Fiscal Year
2022

NIH TECHNOLOGY TRANSFER

ANNUAL REPORT

TABLE OF CONTENTS

Introduction **4**

Mission Statement **5**

Inventions and Agreements **6**

Institute and Center Updates **9**

NCATS - National Center for Advancing Translational Sciences 9
NCI - National Cancer Institute 11
NHGRI - National Human Genome Research Institute 12
NHLBI - National Heart, Lung, and Blood Institute 12
NIAID - National Institute of Allergy and Infectious Diseases 13
NIDDK - National Institute of Digestive 15
NIEHS - National Institute of Environmental Health Sciences 16
NIMH - National Institute of Mental Heath 16
NINDS - National Institute of Neurological Disorders and Stroke 17

Marketing NIH Discovers **18**

NCI and Frederick National Laboratory Technology Showcase 18
CDC Licensed Mpox Virus Diagnostic Technology to Expand Testing Capacity in The US 19
NIAID Develops Monoclonal Antibodies to Prevent Malaria Infection in the U.S. and in Africa 19
NIEHS Contributes To World Health Organization's Covid-19 Technology Access Pool 20
NIEHS OTT's New Website Launched 21
NIH TT Community Awareness Campaign 21

Innovative Collaborations **22**

NCI TTC Helps Support Growing Evidence of One-Dose HPV Vaccination Effectiveness 22
CRADA Facilitates Collaboration Between NCI Surgery Branch and Turnstone Biologics 23
TTC Supports Co-development of Medicines for Alzheimer's Disease Treatment 24
Clinical Collaboration with Nouscom Will Test Neoantigen-based Vaccines to Prevent Hereditary Cancers 24
CRADA Supports NCI Surgery Branch Tumor Infiltrating Lymphocyte Research 25
License with Neogene Therapeutics to Develop CRISPR-Engineered T Cell Therapy 26
CODEFACS and LIRICS Software Transfer Agreements 26
Clinical Trial Agreement and Data Transfer Agreement Support NCI's New Diffuse Large B-cell

Lymphoma Findings 27
NHGRI Collaborations 27
NIH Initiates Antiviral Program for Pandemics (APP) 28
NIAID Supports Mpox Treatment Clinical Trials in the U.S. and in Democratic Republic of The Congo (DRC) 29
NIEHS Partners with Chromadex to Develop Therapies for Parkinson's Disease 30
NIEHS Performs a Clinical Study of Idiopathic Pulmonary Fibrosis with Halo Biosciences 30
NIEHS Partners with the University of Birmingham to Create Toxicology Tools 30
NIEHS Co-Develops COVID-19 Diagnostic with Emory University 31
NIEHS Collaborates with AbbVie to Seek Treatment for Precocious Puberty 31
Data Transfer Agreements (DTAs) for Pooled Phthalate Project 31

Awards, Presentations, and Publications 32

Sury Vepa wins NCATS Mentorship Award 32
IP, Licensing, Tech Transfer Presentations 32
NCATS Publishes on Technology Transfer Tactics Awards 33
Hiding in Plain Sight: Surprising Pharma and Biotech Connections to NIH's NCI 34
TTC Staff Honored as Part of Team, 2021 NIH Clinical Center CEO Award 34
"RADx® Tech & ATP" Team Recognized with 2021 NIH Director's Award 35
TTC Associate Director Selected for NIH Office of the Director Honor Award 35
TTC TTM Honored with Hubert H. Humphrey Award 35
NHGRI Awards, Presentations, and Publications 36
NIAID Won 2022 FLC Award for Excellence in Technology Transfer 37
NIAID Won 2022 FLC Impact Award for Industry Partnerships 38
NIEHS OTT Director Receives an NIEHS Individual Merit Award 39
NINDS Awards 39
Dr. Lola Olufemi received a NINDS Director's Outstanding Innovation Individual Award 39

Appendix 40

HHS Technology Transfer Offices 40

INTRODUCTION

It has been another impactful year for the NIH technology transfer community. In FY2022 NIH received $704 million in royalty income, by far the highest amount ever received in one year. We also saw the FDA approval of Spikevax®, Moderna's COVID-19 mRNA vaccine and Emergency Use Authorization for Novavax's COVID-19 protein-based vaccine, both incorporating NIAID's SARS spike protein technologies. NIAID was also honored to receive a 2022 USPTO Patents for Humanity award that recognized NIAID's efforts to accelerate vaccine development and global access of these technologies.

