationally and internationally, these awards for Dr. Yanovski represent a rich investment in science promising to positively impact human health.

To further appreciate the impact of these awards in the words of investigators:

- *"The collaboration...between intramural and extramural investigators has resulted in new ideas not originally proposed that extend the work in [a] new direction. Moreover, we have started to put into place additional collaborations with industry partners to allow us to continue the innovation."*
- *"The [Bench-to-Bedside] program has worked precisely as intended, providing the infrastructure to support extramural-intramural collaborative science. The program allows the development of studies that would otherwise be difficult to fund."*
- *"The Bench-to-Bedside project funded through ORDR has enabled us to take our results from basic research in 'humanized' mouse models to the next level, i.e., using patient samples. This has strengthened our collaborative efforts with clinicians in both [the] intramural (NEI) and extramural community and has resulted in recruitment of new patients to our NEI clinic."*
- *"This bench to bedside award has led to a major collaboration between me and (my collaborator at) Harvard School of Public Health. We have three published abstracts...Our plan is to now work on a paper and submit ... an R01 application that will be an intramural-extramural collaboration. It was our 2007*

B2B award that made that collaboration possible. We believe that our research will elucidate important new pathways (for a disease) in people of African descent.”

- *“Very important program [that] gave us the initial funding to generate key proof of concept data and led to follow-up funding from other Government institutions.”*
- *“It’s a wonderful opportunity to develop collaborative research that should be highly praised.”*
- *“Outstanding program that stimulates inter-institutional discussions and collaborations on cutting-edge research projects of high risk.”*

Communicating Award Successes

Efforts have been made in recent years to highlight and share BtB success stories via NIH lectures and programs and with the extramural clinical research community. Promotion and recognition of these collaborative successes are key measures to sustain program interest. At the conclusion of the most recent BtB review session, reviewers embraced enthusiastically the suggestion to create an organized forum for BtB investigators to discuss their collaborative projects. Reviewers commented that an effort to structure such forums would have obvious benefit to ensure BtB program viability. Extramural reviewers commented that they would attend for the benefit of hearing outcomes of funded projects. If resources can be identified, such a formal BtB lectureship will be launched. Following is a sampling of prior efforts to ‘get the word out.’

2009 NIH Translational Research Interest Group (TRIG): A special BtB lecture series was launched by TRIG with assistance from the BtB administrative team to highlight 9 successful BtB projects (see Appendix II). The series was held from October 2010 – March 2011.

2011 Rare Disease Day Highlights BtB Lectures: A component of the first celebration of NIH Rare Disease Day highlighted 2 BtB awardees:

- Joan C. Han, NICHD and Felicitas L. Lacbawan, State University of New York; *“WAGR Syndrome: Clinical Characterization and Correlation with Genotype”*
- Constantine A. Stratakis, NICHD and Su Young Kim, NCI; *“Genetics of Inherited Paragangliomas and Gastric Stromal Tumors”*

2012 NIH Research Festival: A two hour BtB Symposium titled, *“Rare disease research in the Bedside to Bench Program: Intramural-Extramural partnerships advancing translational science at the NIH Clinical Center”* was included in the Research Festival agenda coordinated by ORDR and BtB staff. These lectures and a discussion session comprised the symposium:

- Philip Pearl, Children’s National Medical Ctr: *“GABAB Receptor Antagonist SGS-742 Treatment in SSADH Deficiency”*
- Alexandra Freeman, NIAID: *“Role of Pathogen-specific IgE and histamine release in the hyper-IgE syndrome”*
- Karen Berman, NIMH: *“Brain, Genes, and Behavior, in Williams Syndrome”*
- Craig Blackstone, NINDS: *“Common cellular themes for the hereditary spastic paraplegias”*
- Doug Stewart, NCI: *“The DICER1-related Pleuropulmonary Blastoma Cancer Predisposition Syndrome”*

BtB Poster Presentations from 2008 – 2015:

- December 2008 NIH Summit: The Science of Eliminating Health Disparities; Poster titled: *“NCMHD and NIH Clinical Center Collaborations Promote the Elimination of Health Disparities”*

- May 2009: NCRR 2nd annual conference on “Improving Health with Communities” Poster titled: *“Bridging the Intramural and Extramural Clinical Research Communities through Clinical Research Training and the NIH Bench-to-Bedside Program.”*
- 2011 Science of Team Science Meeting in Chicago: Poster titled: *“Evaluation of the Bench to Bedside Program: Four Years of Collaborations between NIH Intramural and Extramural Researchers”*
- 2011 Rare Disease Day: Poster titled: *“The NIH Office of Rare Diseases Research and the NIH Clinical Center Bench-to-Bedside Program Partnering to Promote Translational Research in Rare Diseases”*
- 2013 NIH Research Festival; Poster titled: *“The Bench-to-Bedside (B2B) Program: Connecting the Clinical Center’s Past and Future”*
- April 2014: Translational Science Meeting, Washington, DC; Poster titled: *“Partnering with Extramural Investigators To Foster Clinical Research Collaborations”*
- April 2015: Translational Science Meeting, Washington, DC; Poster titled: *“Partnering for Success: Three Unique Programs, One Mission!”*
- June 2015: Science of Team Science Conference, Bethesda, MD; Poster titled: *“Two Unique Programs with One Mission: Partnering for Success!”*

ALIGNMENT WITH U01 PROGRAM

The Scientific Management Review Board (SMRB) was established by Congress under the authority, 42 U.S.C. 281(e), section 401(e) of the Public Health Service (PHS) Act, as amended, for the purpose of advising the NIH Director and other appropriate agency officials, through reports to the NIH Director, on the use of these organizational authorities and identifying the reasons underlying the recommendations.

One of the first areas of focus for this group was the Clinical Center, and promoting extramural access to the Clinical Center was a primary recommendation in their 2010 report (see: http://smrb.od.nih.gov/documents/reports/CC_122010.pdf). The SMRB specifically noted that, “...The successful Bench-to-Bedside Program, which creates partnerships between intramural and extramural investigators, would benefit from a stable funding model with increased resources.”

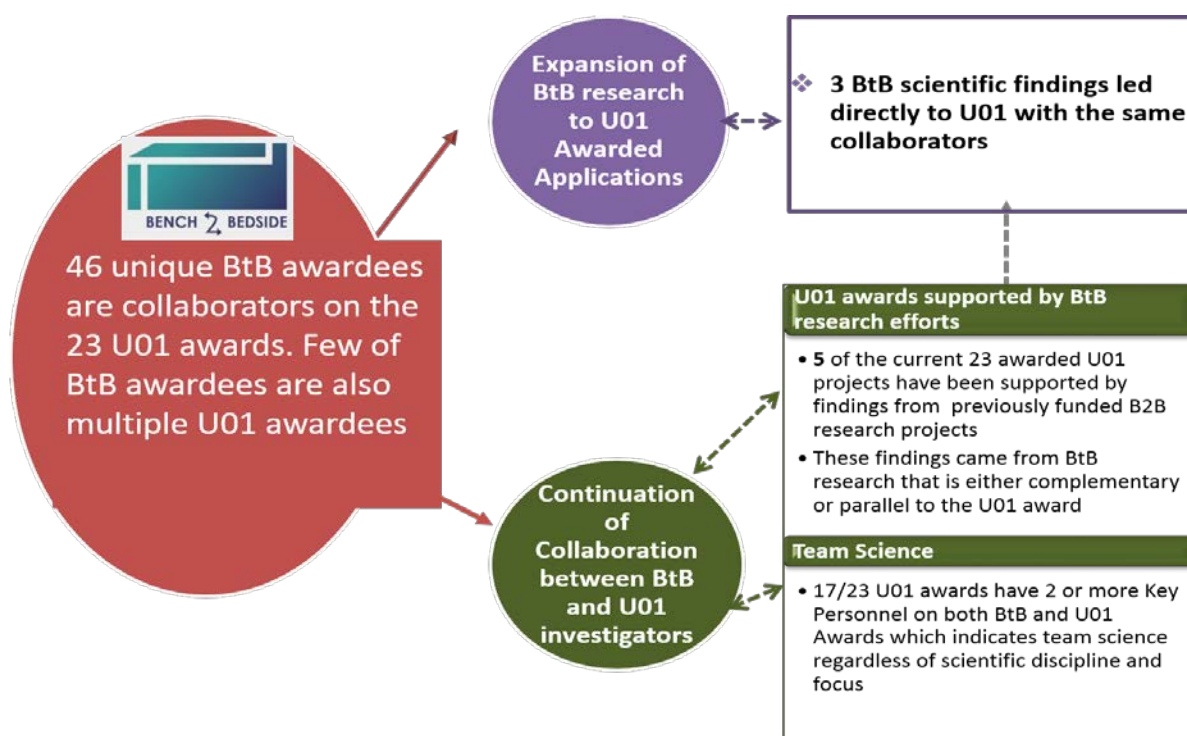
To implement these recommendations a Clinical Center-Extramural Committee, co-chaired by Drs. Sally Rockey and John Gallin, was established in 2011 and included leadership from the NIH Office of the Director, and both the extramural and intramural NIH programs. One subcommittee was charged to consider the recommendation regarding Bench-to-Bedside Program funding.

As this committee explored potential options, the Clinical Center Governing Board recommended keeping the Bench-to-Bedside Program intact and using its success as a template for seeding a new program. The BtB Program has served as an ideal model for a new U01 funding opportunity, “Opportunities for Collaborative Research at the Clinical Center” (<http://grants.nih.gov/grants/guide/pa-files/PAR-15-287.html>) since BtB accomplishes many of the same goals set for this U01 mechanism.

BtB Awards have fostered repeated team research for many U01 projects

BtB supports collaborative research projects undertaken by a team of two or more scientific scholars from multiple institutes within NIH and other research institutions, private and/or academic. Here we present some data that exemplify the essence of collaborative research, which has resulted in U01 awards.

The following figure represents the relationship between the BtB and the U01 awardees at different levels. In some cases, awardees sustained their collaborative efforts in the same scientific disciplines, wherein the BtB awards served as seed funds to pilot test new ideas and resulted in expanding their scientific endeavors to larger studies. In other cases, BtB award(s) fostered major and productive collaborations between intramural and extramural teams and then led to other teamwork in different scientific areas of research.



Since 2006, the BtB program has aimed to foster productive partnerships between intramural and extramural partners, and over 90% of projects have included at least one extramural partner. The BtB program has resulted in long-lasting intramural-extramural collaborations and increased knowledge of NIH Clinical Center research facilities among extramural partners on BtB awards.

Distinctions between the BtB and U01 Award Programs

While the BtB program has some similarities to the U01 program, “*Opportunities for Collaborative Research at the NIH Clinical Center (U01)*,” the two funding opportunities differ and serve as important complements to one another. Both programs fund research that takes advantage of the resources in the NIH Clinical Center, but the types of projects are categorically different.

Specifically, the BtB program provides seed money to teams to initiate high-risk, small-scale pilot projects to build the data for larger-scale work and to successfully apply for future awards. The U01 targets high quality science demonstrating the potential to result in understanding an important disease process or lead to a new therapeutic intervention in topics relevant to the research interests and priorities of the participating Institutes/Centers in this funding opportunity. This distinction is important both for generating a diverse portfolio of research projects at the NIH, and also for attracting a greater diversity of applicants.

