

DEPARTMENT OF HEALTH AND HUMAN SERVICES

NATIONAL INSTITUTES OF HEALTH

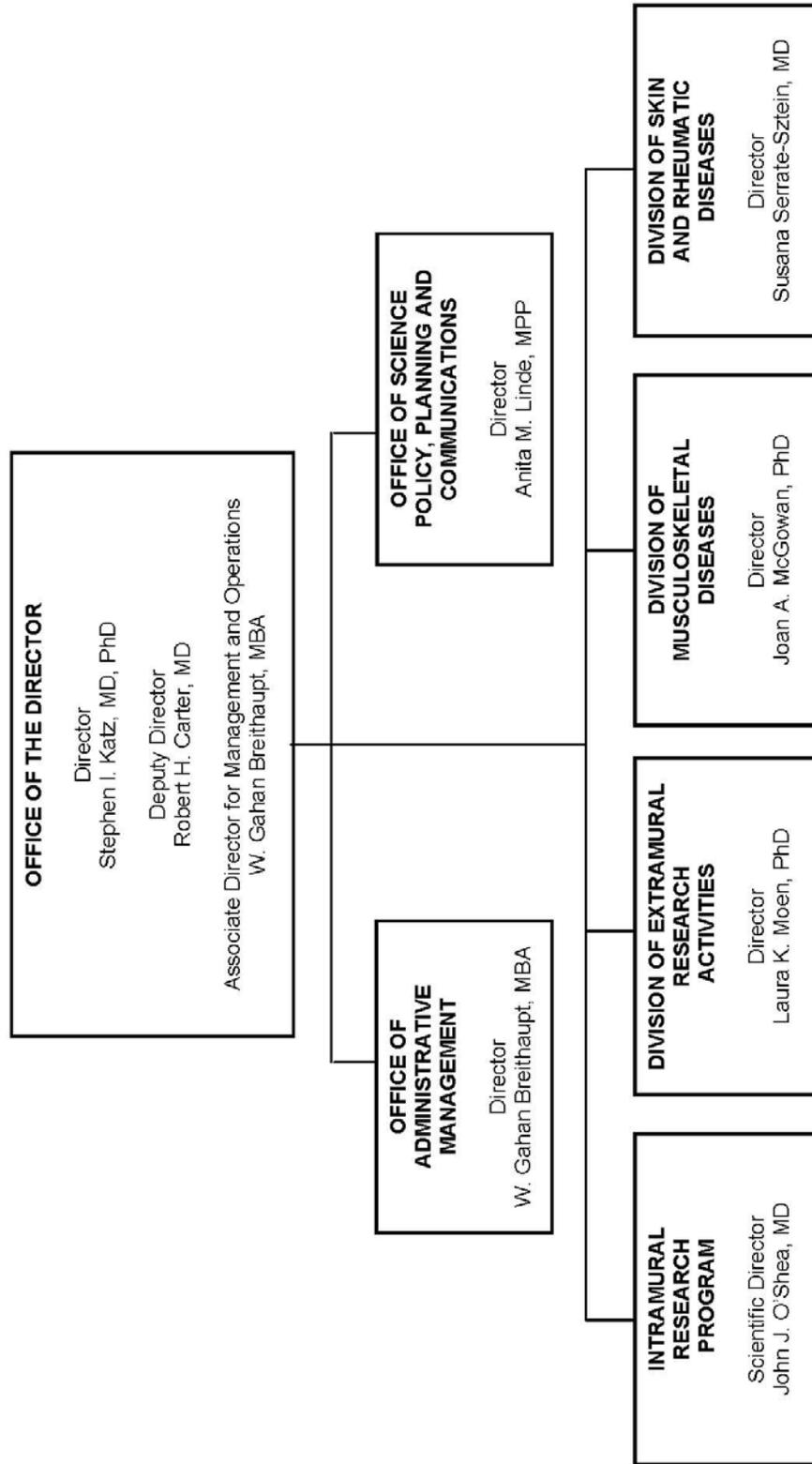
National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS)

<u>FY 2014 Budget</u>	<u>Page No.</u>
Organization Chart.....	2
Appropriation Language.....	3
Amounts Available for Obligation.....	4
Budget Mechanism Table.....	5
Major Changes in Budget Request.....	6
Summary of Changes.....	7
Budget Graphs.....	9
Budget Authority by Activity.....	10
Authorizing Legislation.....	11
Appropriations History.....	12
Justification of Budget Request.....	13
Budget Authority by Object Class.....	22
Salaries and Expenses.....	23
Detail of Full-Time Equivalent Employment (FTE).....	24
Detail of Positions.....	25

**NATIONAL INSTITUTES OF HEALTH**

**National Institute of Arthritis and Musculoskeletal and Skin Diseases**

**Organizational Structure**



NATIONAL INSTITUTES OF HEALTH

National Institute of Arthritis and Musculoskeletal and Skin Diseases

*For carrying out section 301 and title IV of the PHS Act with respect to arthritis and musculoskeletal and skin diseases, \$540,993,000.*

**NATIONAL INSTITUTES OF HEALTH**  
**National Institute of Arthritis and Musculoskeletal and Skin Diseases**

**Amounts Available for Obligation<sup>1</sup>**  
(Dollars in Thousands)

Source of Funding	FY 2012 Actual	FY 2013 CR	FY 2014 PB
Appropriation	536,801	539,065	540,993
Rescission	(1,015)	0	0
Subtotal, adjusted appropriation	535,786	539,065	540,993
Real Transfer under Secretary's Transfer authority	(153)	0	0
Secretary's Transfer for Alzheimer's disease (AD)	(353)		
Comparative Transfers to NLM for NCBI and Public Access	(490)	(634)	0
Subtotal, adjusted budget authority	534,791	538,431	540,993
Unobligated balance, start of year	0	0	0
Unobligated balance, end of year	0	0	0
Subtotal, adjusted budget authority	534,791	538,431	540,993
Unobligated balance lapsing	(80)	0	0
Total obligations	534,711	538,431	540,993

<sup>1</sup> Excludes the following amounts for reimbursable activities carried out by this account:

FY 2012 - \$9,826    FY 2013 - \$11,000    FY 2014 - \$7,500

**NATIONAL INSTITUTES OF HEALTH**  
**National Institute of Arthritis and Musculoskeletal and Skin Diseases**  
**Budget Mechanism - Total <sup>1</sup>**  
(Dollars in Thousands)

