

NCI Director's Report

Ned Sharpless, M.D.
Director

*Joint Meeting of the National Cancer
Advisory Board & Board of Scientific Advisors
December 3, 2019*

@TheNCI
@NCIDirector



What I did on my summer vacation

By Ned Sharpless





**U.S. Department of Health and Human Services
Food and Drug Administration**



FDA Therapeutic Oncology Approvals

April thru October 2019

New (7)

- **Entectinib** for ROS1 NSCLC, NTRK+ tissue agnostic
- **Pexidartinib** for symptomatic tenosynovial giant cell tumor
- **Alpelisib** with fulvestrant for PIK3CA-mutated breast cancer
- **Eradfitinib** for FGFR+ advanced refractory bladder cancer
- **Darolutamide** for nonmetastatic castration resistant prostate cancer
- **Selinexor** for refractory myeloma
- **NovoTTF-100L System** (device) for mesothelioma

Supplements (13)

- **Pembrolizumab** in metastatic Small Cell Lung Cancer after at least one prior line of therapy
- **Ramucirumab** in HCC AFP+ treated with sorafenib
- **Ruxolitinib** for acute GVHD
- **Niraparib** for ovarian cancer treated with 3 or more prior chemotherapy regimens
- **Olaparib** for maintenance treatment of patients with deleterious germline or somatic BRCA-mutated ovarian cancer
- **Avelumab with axitinib** in advanced RCC
- **Lenvatinib with pembrolizumab** for endometrial
- **Ivosidenib** for AML
- **Venetoclax** with **obinutuzumab** in CLL
- **Revlimid with Rituxan** in Follicular Lymphoma
- **Pembrolizumab** in 1st line mNSCLC PDL1+
- **Pembrolizumab** in metastatic squamous cell carcinoma of the esophagus PD-L1 > 10

Take-aways



The engine producing new cancer therapies and diagnostics is doing very well.



Regulatory decisions are extremely complex and must be made with the data available.



NCI has resources other agencies don't and intra-governmental collaboration is critical.