Additionally, the BtB program promotes unique intramural opportunities. BtB provides intramural investigators the chance to apply for funding in translational research. While other intramural awards programs have been introduced in the last several years, in 1999, BtB was a truly unique opportunity for intramural investigators. The opportunity to apply for awards is important for two reasons: (1) investigators practice the skills needed for successful applications; and (2) investigators can apply for award funds to take their research in promising new directions, outside the restrictions of their annual budgets. It is critical to distinguish the Bench to Bedside program from the *Opportunities for Collaborative Research at the NIH Clinical Center (U01)* program. These programs are fundamentally different in their missions and values, and program processes and funding also differ significantly. The table on the following page summarizes these distinctions.

Table 8. Comparison of BtB and U01

	BtB	U01
Type of research funded	Innovative, high risk/high reward translational research pilot projects (or “seed” projects)	Larger-scale translational research projects, with potentially larger amount of supporting data required for successful application
Goal of CC Utilization	Secondary. CC utilization is preferred, not required, and more weight is placed on quality of science and collaborations generated	Primary. Encourages extramural PIs to take advantage of CC resources in response to SMRB recommendation to “Open the Doors of the NIH Clinical Center”
Lead investigators	Typically, intramural PI is lead with encouragement to collaborate with extramural PIs BtB offers intramural PIs a rare opportunity to apply for funding	The extramural PI is the lead investigator with a mandate to collaborate with an intramural PI
Partnerships	BtB allows for intramural-led and/or intramural-only projects. While over 90% of BtB projects include an extramural co-PI, approximately 10% of funded projects are new partnerships solely among intramural investigators. Thus excellent intramural teams, often between investigators from different institutes, may still compete successfully for BtB funds.	Extramural-driven U01 program does not support intramural-only projects
Award Amount/ Duration	\$135K/year direct costs for two years, plus applicable F&A for extramural investigators	Up to \$500K/year direct costs for 4 years renewable, plus applicable F&A for extramural investigators
Funds Distribution	BtB awards more funds to intramural investigators; between 2006 and 2015, ~65% of direct project costs were attributed to intramural investigators	The U01 program, typically exhibits the reverse funding ratios, and with the majority of direct costs awarded to extramural investigators
Application Process	Short application; timeline from application to award notice ~6 months	Extramural grants timeline: applications due in April; earliest start date December (~8 months)
Review Process	Intramural leadership (Scientific & Clinical Directors); BtB Review Team (intramural & extramural experts)	Scientific Merit Review; Advisory Council; U01 includes a pre-application X02 component that is strongly encouraged
International Collaborations	International partners have been involved on BtB awards since the program’s early days	In the third cycle of the program, foreign institutions were invited to apply for U01 awards.

1999 Bench-to-Bedside Projects

Institute(s)	Project	Investigators
NCI CC	"Pretargeted Therapy of Epithelial Cancers with Radiolabeled Monoclonal Antibody B3"	NCI: Pastan, Ira CC: Carrasquillo, J.
NHGRI NIAID	"Gene Therapy for X-Linked Severe Combined Immunodeficiency"	NHGRI: Puck, J. NIAID: Malech, Harry
NHLBI	"Vasculogenesis Using Progenitor Cells"	NHLBI: Quyyumi, Arshed Ali; Finkel, Toren
NIDCR NINR	"Evaluation of Therapeutic Reduction of Raised Elastase Levels by Reducing Neutrophil Numbers or by Direct Anti-Elastase Treatment on the Acceleration of the Rate of Healing"	NIDCR: Wahl, Sharon NINR: Wysocki, Annette
NIDCR NIAMS	"Gene Therapy for Chronic Cancer and Arthritic Pain"	NIDCR: Iadarola, Michael; Dionne, A. NIAMS: Kippel, Jack
NIDDK NCI CC	"Development of Novel Therapies for Sickle Cell Disease"	NIDDK: Schechter, Alan NCI: Keefer, Larry CC: Ognibene, Fred
NINDS	"Production of Clinical Grade ITX to Perform Clinical Trials for Treatment of Muscle Spasm Disorders (e.g., Torticollis and Blepharospasm) at NIH"	NINDS: Youle, Richard; Hallet, Mark
NINDS NCI	"Study of the Effect of the Humanized Monoclonal Antibody Against the Interleukin-2 Receptor alpha Subunit (IL-2R _α ; ZenapaxR) on Inflammatory Activity in the Central Nervous System in Multiple Sclerosis in a Baseline-to-Treatment, Cross-Over, MRI-Controlled Single-Center Phase I/II Trial"	NINDS: Martin, R.; McFarland, Henry NCI: Waldmann, Thomas

2000 Bench-to-Bedside Projects

Institute(s)	Project	Investigators
NCI NICHD	"Phase 1 Study of 5-aza-2'dexycytidine in lung cancer: Tumor response and analysis of altered gene expression and chromatin structure following DNA demethylation in vivo"	NCI: Kaye, FJ; Schrupp, David NICHD: Wolfe, AP
NICHD NEI	"Treatment of infantile neuronal ceroid lipofuscinosis patients with phospho-cysteamine"	NICHD: Mukherjee, Anil NEI: Caruso, R Partners: Levin, S.; Zhang, Z.; Butler, D.
NCI NIAID	"Tumor Specific Replicating Vaccinia Virus Expressing Cytosine Deaminase for Therapy of Metastatic Colorectal Cancer"	NCI: Bartlett, D.; Alexander, H.; Libutti, Steven NIAID: Moss, Bernard CC: Chang, Richard; Chen, Clara
NIAID NCI	"Anti-Tumor Immunotherapy: Linking the mouse to the human experience through the Danger Model"	NIAID: Matzinger, Polly NCI: Marincola, Francesco
NICHD NIDCR	"Effect of the aromatase inhibitor letrozole on estrogen levels and fibrous dysplasia of bone in patients with the McCune-Albright syndrome"	NICHD: Feuillan, P. NIDCR: Robey, Pamela
NHGRI NIDCD CC NIMH NCI	"Genotypic & Phenotypic Dissection of the Smith-Magenis syndrome: An Interdisciplinary Study of Physical, Cognitive and Neurobehavioral abnormalities in SMS"	NHGRI: Smith, Ann; Gropman, Andrea; Sonies, Barbara NIDCD: Friedman, Thomas; Griffith, Andrew CC: Solomon, Beth NIMH: Rapoport, Judy; Giedd, Jay; Nicholson, R. NCI: Wolters, Pamela
NIAMS CC NHLBI	"Inhibition of Angiogenesis in Severe Early Rheumatoid Arthritis"	NIAMS: El-Gabalawy, Hani CC: Eckelman, William; Carrasquillo, J. NHLBI: Balaban, Robert
NHLBI CC	"Selective depletion of donor lymphocytes causing graft-versus-host disease to improve outcome of allogeneic stem cell transplantation"	NHLBI: Barrett, John CC: Read, E.
NCI NIAID	"Studies of CD40 Ligand Trimer as an Adjuvant for HIV-1 Vaccines"	NCI: Franchini, Genoveffa NIAID: Strober, Warren; Jain, A. ; Kovacs, Joseph; Seder, Robert

2001 Bench-to-Bedside Projects

Institute(s)	Project	Investigators
NINDS NIBIB	"Magnetic Resonance Elastography - A Clinical Technique in the Management of Malignant Acute Hemispheric Stroke with Implications for Patient Intervention by Hemispherectomy and Duroplasty"	NINDS: Moore, D. NIBIB: Dimitriadis, Emilios
NEI NCI	"A Phase I/II Pilot Study to Evaluate the Induction of Immune Tolerance in Patients with Sight Threatening Autoimmune Uveitis treated with Zenapax and Rapamycin"	NEI: Nussenblatt, Robert; Ragheb, J. NCI: Waldmann, Thomas
NCI	"Use of IL-10 to Improve the Therapeutic Window of Cisplatin"	NCI: Bartlett, D.; Alexander, R. NIDDK: Star, Robert
CC NHLBI	"Targeted Delivery of Nitric Oxide by Hemoglobin to Improve Regional Blood Flow in Sickle Cell Disease"	CC: Ognibene, Fred; Gladwin, M. NHLBI: Cannon, Richard
NIDDK CC	"Combination Anti-Viral and Immunomodulatory Therapy for Chronic Hepatitis B"	NIDDK: Ghany, Marc; Rehermann, Barbara CC: Alter, Harvey
NIDCR NICHD	"Mutation of human growth hormone (hGH) sorting motifs to facilitate gene therapeutics applications with salivary glands in adult hGH-deficient patients"	NIDCR: Baum, Bruce NICHD: Loh, Y Peng
NCI (DCEG) NCI (DCS)	"Genomic Changes in Pre-malignant, Pre-invasive and Invasive Breast Cancer in Women Genetically at High Risk for Breast Cancer"	NCI: Guisti, R.; Ried, Thomas
NICHD	"Treatment of Smith-Lemli-Opitz Syndrome with Simvastatin"	NICHD: Porter, Forbes; Yergey, Alfred; Tierney, Elaine
NIDCR CC	"New Treatments for Intractable Pain"	NIDCR: Berger, A. CC: Iadarola, Michael

2002 Bench-to-Bedside Projects

Institute(s)	Project	Investigators
NINDS	"T Cell-Depleting Monoclonal Antibody Campath-1H in Patients with Inclusion Body Myositis: Correlation of Clinical Response with Changes in Endomysial T-Cell Epitopes, Inflammatory Cytokines, and Costimulatory Molecules"	NINDS: Dalakas, Marinos; Muraro, Paulo Partners: Martin, R.; Vasconcelos, O.; Raju, R.
NHLBI CC	"Alloreactive natural killer (NK) cell immunotherapy to improve outcome of allogeneic stem cell transplantation"	NHLBI: Barrett, John CC: Read, E.
NICHD	"Intracellular Calcium Measurement in Adipocytes (ICMA): An Adjunct to the Study of Supplemental Calcium in Overweight Out Patients (SCOOP) Study"	NICHD: Parikh, S.; Blank, Paul
CC	"Impact on platelet survival of donor/recipient selection based on definitive sequence-based HLA typing"	CC: Leitman, Susan; Marincola, Francesco
NCI	"Characterization of High Risk Breast Duct Epithelium by Cytology Breast Duct Endoscopy, and cDNA Gene Expression Profile"	NCI: Danforth, David; Steeg, Pat; Zujewski, Jo Anne; Giust, R.; Abati, A.; Simon, Richard; Ried, Thomas
NIEHS	"Potential involvement of a brain-specific isoform of the winged helix transcription factor RFX4 in human congenital hydrocephalus"	NIEHS: Blackshear, Perry; Zeldin, Darryl

2003 Bench-to-Bedside Projects

Institute(s)	Project	Investigators
NIMH NINDS	"Investigation of Brain and Behavioral Effects of Specific Gene Deleted in 'Williams Syndrome Critical Area' of Chromosome 7q11.23 in Patients and Knockout Mice"	NIMH: Berman, Karen; Crawley, Jacqueline NINDS: Koretsky, Alan
NHGRI NINDS	"Sialic Acid Replacement in Patients with Hereditary Inclusion Body Myopathy (HIBM) Caused by Mutations in the GNE (Sialuria) Gene"	NHGRI: Juizing, M. NINDS: Dalakas, Marinos Partners: Krasnewich, D.; Vasconcelos, O.
CC NINDS	"MRI Detection of the Migration of Feridex -PPL Labeled Cells into the CNS in Multiple Sclerosis Patients"	CC: Frank, Joseph; Jordan, Elaine; Leitman, Susan; Read, E. NINDS: McFarland, Henry; Bielekova, Bibi; Duyn, Jeff Partners: Ali, S.; Lewis, B.; Martin, R.; Richert, N.; Anderson, S.; Carter, C.
NCI NHLBI CC	"Dendritic cell vaccination to augment graft-versus-leukemia after allogeneic stem cell transplantation for acute lymphoblastic leukemia (ALL)"	NCI: Mackall, Crystal; Fry, Terry; Gress, Ron NHLBI: Barrett, John CC: Kurlander, Roger; Read, E. Partners: Bishop, M.; Carter, C.; Grubbe, M.
NHLBI NIAID	"Evaluation of HMG-CoA reductase inhibitors in pulmonary sarcoidosis"	NHLBI: Manganiello, Vincent; Farber, Joshua NIAID: Park, Matthew
NHLBI NIAID NHGRI	"Clonal Insertion Site Analysis in Rhesus Macaques & NIH Patients Transplanted with Retrovirally-Transduced Hematopoietic Progenitor & Stem Cells: Implications for Leukemogenesis"	NHLBI: Dunbar, Cynthia; Donahue, Robert NIAID: Malech, Harry NHGRI: Candotti, Fabio; Baxeavanis, Andy