MECHANISM	FY 2012 Actual		FY 2013 CR		FY 2014 PB		Change vs. FY 2012	
	No.	Amount	No.	Amount	No.	Amount	No.	Amount
Research Grants								
<u>Research Projects</u>								
Noncompeting	749	\$266,259	738	\$263,771	699	\$249,998	-50	-\$16,261
Administrative Supplements	(20)	1,019	(20)	1,019	(20)	1,019	(0)	0
Competing:								
Renewal	48	21,778	74	23,445	85	26,940	37	5,162
New	184	51,553	175	55,500	202	63,774	18	12,221
Supplements	2	827	3	891	3	1,024	1	197
Subtotal, Competing	234	\$74,158	252	\$79,836	290	\$91,738	56	\$17,580
Subtotal, RPGs	983	\$341,436	990	\$344,626	989	\$342,755	6	\$1,319
SBIR/STTR	32	13,261	34	13,935	35	14,674	3	1,413
Research Project Grants	1,015	\$354,697	1,024	\$358,561	1,024	\$357,429	9	\$2,732
<u>Research Centers</u>								
Specialized/Comprehensive	42	41,533	42	41,533	42	41,533	0	0
Clinical Research	0	0	0	0	0	0	0	0
Biotechnology	0	0	0	0	0	0	0	0
Comparative Medicine	0	29	0	30	0	30	0	1
Research Centers in Minority Institutions	0	0	0	0	0	0	0	0
Research Centers	42	\$41,562	42	\$41,563	42	\$41,563	0	\$1
<u>Other Research</u>								
Research Careers	154	19,330	154	19,330	154	19,330	0	0
Cancer Education	0	0	0	0	0	0	0	0
Cooperative Clinical Research	0	0	0	0	0	0	0	0
Biomedical Research Support	0	0	0	0	0	0	0	0
Minority Biomedical Research Support	0	264	0	264	0	264	0	0
Other	25	3,045	25	3,045	25	3,045	0	0
Other Research	179	\$22,639	179	\$22,639	179	\$22,639	0	\$0
Total Research Grants	1,236	\$418,898	1,245	\$422,763	1,245	\$421,631	9	\$2,733
<u>Ruth L. Kirschstein Training Awards</u>	<u>FTEPs</u>		<u>FTEPs</u>		<u>FTEPs</u>		<u>FTEPs</u>	
Individual	59	2,973	58	2,973	59	3,065	0	92
Institutional	237	12,112	233	12,112	237	12,448	0	336
Total Research Training	296	\$15,085	291	\$15,085	296	\$15,513	0	\$428
Research & Development Contracts	48	18,383	51	18,158	51	21,424	3	3,041
<i>SBIR/STTR (non-add)</i>	(0)	(28)	(0)	(28)	(0)	(28)	(0)	(0)
Intramural Research	<u>FTEs</u>		<u>FTEs</u>		<u>FTEs</u>		<u>FTEs</u>	
Research Management and Support	134	54,413	139	54,413	139	54,413	5	0
Construction	105	28,012	114	28,012	114	28,012	9	0
Buildings and Facilities		0		0		0		0
Total, NIAMS	239	\$534,791	253	\$538,431	253	\$540,993	14	\$6,202

<sup>1</sup> All items in italics and brackets are "non-adds."

## **Major Changes in the Fiscal Year 2014 Budget Request**

Major changes by budget mechanism and/or budget activity detail are briefly described below. Note that there may be overlap between budget mechanism and activity detail and these highlights will not sum to the total change for the FY 2014 President's Budget for NIAMS, which is \$6.2 million more than the FY 2012 Actual level, for a total of \$541.0 million.

Research Project Grants (+\$2.732 million; total \$357.429 million): NIAMS will support a total of 1,024 Research Project Grant (RPG) awards in FY 2014. Noncompeting awards will decrease by 50 awards and \$16.3 million. Competing RPGs will increase by 56 awards and \$17.6 million. NIH budget policy for RPGs in FY 2014, continues FY 2012 policy of eliminating inflationary increases for future year commitments. However adjustments for special needs (such as equipment and added personnel) will continue to be accommodated. NIAMS continues to place a priority on support to new investigators.

Research Training (+\$0.428 million; total \$15.513 million): NIAMS will support 296 pre- and postdoctoral trainees in full-time training positions, a flat level from the number funded in FY 2012. Support for NRSA training mechanism will be increased by \$0.4 million amount to cover the cost of increased stipends. The Ruth L. Kirschstein NRSA budget reflects a stipend increase to \$42,000 for the entry level postdoctoral trainees and fellows along with 4 percent increases for each subsequent level of experience. These increases are consistent with stipend increases recommended by the Advisory Committee to the NIH Director and the National Research Council. In addition, this increase is consistent with 42 USC 288(b)(5), which anticipates periodic adjustments in stipends "to reflect increases in the cost of living."

**NATIONAL INSTITUTES OF HEALTH**  
**National Institute of Arthritis and Musculoskeletal and Skin Diseases**  
**Summary of Changes**  
(Dollars in Thousands)

<b>FY 2012 Actual</b>		<b>\$534,791</b>		
<b>FY 2014 President's Budget</b>		<b>\$540,993</b>		
<b>Net change</b>		<b>\$6,202</b>		
<b>CHANGES</b>	<b>2014 President's Budget</b>		<b>Change from FY 2012</b>	
	<b>FTEs</b>	<b>Budget Authority</b>	<b>FTEs</b>	<b>Budget Authority</b>
A. Built-in:				
1. Intramural Research:				
a. Annualization of March				
2013 pay increase & benefits		\$18,870		\$48
b. January FY 2014 pay increase & benefits		18,870		139
c. One more day of pay		18,870		72
d. Differences attributable to change in FTE		18,870		0
e. Payment for centrally furnished services		9,394		169
f. Increased cost of laboratory supplies, materials, other expenses, and non-recurring costs		26,149		75
Subtotal				\$504
2. Research Management and Support:				
a. Annualization of March				
2013 pay increase & benefits		\$15,701		\$42
b. January FY 2014 pay increase & benefits		15,701		116
c. One more day of pay		15,701		59
d. Differences attributable to change in FTE		15,701		0
e. Payment for centrally furnished services		4,024		72
f. Increased cost of laboratory supplies, materials, other expenses, and non-recurring costs		8,287		1
Subtotal				\$290
Subtotal, Built-in				\$794

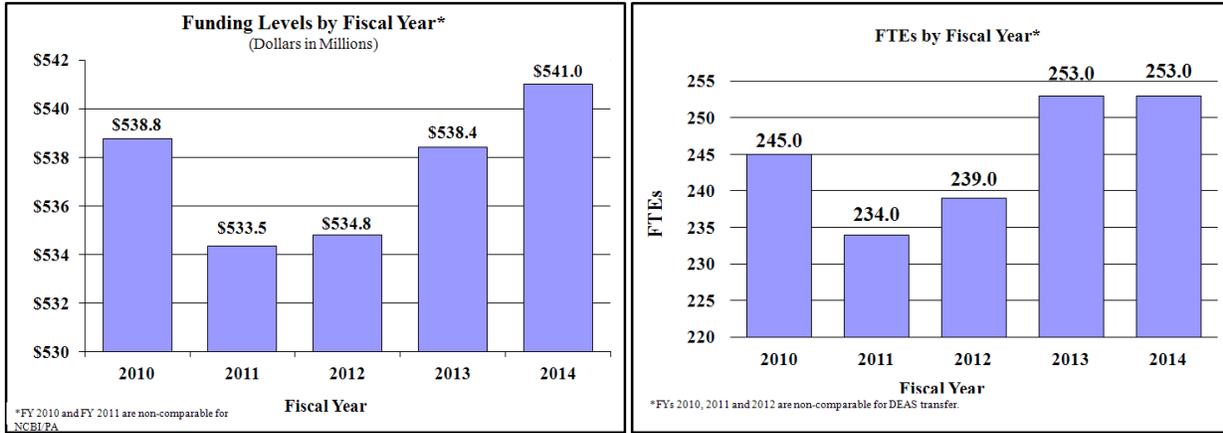
**NATIONAL INSTITUTES OF HEALTH**  
**National Institute of Arthritis and Musculoskeletal and Skin Diseases**

