NCI Director’s Update

Joint NCAB-BSA Meeting

Norman E. Sharpless, M.D.
December 4, 2018
NCI Appropriations
FY 2015 - 2019 (in millions)

Source: NCI Office of Budget and Finance

21st Century Cures Act funding shown in orange.
FY 2018 RPG FUNDING

Largest increase since FY 2003

FY 2018 ESIs

NCI exceeded its goal of funding 25% more Early-Stage Investigators

FY 2018 NEW RPGs

Total number of awards up from FY 2017
FY19 Realities: Increasing Costs

- Rent and utilities
- Mandatory assessments and transfers
- NRSA stipend increases
- Increasing award sizes
- Non-competing commitments to RPGs
### Competing R01 Applications

<table>
<thead>
<tr>
<th>Fiscal Year</th>
<th>2013</th>
<th>2014</th>
<th>2015</th>
<th>2016</th>
<th>2017</th>
<th>2018</th>
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</thead>
<tbody>
<tr>
<td>NCI</td>
<td>4,175</td>
<td>4,240</td>
<td>4,640</td>
<td>5,019</td>
<td>5,572</td>
<td>6,113</td>
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<tr>
<td>NIH Total</td>
<td>27,939</td>
<td>27,399</td>
<td>28,873</td>
<td>29,968</td>
<td>30,516</td>
<td>30,874</td>
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Guiding Principles for FY19

1. Preserve the RPG Pool
2. Stay true to the Moonshot Vision
3. Continue to Prioritize ESIs
As director of the National Cancer Institute (NCI), I am pleased to share our Annual Plan and Budget Proposal for Fiscal Year 2020. Having been sworn in to my position a little less than a year ago, this marks my first opportunity to present, in this form, the promising results of our country’s investments in biomedical research. This plan directs attention to areas where additional support has unique potential to improve cancer prevention, detection, and treatment.

To place the plan’s focus squarely on those most likely to benefit from NCI research, we have included stories of patients. While each story is unique, they are not that different from that of Mike, a patient I treated for acute leukemia.

Mike started feeling poorly in 2016, and a bone marrow biopsy revealed acute myeloid leukemia (AML). I began his initial treatment with aggressive chemotherapy, which caused difficult side effects and required him to spend more than a month in the hospital. After further therapy, Mike fully recovered, and he has been in remission for more than 2 years.

Mike and I talk about cancer research. In addition, NCI has benefitted from concerted, sustained, and bipartisan support from Congress.
Researchers use immune-cell ‘army’ to battle another tough cancer

By Laurie McGinley June 4 Email the author

WHAT IS IMMUNOTHERAPY?: WOMAN WITH TERMINAL BREAST CANCER SAVED BY PIONEERING TREATMENT

BY KASHMIRA GANDER ON 8/8/18 AT 7:03 AM

NIH grantee wins 2018 Nobel Prize in Physiology or Medicine

The 2018 Nobel Prize in Physiology or Medicine was awarded to National Institutes of Health grantee James Allison, Ph.D., of the University of Texas MD Anderson Cancer Center, Houston, Texas, and Tasuku Honjo, M.D., Ph.D., of the Kyoto University, Japan, for their discovery of immune checkpoint inhibition of negative immune regulation.

The Royal Swedish Academy of Sciences said the discovery, which involves stimulating the inherent ability of the body to attack tumor cells, has established an entirely new principle for the treatment of cancer.
Moxetumomab Approved by FDA for Hairy Cell Leukemia

Subscribe

September 14, 2018, by NCI Staff

The Food and Drug Administration (FDA) has approved moxetumomab pasudotox (Lumoxiti), a bacterial toxin-based drug, for the treatment of some patients with hairy cell leukemia (HCL). The approval covers the use of moxetumomab in patients with HCL who have already undergone at least two lines of standard treatments.

The action by FDA makes moxetumomab the first treatment approved for this group of patients. The approval was based on the findings from an 80-patient clinical trial sponsored by the drug’s manufacturer, MedImmune.

In the trial, approximately 30% of patients had a complete disappearance of their cancer (complete response) that lasted for a long period, and side effects from the therapy were few and mostly minor. Overall, 75% of patients in the trial had either a partial response or complete response.

Moxetumomab was originally discovered by Ira Pastan, M.D., and colleagues in NCI’s Center for Cancer Research (CCR), and later licensed to MedImmune/AstraZeneca for clinical development.
Effect of Aspirin on All-Cause Mortality in the Healthy Elderly


A Death Related to Cancer

Hazard ratio, 1.31 (95% CI, 1.10–1.56)

Cumulative Incidence (%)

<table>
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<tr>
<th>Years since Randomization</th>
<th>Aspirin</th>
<th>Placebo</th>
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<tr>
<td>0</td>
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<td>0</td>
</tr>
<tr>
<td>1</td>
<td>0.5</td>
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<td>6</td>
<td>5.1</td>
<td>4.1</td>
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No. at Risk

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<tr>
<th>Group</th>
<th>No. at Risk</th>
<th>No. at Risk</th>
<th>No. at Risk</th>
<th>No. at Risk</th>
<th>No. at Risk</th>
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<tbody>
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<td>Aspirin</td>
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<td>9408</td>
<td>8286</td>
<td>6291</td>
<td>4016</td>
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<tr>
<td>Placebo</td>
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<td>9545</td>
<td>9466</td>
<td>8369</td>
<td>6367</td>
<td>4077</td>
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FDA approves larotrectinib for solid tumors with NTRK gene fusions

On November 26, 2018, the Food and Drug Administration granted accelerated approval to larotrectinib (VITRAKVI, Loxo Oncology Inc. and Bayer) for adult and pediatric patients with solid tumors that have a neurotrophic receptor tyrosine kinase (NTRK) gene fusion without a known acquired resistance mutation, that are either metastatic or where surgical resection is likely to result in severe morbidity, and who have no satisfactory alternative treatments or whose cancer has progressed following treatment.