AIDS Category

Institute(s)	Project	Investigators
NCI NIAID	"Use of 5 Fluorouracil (5FU) in Combination with Antiretroviral Drugs as a Salvage Strategy to Overcome Drug Resistance in Heavily Treated HIV-Infected Pediatric Patients"	NCI: Wood, Lauren NIAID: Womack, C.
NIAID CC	"Imaging Probe for the In Vivo Assessment of HIV-1 Dynamics"	NIAID: DiMascio, Michelle; Imamichi, Tomozumi; Lane, Cliff CC: Drs. Pandit and Li
NIDCR CC Dartmouth	"Therapeutic Targeting of a Virally Regulated Host Cell Molecule in HIV Infection"	NIDCR: Wahl, Sharon; Vazquez, N. CC: Masur, Henry Dartmouth University: Sporn, M.B.
NCI NIAID	"Role of Recombination in HIV-1 Drug Resistance in vitro <i>and</i> in vivo"	NCI: Maldarelli, Frank; Palmer, S; Coffin, J NIAID: Polis, N; Michan, J
NCI NIAID	"Treatment of Drug Resistant and Persistent HIV-1 Infection with the Designed Proteins 5-Helix and 5-Helix-PE"	NCI: Hamer, Dean NIAID: Kovacs, Joseph
CBER, FDA NIAID	"HIV-1 Infections During Vaccine Trials: Identifying New Peptides for the Differential Diagnosis of HIV-1 Infection in the Face of Vaccine-Generated Antibodies"	FDA: Goldberg, Hana NIAID: Graham, Barney
NCI NIAID	"Effect on Immune Responses and Sustainability of Viral Suppression in HIV-Infected Children of a Therapeutic Vaccination Strategy with a Multiclude HIV-1 DNA Plasmid Vaccine Prime and a Recombinant Adenovector Boost"	NCI: Zeichner, S NIAID: Kroup, Richard

2004 Bench-to-Bedside Projects

Institute(s)	Project	Investigators
NIAMS NINDS	"Neural substrates underlying motor control of a newly implanted hand transplant"	NIAMS: Cendales; Linda NINDS: Cohen, Leonardo
NIEHS NCI NICHD CC	"Development of Non-invasive treatment for uterine leiomyoma (fibroids)"	NIEHS: Davis, B. NCI: Partners NICHD: Stratton, Pam CC: Partners
NIDDK NCI ORS	"Infrared and Near Infrared Image Guided Minimally Invasive Assessment of Renal Perfusion during Donor Nephrectomies and Partial Nephrectomies"	NIDDK: Elster, E.; Kirk, Allan NCI: Partners ORS: Partners
NCI NIDCD	"Novel Approach for treatment of squamous cell malignancies with 17-AAG and bortezomib"	NCI: Gius, D.; Neckers, Len NIDCD: Van Waes, Carter
CC NHLBI	"Therapeutic application of intravascular nitrite for sickle cell disease"	CC: Gladwin, M NHLBI: Cannon, Richard
NIMH NIAAA	"Intermediate phenotype and genetic mechanisms for psychosis and cognitive disturbance in 22q11.2-hemideletion syndrome"	NCI: Meyer-Lindenberg, A. NIAAA: Goldman, David NIMH: Partners
NHLBI NCI	"Molecular profiling of response to proteasome inhibition by bortezomib (PS341) in a clinical trial of mantle cell lymphoma"	NHLBI: Weistner, Adrian NCI: Wilson, Wyndham
NEI NCI	"A Phase I/II pilot study to evaluate the treatment of intraocular lymphoma with BL22 immunotoxin"	NEI: Nussenblatt, Robert NCI: Pastan, Ira
NHLBI NIAID NCI	"Immunotherapy for Myelodysplastic Syndrome"	NHLBI: Barrett, John NIAID: Douek, Daniel NCI: Rosenberg, Steve
NHGRI CC	"A Phase I Treatment Trial of the Circadian Sleep Disturbance in Smith-Magenis Syndrome (SMS)"	NHGRI: Smith, Ann CC: Grimes, George
NIAID NHLBI	"Pre-clinical non-human primate studies of an in vivo selectable vector intended for use in a planned clinical trial of gene therapy for chronic granulomatous disease"	NIAID: Malech, Harry NHLBI: Dunbar, Cynthia
CC CIT	"Isolation and Characterization of Circulating Endothelial Cells in Primary Pulmonary Hypertension: Implications for Early Diagnosis and Novel Therapeutic Targets"	CC: Solomon, Michael; Danner, Robert CIT: Partners
NINDS NCI	"Evaluation of the anti-IL-2/IL-15R α monoclonal antibody Hu MiK-b-1 in a therapeutic trial in patients with HAM/TSP and a test of hypotheses concerning the role of IL-15 in the maintenance of CD8 $^{+}$ memory T-cells and in the pathogenesis of HAM/TSP"	NINDS: Jacobson, Steven NCI: Waldmann, Thomas CC: Fleisher, Thomas
NCI NHGRI	"Childhood Cancer and Plexiform Neurofibroma Tissue Microarray for Molecular Target Screening and Clinical Drug Development"	NCI: Fox, E. NHGRI: Baird, K.
NCI NHGRI	"Molecular profiling and drug discovery for patients with PTEN Hamartomatous Tumor Syndromes (PHTS)"	NCI: Dennis, P. NHGRI: Elkahoun, A.
NIAID NCI	"Assessing the safety and immunogenicity of a therapeutic vaccination strategy using a DNA plasmid vaccine prime and a recombinant adenovector boost in HIV infected adults"	NIAID: Graham, Barney NCI: Partners
NCI NIAID	"Targeting vascular endothelial growth factor-A (VEGF-A) and receptor-2 (VEGFR-2) for the treatment of Primary Effusion Lymphoma"	NCI: Tosato, Giovanna; Yarchoan, Robert NIAID: Partners
NCI NINDS	"Pediatric NeuroAIDS in the HAART Era: Identification of Disease Markers and Therapeutic Targets"	NCI: Hazra, R. NINDS: Major, Eugene; Schwartz, L.
NCI NIAID	"Treatment of Primary-Effusion Lymphoma with Combination Virolytic and Cytotoxic Chemotherapy"	NCI: Yarchoan, Robert; Little, Richard NIAID: Cohen, Jeffrey

2005 Bench-to-Bedside Projects

GENERAL CATEGORY

Institute(s)	Project	Investigators
NINDS NIDCD	"Induction of volitional swallowing in chronic dysphagia post stroke: A novel mechanism-based intervention"	NINDS: Ludlow, Christy; Cohen, Leonardo NIDCD: Horwitz, Barry
NHLBI NINDS	"Induction of Mucosal Tolerance to Recombinant Human E-Selectin for the treatment of Myocardial Ischemia-Reperfusion Injury and Symptomatic Coronary Atherosclerosis. A Collaborative NHLBI/NINDS Investigation"	NHLBI: Desilva, Ranil; Finkel, Toren NINDS: Hallenbeck, J.

RARE DISEASES CATEGORY

Institute(s)	Project	Investigators
NCI NHGRI	"Natural history, biology and treatment of dermal neurofibromas in neurofibromatosis type 1 (NF1)"	NCI: Widemann, Brigitte NHGRI: Stewart, Douglas
NHGRI NHLBI	"Testing Treatment of Hutchinson-Gilford Progeria Syndrome with Farnesyl Transferase Inhibitors"	NHGRI: Gordon, L.; Gahl, William; Introne, Wendy; Collins, Francis NHLBI: Orlic, Donald; Nabel, Elizabeth
NIEHS NINDS	"Analysis of global gene expression patterns and mitochondrial DNA damage in lymphocytes of Friedreich's Ataxia patients undergoing idebenone treatment in Phase II double-blind placebo controlled study"	NIEHS: Van Houten, B.; Haugen, Astrid NINDS: Fischbeck, Karen Other Collaborators: Dr. DiProspero
NCI CC	"Adoptive Cell Therapy for Ewing's Sarcoma Using Artificial Antigen Presenting Cells"	NCI: Mackall, Crystal CC: Partners
NICHD NHGRI	"Ganaxolone Therapy for Niemann-Pick Type C"	NICHD: Porter, Forbes NHGRI: Pavan, William
NIEHS NICHD	"UVA Sensitivity in Smith-Lemli-Opitz Syndrome: Possible Involvement of - α -Cholesta -5,7,9(11)-trien-3	NIEHS: Chignell, C. NICHD: Porter, Forbes
CC NCI NIAID	"Pre-clinical and clinical investigations into the mechanisms and efficacy of extracorporeal photopheresis (ECP) in the abrogation of graft-vs-host disease (GVHD) and facilitation of graft-vs-tumor (GVT) immunity in pediatric patients"	CC: Wayne, A.; Leitman, Susan NCI: Fry, Terry; Pavletic, Steven NIAID: Malech, Harry Other Collaborators: Dr. Bolan
NCI NICHD	"Site-Selective cAMP Analogs for Treatment of Carney Complex"	NCI: Cho-Chung, Y. NICHD: Stratakis, Constantine
NIAMS NHGRI	"Pathogenesis of and risk factors for autoimmunity in the Wiskott-Aldrich Syndrome"	NIAMS: Siegel, Richard; Nikolov, Nikolay NHGRI: Candotti, Fabio
NIAID NCI	"Development of a Specific Drug treatment for WHIM Syndrome"	NIAID: McDermott, David; Murphy, Philip; Malech, Harry; Kawai, T. NCI: Hwang, S.