**Summary of Changes--continued**

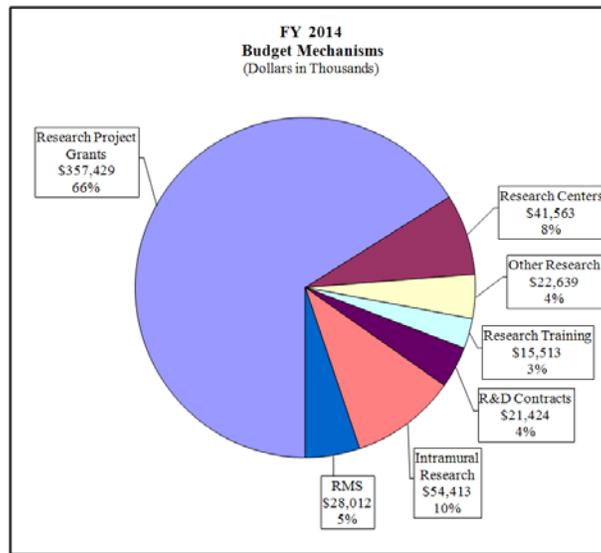
CHANGES	2014 President's Budget		Change from FY 2012	
	No.	Amount	No.	Amount
<b>B. Program:</b>				
1. Research Project Grants:				
a. Noncompeting	699	\$251,017	-50	-\$16,261
b. Competing	290	91,738	56	17,580
c. SBIR/STTR	35	14,674	3	1,413
Total	1,024	\$357,429	9	\$2,732
2. Research Centers	42	\$41,563	0	\$1
3. Other Research	179	22,639	0	0
4. Research Training	296	15,513	0	428
5. Research and development contracts	51	21,424	3	3,041
Subtotal, Extramural		\$458,568		\$6,202
6. Intramural Research	<u>FTEs</u> 139	\$54,413	<u>FTEs</u> 5	-\$504
7. Research Management and Support	114	28,012	9	-290
8. Construction		0		0
9. Buildings and Facilities		0		0
Subtotal, program	253	\$540,993	14	\$5,408
Total changes				\$6,202

## Fiscal Year 2014 Budget Graphs

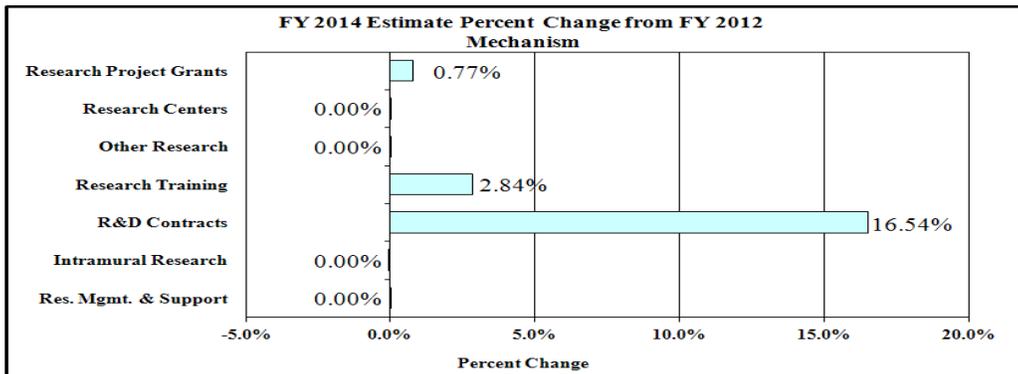
### History of Budget Authority and FTEs:



### Distribution by Mechanism:



### Change by Selected Mechanism:



**NATIONAL INSTITUTES OF HEALTH**  
**National Institute of Arthritis and Musculoskeletal and Skin Diseases**  
**Budget Authority by Activity**<sup>1,2</sup>  
(Dollars in Thousands)

	FY 2012 Actual		FY 2013 CR		FY 2014 PB		Change vs. FY 2012	
	FTEs	Amount	FTEs	Amount	FTEs	Amount	FTEs	Amount
<b>Extramural Research</b>								
<u>Detail:</u>								
Arthritis and Rheumatic Diseases		\$115,296		\$116,225		\$116,879		\$1,583
Skin Biology and Diseases		67,450		67,992		68,373		923
Muscle Biology and Diseases		76,058		76,671		77,103		1,045
Musculoskeletal Biology and Diseases		125,404		126,412		127,121		1,717
Bone Biology and Diseases		68,158		68,706		69,092		934
<b>Subtotal, Extramural</b>		\$452,366		\$456,006		\$458,568		\$6,202
<b>Intramural Research</b>	134	\$54,413	139	\$54,413	139	\$54,413	5	\$0
<b>Research Management &amp; Support</b>	105	\$28,012	114	\$28,012	114	\$28,012	9	\$0
<b>TOTAL</b>	239	\$534,791	253	\$538,431	253	\$540,993	14	\$6,202

1. Includes FTEs whose payroll obligations are supported by the NIH Common Fund.

2. Includes Transfers and Comparable Adjustments as detailed in the "Amounts Available for Obligation" table.

**NATIONAL INSTITUTES OF HEALTH  
National Institute of Arthritis and Musculoskeletal and Skin Diseases**

**Authorizing Legislation**

	<b>PHS Act/ Other Citation</b>	<b>U.S. Code Citation</b>	<b>2013 Amount Authorized</b>	<b>FY 2013 CR</b>	<b>2014 Amount Authorized</b>	<b>FY 2014 PB</b>
Research and Investigation	Section 301	42§241	Indefinite	\$538,431,000	Indefinite	\$540,993,000
National Institute of Arthritis and Musculoskeletal and Skin Diseases	Section 401(a)	42§281	Indefinite		Indefinite	
<b>Total, Budget Authority</b>				<b>\$538,431,000</b>		<b>\$540,993,000</b>

**NATIONAL INSTITUTES OF HEALTH**  
**National Institute of Arthritis and Musculoskeletal and Skin Diseases**

**Appropriations History**

Fiscal Year	Budget Estimate to Congress	House Allowance	Senate Allowance	Appropriation
2005	\$515,378,000	\$515,378,000	\$520,900,000	\$515,378,000
Rescission				(\$4,221,000)
2006	\$513,063,000	\$513,063,000	\$525,758,000	\$513,063,000
Rescission				(\$5,131,000)
2007	\$504,533,000	\$504,533,000	\$508,585,000	\$508,240,000
Rescission				-
2008	\$508,082,000	\$516,044,000	\$519,810,000	\$517,629,000
Rescission				(\$9,043,000)
Supplemental				\$2,705,000
2009	\$509,080,000	\$526,583,000	\$523,246,000	\$524,872,000
Rescission				-
2010	\$530,825,000	\$543,621,000	\$533,831,000	\$539,082,000
Rescission				-
2011	\$555,715,000	-	\$554,846,000	\$539,082,000
Rescission				(\$4,733,461)
2012	\$547,891,000	\$547,891,000	\$528,332,000	\$536,801,000
Rescission				(\$1,014,554)
2013	\$535,610,000	-	\$537,233,000	-
Rescission				-
2014	\$540,993,000	-	-	-

## Justification of Budget Request

### National Institute of Arthritis and Musculoskeletal and Skin Diseases

Authorizing Legislation: Section 301 and title IV of the Public Health Service Act, as amended.