Tara Kirby

Another highlight of FY2022 was NIH's participation in the World Health Organization's COVID-19 Technology Access Pool. Multiple NIH Institutes and Centers (ICs) including NIAID, NCATS, NCI, NIEHS, and NEI licensed their COVID-19 technologies to this pool to facilitate wide availability of lifesaving COVID-19 intervention around the world. An announcement of the licenses was made on May 12th by President Biden at the second Global COVID-19 Summit.

The Office of Technology Transfer (OTT) contributed to NIH's technology transfer community in multiple important ways during FY2022. The Licensing Administration and Compliance Unit (LCAU) met the challenge of administering the record-breaking income received from COVID-19 licenses and initiated royalty audits of multiple commercialization licenses. The Intramural Technology Transfer Portfolio Management Unit finalized enhancements and data integrity efforts for the Enterprise Technology Transfer (ETT) data system, which officially launched to the Technology Transfer Community in December as the system of record for all intramural technology transfer activities. OTT also launched a study to better understand the health and economic impacts of the intramural licensing program, which was recently made available to the public.

In addition to these important projects, OTT continued to provide key services and support functions for all NIH TTOs and the CDC, including management and oversight of royalty collection and disbursement, monitoring and enforcement of patent rights and licenses, coordination of patent annuity payments, outreach to existing and potential licensees, patent docketing services, reporting, and support of ETT and the Technology Transfer Community SharePoint and public websites.

We invite you to look through the report to learn more about the achievements and scientific advancements made at the NIH and the CDC during the past year.

You can learn even more about NIH intramural technology transfer at www.techtransfer.nih.gov - or try the QR code to the right!

Sincerely,

Tara Kirby

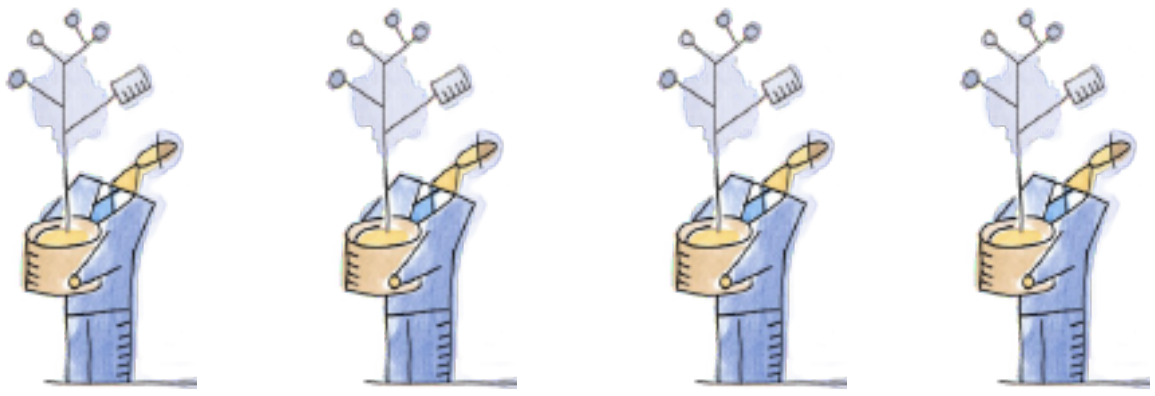
Director, Office of Technology Transfer

MISSION STATEMENT

The mission of Technology Transfer at National Institutes of Health (NIH) is to facilitate partnerships with a wide array of stakeholders, and effectively manage the inventions conceived by scientists working at the NIH and the Centers for Disease Control and Prevention (CDC). In doing so, NIH Technology Transfer supports the larger NIH mission to seek fundamental knowledge about the nature and behavior of living systems and the application of that knowledge to enhance health, lengthen life, and reduce illness and disability.