NCI Funding for Fiscal Year 2020

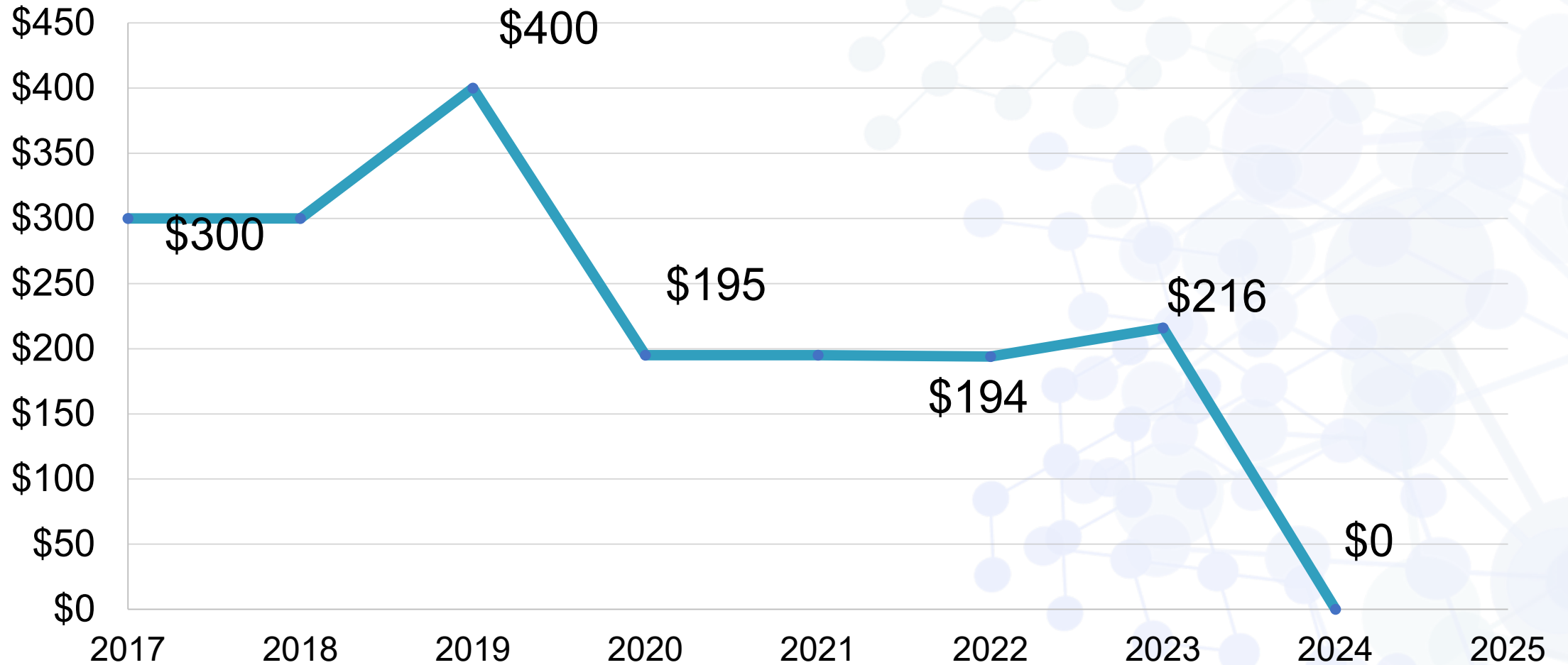
Dollars in millions

	House	Senate
Base appropriation	\$6,249	\$6,157
Moonshot	\$195	\$195
Total	\$6,444 (\$300 increase over FY 19)	\$6,352 (\$208 increase over FY 19)

Both the House and Senate bills include \$50M for Childhood Cancer Data Initiative (CCDI) in the Base appropriation amount.