Budget Authority (BA):

	FY 2012 Actual	FY 2013 CR	FY 2014 President's Budget	FY 2014 +/- FY 2012
BA	\$534,791,000	\$538,431,000	\$540,993,000	+\$6,202,000
FTE	239	253	253	+14

Program funds are allocated as follows: Competitive Grants/Cooperative Agreements; Contracts; Direct Federal/Intramural and Other.

### Director's Overview

The National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS) supports a broad range of research, training, and information dissemination activities. Some conditions within the NIAMS mission are rare, affecting only a few thousand people world-wide; others are very common. All have a major influence on the quality of people's lives. Diseases addressed by NIAMS affect individuals of all ages and of all racial and ethnic backgrounds; many disproportionately affect women and minorities. Over the years, NIAMS-funded research teams have made significant progress in uncovering the causes and improving the treatment of many disorders of the bones, muscles, joints, and skin. The activities described below are a subset of Institute efforts that are advancing the NIH Director's priority areas for FY 2014.

Much of the NIAMS budget supports basic research into the biological processes underlying health and disease. Over time, discoveries have led to new treatments for people with a range of debilitating conditions. For example, NIAMS-funded basic research during the 1990s enhanced the understanding of molecular mechanisms of inflammation and immune system dysfunction that causes rheumatoid arthritis. The laboratory results were translated to biologics—drugs in the form of biological molecules that are widely prescribed for millions of patients who have rheumatoid arthritis. Ongoing NIAMS-funded studies are identifying the factors that contribute to differences in symptoms and severity. These findings are helping to match appropriate drugs for the management of individual patients.

Scientists also are focusing on genetic changes that are involved in both common and rare diseases within the NIAMS mission. The American Recovery and Reinvestment Act of 2009

(ARRA) enabled researchers to map genetic variations at millions of sites in the genomes of several thousand men and women who have or are at high risk of osteoporotic fracture or knee osteoarthritis. The data will be a valuable resource that many research groups can use to uncover the genetic underpinnings of musculoskeletal diseases and to identify drug discovery targets.

Already, analysis of the ARRA-funded data sets suggests that defects in the same biologic pathways can cause both osteoporosis and osteoarthritis. This finding is consistent with the increasing recognition that musculoskeletal diseases and disorders such as osteoporosis, osteoarthritis, and muscle atrophy share certain biologic processes. Because all have at least some common pathways, strategies for rebuilding any given tissue are likely to have multiple effects. This is prompting NIAMS to encourage additional basic and preclinical research into the implications that these connections may have for the development and testing of compounds to build, restore, or preserve musculoskeletal tissues or treat bone, joint, or muscle disorders.

The discovery that adult skin or blood cells can be reprogrammed to produce “induced pluripotent stem (iPS) cells,” which then can become any cell type, was recognized by the 2012 Nobel Prize in Physiology or Medicine. Because iPS cell-based interventions can generate patient-specific cells that should not trigger immune rejection when returned to a donor, the advance has dramatic implications for cellular therapies and regenerative medicine. In addition, iPS cells can produce disease- and patient-specific models to study the development of diseases such as muscular dystrophies and blistering skin diseases, and the toxicity and effectiveness of drugs against these and other conditions. Consistent with the translational science opportunities afforded by the NIH’s Center for Regenerative Medicine, NIAMS is exploring strategies to expand the application of iPS cells to studies of diseases in the Institute’s mission areas (see **Program Portrait: Regenerative Medicine in Skin and Musculoskeletal Disorders**, below).

Testing promising cellular, molecular, surgical, or behavioral interventions in clinical trials is an essential step in the translation of discoveries into improvements in health and clinical care. To improve the cost-effectiveness and timeliness of its studies, NIAMS funds most clinical trials through a two-part grant process. Begun in FY 2012, this phased process will continue for grants funded through FY 2014. NIAMS also will continue mechanisms to support arthritis and musculoskeletal and skin disease research that utilizes resources from ongoing clinical projects and preliminary studies on which larger clinical trials could be based. NIAMS encourages investigators to use existing resources, such as the research infrastructures that the Clinical Effectiveness Research Network developed for studies of psoriasis, and the Childhood Arthritis and Rheumatology Research Alliance implemented for pediatric rheumatic diseases. Already, studies using these programs have yielded results on which doctors can base treatment decisions.

Basic science and technologic improvements in imaging allow for assessments of bone quality that were impossible a decade ago. Looking to FY 2014, the Institute is exploring ways to help researchers leverage new imaging tools and approaches for examining bone health. These techniques, many of which were developed or refined with NIAMS funding, allow investigators to probe the relationship between bone’s inner structure and its strength. The ability to visualize how bone changes with disease is guiding the development and use of surrogate markers for fracture and is expected to provide clear evidence of whether and how treatments are working.

Many diseases of interest to NIAMS cause patients to have pain or experience fatigue, impact patients' sleep, alter patients' ability to physically or mentally function on a daily basis, or otherwise interfere with social relationships and overall quality-of-life. Building on the NIH Common Fund initiative called the Patient Reported Outcomes Measurement Information System (PROMIS®), NIAMS is encouraging investigators to integrate patients' self-assessments in chronic diseases representative of the NIAMS portfolio. The goal of these projects, which will run through FY 2016, is to facilitate the availability of improved and more personalized therapeutic options for people who have arthritis or musculoskeletal or skin conditions.

The Institute remains highly committed to supporting the next generation of talented researchers. Recognizing that the transition from a mentored environment to an independent tenure-track faculty position is a critical point in the development of junior investigators' research careers, NIAMS convened a roundtable discussion in May 2012 to address the challenges rheumatologists who are interested in research face during this career phase. NIAMS expanded the effort in December 2012 to include scientists across the full spectrum of research topics within its mission. The Institute hosted a forum for physician-scientists who have NIAMS-funded mentored career development awards, former award recipients who have recently begun independent research careers, and established investigators. Approximately thirty researchers discussed the challenges that junior scientists face when pursuing research independence.

Overall Budget Policy: The FY 2014 request for NIAMS is \$540.993 million or 1.1 percent above the FY 2012 Actual level. Investigator-initiated research project grants and research conducted by new scientists continue to be the Institute's highest priorities. As part of this effort, NIAMS is committed to training and supporting a diverse biomedical research community that will continue to make advances for generations to come. Funds are included in R&D contracts to support trans-NIH initiatives, such as the Basic Behavioral and Social Sciences Opportunity Network (OppNet).