Working on behalf of the NIH and the CDC, all agencies of the Department of Health and Human Services (HHS), Technology Transfer offices[1] across the NIH apply responsive, and sometimes creative, approaches to meet the needs of all parties involved, operating with a goal of moving scientific research and discovery forward for the benefit of public health. Technology Transfer at NIH:

- Protects U.S. intellectual property and the discoveries conceived by NIH and CDC intramural researchers. This includes working with researchers to determine if an invention warrants patent protection, overseeing the filing of Employee Invention Reports (EIRs), and coordinating the patent filing and prosecution process.
- Serves as a bridge through marketing and communications, connecting the inventive discoveries made by scientists in the NIH and CDC research programs to commercial partners with the capability of developing these technologies into products and services to benefit public health. Without TT, the full potential of these inventions would not be realized, and the public would not receive the full benefit of these biomedical discoveries.
- Facilitates partnerships with outside parties to allow for collaboration.
- Negotiates licenses and collaborative agreements such as Cooperative Research and Development Agreements (CRADAs) to ensure the timely development of federal technologies that contribute to society by driving economic growth and productivity; these collaborations leverage the strengths of each institution to advance basic and clinical research objectives.
- Monitors the development of these technologies to ensure commercialization milestones are reached, products are brought to the market, and royalty fees are paid.
- Facilitates the transfer of thousands of research materials and data into and out of NIH.



1 Please see the Appendix for a list of all the HHS Technology Transfer Offices within the NIH that contributed to this report.

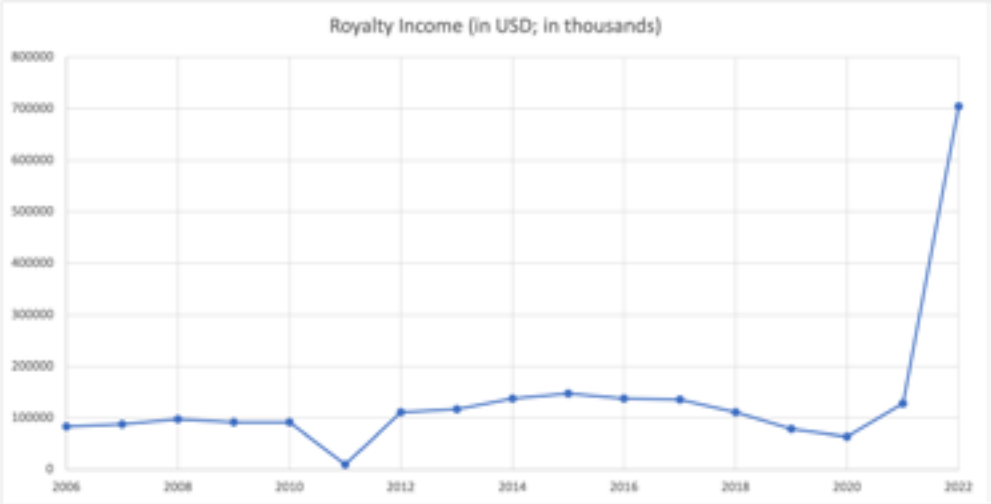
INVENTIONS AND AGREEMENTS

The TT Program at NIH is the focal point for implementation of the Federal Technology Transfer Act. Technology licensing specialists in the NIH ICs license patented inventions to pharmaceutical, medical device, and biotechnology companies in order to stimulate development of technologies into commercial products. These licensing specialists also transfer materials to non-profit research institutions and license for royalties to commercial entities unpatented research tools to increase their availability to the scientific community. These activities support the NIH's mission to benefit the public health and to provide a financial return on public investment.

In addition, the TT Program negotiates terms for research collaborations between NIH and commercial and academic organizations. These collaborations leverage the strengths of each institution to advance basic and clinical research objectives. The TT Program also facilitates the transfer of thousands of research materials and data into and out of NIH.

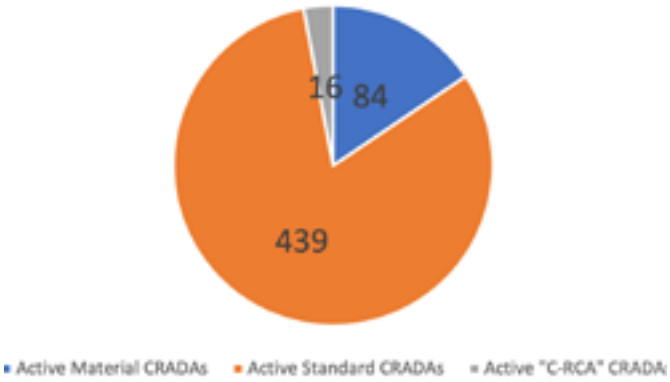
In FY2022 NIH brought in $704 million in royalty income. There were 251 invention disclosures, 204 patent applications filed, 96 U.S. patents issued, and 332 executed licenses. A graphical breakdown of these numbers is provided on the following pages.