AIDS CATEGORY

Institute(s)	Project	Investigators
NCI NIAID	"Role of HIV-1 RNase in Developing Resistance to Nucleoside Analogs"	NCI: Maldarelli, Frank; Pathak, Vinay NIAID: Morse, Caryn
NHLBI CC	"Endothelial Dysfunction in HIV-associated Pulmonary Hypertension"	NHLBI: Machado, R.; Gladwin, M.; Lederman, Robert CC: Raghavachari, Nalini; Masur, Henry Other Collaborators: Drs. Barnett, McCoy, Solomon
NCI NIAID	"Clinical Development of E. coli Nissle 1917 as Host for a Live Anti-HIV Microbicide"	NCI: Hamer, D. NIAID: Sereti, Irini Other Collaborators: Drs. Asdhya, Henry, Mannon
NIAID CC	"Persistent HIV Reservoirs in Infected Patients Receiving Effective Antiviral Therapy for Prolonged Periods of Time: Delineation of the Mechanism and Source of Viral Replication and Execution of a New Therapeutic Strategy"	NIAID: Chun, Tae-Wook; Moir, Susan; Kottlil, Shyam CC: Healey, L.; Kovacs, Joseph
CC NIAID NINDS	"Identification of Biomarkers of Mitochondrial-related Drug Toxicities in HIV-infected Patients Receiving Highly Active Anti-retroviral Therapy"	CC: Kovacs, Joseph NIAID: Mican, J. NINDS: Partners

2006 Bench-to-Bedside Projects

RARE DISEASES CATEGORY

Institute(s)	Project	Investigators
NHLBI CC Harvard Georgetown National Naval Medical Center	"Role of Cyclin D1 in Myelodysplasia"	NHLBI: Sloand, Elaine; Young, Neal; Scheinberg, Phillip; Barrett, John CC: Kurlander, Roger Harvard University: Groopman, Jerome Georgetown University: Blancato, Jan National Naval Medical Center: More, Kenneth Salisbury District Hospital (UK): Crolla, John
NHLBI CC NCI Walter Reed Army Medical Center	"Exploring the Anti-Tumor Effects of in vitro Expanded Natural Killer (NK) Cells Against Renal Cell Carcinoma Sensitized to NK-TRAIL Cytotoxicity with Bortezomib"	NHLBI: Childs, Richard; Lundqvist, Andreas CC: Read, E.J.; Suffredini, Anthony Walter Reed Army Medical Center: Gorak, Edward; Alvarez, Gauri
NHLBI MD Anderson Cancer Center	"A New Global Function for a Rare Disease Gene: Clinical Significance of the Regulation of Mitochondrial Respiration by Tumor Suppressor p53 in Li-Fraumeni Syndrome"	NHLBI: Hwang, Paul MD Anderson Cancer Center: Strong, Louise; Khakoo, Aarif; Arena, Ross
NCI, NHLBI, NINDS Memorial Sloan- Kettering Cancer Center	"Therapeutic Approaches for Cancer Stem Cells in Small Cell Neuroendocrine Carcinomas"	NCI: Harris, Curtis; Varticovski, Lyuba; Dennis, Phillip A.; Bates, Susan NHLBI: Dunbar, Cynthia NINDS: McKay, Ron Memorial Sloan-Kettering Cancer Center: Travis, William D.
NCI USC Karmanos Cancer Institute and Wayne State University Fred Hutchinson Cancer Research Center and Univ of WA Mayo Clinic College of Medicine	"High Density Genotyping in Diffuse Large B-cell Lymphoma (DLBCL) and Follicular Lymphoma – Translating Etiologic Clues into Prognostic Relevance Within the NCI-SEER NHL Case Control Study"	NCI: Chanock, Stephen; Wang, Sophia; Hartge, Patricia; Staudt, Lou; Morton, Lindsay; Wacholder, Sholom; Rothman, Nathaniel Mayo Clinic College of Medicine: Cerhan, James University of Southern California: Cozen, Wendy Fred Hutchinson Cancer Research Center: Davis, Scott Karmanos Cancer Center & Wayne State University: Severson, Richard
CC NCI University of Toronto/ Ontario Cancer Institute University of Illinois	"Novel Suicide Gene-Modified Donor Th2 Cells for GVHD Prevention"	CC: Reed, Elizabeth J.; Carter, Charles NCI: Fowler, Daniel University of Toronto/Ontario Cancer Institute: Medin, Jeffrey University of Illinois: Lavie, Arnon
NIDDK NHLBI University of Maryland	"A Nutrigenomics Intervention for the Study of the Role of Dietary Sitosterol on Lipid, Glucose and Energy Metabolism"	NIDDK: Celi, Francesco NHLBI: Sachdev, Vandana University of Maryland: Horenstein, Richard; Fried, Susan; Shuldiner, Alan
NHLBI CC NIDDK NCI Drexel University	"Pilot Trial of Intravenous Nitrite for Sickle Cell Vaso-Occlusive Pain Crisis"	NHLBI: Gladwin, Mark; Machado, Roberto; Taylor, James; Wang, Xunde CC: Kato, Gregory NIDDK: Schechter, Alan; Shiva, Sruti NCI: Mack, Kyle Drexel University: Hsu, Lewis

AIDS CATEGORY

Institute(s)	Project	Investigators
NIAID St. Michael's Medical Center	"The effect of HIV-1 Infection on Endogenous miRNA Expression <i>in vivo</i> "	NIAID: Jeang, Kuan-The St. Michael's Medical Center: Smith, Stephen
NCI NIAID Johns Hopkins University	"Genetic Characteristics of HIV-1 During Suppressive Antiretroviral Therapy"	NCI: Maldarelli, Frank; Coffin, John; Palmer, Sarah; Weigand, Ann; Kearney, Mary NIAID: Kottilil, Shyamasundaran Johns Hopkins University: Persaud, Deborah
CC San Francisco General Hospital Science Applications International Corporation (SAIC) Makerere University, Mulago Hospital	"Evaluation of Molecular Methods for the non-invasive Diagnosis of Pneumocystis and Tuberculosis and Molecular Evaluation of Non-subtype B HIV Quasispecies in the Lung"	CC: Kovacs, Joseph; Fischer, Steven NCI: Maldarelli, Frank University of California, San Francisco: Huang, Laurence San Francisco General Hospital: Davis, J. Lucian SAIC: Imamichi, Hiromi Makerere University, Mulago Hospital: Worodria, William; Yoo, Samuel
NIDDK Children's National Medical Center	"Microalbuminuria and Podocyturia in Patients with HIV disease: Detection, Characterization, and Therapy"	NIDDK: Kopp, Jeffrey; Howard, Lilian Children's National Medical Center: McBryde, Kevin

MINORITY HEALTH AND HEALTH DISPARITIES CATEGORY

Institute(s)	Project	Investigators
NHLBI CC NIDDK	"Hemolysis, HIV/AIDS and Parasitic Infections Associated Secondary Pulmonary Arterial Hypertension in Sickle Cell Diseases"	NHLBI: Aliyi, Zakari; Gladwin, Mark; Taylor, James, G.; Machado, Roberto CC: Cato, Gregory NIDDK: Rogers, Griffin
NIDA University of Pennsylvania	"Novel Bench-to-Bedside Research Methods for Drug Addiction: Development, Validation and Application"	NIDA: Stein, Elliott University of Pennsylvania: Childress, Anna Rose; Peoples, Laura
NHGRI Fred Hutchinson Cancer Research Center	"Breast Cancer among African American Women: The role of Missense Changes in the BRCA1 and BRCA2 Breast Cancer Susceptibility Genes Using a Population-Based Approach"	NHGRI: Ostrander, Elaine Fred Hutchinson Cancer Research Institute: Malone, Kathleen
NICHD NIDDK University of Wisconsin	"Melanocortin 3 receptor Mutations as an Etiology for Obesity in African American and Caucasian children"	NICHD: Yanovski, Jack; Ning, Cong; Koo, Ja-Shin; Westphal, Heiner J. NIDDK: Gavrilova, Oksana University of Wisconsin: Schoeller, Dale

WOMEN'S HEALTH CATEGORY

Institute(s)	Project	Investigators
NIDDK Oregon State University	"Vitamin E Pharmacokinetics and Oxidative Biomarkers in Normal and Obese Women"	NIDDK: Levine, Mark Oregon State University: Traber, Maret

CO-FUNDED (INSTITUTE AND NCRR)

Institute(s)	Project	Investigators
NIDDK Washington Hospital Center	"Immunosuppression Minimization by Biological Response Monitoring"	NIDDK: Kirk, Allen Washington Hospital Center: Light, Jimmy
NINDS NEI Johns Hopkins School of Medicine University of Pennsylvania	"A Preliminary Assessment of the use of Ocular Coherence Tomography and Magnetic Resonance Imaging as Outcome Measures for Studying the Optic Nerve in Studies of Neuroprotection in Multiple Sclerosis" project to evaluate	NINDS: McFarland, Henry NEI: Nussenblatt, Robert Johns Hopkins School of Medicine: Calabresi, Peter University of Pennsylvania: Balcer, Laura

2007 Bench-to-Bedside Projects

RARE DISEASES CATEGORY

Institute(s)	Project	Investigators
NHLBI CC INOVA Fairfax Hospital	"Antiproliferative Therapy for Severe Pulmonary Arterial Hypertension"	NHLBI: Boehm, Manfred; Machado, Roberto; Gladwin, Mark CC: Cuttica, Michael J.; Cochard, Audrey; Barnett, Christopher Inova Fairfax Hospital: Nathan, Steven
NICHD University of Oxford (UK)	"Characterization of Glycosphingolipid Accumulation in Smith-Lemli-Opitz Syndrome and Treatment with N-butylidoxynojirimycin"	NICHD: Porter, Forbes University of Oxford (UK): Platt, Fran
NHLBI CC, University of Maryland	"Life-Threatening Pulmonary Complications of Organ Transplantation: An Investigation of the Pathogenesis of Bronchiolitis Obliterans and Its Novel Treatment with Aerosolized Liposomal Cyclosporine A"	NHLBI: Childs, Richard CC: Suffredini, Anthony; Danner, Robert University of Maryland: Iacono, Aldo; Griffith, Bartley; Terrin, Michael
NIAID Uniformed Services University of the Health Sciences NCI, NHGRI, CC, University of California, San Francisco	"Role of Pathogen-specific IgE and histamine release in the hyper-IgE syndrome"	NIAID: Freeman, Alexandra; Holland, Steve; Welch, Pam; DeCastro, Rosamma; Anderson, Victoria NCI: Turner, Maria NHGRI: Davis, Joie CC: Darnell, Dirk Uniformed Services University of Health Sciences: Mitre, Edward; Torrero, Marina; Hubner, Marc; Larson, David University of California, San Francisco: Puck, Jennifer
NICHD NEI NIDCR NIMH NIDDK Georgetown University Medical Center Uniformed Services University of the Health Sciences	"WAGR Syndrome: Clinical Characterization and Correlation with Genotype"	NICHD: Han, Joan; Lu, Bai; Rennert, Owen; Yanovski, Jack NEI: Brooks, Brian NIDCR: Domingo, Demetrio NIMH: Swedo, Susan NIDDK: Kopp, Jeffrey Georgetown University Medical Center: Lacbawan, Felicitas Uniformed Services University of Health Sciences: Sharp, Stephen Johns Hopkins University: Bengel, Frank M.
NHLBI NCI University of Virginia	"Sensitivity and resistance to Rituximab therapy in SLL/CLL: the role of antigenic modulation, immune effector mechanisms and direct pro-apoptotic signaling"	NHLBI: Weistner, Adrian NCI: Wilson, Wyndham University of Virginia: Weiss, Geoffrey; Taylor, Ron
NINDS University of Pennsylvania	"Quantification of urinary oxidized lipids, 8-hydroxyguanine, and 8-hydroxy-2'-deoxyguanosine in Friedreich ataxia patients undergoing idebenone treatment in a phase II double-blind placebo-controlled study"	NINDS: Fischbeck, Kenneth; DiProspero, Nicholas University of Pennsylvania: Blair, Ian; Wilson, Robert
NINDS NIDDK	"Translational Studies of Hereditary Spastic Paraplegias types SPG4 and SPG20"	NINDS: Blackstone, Craig; Bakawska, Joanna NIDDK: Hurley, James; Yang, Dong
NICHD Cleveland Clinic Genomic Medicine Institute	"Genetics of inherited paragangliomas and gastric stromal tumors associated with adrenal and other tumors"	NICHD: Stratakis, Constantine Cleveland Clinic Genomics Medicine Institute: Eng, Charis