### **Program Descriptions and Accomplishments**

**Arthritis and Rheumatic Diseases:** This program advances high-quality basic, translational, and clinical biomedical and biopsychosocial research to treat, cure, and prevent arthritis and other rheumatic diseases. It supports the application of new insights in the fields of genetics, genomics, proteomics, immunology, and imaging to understand how the immune system interacts with various tissues in normal and pathological conditions, and to ensure a continuous supply of new targets on which therapies can be based. For example, a growing body of evidence points to how microbes contribute to inflammatory diseases. NIAMS-supported studies found that some cases of Lyme arthritis (joint inflammation that can result from Lyme disease) are due to lingering effects of the disease-causing bacteria. Other new NIAMS-funded discoveries about the immune system's reaction to bacterial infections and its regulation of subsequent inflammatory responses may help control diseases such as rheumatoid arthritis and gout. In addition, NIAMS-supported researchers studying pediatric and adult lupus patients have identified abnormalities in a gene that contributes to a microbe-fighting immune system pathway; these genetic changes may cause dysregulated inflammation in a subset of lupus patients.

Budget Policy: The FY 2014 President's Budget estimate is \$116.879 million, an increase of \$1.583 million, or 1.3 percent over the FY 2012 Actual level. Plans for FY 2014 include encouraging late-stage development and validation of molecular indicators, or biomarkers, for monitoring disease progression and therapeutic responses in inflammatory diseases, such as arthritis, lupus, and psoriasis. As part of a Center of Research Translation that NIAMS began funding in FY 2012, the program will continue to support studies into the clinical and societal factors that contribute to an increased prevalence of gout. The program also will build on a January 2013 roundtable discussion about partnerships to advance therapies for rheumatic diseases, which NIAMS convened with representatives from the research community, the U.S. Food and Drug Administration, and industry to discuss strategies for scientific collaborations that will lead to better, safer, and more efficient clinical studies.

**Musculoskeletal Biology and Diseases:** The Musculoskeletal Biology and Diseases program focuses on understanding the fundamental biology of tissues that constitute the musculoskeletal system, and on translating and applying this knowledge to a variety of diseases and conditions including osteoarthritis (OA). It oversees research into the causes and treatment of acute and chronic injuries, including repetitive stress and sports injuries, and is funding a long-term study into the effectiveness of various treatments for patients who have low back pain. The program supports the development of technologies—such as bone and joint imaging, tissue engineering, and regenerative medicine—to improve the diagnosis and treatment of skeletal disorders, or to facilitate repair of damage caused by trauma to otherwise healthy tissue (see **Program Portrait: Regenerative Medicine in Skin and Musculoskeletal Disorders**, below). Its longstanding investment in the Osteoarthritis Initiative, a trans-NIH public-private partnership to facilitate development of treatments for people who have knee OA, is the basis for a new Foundation for the National Institutes of Health Biomarkers Consortium project to evaluate biochemical and imaging biomarkers for more precise ways of measuring OA progression, and providing tools to assess the effectiveness of new treatments.

Budget Policy: The FY 2014 President's Budget estimate is \$127.121 million, an increase of \$1.717 million, or 1.3 percent over the FY 2012 Actual level. NIAMS plans a focused effort to explore the biology of the hard-to-heal tendons and ligaments that comprise the joints that could hold promise for new strategies to help people recover from sports injuries or other traumatic events. NIAMS will continue to support translational research to improve the care patients receive following orthopaedic trauma; this includes strategies that clinicians could use to assess a person's risk of developing OA, as well as efforts to forestall or prevent OA onset following a joint injury.

**Bone Biology and Diseases:** The Bone Biology and Diseases program covers a broad spectrum of research designed to better understand genetic and cellular mechanisms involved in the build-up and break down of bone. It studies regulation of bone remodeling; bone formation, bone resorption, and mineralization; and effects of hormones, growth factors, and cytokines on bone cells. The program supports several large epidemiologic cohorts for characterization of the natural history of osteoporosis, and for identification of genetic and environmental risk factors that contribute to bone disease. Over the past decades, these studies have provided information that health care providers are using to assess people's bone health. The NIH Study of

Osteoporotic Fractures (SOF) finding that bone mineral density (BMD) relates closely to fracture risk, for example, contributed to Medicare's decision to pay for numerous people to get their BMD measured every two years. Many started taking bone-preserving drugs because of their results, and the rate of hip fractures dropped nearly 25 percent among female beneficiaries.<sup>1</sup> New, longer-term data from SOF is refining the screening guidance: women at the highest risk of osteoporosis might benefit from annual exams, while women with the lowest risk could be tested much less frequently unless other aspects of their health change.

**Budget Policy:** The FY 2014 President's Budget estimate is \$69.092 million, an increase of \$0.934 million, or 1.3 percent over the FY 2012 Actual level. NIAMS plans for this program include encouraging basic and translational research to inform the development of bone-building and joint-preserving therapies for people who have brittle bones or deteriorating joints. Capitalizing on recent discoveries related to how bone and muscle communicate with each other and with other organ systems in processes as diverse as fracture healing and fat storage, the program will encourage bone researchers to collaborate with muscle experts to develop and test new hypotheses related to human health and disease.

**Muscle Biology and Diseases:** This program supports a wide range of basic, translational, and clinical research projects in skeletal muscle biology and diseases. It focuses on the fundamental biology of muscle development, physiology, and muscle imaging. Its overarching objective is to advance the understanding of the role that muscle plays in musculoskeletal and whole body health and, ultimately, to treat or prevent skeletal muscle diseases and disorders (including muscular dystrophies, inflammatory myopathies, muscle ion channel diseases, disuse atrophy, skeletal muscle injury and loss of muscle mass associated with aging and diseases). The development of tools that allow researchers to study genetic material that does not normally code for proteins in adult muscle led to several basic science discoveries in FY 2012 that will pave the way for tomorrow's breakthroughs. For example, a new strategy for degradation of toxic RNA may lead to novel drugs for myotonic dystrophy. Two other recent discoveries—that small RNA fragments called microRNAs can regulate the behavior of dormant muscle stem cells, and that subtle differences in muscle RNA influence whether genes produce proteins—may one day enable therapies for muscle diseases and disorders where repair and regeneration of muscle are inadequate. Additional information about NIAMS-funded progress and plans regarding muscular dystrophy is in the **Program Portrait: Innovative Approaches to Muscular Dystrophy Treatments**, below.

**Budget Policy:** The FY 2014 President's Budget estimate is \$77.103 million, an increase of \$1.045 million, or 1.3 percent over the FY 2012 Actual level. For FY 2014, the Institute will continue to participate in the Senator Paul D. Wellstone Muscular Dystrophy Cooperative Research Centers Program, and to support a Center of Research Translation grant to assess a strategy to repair the genetic defect that causes Duchenne muscular dystrophy. Other plans include continuing to foster research into the role skeletal muscle plays in influencing other organ systems in addition to skeletal health (as described above), and to support clinician scientists who are committed to a muscular dystrophy research career.