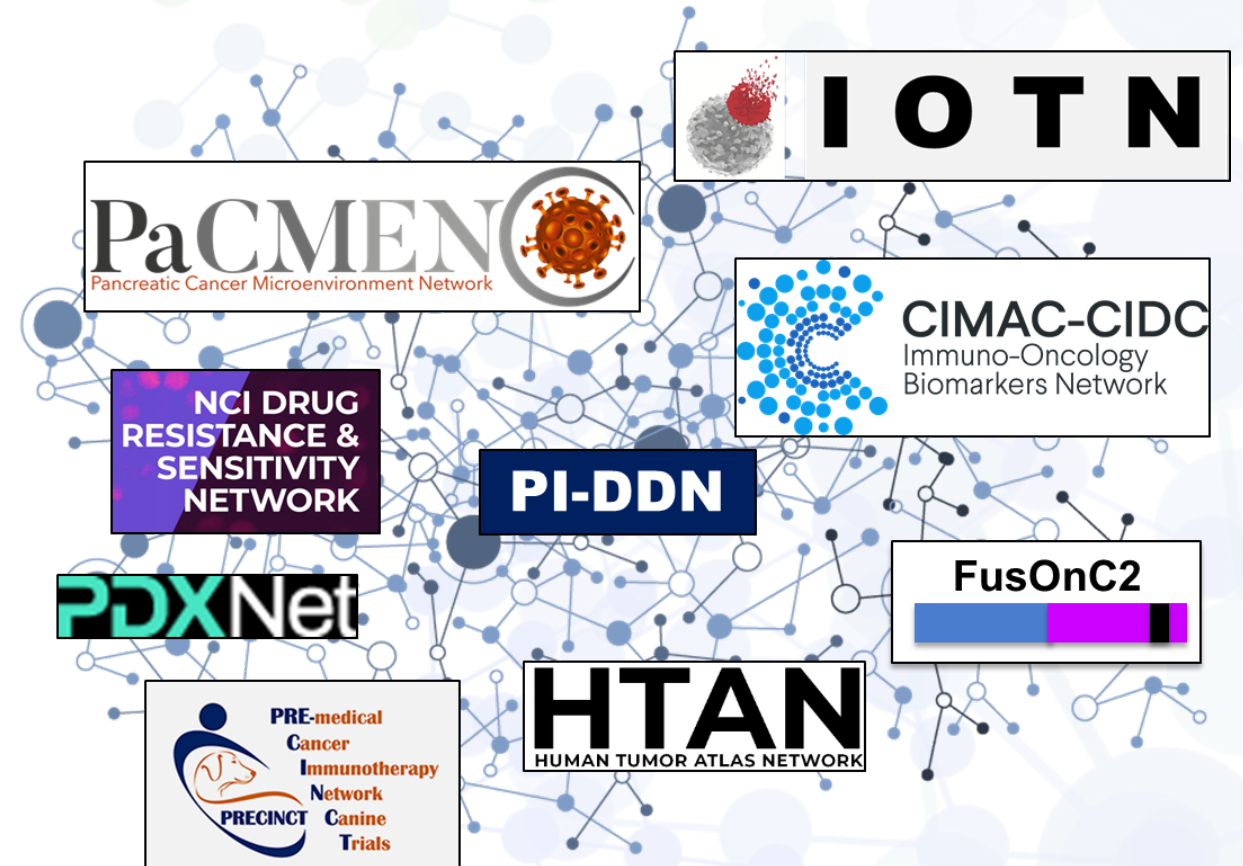
Cancer Moonshot

Funding Authorized under 21st Century Cures Act (dollars in millions)



Cancer Moonshot Networks

- Individual network meetings November 17-19
- Collaborative meeting of 8 networks November 20 to catalyze inter-network communication and collaboration



Key Focus Areas

BASIC SCIENCE

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

WORKFORCE DEVELOPMENT

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

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

Childhood Cancer Research Presidential Initiative



- President's FY 2020 budget proposal includes \$50M.
- NCI held a stakeholder meeting in July 2019.
- Planning is underway in anticipation of funding.

NCI Cloud Resources

- Provide access to popular analysis tools and pipelines, as well as large cancer data sets without need to download
- Allow researchers to bring their own data, tools and pipelines to the Cloud Resources
- Offer workspaces for researchers to save and share their data, results, and analyses

- Access and analyze over 2PB multi-omics cancer data without having to download
- Upload your own data for analysis

Data



- Perform large scale analysis using the elastic compute power of commercial cloud platforms

Compute



- dbGaP-authorized users can access controlled TCGA data
- Systems meet strict Federal security guidelines

Security



Study quantifies impact of NCI-sponsored trials on clinical cancer care



Original Investigation | Oncology

Association of National Cancer Institute-Sponsored Clinical Trial Network Group Studies With Guideline Care and New Drug Indications

Joseph M. Unger, PhD, MS; Van T. Nghiem, PhD; Dawn L. Hershman, MD, MS; Rsha Vaidya, PhD; Michael LeBlanc, PhD; Charles D. Blanke, MD

Abstract

IMPORTANCE National Cancer Institute Clinical Trial Network (NCTN) groups serve a vital role in identifying effective new antineoplastic regimens. However, the downstream clinical effect of their trials has not been systematically examined.

OBJECTIVE To examine the association of NCTN trials with guideline care and new drug indications.

DESIGN, SETTING, AND PARTICIPANTS This retrospective cohort study evaluated phase 3 SWOG Cancer Research Network clinical trials from January 1, 1980, through June 30, 2017. Only completed trials with published results were included. To be considered practice influential (PI), a trial must have been associated with guideline care through its inclusion in National Comprehensive Cancer Network (NCCN) clinical guidelines or US Food and Drug Administration (FDA) new drug approvals in favor of a recommended treatment. Data were analyzed from June 15, 2018, through March 29, 2019.