Royalty Income

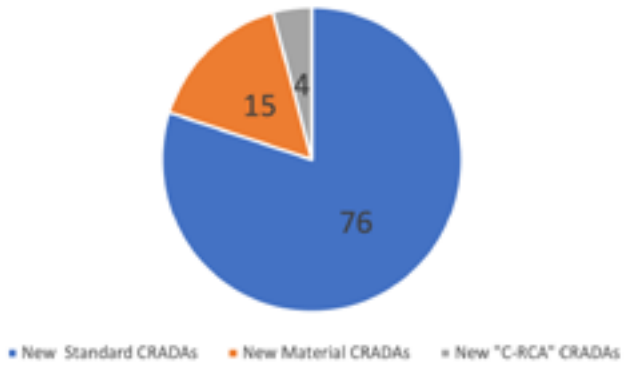


CRADA Metrics

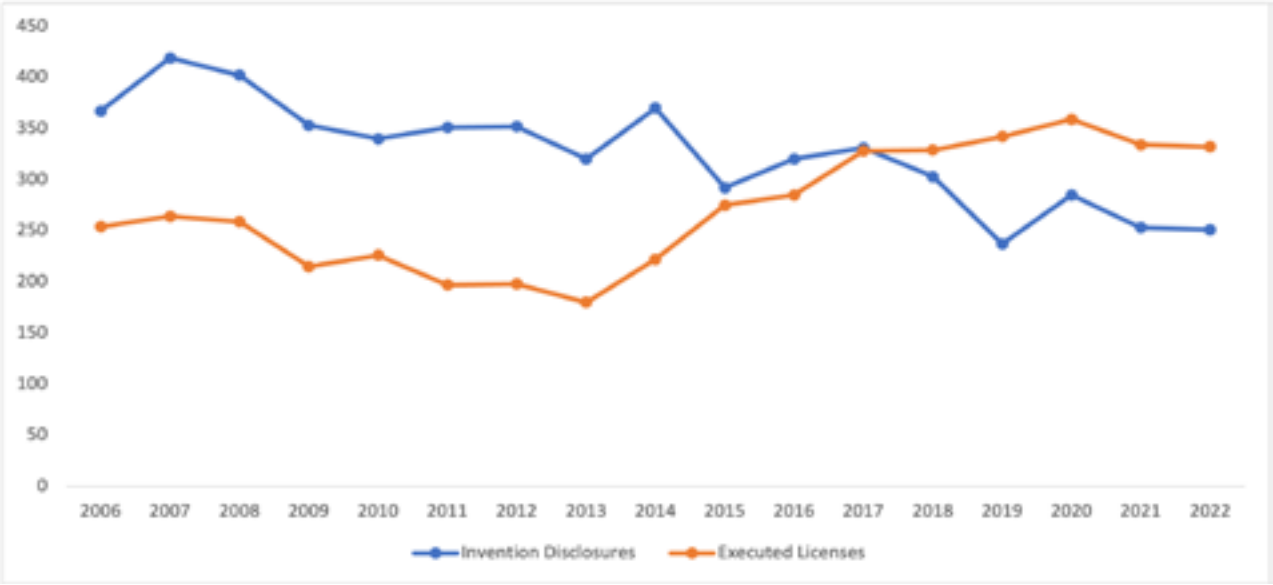
Active CRADAs



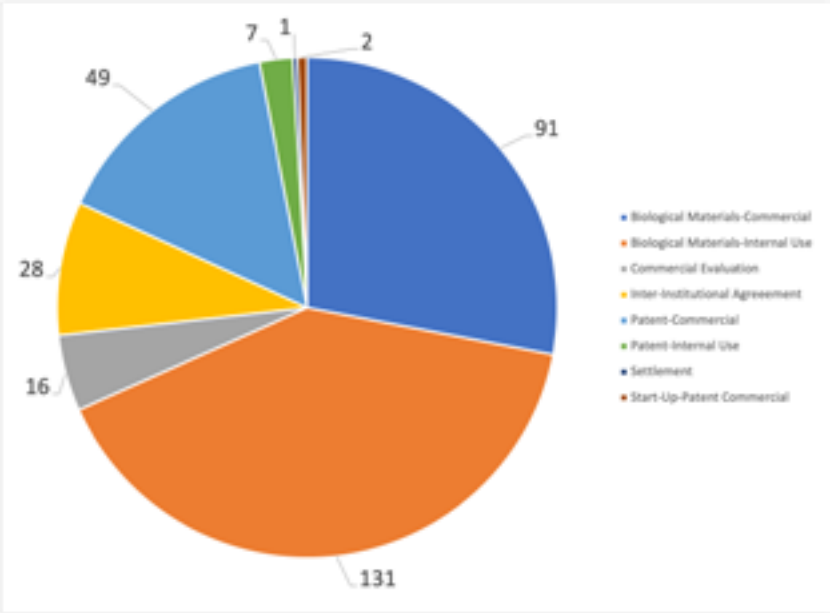
New CRADAs



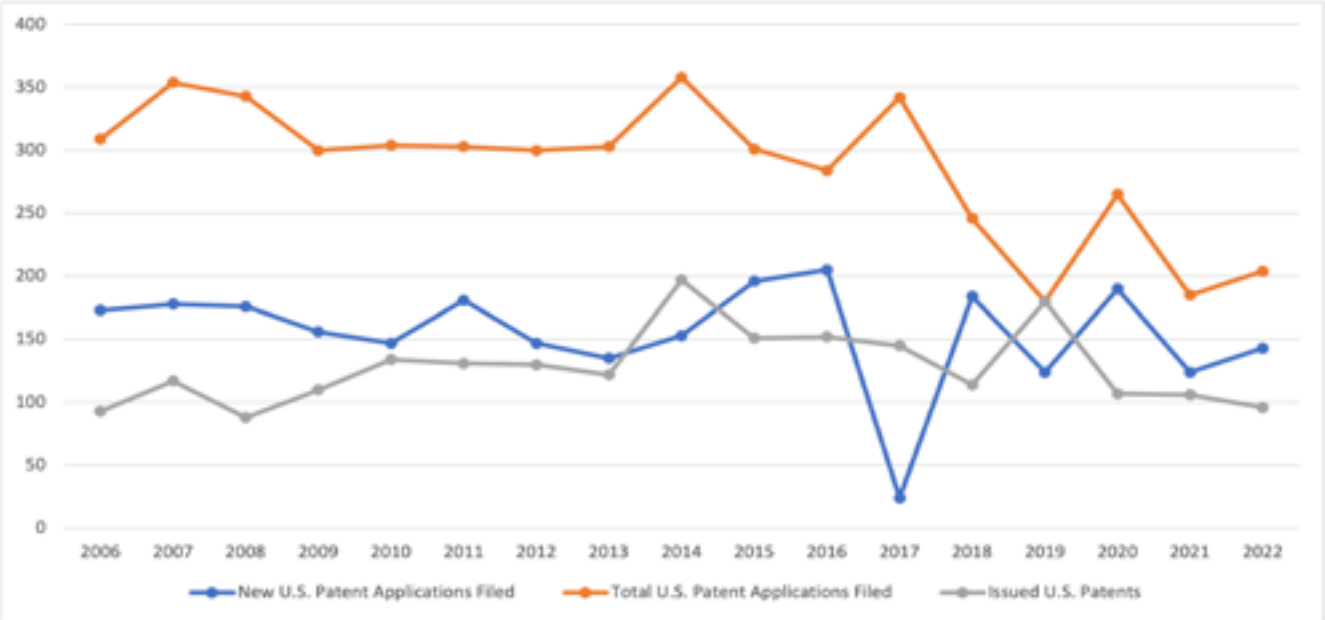
Inventions and Licenses



Licenses in a Fiscal Year by Type of Agreements



Patents



INSTITUTE AND CENTER UPDATES

NCATS - National Center for Advancing Translational Sciences

The success of The National Center for Advancing Translational Sciences (NCATS) in advancing translational sciences is built on effective management of three core pillars: collaboration, innovation, and acceleration. The expertise, capabilities, and resources required to successfully advance a drug, device, or intervention resides in different groups as these efforts progress through the translational science spectrum. Partnerships and collaborations across individuals, organizations and sectors are essential to efficient progress. The creation of productive and mutually beneficial collaborations depends not only on individual excellence, but on teamwork, coordination, cooperation, and communication.

Traditional professional incentive structures focus on individual accomplishment and make teamwork difficult to navigate. Embracing patients and communities as research partners also holds great potential for the development treatments with meaningful outcomes for the populations affected by disease. With these needs in mind, NCATS tests novel partnership structures that cut across traditionally siloed scientific disciplines, organizations, and sectors.