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<sup>1</sup> Brauer CA, et al. JAMA. 2009. PMID: 19826027

### **Program Portrait: Innovative Approaches to Muscular Dystrophy Treatments**

**FY 2012 Level:** \$30.1 million

**FY 2014 Level:** \$30.4 million

**Change:** +\$0.3 million

Over the past decade, investments in basic and translational research have uncovered numerous candidate therapies for muscular dystrophies. The diversity of the options developed by NIAMS-funded investigators is as varied as the diseases themselves. A strategy to replace the defective gene that causes Duchenne muscular dystrophy has shown promise in dogs, while a different approach to get cells to produce a protein involved in muscle repair improves heart function in a hamster model of limb-girdle muscular dystrophy. NIAMS-funded researchers are studying small molecules that can skip over the mutated DNA fragment to allow the defective gene to make a shorter, but functional, version of the protein that is missing in people who have Duchenne muscular dystrophy; others are collecting data on similar molecules that allow muscle proteins to assemble normally in a mouse model of myotonic dystrophy.

A major component of the NIAMS muscular dystrophy research portfolio includes funding for two of the six NIH-supported Senator Paul D. Wellstone Muscular Dystrophy Cooperative Research Centers (MDCRCs). Each center brings together expertise, infrastructure, and resources focused on major questions about muscular dystrophy. NIH responded to the burgeoning number of basic research findings in muscular dystrophy by changing the focus of the FY 2008 and FY 2010 Wellstone MDCRC competitions to encourage research that translates basic findings about the disease to human studies and applications in the clinic. To further capitalize on basic and translational research investments and accelerate progress toward effective treatments or other improvements in patients' lives, new or renewing centers funded in FY 2013 and FY 2015 will be expected to include clinical research with direct interactions between researchers and muscular dystrophy patients. In addition to therapy development, NIH will also encourage studies of the natural history of diseases, biomarker identification and validation, biopsychosocial studies, and other patient-oriented research.

**Skin Biology and Diseases:** This program supports a broad portfolio of basic, translational, and clinical research in skin, including work on the developmental and molecular biology of skin, the study of skin as an immune organ, and the genetics of skin diseases. The Institute is pursuing tissue engineering and regenerative medicine strategies involving skin tissue, and imaging tools that will facilitate studies of skin biology and diseases (see **Program Portrait: Regenerative Medicine in Skin and Musculoskeletal Disorders**, below). Although skin provides important protection from outside elements, it can also be a barrier to delivery of therapeutic agents. NIAMS-supported researchers have created a potential approach for gene therapy of skin diseases, such as psoriasis and skin cancer, by combining nanoscale gold particles and genetic material. These complexes can penetrate the skin after topical application, and change the expression of disease-associated genes. Atopic dermatitis, or eczema, is a chronic, inflammatory skin condition, resulting from disruption of the skin barrier function. Due to severe itching, atopic dermatitis has a tremendous impact on quality of life and was a major topic at the NIAMS 2012 scientific planning retreat. Institute staff and extramural experts discussed emerging targets, future research directions, and opportunities for intervention. Keratinocytes, the cells in the outer layer of skin, proliferate in a regulated fashion in skin wound healing, and in an unregulated manner in psoriasis. Recent NIAMS-funded research identified a molecule that controls keratinocyte growth and may be a therapeutic target for both wound healing and psoriasis. Work also continues on rare genetic diseases of the skin, such as epidermolysis bullosa and pachyonychia congenita.

### **Program Portrait: Regenerative Medicine in Skin and Musculoskeletal Disorders**

**FY 2012 Level:** \$62.7 million

**FY 2014 Level:** \$63.4 million

**Change:** +\$0.7 million

Loss of tissue or organ function due to disease, injury, or aging leads to significant human suffering. Tissue engineering and regenerative medicine approaches to rebuild or repair tissue are based on a combination of cells, scaffolds, and biomolecules such as proteins. Stem cells are specialized populations of cells that, through activation and division, provide the cells needed for tissue renewal. They come from many sources and are being considered as treatments for many conditions. Because hair follicles are a reliable, easily accessible source of stem cells, they have been widely used to study how stem cells divide and form other tissues. A new, innovative, non-invasive imaging technology allows visualization of entire, regenerating hair follicles in live mice, which could have tremendous impact in understanding of tissue regeneration and, potentially, other dynamic processes in skin.

As noted in the Director's Overview, adult skin cells can be reprogrammed to become iPS cells, which can be converted into all other cell types. NIAMS plans for FY 2014 include leveraging resources available through the NIH's Center for Regenerative Medicine to develop protocols that researchers can use to generate cells and to create cell-based models of diseases and disorders within the NIAMS mission for use when testing potential drugs.

NIAMS also will continue to support regenerative medicine approaches for other tissues, such as muscle, bone, and the tendons and ligaments that connect them. Tendons and ligaments are complex tissues that are highly prone to injury, especially during athletic activity. Tendon grafts are the standard treatment for anterior cruciate ligament injuries in the knee. Recent NIAMS-funded research has shown that attaching a "bioenhanced" tendon graft, consisting of a collagen scaffold enriched with platelet plasma, led to significant joint improvements in comparison with untreated injuries. Repair of torn patellar tendons in the knee, either through natural healing or surgery, is rarely sufficient to return the tissue to full strength. NIAMS-supported researchers are identifying the molecular and cellular components in regenerated patellar tendons to improve the outcomes of tissue repair.

**Budget Policy:** The FY 2014 President's Budget estimate is \$68.373 million, an increase of \$0.923 million, or 1.3 percent over the FY 2012 Actual level. There have been significant gains in understanding itch sensation in recent years, which is a major quality of life issue in many skin conditions. NIAMS is encouraging research that will lead to more discoveries in the mechanisms of itch sensation, and developing interventions to modulate and control itch in disease. The Institute will continue to support shared facilities and services for skin research that will enhance and coordinate activities, and foster new innovations.

**Intramural Research Program:** The mission of the Intramural Research Program (IRP) is to conduct innovative basic, translational, and clinical research relevant to the health concerns of the Institute, and to provide training for investigators who are interested in related research careers (see **Program Portrait: Rare Autoinflammatory Diseases**, below, for examples of NIAMS intramural research projects). The program conducts intensive basic scientific and clinical studies on the genetics, etiology, pathogenesis, and treatment of a variety of rheumatic, autoimmune, inflammatory, joint, skin, and muscle diseases. The NIAMS IRP consists of basic researchers and physician-scientists who have contributed many noteworthy discoveries. For example, scientists studying atopic dermatitis showed that mice that could not process a byproduct of vitamin A had skin defects which rendered them more susceptible to infectious organisms and allergens. This better understanding of how people develop atopic dermatitis may lead to new treatments for several skin conditions. Other intramural researchers are working with industry partners to translate basic research findings into clinical treatments. New breakthroughs have been made in the development of small molecules that block multiple key

inflammatory pathways that underlie many autoimmune diseases. NIAMS scientists have also resolved longstanding questions about the origin of recurrent chromosomal rearrangements that drive human lymphomas and leukemias. In addition, researchers identified an enzyme that plays a key role in promoting such rearrangements, which could be a potential therapeutic target for the prevention of certain human cancers.