GENERAL CATEGORY

Institute(s)	Project	Investigators
NCI Hackensack University Medical Center	"Dynamic Measurement and modeling of Immune Homeostasis and Reconstitution in Pre-clinical and Clinical Studies of Cytokine Therapy and Allogeneic Hematopoietic Stem Cell Transplantation (AHSCT)".	NCI: Gress, Ron; Chu, Yu-Way; Hakim, Fram; Telford, William; Sidorov, Igor; Dimitrov, Dimitar Hackensack University Medical Center: Korngold, Robert

AIDS CATEGORY

Institute(s)	Project	Investigators
NIAID CC University of Toronto George Washington University	"Intensification of Antiretroviral Therapy Using HIV Integrase Inhibitor (MK-0518) to Assess Decay of Viral Reservoirs in Peripheral Blood and Gut-Associated Lymphoid Tissue of Chronically Infected Patients"	NIAID: Chun, Tae-Wook ; Moir, Susan; Kottlil, Shyam CC: Healy, Letha; Penzak, Scott R. University of Toronto: Kovacs, Colin; Loufty, Mona George Washington University: Ward, Douglas
NIDCR CC	"Humoral Response Profiling of Viral and Cellular Tumor Antigens for Predicting, Diagnosing, and Monitoring HIV Malignancies"	NIDCR: Burbelo, Peter; Iadorola, Michael CC: Kovacs, Joseph; Healy, Letha
NIAID CC Massachusetts General Hospital	"1H Magnetic Resonance Spectroscopy for Quantification of Hepatic Triglyceride Content: Validation and Application in HIV-infected Patients"	NIAID: Hadigan, Colleen; Kottlil, Shyam CC: Louie, Adeline; Thomasson, David; Morse, Caryn Massachusetts General Hospital: Sahani, Dushyant; Torriani, Martin
NIAID University of Washington	"Development of Immunotoxins against Kaposi's Sarcoma Associated Herpesvirus for Treatment of Multicentric Castleman's Disease (MCD)"	NIAID: Berger, Ed University of Washington: Casper, Corey

MINORITY HEALTH & HEALTH DISPARITIES CATEGORY

Institute(s)	Project	Investigators
NIDDK Univ of California, Los Angeles	"Effects of Beta Cell Rest in Young Individuals with Type 2 Diabetes and in Relevant Animal Model"	NIDDK: Rother, Kristina; Brown, Rebecca UCLA: Butler, Peter
NHLBI Johns Hopkins University	"Identification of Predictive Biomarkers of Asthma Exacerbations in Exhaled Breath Condensates from High Risk Patients"	NHLBI: Levine, Stewart; Kaler, Maryann Johns Hopkins University: Jackman, Joany; Rothman, Richard
NIDDK NHLBI Harvard School of Public Health Brigham & Women's Hospital	"Ethnic Differences in Triglyceride Levels and Vascular Disease: A Study of Premenopausal African American and Caucasian Women"	NIDDK: Sumner, Anne; Ricks, Madia NHLBI: Sachdev, Vandana CC: Denking, Blakeley Harvard School of Public Health: Sacks, Frank Brigham & Women's Hospital: Aikawa, Masanori
NHLBI CC	"Contribution of Stromal Free Hemoglobin, Red Cell Membranes, and Red Cell Lysate on Nitric Oxide Inactivation in the Chronic Hemolytic State"	NHLBI: Gladwin, Mark; Machado, Roberto CC: Solomon, Steven; Klein, Harvey; Natanson, Charles

WOMEN'S HEALTH CATEGORY

Institute(s)	Project	Investigators
NCI Gynecologic Oncology Group Univ of Miami Univ of Oklahoma	"Targeting HPV E2 as a vaccine against HPV mediated CIN1 and CIN2"	NCI: Khleif, Samir; Berzofsky, Jay; Qian, Jiahua Gynecologic Oncology Group: Vaccine Working Group, Vaccine Subcommittee, GOG University of Miami: Lucci, Joseph University of Oklahoma: Walker, Joan

2008 Bench-to-Bedside Funded Projects

GENERAL CATEGORY

Institute(s)	Project	Investigators
NCI NIAID Ludwig Institute for Cancer Research Weill Cornell Medical College	"Targeting cancer-testis gene expression for lung cancer therapy"	NCI: Schrump, David; Rao, Mashadev; Hong, Julie NIAID: Lobanenko, Victor; Loukinov, Dmitri Ludwig Institute for Cancer Research: Old, Lloyd Weill Medical College at Cornell: Altorki, Nasser
NINR CC University of Maryland	"Molecular mechanisms of glial cell modulation of chemotherapy-induced painful peripheral neuropathy"	NINR: Wang, Xiao Min; Saligan, Leo CC: Mannes, Andrew University of Maryland: Dorsey, Susan; Renn, Cindy

AIDS CATEGORY

Institute(s)	Project	Investigators
NCI University of California, San Diego	"New strategies to decrease and eradicate HIV-1 reservoirs"	NCI: Maldarelli, Frank; Freed, Eric; Palmer, Sarah; Pathak, Vinay University of California, San Diego: Reid, Erin
NICHD NCI Case Western Reserve University	"HIV-1 suppression by Acyclovir in patients coinfecting with human herpesviruses from basic mechanism to clinical application"	NICHD: Margolis, Leonid; Biancotto, Angelique; Brichacek, Beda; Grivel, Jean-Charles; Lisco, Andrea; Vanpouille, Christophe Case Western Reserve: Lederman, Michael; Rodriguez, Benigno
NIAID NCI Children's National Medical Center	"Non-invasive cardiac 3T MRI for evaluation of premature coronary artery disease and myocardial dysfunction in adolescents and young adults with HIV acquired in infancy and childhood"	NIAID: Hadigan, Colleen NHLBI: Gharib, Ahmed NCI: Hazra, Rohan; Purdy, Julia Children's National Medical Center: Cross, Russell; Clauss, Sarah

AIDS/MINORITY HEALTH CATEGORY

Institute(s)	Project	Investigators
NIAID Johns Hopkins University Uganda (Makere Medical School, Mulago Hospital)	"Hepatitis B and HIV co-infection in Uganda"	NIAID: Quinn, Thomas; Stabinski, Lara; Reynolds, Steve Johns Hopkins University: Thio, Chloe; Kirk, Gregory; Gray, Ron; Thomas, David Uganda (Makere Medical School, Mulago Hospital): Ocama, Ponsiano; Serwadda, David
NCI FDA University of Washington	"Development and evaluation of a nanoparticle-based HIV-1 p24 antigen assay for monitoring therapy in resource limited settings"	NCI: Maldarelli, Frank FDA: Hewlett, Indira University of Washington: Frenkel, Lisa

MINORITY HEALTH & HEALTH DISPARITIES CATEGORY

Institute(s)	Project	Investigators
NIDDK University of Michigan	"Towards molecular marker based management of diabetic nephropathy in Pima Indians"	NIDDK: Nelson, Robert; Weil, Jennifer; Knowler, William University of Michigan: Kretzier, Matthias; Pennathur, Subbrannian; Woolf, Peter

RARE DISEASES CATEGORY

Institute(s)	Project	Investigators
NHGRI University of South Carolina University of Texas, MD Anderson	"Predicting the response to treatment using gene mutation profiling in metastatic melanoma patients"	NHGRI: Samuels, Yarden University of South Carolina: Buckhaults, Phillip University of Texas-MD Anderson: Hwu, Patrick
NHLBI CC	"Graft-Versus-Host Disease: novel cellular therapy using selective thawing of umbilical cord blood to obtain an aliquot for ex-vivo natural killer cell expansion and infusion following allogeneic hematopoietic stem cell transplantation"	NHLBI: Childs, Richard; Lundqvist, Andreas; Berg, Maria CC: Vasu, Sumithira
CC NHLBI INOVA Fairfax Hospital	"Evaluation of the platelet transcriptome expression profile in pulmonary arterial hypertension"	CC: Machado, Roberto; Cuttica, Michael NHLBI: Raghavachari, Nalini; Gladwin, Mark Inova Fairfax Hospital: Nathan, Steven
NHLBI, CC Harvard University Temple University	"Characterization of Jak/Stat activation in patients with monosomy 7 and the development of targeted therapy for patients using a Jak2 inhibitor"	NHLBI: Sloland, Elaine; Young, Neal; Pfannes, Loretta; Scheinberg, Phillip; Olnes, Matthew CC: Leitman, Susan Harvard University: Groopman, Jerome Temple University: Reddy, E. Premkumar Onconova Therapeutics, Inc.: Maniar, Manoj
NHLBI Imperial College, London	"Development of immunotherapeutic strategies to overcome tolerance in leukemia"	NHLBI: Barrett, Austin John; Yong, Agnes; Le, Quan Imperial College of London: Rezvani, Katy; Apperley, Jane; Altmann, Danny
NIAID NCI	"Recombinant human IL-6 (CYT107) as immunomodulatory therapy for idiopathic CD4 lymphopenia: a phase I/IIA open-label pilot study"	NIAID: Brenchley, Jason; Sereti, Irini; Hodge, Jessica; Porter, Brian; Mannon, Peter NCI: Estes, Jake

WOMEN'S HEALTH CATEGORY

Institute(s)	Project	Investigators
NHLBI NCI	"Immunogenicity of quadrivalent human papilloma virus vaccine (HPV Types 6, 11, 16, 18) in recipients of reduced intensity hematologic stem cell transplantation (HSCT)"	NICHD: Stratton, Pamela NHLBI: Shenoy, Aarthi NCI: Wood, Lauren; Pinto, Ligia
NICHD NIDDK CC Sackler Sch of MedTel Aviv, Israel	"Histaminergic pathways and energy intake in obese women"	NICHD: Yanovski, Jack; Yanoff, Lisa; Cropp, Cheryl; Han, Joan; Savastano, David NIDDK: Chen, Kong CC: Calis, Karim Sackler School of Medicine, Tel Aviv, Israel: Barak, Nir

2009 Bench-to-Bedside Funded Projects

GENERAL CATEGORY

Institute(s)	Projects	Investigators
NIDCD NINDS Johns Hopkins University	"Connectivity Analysis for Investigation of Auditory Impairment in Epilepsy"	NICHD: Horowitz, Barry NINDS: Theodore, William Johns Hopkins University: Boatman, Diana
NIAID Tufts Yale	"Searching for Persistence of Infection in Lyme Disease"	NIAID: Marques, Adriana Tufts University: Hu, Linden; Telford III, Sam Yale University: Krause, Peter

AIDS CATEGORY

Institute(s)	Project	Investigators
NIDDK NCI NEI PG County Hospital Washington Hospital Center	"MYH9 Genetic Variation in Kidney Disease Among African-Americans"	NIDDK: Kopp, Jeffrey; Gharib, Ahmed NCI: Winkler, Cheryl NEI: Chew, Emily P.G. County Hospital: Berhane, Daniel Washington Hospital Center: Light, Jimmy
NIDCR CC	"Development of a Diagnostic Test for Latent Tuberculosis Infection"	NIDCR: Burbelo, Peter CC: Kovacs, Joseph
NIAID NIDDK CC Johns Hopkins University	"Cardiometabolic Assessment in HIV"	NIAID: Hadigan, Colleen NIDDK: Gharib, Ahmed; Kong, Chen; Skarulis, Monica CC: Bluemke, David Johns Hopkins University: Lai, Shenghan