The NCATS Office of Strategic Alliances (OSA) aims to make it easy for industry, small businesses, and academia to interact and partner with NCATS scientists. OSA staff help develop formal partnerships that proactively address complex issues, such as intellectual property and project management roles, to make for smoother, more effective collaborations.

NCATS OSA typically negotiates and executes an average of 400 agreements annually; additionally, there has been a concerted effort to assure that all agreements with term limits were either closed due to project completion, or amended to enable the project to continue. While some of these executed agreements were built from institutional template agreements, most required customization as well as substantial input of time for negotiation of terms acceptable to the NIH. Given the varied nature of NCATS' collaborations with industry, academia, patient groups, etc, may agreement negotiations require significant time and effort to educate our counterparts on the particulars and requirements of collaborating with the federal government, and in particular NCATS/NIH.

While implementing the mission-related programs and activities, NCATS has built and continues to build a large and complex intellectual property (IP) portfolio. In numerical terms, the NCATS portfolio includes more than 300 inventions, the majority of which (more than 200) are jointly

owned with collaborators. These inventions have resulted in: 89 issued US patents; 283 issued foreign patents; and 1,178 pending patent applications.

Further, as a means for accelerating innovation and commercial development, NCATS has licensed many of its technologies (nearly 50 commercial licenses and over 70 Inter-Institutional license agreements). The NCATS IP portfolio reflects the great strides being made in forming effective collaborations, which result in significant innovations in the form of novel IP and further which culminate in accelerating development of diagnostics and therapeutics that will benefit patients.

Lili Portilla Bids Adieu

Lili M. Portilla, the Director of Strategic Alliances at NCATS has accepted a position at Novavax as their new Senior Director of Government Programs and will be bidding farewell to us after 30 plus years of dedicated service to the NIH. As a subject matter expert, deep thinker, supportive mentor, and dynamic leader, Lili has enriched many lives at NIH. During her NIH career at NHLBI, NCRR, and most recently at NCATS, Lili has initiated, nurtured, and developed many initiatives in technology transfer, animal and molecule repositories, and small business and entrepreneurship programs. Her excellent tenure is reflected in her twenty NIH and NCATS Director's awards. The NIH community will miss Lili deeply but will be eternally grateful for her numerous contributions as we wish her much joy in her next chapter. To stay in touch with Lili, please connect with her on LinkedIn.

Lili Portilla

NCATS

COLLABORATE. INNOVATE. ACCELERATE.

NCI - National Cancer Institute

TTC Support of NCI's New Connect for Cancer Prevention Study (Connect)

In summer 2022, NCI launched its new Connect for Cancer Prevention Study (Connect) in partnership with nine health care systems across the United States. Connect is a prospective cohort study that follows people over time to learn how different factors affect health. As part of this project, researchers aim to study 200,000 people between the ages of 40 and 65 with no history of cancer to help understand more about the causes of cancer and how to better prevent it. TTC Unit Supervisor, Lisa Finkelstein, Ph.D. executed two MTAs with partnering health care systems for NCI to receive human biospecimens and electronic health data in support of this project.

TTC Helps Support the DCEG Cancer Moonshot Project Initiative to Accelerate Cervical Cancer Control

Senior Investigator, Mark Schiffman, M.D., M.P.H., and his colleagues in the NCI's Division of Cancer Epidemiology and Genetics, are spearheading a herculean effort to reduce cervical cancer burden worldwide through the DCEG Cancer Moonshot Project Initiative to Accelerate Cervical Cancer Control ("Moonshot"). Cervical cancer is the fourth most common cancer in women and is almost always caused by HPV infection. Although effective vaccines exist against HPV, challenges remain for cervical cancer control including inefficient or unsustainable screening, over-treatment, low vaccine uptake rates, and reduced access to vaccines.The goal of the Moonshot is to overcome these barriers to create efficient and effective cervical cancer screening approaches to reduce global cervical cancer burden.

One approach the Moonshot team has been pursuing is the development of an artificial intelligence (AI) tool, called automated visual evaluation, that digitally analyzes cervical images and accurately identifies cervical precancers. This tool would require minimal training and simple technologies to operate and can serve as a more easily accessible, simpler, more expedient, and reliable way to improve screening for cervical cancer. Since 2016, NCI TTC has negotiated over 18 MTAs, 10 DTAs, 2 CDAs, and 1 MOU with over 24 institutions from more than 16 countries to support the Moonshot research efforts. The Moonshot continues today, and you can read about some recent findings. Schiffman's technology transfer (TT) docket is currently managed by TTM Michaela McCrary, Ph.D., NCI TTC.