**Budget Policy:** The FY 2014 President's Budget estimate is \$54.413 million, the same as the FY 2012 Actual level. NIAMS plans for FY 2014 include maintaining its focus on translational research, in order to facilitate patient-oriented studies in the areas of arthritis, musculoskeletal, and skin diseases, including their genetic, inflammatory, and immune underpinnings. NIAMS will also continue its commitment to multidisciplinary training of rheumatology research fellows to strengthen the pipeline of highly qualified physician-scientists in this field. Both efforts will continue to synergize with the NIAMS Community Health Center facility in Silver Spring, Maryland, and will be enhanced by the recent appointment of a new clinical director to oversee clinical and translational intramural research. NIAMS is also participating in the NIH Funding Opportunity Announcement that is designed to promote collaborations between intramural and extramural investigators and take advantage of the unique research opportunities available at the NIH Clinical Center.

**Program Portrait: Rare Autoinflammatory Diseases**

**FY 2012 Level:** \$1.9 million

**FY 2014 Level:** \$1.9 million

**Change:** \$0.0 million

The NIAMS Intramural Research Program (IRP) continues to support a wide range of basic, translational, and clinical research activities. Leveraging the unique opportunities at the NIH Clinical Center, intramural scientists have sought to identify and treat rare autoinflammatory diseases that often severely impact pediatric patients. As part of an international team, NIAMS researchers helped identify a genetic mutation that causes a rare childhood disease characterized predominantly by inflammation and fat loss. The research suggests that the disorder, named chronic atypical neutrophilic dermatosis with lipodystrophy and elevated temperature (CANDLE), represents a spectrum of diseases that have been described in the literature under a variety of names. More importantly, since no effective treatment for this disease currently exists, the findings may have uncovered a possible target for future therapies.

Additionally, intramural investigators showed that the medication etanercept reduces the frequency and severity of symptoms of TNF receptor-associated periodic syndrome (TRAPS), a rare inherited condition characterized by recurrent fevers, abdominal pain and skin rashes. Etanercept belongs to a class of drugs that block tumor necrosis factor, a protein implicated in inflammation in TRAPS and in rheumatic diseases such as rheumatoid arthritis. While it has been prescribed to TRAPS patients for a decade, this is the first formal study of its long-term effectiveness.

IRP researchers also showed that another rheumatoid arthritis medication, anakinra, stopped the progression of organ damage in people with neonatal-onset multisystem inflammatory disease (NOMID). This rare and debilitating genetic disorder causes persistent inflammation and tissue damage. Anakinra, one of a relatively new class of drugs known as biologic response modifiers or biologics, blocks the activity of interleukin-1 (IL-1), a protein made by cells of the immune system. Previous work by the same NIAMS group showed that blocking IL-1 relieved symptoms of NOMID. However, this is the first study to show that anakinra works over the long-term and, at higher doses, can prevent vision and hearing loss and brain lesions.

**Research Management and Support (RMS):** NIAMS' RMS budget supports the scientific, administrative management, and information technology activities associated with day-to-day operations. In FY 2012, the Institute managed more than 1,236 research grants and centers, as well as 48 research and development contracts and 296 individual and institutional full-time research training positions. NIAMS supported 496 clinical research studies, including 72 clinical trials. In FY 2012, the NIAMS continued to expand its information dissemination efforts. For example, an English and Spanish fotonovela was created to teach children and teens how to avoid sports injuries. "Ana's Story" uses a graphic format to engage readers and deliver important health messages. Social media efforts, such as daily updates on Twitter and Facebook, have enhanced public engagement and awareness of NIAMS and NIH information resources by increasing traffic to the NIAMS website. The NIAMS also hired a new Chief Information Officer to manage the information technology resources of the Institute.

Budget Policy: The 2014 President's Budget estimate is \$28.012 million, the same as the FY 2012 Actual level. In FY 2014, NIAMS will continue its efforts to improve access to, and availability of, meaningful health information for racial and ethnic minority populations through its National Multicultural Outreach Initiative (NMOI). Following the pilot testing of two health planners—targeting Hispanics/Latinos and American Indians, Alaska Natives, and Native Hawaiians—that raise awareness about the NIH and the NIAMS, two additional health planners for African Americans and Asian Americans/Pacific Islanders were created for the 2013 calendar year. All four planners are available free of charge to the public and can be used in conjunction with a newly developed online toolkit. Outreach to intermediary organizations will facilitate the distribution of health planners and the electronic toolkit in multicultural communities; the Institute will assess the effectiveness of the outreach and distribution efforts later in the year. The Institute also will continue to sponsor roundtable discussions and a scientific retreat with extramural investigators and lay representatives to inform the research priority-setting and strategic planning process. The apparent increase in estimated FY 2014 FTE compared to the FY 2012 actual FTE usage level is due to the effect of transferring positions previously funded from a centralized support operation (Division of Extramural Activities Support) to individual ICs as of year-end 2012. As a result of the DEAS transfer, estimated salaries and benefits for FY 2014 are proportionately higher than those identified for FY 2012 and previous years.

**NATIONAL INSTITUTES OF HEALTH**  
**National Institute of Arthritis and Musculoskeletal and Skin Diseases**

**Budget Authority by Object Class**  
(Dollars in Thousands)

	FY 2012 Actual	FY 2014 PB	Increase or Decrease
Total compensable workyears:			
Full-time employment	239	253	14
Full-time equivalent of overtime and holiday hours	0	0	0
Average ES salary (in whole dollars)	\$179,700	\$179,700	\$0
Average GM/GS grade	11.8	11.6	(0.2)
Average GM/GS salary (in whole dollars)	\$94,292	\$92,668	(\$1,624)
Average salary, grade established by act of July 1, 1944 (42 U.S.C. 207) (in whole dollars)	\$88,992	\$85,542	(\$3,450)
Average salary of ungraded positions (in whole dollars)	\$115,209	\$116,508	\$1,299
<b>OBJECT CLASSES</b>	<b>FY 2012 Actual</b>	<b>FY 2014 PB</b>	<b>Increase or Decrease</b>
Personnel Compensation:			
11.1 Full-time permanent	\$14,303	\$15,354	\$1,051
11.3 Other than full-time permanent	8,276	8,783	507
11.5 Other personnel compensation	428	460	32
11.7 Military personnel	326	349	23
11.8 Special personnel services payments	2,092	2,209	117
<b>Total, Personnel Compensation</b>	<b>\$25,425</b>	<b>\$27,155</b>	<b>\$1,730</b>
12.0 Personnel benefits	\$6,661	\$7,115	\$454
12.2 Military personnel benefits	280	301	21
13.0 Benefits for former personnel	0	0	0
<b>Subtotal, Pay Costs</b>	<b>\$32,366</b>	<b>\$34,571</b>	<b>\$2,205</b>
21.0 Travel and transportation of persons	\$646	\$646	(\$0)
22.0 Transportation of things	160	160	(0)
23.1 Rental payments to GSA	4	4	0
23.2 Rental payments to others	0	0	0
23.3 Communications, utilities and miscellaneous charges	551	551	(0)
24.0 Printing and reproduction	10	10	0
25.1 Consulting services	1,522	1,537	15
25.2 Other services	2,780	1,135	(1,645)
25.3 Purchase of goods and services from government accounts	46,154	49,343	3,189
25.4 Operation and maintenance of facilities	72	72	(0)
25.5 Research and development contracts	8,616	9,743	1,127
25.6 Medical care	4,877	4,838	(39)
25.7 Operation and maintenance of equipment	997	939	(58)
25.8 Subsistence and support of persons	0	0	0
<b>25.0 Subtotal, Other Contractual Services</b>	<b>\$65,018</b>	<b>\$67,607</b>	<b>\$2,589</b>
26.0 Supplies and materials	\$4,321	\$4,010	(\$311)
31.0 Equipment	4,716	4,273	(443)
32.0 Land and structures	17	17	0
33.0 Investments and loans	0	0	0
41.0 Grants, subsidies and contributions	426,983	429,144	2,161
42.0 Insurance claims and indemnities	0	0	0
43.0 Interest and dividends	0	0	(0)
44.0 Refunds	0	0	0
<b>Subtotal, Non-Pay Costs</b>	<b>\$502,426</b>	<b>\$506,422</b>	<b>\$3,996</b>
<b>Total Budget Authority by Object Class</b>	<b>\$534,791</b>	<b>\$540,993</b>	<b>\$6,202</b>