RARE DISEASES CATEGORY

Institute(s)	Project	Investigators
NIAID NCI MD Anderson	"Frequency and TCR Diversity of FOXP3+ Regulatory T Cells in Chronic GVHD"	NIAID: Tran, Dat; Shevach, Ethan NCI: Pavletic, Steven MD Anderson: Cooper, Laurence
NINDS University of Pittsburgh Children's National Medical Center	"GABAB Receptor Antagonist SGS-742 Treatment in SSADH Deficiency"	NINDS: Theodore, William University of Pittsburgh: Gibson, Kenneth Children's National Medical Center: Pearl, Phillip
NHLBI NIAID NIDDK NHGRI Johns Hopkins University	"Aneurysm Formation in Patients with Mutations in STAT3"	NHLBI: Boehm, Manfred; Beltran, Leilani; Walts, Avram; San, Hong NIAID: Freeman, Alexandra; Holland, Steven NIDDK: Gharib, Ahmed NHGRI: Davis, Joie Johns Hopkins University: Dietz, Hal Fred Hutchinson Cancer Research Center: Davis, Scott
NIAID NLM Cincinnati Children's Hospital University of Toronto	"Genomic and Stem Cell Approaches to Hemophagocytic Lymphohistiocytosis"	NIAID: Su, Helen; Lenardo, Michael NLM: Schaffer, Alejandro Cincinnati Children's Hospital: Filipovich, Alexandra University of Toronto: Zuniga-Pflucker, Juan Carlos
NCI Fred Hutchinson Cancer Center Johns Hopkins University	"Leukotriene Inhibition for the Amelioration of Bronchiolitis Obliterans"	NCI: Gress, Ron Fred Hutchinson Cancer Center: Lee, Stephanie Johns Hopkins University: Chen, Allen
NCI University of Toronto	"Repositioning Metformin as an Anti-Cancer Agent in Li-Fraumeni Syndrome"	NCI: Dennis, Phillip; Harris, Curtis; Fraumeni, Joseph; Savage, Sharon University of Toronto: Malkin, David

MINORITY HEALTH CATEGORY

Institute(s)	Project	Investigators
NIDCR NIDCD NCI University of SC	"Targeting mTOR as a Novel Mechanism-Based Therapy for Head and Neck Cancer"	NIDCR: Gutkind, J.Silvio; Molinolo, Alfredo NIDCD: Van Waes, Carter NCI: Dennis, Phil; Steinberg, Seth SAIC-Frederick: Veenstra, Timothy University of South Carolina: Day, Terry; Kirkwood, Keith; Rosenzweig, Steven
NHLBI, NCI, CC University of Pittsburgh	"Hemolysis-Associated Hemostatic Activation in Sickle Cell Disease"	NHLBI: Kato, Gregory NCI: Roberts, David CC: Lozier, Jay University of Pittsburgh: Novelli, Enrico; Isenberg, Jeffrey; Ragni, Margaret; Gladwin, Mark

MINORITY HEALTH / RARE DISEASES CATEGORY

Institute(s)	Project	Investigators
NHLBI Boston University Howard University	"Gene Expression Profiling to Predict Sickle Cell Anemia Sub-Phenotypes"	NHLBI: Taylor, James; Kato, Gregory; Minniti, Caterina; Raghavachari, Nalini; Xu, Xiuli Howard University: Gordeuk, Victor; O'Neal, Patricia Boston University School of Medicine: Klings, Elizabeth

PHARMACOGENOMICS CATEGORY

Institute(s)	Project	Investigators
NIMH, NIDDK University of Michigan	"Genetic Markers of CNS Adverse Events During Interferon Treatment"	NIMH: Laje, Gonzalo; McMahon, Francis NIDDK: Ghany, Marc University of Michigan: Fontana, Robert
NCI FDA University of Maryland	"Clopidogrel Pharmacogenetics: Practical Application"	NCI: Figg, William FDA: Pacanowski, Michael; Madabushi, Rajnikanth

BEHAVIORAL & SOCIAL SCIENCES CATEGORY

Institute(s)	Project	Investigators
NICHD NIDDK CC Uniformed Services University of the Health Sciences	"FTO and Eating in Absence of Hunger"	NICHD: Yanovski, Jack; Han, Joan NIDDK: Yanovski, Susan CC: Kozlosky, Merel; Sebring, Nancy Uniformed Services University of Health Sciences: Tanofsky-Kraff, Marian; Shomaker, Lauren

2010 Bench-to-Bedside Funded Projects

AIDS CATEGORY

Institute(s)	Project	Investigators
NCI NIAID Johns Hopkins University University of California Los Angeles Medical Center	"New Bioinformatic Approach to Determine HIV Incidence"	NCI: F. Maldarelli; M. Kearney; W. Shao NIAID: R. Dewar Johns Hopkins University: J. Margolick LA Biomedical Research Institute at Harbor-UCLA Medical Center (LA BioMed): E. Daar
NCI University of Pittsburgh National Naval Medical Center	"Role of Gut-Associated Lymphoid Tissue in HIV-1 Persistence"	NCI: F. Maldarelli; M. Kearney University of Pittsburgh: D. McMahon National Naval Medical Center: A. Ganesan
CC. NIAID, NCI Washington Hospital Center	"Multiplex Microarray Chip-based Diagnosis of Respiratory Infections in HIV"	CC: J. Kovacs; A. Suffredini; P. Murray NIAID: S. Holland; J. Cuellar-Rodriguez NCI: J. Gea-Banacloche; R. Lempicki Washington Hospital Center: M. Smith

BEHAVIORAL & SOCIAL SCIENCES CATEGORY

Institute(s)	Project	Investigators
NIMH Yale University OK Univ Health Sciences Ctr	"Antibody Identification and IVIG Treatment of PANDAS"	NIMH: S. Swedo Yale University: J. Leckman OK Univ Health Sci Center: M. Cunningham

WOMEN'S HEALTH

Institute(s)	Project	Investigators
NICHD State University of New York	"Adrenal Hyperplasia Among Adolescent Patients Polycystic Ovarian Syndrome"	NICHD: C. Stratakis SUNY: S. Ten

MINORITY HEALTH CATEGORY

Institute(s)	Project	Investigators
NCI University of California, Davis	"Control of XMRV Replication in PBMCs and Prostate Carcinomas"	NCI: V. Pathak; A. Rein; W. S. Hu; F. Maldarelli University of California, Davis: R. deVere White; H.J. Kung
NHLBI CC	"In vitro Fucosylation to Augment Cord Blood Stem Cell Engraftment"	NHLBI: R. Childs; J. Pantin CC: D. Stroncek
NIDDK NINR CC Beth Israel Medical Center	"Biochemical Mechanisms of the Etiology of Sickle Cell Pain"	NIDDK: A. Schechter; NINR: R. Dionne CC: D. Stroncek; W. Smith Beth Israel Medical Center: R. Portenoy; R. Cruciani

GENERAL CATEGORY

Institute(s)	Project	Investigators
CC NCI Massachusetts General Hospital	"Optical Guidance for Improved Prostate Cancer Surgery"	CC (Radiology and Imaging Sciences): B. Wood; M. Dreher; A. Kapoor NCI: P. Pinto; W. Linehan MGH: U. Mahmood
NIAID NIBIB NCI Georgetown University	"Imaging CXCR4-Expressing Cancer Using 64CuAMD"	NIAID: J. Farber; I. Weiss NIBIB: X. Chen; O. Jacobson NCI: P. Choyke Georgetown University: C. Isaacs

PHARMACOGENOMICS CATEGORY

Institute(s)	Project	Investigators
NIAID University of Maryland	"Mechanism of Response to Anti-TNF Therapy in Inflammatory Bowel Disease"	NIAID: M. Yao; W. Strober; I. Fuss University of MD: R. Cross; M. Flasar

RARE DISEASES CATEGORY

Institute(s)	Project	Investigators
NIAID NCI NIBIB University of Minnesota Mayo College of Medicine	"Targeting Antigen-Antibody Responses in Systemic Capillary Leak Syndrome"	NIAID: K. Druey; T. Myers; S. Porcella NCI: O. Landgren NIBIB: A. Gorbach University of Minnesota: A. Dudek Mayo College of Medicine: P. Greipp
CC NINDS NHLBI Johns Hopkins University	"Sympathetic Innervation & Myocardial Injury in Acute Stress Cardiomyopathy"	CC: C. Sibley; D. Bluemke NINDS: D. Goldstein NHLBI: D. Rosing JHU: I. Wittstein; F. Bengel; J. Mudd; J. Lima
NCI Children's Hospital Medical Ctr	"Preclinical Testing of Targeted Agents for Clinical Development in NF1"	NCI: A. Kim; B. Widemann; E. Dombi Children's Hospital Medical Ctr: N. Ratner; J. Wu
NCI National Children's Medical Center Children's Hospital & Clinics of MN	"The DICER1-related Pleuropulmonary Blastoma Cancer Predisposition Syndrome"	NCI: C. Kratz; B. Alter; P. Rosenberg National Children's Medical Center: A. Hill Children's Hosp & Clinics of MN: Y. Messinger; K. Schulz
NIMH University of Louisville	"Brain Development in Children with Williams Syndrome, and the LIMK1 Gene"	NIMH: K. Berman; J. Kleinman University of Louisville: C. Mervis
NCI Yale University	"The Role of EGFR in Endolymphatic Sac Tumors"	NCI: P. Dennis Yale University: A. Vortmeyer

RARE DISEASES DRUG DEVELOPMENT

Institute(s)	Project	Investigators
NHLBI Cincinnati Children's Hospital Medical Center	"A Novel Therapy to Treat Acid Lipase-Deficiency by LCAT Inhibition"	NHLBI: A. Remaley; K. Vickers; R. Shamburek Cincinnati Children's Hospital Medical Center - Research Foundation: G. Grabowski; H. Du
NICHD NHGRI Washington University	"Development of Combination Therapy for Niemann-Pick Disease, Type C"	NICHD: F. Porter; A. Yergey; S. Bianconi NHGRI: W. Pavan Washington University: D. Ory

NCI University of Washington Medical Center Cincinnati Children's Hospital Medical Center	"Gene Therapy Clinical Trial for LAD-1 Using a Foamy Viral Vector"	NCI: D. Hickstein University of Washington Medical Center: D. Russell Cincinnati Children's Hospital Medical Center: P. Malik
CC NIAID NIDDK	"BH3 Mimetics for the Treatment of Autoimmune Lymphoproliferative Syndrome"	CC: J. Oliveira Filho; T. Fleisher NIAID: V. Rao; K. Dowdell NIDDK: D. Appella
NICHD NHLBI Johns Hopkins University	"Preventing Aortic Dilation in Women with Turner Syndrome"	NICHD: C. Bondy; V. Bakalov; J. Zhou NHLBI: A. Arai; D. Rosing; M. Boehm; V. Sachdev Johns Hopkins University: J. Van Eyk; Q. Fu
NIAID Uniformed Services University of the Health Sciences George Washington University	"Immunogenicity and Leishmania vaccine Potential of Sandfly Saliva in Humans"	NIAID: J. Valenzuela; S. Kamhawi Uniformed Services University of the Health Sciences/Walter Reed Army Medical Center: N. Aronson George Washington University: M. Bottazzi

INSTITUTE FUNDED

Institute(s)	Project	Investigators
NICHD NIMH Uniformed Services University of the Health Sciences GlaxoSmithKline Pharmaceuticals	"The Role of BDNF in Autism Spectrum Disorder and Cognitive Function"	NICHD: J. Han; C. Pierpaoli NIMH: K. Martinowich; D. Weinberger; C. Golden Williams; S. Swedo; A. Thurm NCI: L. Tessarollo USUHS: S. Sharp GlaxoSmithKline Pharmaceuticals: B. Lu