This is the second tissue-agnostic FDA approval for the treatment of cancer.
6 Ibrutinib Alone or in Combination with Rituximab Produces Superior Progression Free Survival (PFS) Compared with Bendamustine Plus Rituximab in Untreated Older Patients with Chronic Lymphocytic Leukemia (CLL): Results of Alliance North American Intergroup Study A041202

Program: General Sessions
Session: Plenary Scientific Session
Hematology Disease Topics & Pathways:
Diseases, Leukemia, Biological, CLL, Therapies, Elderly, Study relevant, TKI

Sunday, December 2, 2018, 2:00 PM-4:00 PM
Hall AB (San Diego Convention Center)

Jennifer A. Woyach, MD1, Amy S. Ruppert, MAS, PhD2, Nylander M Booth1*, Wei Ding, MD, PhD5, Nancy L. Bartlett, MD6, Daniel C. Rogers, MD10, Sameer A. Parikh, MD11, Steven Coutre, MD12*, Nattam, MD15, Richard A. Larson, MD16, Harry P. Erba, MD, PhD, FRCPC18, Jim Atkins19*, Jeremy S. Abramson, MD, MMSc20, Richard M. Stone, MD23, Sumithra J Mandrekar, PhD4* and John K. Motz, MBBS2

LBA-4 A Randomized Phase III Study of Ibrutinib (PCI-32765)-Based Therapy Vs. Standard Fludarabine, Cyclophosphamide, and Rituximab (FCR) Chemoimmunotherapy in Untreated Younger Patients with Chronic Lymphocytic Leukemia (CLL): A Trial of the ECOG-ACRIN Cancer Research Group (E1912)

Program: General Sessions
Session: Late-Breaking Abstracts Session
Hematology Disease Topics & Pathways:
Surviviorship, Leukemia, Diseases, Therapies, CLL, Combinations, Clinically relevant, Lymphoid Malignancies, Quality Improvement

Tuesday, December 4, 2018, 7:30 AM-9:15 AM
Hall AB (San Diego Convention Center)

Tait D. Shanafelt, MD1, Victoria Wang2*, Neil E. Kay, MD3, Curtis A. Hanson, MD4, Susan M. O'Brien, MD5, Jacqueline C Barrientos, MD6, Harry P. Erba, MD, PhD7, Richard M. Stone, MD8, Mark R. Litzow, MD5 and Martin S. Tallman, MD9

1Stanford University, Stanford, CA
2Harvard, Boston, MA
3Division of Hematology, Mayo Clinic, Rochester, MN
4Department of Laboratory Medicine and Pathology, Mayo Clinic, Rochester, MN
5UCI Cancer Center, Orange, CA
6Department of Medicine, Hofstra North Shore – LIJ School of Medicine, Great Neck, NY
7University of Alabama at Birmingham Comprehensive Cancer Center, Birmingham, AL
8Dana-Farber Cancer Institute, Boston, MA
9Leukemia Service, Department of Medicine, Memorial Sloan-Kettering Cancer Center, New York, NY
Pediatric and AYA Cancer Research

Recent Advances and Ongoing Studies
- Accelerated Approval for Larotrectinib
- Phase II Trial Results: Selumetinib for NF and Plexiform Neurofibromas
- NCI-COG Pediatric MATCH
- NCI POB Phase 1 Trial: CD19/CD22 CAR T

New Networks and Projects
- Pediatric Immunotherapy Discovery and Development Network
- Fusion Oncoproteins in Childhood Cancers Consortium
- Pediatric Cancer Control Research Across the Lifespan

Relevant Legislation and Implementation
- Childhood Cancer STAR Act
- RACE for Children Act/FDA Reauthorization Act of 2017
Cancer Moonshot Funding Authorized Under the 21st Century Cures Act (dollars in millions)
CTAC Ad Hoc Working groups

Glioblastoma
Co-Chairs:
Walter J. Curran, Jr. M.D., F.A.C.R.
Chi V. Dang, M.D., Ph.D.

Radiation Oncology
• Roster in development
• Kickoff meeting ~Spring 2019
Leadership transitions

- Director, Center for Global Health (CGH)
- Director, Center for Bioinformatics and Information Technology (CBIIT)
- Director, Cancer Therapy Evaluation Program (CTEP)
- Director, Division of Cancer Prevention (DCP)
- Associate Director, Frederick
Key Focus Areas

WORKFORCE DEVELOPMENT
Support the cancer research enterprise by focusing on the workforce of cancer investigators

BASIC SCIENCE
Reaffirm our commitment to basic science to drive novel approaches and technologies

BIG DATA
Increase data aggregation and interpretation to speed our work across the cancer enterprise

CLINICAL TRIALS
Fully realize the power of clinical trials through innovative design, administration, and analyses