MAIN OUTCOMES AND MEASURES Estimated overall rate of PI trials, as well as trends over time. The total federal investment supporting the set of trials was also determined.

RESULTS In total, 182 trials consisting of 148 028 patients were studied. Eighty-two studies (45.1%; 95% CI, 37.7%–52.6%) were PI, of which 70 (38.5%) influenced NCCN guidelines, 6 (3.3%) influenced FDA new drug approvals, and 6 (3.3%) influenced both. The number of PI trials was 47 of 65 (72.3%) among those with positive findings and 35 of 117 (29.9%) among those with negative findings. Thus, 35 of 82 PI trials (42.7%) were based on studies with negative findings, with nearly half of these studies (17 of 35 [48.6%]) reaffirming standard of care compared with experimental therapy. The total federal investment spent in conducting the trials was \$1.35 billion (2017 US dollars), a rate of \$7.5 million per study or \$16.6 million per PI trial.

CONCLUSIONS AND RELEVANCE Nearly half of all phase 3 trials by one of the NCTN's largest groups were associated with guideline care or new drug indications, including those with positive and negative findings. Compared with the costs of a new drug approval in pharmaceutical companies, typically estimated at more than \$1 billion, the amount of federal funds invested to provide this valuable evidence was modest. These results suggest that the NCTN program contributes clinically meaningful, cost-efficient evidence to guide patient care.

JAMA Network Open. 2019;2(9):e1910593. doi:10.1001/jamanetworkopen.2019.10593

Key Points

Question What proportion of National Cancer Institute-sponsored, phase 3 Clinical Trial Network program studies are associated with guideline care or new drug indications?

Findings In this cohort study based on 182 trials including 148 028 patients, 82 trials (45.1%) were associated with guideline care or new drug indications, including trials with positive and negative findings. The estimated federal investment for each practice-influential trial was \$16.6 million.

Meaning The National Cancer Institute's Clinical Trial Network program contributes clinically meaningful, cost-efficient evidence to guide patient care.

Supplemental content

Author affiliations and article information are listed at the end of this article.

NCI Bottom Line: A Blog About Grants & More

New blog featuring 1-2 posts per month addressing:

- budget- and funding-related milestones
- funding trends and patterns
- emerging policy or fiscal issues
- analyses of NCI's grants portfolio

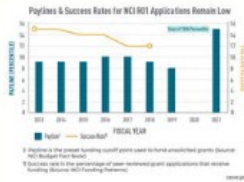
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NCI Bottom Line: A Blog about Grants and More

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This grantee-focused blog covers the latest on NCI's fiscal landscape, funding decisions, grants policy news, processes, and more.



Envisioning NCI's Future: Annual Plan and Budget Proposal for FY 2021

October 8, 2019, by L. Michelle Bennett, Ph.D.

For this edition of the NCI Bottom Line, Dr. Michelle Bennett, director of NCI's Center for Research Strategy, provides an overview of NCI's FY2021 Annual Plan and Budget Proposal. In the blog, Dr. Bennett highlights priorities from the Plan that are especially relevant to current and future NCI-funded researchers, including supporting more investigator-initiated research and maximizing opportunities in emerging areas of science.

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Leadership Vacancies

Director, Division of Cancer Prevention (DCP)

Debbie Winn, Acting

Director, Division of Cancer Biology

Daniel Gallahan, Acting



Jonathan S. Wiest, Ph. D.
Director, Center for Cancer Training

Recent Leadership Appointments

Dinah Singer, Ph.D.

Deputy Director for Scientific Strategy & Development

Meg Mooney, MD, MBA

Associate Director, Cancer Therapy Evaluation Program (CTEP)

Joy Wiszneauckas

Director, Committee Management Office

Satish Gopal, MD, MPH *(pending NIH approvals)*

Director, Center for Global Health

Oliver Bogler, Ph.D. *(pending NIH approvals)*

Director, Center for Cancer Training





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