2011 Bench-to-Bedside Funded Projects

AIDS CATEGORY

Institute(s)	Project	Investigators
NICHD Case Western Reserve University	"Valacyclovir for Treatment of HIV-1 Infection"	NICHD: L. Margolis Case Western: M. M. Lederman
NIAID CC University of Cincinnati	"Effect of CCR5 Blockade on liver disease progression in HIV patients"	NIAID: A. Kohli; S. Kottilli; D. Fishbein; A. Osinusi CC: A. F. Suffredini University of Cincinnati: K.E. Sherman
NIAID NHLBI NIDDK George Washington University	"HDL Metabolism and Atherosclerosis in HIV-infected Patients"	NIAID: C. Hadigan NHLBI: A. Remaley NIDDK: A. Gharib; K. Abd-Elmoniem George Washington University: M. Bukrinsky
NIAID NCI NIDDK Johns Hopkins University	"Liver Fibrosis in HIV-infected persons of African descent"	NIAID: L. Stabinski; T. Quinn; S. Reynolds; A. Redd; S. Kottilli; A. Klion NCI: D. Kleiner NIDDK: T. Heller Johns Hopkins University: G. D. Kirk

BEHAVIORAL & SOCIAL SCIENCES CATEGORY

Institute(s)	Project	Investigators
NICHD NIAAA NIDDK CC Uniformed Services University of the Health Sciences	"Depression and Insulin Resistance in Adolescent Girls"	NICHD: J. Yanovski; L. B. Shomaker NIAAA: M. Heilig NIDDK: K. Chen CC: M. Kozlosky, A.B. Courville USUHS: M. Tanofsky-Kraff University of Minnesota: Z. M. Kukoska

WOMEN'S HEALTH CATEGORY

Institute(s)	Project	Investigators
CC NIDDK University of Pennsylvania	"Role of Androgen and Estrogen Receptor Signaling in Pulmonary Arterial Hypertension"	CC: R. Danner; M. Solomon; J. Elinoff NIDDK: A. M. Gharib; K. Abd-Elmoniem University of Pennsylvania: J. E. Rame; K. B. Margulies; P. Forfia

MINORITY HEALTH CATEGORY

Institute(s)	Project	Investigators
NHLBI University of Pennsylvania	"Reverse cholesterol transport in humans with Mendelian low HDL disorders"	NHLBI: A. Remaley University of Pennsylvania: M. Cuchel
NICHD NIDDK CC Mayo Clinic, Rochester, MN	"Fat Metabolism and Function-Altering Polymorphisms in MC3R"	NICHD: J. Yanovski; H. Westphal; D. Adler-Wailes NIDDK: O. Gavrilova; K. Chen CC: J. C. Reynolds; D. M. Dellavalle Mayo Clinic, Rochester, MN: M. D. Jensen

GENERAL CATEGORY

Institute(s)	Project	Investigators
NICHD NIMH	"Near Infra-Red Functional Imaging in Autistic Spectrum Disorder Patients"	NICHD: A. Gandjbakhche; J. Riley; L. Najafizadeh NIMH: S. Swedo; A. Thurm; M. Gozzi
CC, NIDDK, NIAID Johns Hopkins	"Imaging bacterial infection in immunocompromised patients"	CC: D. Hammoud NIDDK: A. M. Gharib; K. Abd-Elmoniem NIAID: A. Freeman; S. Holland Johns Hopkins: M. G. Pomper; C. Endres; S. Cho

RARE DISEASES CATEGORY

Institute(s)	Project	Investigators
NEI Benaroya Research Institute	"Development of antigen specific biomarkers for autoimmune uveitis disease"	NEI: R. Caspi; M. Mattapallil; R. B. Nussenblatt; N. Sen Benaroya Research Institute at VA: G. T. Nepom; B. W. Kwok; E. James
NCI NHLBI Hospital A.C. Camargo - Fundação Antonio Prudente International Agency for Research on Cancer The Hospital for Sick Children	"Mitochondria, Telomeres, and Lifestyle in Li-Fraumeni Syndrome Outcomes"	NCI: S. A. Savage; I. Wentzensen; P. Mai; M. Greene; R. Sinha; J. Fraumeni NHLBI: P. M. Hwang Hospital A. C. Camargo - Fundação Antonio Prudente: M. A. Achatz International Agency for Research on Cancer: P. Hainaut The Hospital for Sick Children: D. Malkin
Washington University NHGRI Albert Einstein Coll of Medicine	"Cyclodextrin therapy for Niemann-Pick C1 disease"	Washington University: D. Ory NHGRI: J. J. Marugan; NICHD: F. D. Porter Albert Einstein College of Medicine: S. U. Walkley

NICHD NIA NIMH Medical University of South Carolina	"Amitriptyline for the Treatment of BDNF Haploinsufficiency"	NICHD: J. Han NIA: B. Martin; S. Maudsley; W. Chadwick NIMH: S. Swedo; A. Thurm; C. Golden Williams Medical University of South Carolina: L. M. Luttrell
NICHD NHLBI Howard University Medical Center	"Exacerbation of HIF2alpha-dependent polycythemia by iron deficiency"	NICHD: T. Rouault NHLBI: G. J. Kato; NCI: W. M. Linehan; J. Mitchel Howard University Medical Center: V. Gordeuk
NICHD NHLBI NIMH Kennedy Krieger Institute	"Biomarkers of neurodevelopment in Smith-Lemli-Opitz Syndrome"	NICHD: F. D. Porter; A. Yergey NHLBI: A. Remaley NIMH: J. Geidd; CC: E. Baker Kennedy Krieger Institute/Johns Hopkins University: E. Tierney; S. Mori; R. W. Lee

2012 Bedside-to-Bench Projects

AIDS CATEGORY

Institute(s)	Project	Investigators
NIAID CC Nationwide Children's Hospital	"Anti-PD-1 antibody to eradicate HBV in HBV/HIV co-infection"	NIAID: L. Barrett; S. Kottlil; A. Kohli; E. Meissner CC: A. Suffredini; SAIC-Frederick: A. Osinusi Nationwide Children's Hospital: C. Walker
NIMH NINDS Univ of California, San Diego	"Inflammation and Function of P-gp in HIV Infection of Brain"	NIMH: W. Kreis; R. Innis; S. Kapetanovic NINDS: A. Nath; CC: C. Morse; S. Penzak U of Cal, San Diego: R. Ellis; S. Letendre; R. Heaton; B. Best
CC, NIAID, NCI, NIDDK Inova Health System University of Maryland	"Targeting Lysyl Oxidase-like-2 to Inhibit Hepatic Fibrogenesis in HIV"	CC: C. Morse; J. Kovacs; B. Wood; NIAID: E. Meissner; S. Kottlil; NCI: D. Kleiner NIDDK: T. Heller; A. Gharib; Inova Health System: Z. Goodman; U of MD: R. Redfield; R. Talwani
NIAID Johns Hopkins University	"Effect of HIV Superinfection on Disease Progression and Immune Response"	NIAID: T. Quinn; S. Porcella Johns Hopkins University: R. Gray; G. Kirk

WOMEN'S HEALTH CATEGORY

Institute(s)	Project	Investigators
NCI, CC Johns Hopkins University U Mass, Amherst	"Molecular Epidemiology of Postpartum Involution of the Breast: Demonstration of Tools for Understanding Influences of Pregnancy on Breast Cancer Risk"	NCI: M. Sherman; J. Faupel-Badger; S. Hewitts; J. Balkam; G. Gierach; K. Flanders; CC: D. Bluemke; JHU: A. Meeker; U Mass Amherst: K. Arcaro

GENERAL CATEGORY

Institute(s)	Project	Investigators
NCI; CC UPenn; Children's Hosp of Philadelphia	"CD22 Chimeric Antigen Receptors Targeting Acute Lymphoblastic Leukemia"	NCI: C. Mackall; A. Wayne; T. Fry; R. Orentas; W. Haso; CC: D. Stroncek University of Pennsylvania: C. June; B. Levine Children's Hospital of Philadelphia: S. Grupp
NIAMS, NHGRI, NIDCR Cincinnati Children's Hospital	"Defining the Functional Role of STAT4 in human systemic lupus erythematosus"	NIAMS: J. O'Shea NHGRI: D. Kastner; E. Remmers; NIDCR: G. Illei Cincinnati Children's Hospital: J. Harley

RARE DISEASES CATEGORY

Institute(s)	Project	Investigators
NHLBI NIAID Baylor College of Medicine	"T cell adoptive therapy for viral infection after stem cell transplantation"	NHLBI: J. Barrett NIAID: D. Douek; CC: D. Stroncek Baylor College of Medicine: C. Bollard
NINDS Children's Research Institute University of California, Davis	"Genetic Characterization and Outcome in Congenital Disorders of Muscle"	NINDS: C. Bonnemann Children's Research Institute (CNMC): E. Hoffman University of California, Davis: C. McDonald
NICHD, NHLBI Children's Hospital of Philadelphia	"Choroid Plexus-Directed Gene Therapy for Lysosomal Storage Disease"	NICHD: S. Kaler NHLBI: R. Kotin Children's Hospital of Philadelphia: J. Wolfe
NCI NICHD University of Louisville	"Elucidation of cancer metabolism by Stable isotope-Resolved Metabolomics"	NCI: W. Linehan NICHD: T. Rouault University of Louisville: A. Lane; T. Fan
NINDS CC University of Pennsylvania	"Treatment of Anti-NMDA receptor encephalitis"	NINDS: A. Nath; I. Cortese; S. Jacobson; K. Roche CC: D. Hammoud Univ of Pennsylvania: J. Dalmau; R. Balice-Gordon
NHLBI, NINDS University of Central Florida	"Metabolic phenotyping of Parkin mutation associated Parkinson's Disease"	NHLBI: M. Sack, NINDS: R. Youle University of Central Florida: X. Han
NHGRI, NIDDK Children's National Medical Ctr	"Metabolic Phenotyping in Methylmalonic Acidemia: Markers and Drug Response"	NHGRI: C. Venditti; E. Manoli; NIDDK: K. Chen Children's National Medical Center: M. Tuchman
NICHD NINDS University of Massachusetts Memorial Medical Center University of Maryland, Baltimore	"Developing and validating membrane biomarkers in the muscular dystrophies"	NICHD: J. Zimmerberg; P. Blank; P. Basser; G. Humphrey NINDS: C. Bonnemann Univ of MA Memorial Med Ctr: R. Brown University of Maryland, Baltimore: R. Bloch

INSTITUTE FUNDED

Institute(s)	Project	Investigators
NICHD Harvard Medical School Massachusetts General Hospital	"Blockade of Kisspeptin Signaling in Women"	NICHD: A. Delaney Harvard Medical School: S. Seminara Massachusetts General Hospital: W. Crowley
NIAID Stanford University	"Transcriptional responses to LAIV in bulk and single resident URT cells"	NIAID: K. Subbarao Stanford University: H. Greenberg; A. Sen; X. He

2013 Bedside-to-Bench Projects

AIDS CATEGORY

Institute(s)	Project	Investigators
NIAID NINDS University of Minnesota School of Public Health University of New South Wales	"PAR1 signaling in T cells and its role in HIV infection"	NIAID: Catalfamo, Marta; Lane, H. Clifford; Sneller, Michael NINDS: McGavern, Dorian Univ of MN School of Public Health: Neaton, James University of New South Wales: Emery, Sean
NCI University of Pittsburgh Catholic University of America	"Targeting Persistent HIV Reservoirs During Suppression with ART"	NCI: Kearney, Mary; Maldarelli, Frank University of Pittsburgh: Mellors, John Catholic University of America: Rao, Venigalla
CC NIDCR NIAID SAIC Georgetown University Hospital	"Evaluation of Impaired Immunologic Responses to Antiretroviral Therapy"	CC: Kovacs, Joseph; Morse, Caryn NIDCR: Burbelo, Peter; NIAID: Sereti, Irini SAIC/NCI Frederick: Estes, Jacob SAIC-Frederick, Inc. (NIAID): Lempicki, Richard Georgetown University Hospital: Young, Mary; Kumar, Princy
NCI CC University of Pittsburgh	"Localizing Reservoirs of HIV Persistence in Lymphoid Tissue"	NCI: Maldarelli, Frank; Kearney, Mary CC: Wood, Bradford; Venkatesan, Aradhana; Hammoud, Dima University of Pittsburgh: Mellors, John