Includes FTEs whose payroll obligations are supported by the NIH Common Fund.

**NATIONAL INSTITUTES OF HEALTH**  
**National Institute of Arthritis and Musculoskeletal and Skin Diseases**

**Salaries and Expenses**  
(Dollars in Thousands)

OBJECT CLASSES	FY 2012 Actual	FY 2014 PB	Increase or Decrease
<b>Personnel Compensation:</b>			
Full-time permanent (11.1)	\$14,303	\$15,354	\$1,051
Other than full-time permanent (11.3)	8,276	8,783	507
Other personnel compensation (11.5)	428	460	32
Military personnel (11.7)	326	349	23
Special personnel services payments (11.8)	2,092	2,209	117
<b>Total Personnel Compensation (11.9)</b>	<b>\$25,425</b>	<b>\$27,155</b>	<b>\$1,730</b>
Civilian personnel benefits (12.1)	\$6,661	\$7,115	\$454
Military personnel benefits (12.2)	280	301	21
Benefits to former personnel (13.0)	0	0	0
<b>Subtotal, Pay Costs</b>	<b>\$32,366</b>	<b>\$34,571</b>	<b>\$2,205</b>
Travel (21.0)	\$646	\$646	\$0
Transportation of things (22.0)	160	160	0
Rental payments to others (23.2)	0	0	0
Communications, utilities and miscellaneous charges (23.3)	551	551	0
Printing and reproduction (24.0)	10	10	0
<b>Other Contractual Services:</b>			
Advisory and assistance services (25.1)	1,522	1,537	15
Other services (25.2)	2,780	1,135	(1,645)
Purchases from government accounts (25.3)	31,261	31,336	75
Operation and maintenance of facilities (25.4)	72	72	0
Operation and maintenance of equipment (25.7)	997	939	(58)
Subsistence and support of persons (25.8)	0	0	0
<b>Subtotal Other Contractual Services</b>	<b>\$36,632</b>	<b>\$35,019</b>	<b>(\$1,613)</b>
Supplies and materials (26.0)	\$4,212	\$3,901	(\$311)
<b>Subtotal, Non-Pay Costs</b>	<b>\$42,211</b>	<b>\$40,287</b>	<b>(\$1,924)</b>
<b>Total, Administrative Costs</b>	<b>\$74,577</b>	<b>\$74,858</b>	<b>\$281</b>

**NATIONAL INSTITUTES OF HEALTH**  
National Institute of Arthritis and Musculoskeletal and Skin Diseases

**Details of Full-Time Equivalent Employment (FTEs)**

OFFICE/DIVISION	FY 2012 Actual			FY 2013 CR			FY 2014 PB		
	Civilian	Military	Total	Civilian	Military	Total	Civilian	Military	Total
Office of the Director									
Direct:	59	-	59	59	-	59	59	-	59
Reimbursable	-	-	-	-	-	-	-	-	-
Total:	59	-	59	59	-	59	59	-	59
Office of Extramural Activities									
Direct:	44	2	46	53	2	55	53	2	55
Reimbursable	-	-	-	-	-	-	-	-	-
Total:	44	2	46	53	2	55	53	2	55
Intramural Research Program									
Direct:	126	2	128	130	3	133	130	3	133
Reimbursable	6	-	6	6	-	6	6	-	6
Total:	132	2	134	136	3	139	136	3	139
<b>Total</b>	<b>235</b>	<b>4</b>	<b>239</b>	<b>248</b>	<b>5</b>	<b>253</b>	<b>248</b>	<b>5</b>	<b>253</b>
Includes FTEs whose payroll obligations are supported by the NIH Common Fund. FTEs supported by funds from Cooperative Research and Development Agreements.	2	0	2	2	0	2	2	0	2
<b>FISCAL YEAR</b>	<b>Average GS Grade</b>								
2010	11.6								
2011	11.6								
2012	11.8								
2013	11.6								
2014	11.6								

**NATIONAL INSTITUTES OF HEALTH**  
**National Institute of Arthritis and Musculoskeletal and Skin Diseases**

**Detail of Positions**

GRADE	FY 2012 Actual	FY 2013 CR	FY 2014 PB
Total, ES Positions	1	1	1
Total, ES Salary	\$ 179,700	\$ 179,700	\$ 179,700
GM/GS-15	17	17	17
GM/GS-14	32	32	32
GM/GS-13	41	41	41
GS-12	26	27	27
GS-11	8	10	10
GS-10	0	0	0
GS-9	12	12	12
GS-8	12	14	14
GS-7	8	10	10
GS-6	5	5	5
GS-5	0	4	4
GS-4	3	3	3
GS-3	0	0	0
GS-2	0	0	0
GS-1	0	0	0
Subtotal	164	175	175
Grades established by Act of July 1, 1944 (42 U.S.C. 207):			
Assistant Surgeon General	1	1	1
Director Grade	1	1	1
Senior Grade	2	2	2
Full Grade	0	1	1
Senior Assistant Grade	0	0	0
Assistant Grade	0	0	0
Subtotal	4	5	5
Ungraded	96	98	98
Total permanent positions	165	176	176
Total positions, end of year	265	279	279
Total full-time equiv (FTE) at YE	239	253	253
Average ES salary	\$ 179,700	\$ 179,700	\$ 179,700
Average GM/GS grade	11.8	11.6	11.6
Average GM/GS salary	\$ 94,292	\$ 91,864	\$ 92,668

Includes FTEs whose payroll obligations are supported by the NIH Common Fund.