NHGRI - National Human Genome Research Institute

In FY2022, NHGRI continued to manage its robust technology transfer portfolio and coordinate its activities with many teams at the Institute (including Ethics, Bioethics, and Financial Management) to provide excellent service to its investigators and promote and translate its technologies into commercial space.

The Office seamlessly transitioned from 100% telework to a mix of in-person and telework in the summer of 2022.

NHLBI - National Heart, Lung, and Blood Institute

The National Heart, Lung, and Blood Institute (NHLBI) had a busy FY22! The institute executed 94 transactional, license, and CRADA agreements and its licensed product, Hemgenix®, was awaiting FDA approval as of the end of the fiscal year. They also oversaw the technology transfer needs of their service center clients: the National Institute on Alcohol Abuse and Alcoholism (NIAAA), the National Institute of Arthritis and Musculoskeletal and Skin Diseases (NIAMS), the National Institute of Biomedical Imaging and Bioengineering (NIBIB), the National Institute on Deafness and Other Communication Disorders (NIDCD), the National Institute of Environmental Health Sciences (NIEHS), and the National Institute of Nursing Research (NINR).

Hemgenix® is a one-time gene therapy for the treatment of adults living with hemophilia B. Hemgenix® (etranacogene dezaparvovec) is an adeno-associated virus vector-based gene therapy for adults with Hemophilia B, or have a life-threatening risk of hemorrhage, or have repeated spontaneous bleeding episodes.

Hemophilia B is a genetic bleeding disorder that is a result of insufficient levels of blood clotting Factor IX, which is a protein needed to produce blood clots to stop bleeding. Previously, treatment involved intravenous (IV) infusions of Factor IX replacement products to aid the body's ability to stop bleeding and prevent future bleeding episodes. Hemgenix® is a one-time gene therapy given as a single dose by IV infusions. The gene is expressed in the liver to produce Factor IX protein which increases blood levels of Factor IX which in turn limits bleeding episodes.

Hemgenix® was developed and launched by NHLBI's licensee uniQure , N.V, and its sublicensee CSL Behring. NHLBI's contributions were a method of delivering a heterologous nucleic acid or gene of interest to particular target cells using an Adeno-Associated Virus of serotype 5 (AAV5) as well as a novel method of producing the virus in insect cells. Hemgenix® is waiting to receive its approval from the FDA.

NIAID - National Institute of Allergy and Infectious Diseases

NIAID Makes COVID-19 Technologies Available to Global Manufacturers Through WHO Program

NIH licensed COVID-19 technologies arising from NIH intramural research to the Medicines Patent Pool (MPP) for access through the World Health Organization's (WHO) COVID-19 Technology Access Pool (C-TAP). The announcement of the licenses was made on May 12th by President Biden at the second Global COVID-19 Summit, cohosted by the United States, Belize, Germany, Indonesia and Senegal.

Controlling COVID-19 and addressing other public health needs are only possible if all communities around the world have access to lifesaving vaccines, treatments, and diagnostics. While NIH's early-stage technology contributions are only one component in the development process and do not alone allow full development of medical countermeasures, such contributions are an important step toward facilitating wider availability of lifesaving interventions around the world.

The MPP's purpose is to sublicense medicines and health technologies to increase access to and facilitate development of essential medicines for people living in low-and middle-income countries. NIH has been involved with MPP since its founding in 2010 when NIH contributed the first patent to MPP. C-TAP's goal is to increase the global supply of vaccines, treatments, and diagnostics for COVID-19. The licensing of patents through C-TAP is handled by MPP. NIH has agreed to license these technologies to the MPP to help low- and middle-income countries have access to lifesaving treatments, vaccines, and diagnostics.