BEHAVIORAL & SOCIAL SCIENCES CATEGORY

Institute(s)	Project	Investigators
NIAAA NIDA CC NIMH	"Oxytocin in Alcohol Dependence: A Novel and Translational Approach"	NIAAA: Lee, Mary; Momenan, Reza; Leggio, Lorenzo NIDA: Kimes, Alane CC: Herscovitch, Peter NIMH: Averbach, Bruno

RARE DISEASES CATEGORY

Institute(s)	Project	Investigators
NINR NINDS The Hospital for Sick Children	"N-acetylcysteine trial in RYR1-related congenital myopathy"	NINR: Meilleur, Katherine NINDS: Bonnemann, Carsten The Hospital for Sick Children: Dowling, James
NCI Johns Hopkins University School of Medicine	"Highly Active Anti-Tumor Therapy (HAAT): Targeting Tumor Metabolism"	NCI: Widemann, Brigitte; Arnaldez, Fernanda Johns Hopkins: Loeb, David; Meyer, Christian Frederick; Pomper, Martin; Fayad, Laura; Powell, Jonathan; Albert, Catherine

WOMEN'S HEALTH CATEGORY

Institute(s)	Project	Investigators
NIMH The Rockefeller University	"BDNF Genotype-Estrogen Interactions in Brain & Mood Disorders in Women"	NIMH: Berman, Karen; Schmidt, Peter The Rockefeller University: McEwen, Bruce

INSTITUTE FUNDED

Institute(s)	Project	Investigators
NICHHD, NIMH, CC University of North Dakota School of Medicine Uniformed Services University of the Health Sciences	"Testing Neurobehavioral Endophenotypes of Loss of Control Eating"	NICHHD: Yanovski, Jack NIMH: Pine, Daniel; Nelson, Eric; Coppola, Richard CC: Psota, Tricia Univ of North Dakota Sch of Med: Engel, Scott Uniformed Services University of the Health Sciences: Tanofsky-Kraff, Marian; Vannucci, Anna
NIMH Columbia Univ Medical Center Univ of California, San Diego	"Visual processing of dynamic social behavior in the schizophrenic brain"	NIMH: Leopold, David Columbia University Medical Center: Javitt, Daniel Univ of California, San Diego: Martinez, Antigona

2014 Bedside-to-Bench Projects

AIDS CATEGORY

Institute(s)	Project	Investigators
NIAID CC NINR Massachusetts General Hospital	"Cardiometabolic Effects of Eplerenone in HIV Infection"	NIAID: Hadigan, Colleen CC: Bluemke, David; Liu, Chia-Ying; Morse, Caryn NINR: Henderson, Wendy Massachusetts General Hospital: Grinspoon, Steven; Takara, Stanley
NIAID University of Pittsburgh	"Targeting tissue factor in HIV/SIV infection"	NIAID: Sereti, Irini; Franchischetti, Ivo; Andrade, Bruno de Bezerril; Schechter, Melissa University of Pittsburgh: Pandrea, Ivona
NINDS NIAID CC NCI NIA NIMH NIAMS Johns Hopkins University Uniformed Services University of Health Sciences	"Anakinra, an IL-1 receptor antagonist, for neuroinflammation in HIV-1"	NINDS: Nath, Avindra; Smith, Bryan; Bhagavatheeshwaran, Govind; Auh, Sungyoung; Jacobson, Steven NIAID: Lau, Chuen-Yen; Tramont, Edmund; DerSimonian, Rebecca CC: Morse, Caryn; Kovacs, Joseph NCI: Maldarelli, Frank NIA: Rapoport, Stanley NIMH: Snow, Joseph; Kapetanovic, Suad NIAMS: Goldbach-Mansky, Raphaela Johns Hopkins University: Sacktor, Ned; Pardo, Carlos; McArthur, Justin Uniformed Services University of Health Sciences: Agan, Brian; Ganesan, Anuradha; Anorworanich, Jintanat

BEHAVIORAL & SOCIAL SCIENCES CATEGORY

Institute(s)	Project	Investigators
NICHHD Yale University	"Selective Dopamine D4 Receptor-Targeting Compounds as Pro-Cognitive Drugs"	NICHHD: Buonanno, Andres Yale University: Corlett, Philip; Krystal, John

RARE DISEASES CATEGORY

Institute(s)	Project	Investigators
NCI CC NIAID University of Pennsylvania	"CAR-modified CD8+ memory stem cells targeting B-cell malignances"	NCI: Gattinoni, Luca; Kochenderfer, James CC: Stroncek, David; Sabatino, Marianna NIAID: Roederer, Mario University of Pennsylvania: June, Carl
NIAID CC NIGMS Ichan School of Medicine at Mt. Sinai Novartis	"Targeted therapy in immunodeficient patients with mutations in PI3K genes"	NIAID: Lenardo, Michael; Rao, Koneti; Su, Helen; Uzel, Gulbu CC: Kuehn, Hyesun; Fleisher, Thomas; Rosenzweig, Sergio NIGMS: Lucas, Carrie Icahn School of Medicine at Mt. Sinai: Cunningham-Rundles, Charlotte Novartis: Burkhart, Christoph; Christ, Andreas

DIETARY SUPPLEMENTS CATEGORY

Institute(s)	Project	Investigators
NICH NIA University of Missouri-Columbia	"Mechanisms and treatment of copper-related motor neuron disease"	NICH: Kaler, Stephen; Feldman, Benjamin NIA: Traynor, Bryan University of Missouri-Columbia: Petris, Michael

MINORITY HEALTH CATEGORY

Institute(s)	Project	Investigators
NHLBI NHGRI NICH Massachusetts General Hospital	"Telomere Diseases in Pregnant Women"	NHLBI: Townsley, Danielle; Young, Neal; Dumitriu, Bogdan NHGRI: Merideth, Melissa NICH: Stratton, Pamela Massachusetts General Hospital: Fogerty, Annemarie; Dzik, Walter

IC FUNDED

Institute(s)	Project	Investigators
NIDDK NCI CC University of Colorado	"Mechanisms of microbial translocation in hepatitis C related liver disease"	NIDDK: Heller, Theo; Rehmann, Barbara; Koh, Christopher; Gharib, Ahmed; Abd-Elmoniem, Khaled; Etzion, ohadmd Ohad NCI: Kleiner, David CC: Levy, Elliott University of Colorado: Everson, Gregory

2015 Bedside-to-Bench Projects

AIDS CATEGORY

Institute(s)	Project	Investigators
NIAID University of Minnesota	"Pre-Clinical Development of Anti-HIV Chimeric Antigen Receptors"	NIAID: Berger, Ed; Chung, Taw Wook University of Minnesota: Skinner, Pamela
CC NIAID NIA NINDS NIMH Johns Hopkins University Georgetown University	"Intermittent Caloric Restriction in HIV-related Metabolic Syndrome"	CC: Morse, Caryn; Kovacs, Joseph; Courville, Amber; Bernstein, Sheena NIAID: Hadigan, Colleen NIA: Mattson, Mark NINDS: Nath, Avi; Smith, Bryan NIMH: Snow, Joseph Johns Hopkins University: Margolick, Joseph; Brown, Todd Georgetown University: Young, Mary
NIAID CC	"Herpesviruses reactivation and inflammation in HIV+ women initiating ART"	NIAID: Sereti, Irini; Reynolds, Steven; Quinn, Thomas CC: Fahle, Gary

AIDS CATEGORY: Project co-Funded by Office of AIDS Research (OAR) and National Heart, Lung and Blood Institute (NHLBI)

Institute(s)	Project	Investigators
NHLBI NCI NINDS CC	"Adoptive immunotherapy for polyomavirus-related diseases"	NHLBI: Muranski, Pawel; Barrett, John NCI: Brownell, Isaac; Buck, Chris NINDS: Nath, Avi; Cortese, Irene CC: Stroncek, David

BEHAVIORAL & SOCIAL SCIENCES CATEGORY

Institute(s)	Project	Investigators
NICHD NIMH University of Maryland College Park	"Mirror neuron network dysfunction as an early biomarker of neurodevelopment"	NICHD: Gandjbakhche, Amir NIMH: Thurm, Audrey University of Maryland College Park: Fox, Nathan

RARE DISEASES CATEGORY

Institute(s)	Project	Investigators
NHLBI NCI George Washington University Columbia University McGill University	"Metformin for cancer chemoprevention in Li-Fraumeni Syndrome"	NHLBI: Hwang, Paul NCI: Annunziata, Christina George Washington University: Walcott, Farzana Columbia University: Fojo, Antonio McGill University: Pollack, Michael
NICHD NIMH Los Angeles Biomedical Research Institute University of Minnesota	"Phenotypic effects of choroid plexus gene therapy for Sanfilippo B syndrome"	NICHD: Kaler, Stephen NIMH: Snow, Joseph Los Angeles Biomedical Research Institute: Dickson, Patricia University of Minnesota: Nestril, Igor

MINORITY HEALTH CATEGORY

Institute(s)	Project	Investigators
NIDDK NHGRI Children's National Medical Center	"Therapeutic Targets in African-American Youth with Type 2 Diabetes"	NIDDK: Chung, Stephanie NHGRI: Rotimi, Charles; Bentley, Amy Children's National Medical Center: Magge, Sheela

WOMEN'S HEALTH CATEGORY

Institute(s)	Project	Investigators
NICHD NIMH CC Uniformed Services University of the Health Sciences University of North Dakota School of Medicine Neuropsychiatric Research Institute	"Attention Bias Retraining in Adolescents with Loss of Control Eating"	NICHD: Yanovski, Jack NIMH: Pine, Daniel; Coppola, Richard; Nelson, Eric CC: Courville, Amber Uniformed Services University of the Health Sciences: Tanofsky-Kraff, Marian; Waters, Andrew University of North Dakota School of Medicine: Engel, Scott Neuropsychiatric Research Institute: Crosby, Ross

IC FUNDED

Institute(s)	Project	Investigators
CC NCI Children's National Medical Center The University of Georgia College of Engineering	"Integrated Planning, Navigation, Ablation and Monitoring for Prostate Cancer"	CC: Wood, Bradford; Kwak, Jin Tae; Xu, Sheng NCI: Pinto, Peter; Choyke, Peter; Turkbey, Bari Children's National Medical Center: Cleary, Kevin The University of Georgia College of Engineering: Tse, Zion Tsz Ho

CONTACT US

Julie Orlando
Program Coordinator



Tel 301.402.0102
BenchtoBedside@mail.nih.gov
or
julie.orlando@nih.gov

Pat Piringer
Program Coordinator



Tel 301.402.2435
BenchtoBedside@mail.nih.gov
or
ppiringer@nih.gov

Hana Smith
Science Contributor



Tel 301.496.9425
BenchtoBedside@mail.nih.gov
or
smithha@cc.nih.gov

Program Information

Bench-to-Bedside (BtB) Program
NIH Clinical Center, Bethesda, MD

www.cc.nih.gov/ccc/btb/