The technologies include the stabilized spike protein used in currently available COVID-19 vaccines, research tools for vaccine, drug, and diagnostic development as well as early-stage

vaccine candidates and diagnostics. NIAID Technology Transfer and Intellectual Property Office (TTIPO) manage and protect federal government's intellectual property right for the technologies that NIAID contributed as follows:

Vaccine Development

- Prefusion coronavirus spike proteins and SARS-CoV-2 prefusion spike proteins and their use

Tools for Vaccine Development

- Structure-Based Design of SARS-CoV-2 Spike Immunogens Stabilized in the RBD-All Down Conformation
- SARS-CoV-2 Pseudotyping Plasmid

Research Tool for Drug Development

- ACE2 Dimer construct

Vaccine Candidates

- Newcastle Disease Virus-Like Particles Displaying Prefusion-Stabilized SARS-CoV-2 Spikes as a Single-Dose COVID-19 Vaccine
- Parainfluenza virus 3 based vaccine against COVID-19
- A VSV-EBOV-Based Vaccine Against COVID-19

NIAID Prefusion Spike Protein Technologies Enable Another COVID-19 Vaccine - Novavax COVID-19 Vaccine

NIAID's TTIPO continues to protect and license NIAID intellectual property (IP) rights on coronavirus spike proteins to enable development and commercialization of COVID-19 medical products.

In 2016, Dr Barney Graham, at that time, Deputy Director of the Vaccine Research Center, NIAID, and his team, together with their academic and non-profit collaborators, engineered coronavirus spike proteins to stabilize the spike in its prefusion conformation. The prefusion stabilized spike protein of SARS-CoV-2 is incorporated as the antigen in all SARS-CoV-2 vaccines authorized in the U.S. In 2022, Novavax was the most recent vaccine developer to gain Emergency Use Authorization for its SARS-CoV-2 vaccine which uses NIAID's prefusion stabilized spike technology.

Credit: iStock/ffikretow

NIAID decided to employ a nonexclusive licensing strategy for this foundational technology which in turn enabled development of vaccines using this effective antigen in a variety of platforms. It has also made this technology available for use in diagnostics and as a research material. Since January 2020, NIAID has negotiated 26 license agreements with 24 different commercial entities for this technology. Of these, two license agreements with vaccine developers were executed in 2022 and eight additional license agreements remain in negotiation.

Financial Update

In FY 2022, NIAID received approximately $9.2 million and $11.3 million under conditional gift agreements and cooperative research and development agreements (CRADAs), respectively. TTIPO negotiated these agreements.

NIDDK - National Institute of Diabetes and Digestive and Kidney Diseases

The NIDDK's Technology Advancement Office (TAO) handles not only NIDDK's technology transfer needs, but also its service center clients: The National Institute of Dental and Craniofacial Research (NIDCR), The Fogarty International Center (FIC), Office of Research Services (ORS), and The NIH Office of the Director, Division of Program Coordination, Planning, and Strategic Initiatives (OD/DPCPSI).

LIVMARLI Enters the US Market

LIVMARLI® (maralixibat) entered the US market this year after receiving FDA approval on September 29, 2021. It is the first and only approved medication for the treatment of cholestatic pruritus in patients with Alagille syndrome (ALGS) one year of age and older. LIVMARLI® is a minimally absorbed ileal bile acid transporter (IBAT) inhibitor.

"*Children with Alagille syndrome suffer from cholestatic pruritus, which is serious, unremitting, and debilitating. Their sleep is disrupted, and they endure bleeding and scarring of the skin due to unrelenting scratching*," said Binita M. Kamath, MBBChir, Pediatric Hepatologist, The Hospital for Sick Children (SickKids), Toronto, Ontario, Canada. "*There have been no approved treatments to date for cholestatic pruritus in Alagille syndrome, and many children ultimately require major surgical interventions such as liver transplantation for refractory pruritus. The approval of LIVMARLI® signifies a meaningful shift in the treatment paradigm for Alagille syndrome and provides hope for the many families who have lived with persistent itch for far too long*."

ALGS is a rare genetic disorder caused by abnormalities in bile ducts that can lead to progressive liver disease. Malformed or reduced bile ducts cause cholestasis, the accumulation of bile acids in the liver, which leads to inflammation and liver injury, and prevents the liver from working properly. Cholestasis in ALGS is associated with pruritus which is among the most common indications for liver transplant in ALGS.

The approval of LIVMARLI® was based on the pivotal ICONIC study as well as five years of data from supportive studies conducted in collaboration with NIDDK and their extramural U01 collaboration programs resulting in a robust body of evidence in 86 patients with ALGS. Data from ICONIC demonstrated statistically significant reductions in pruritus, one of the most common and arduous symptoms associated with the disease, which was maintained through four years.

Can-Fite Biopharma's Piclidenoson Seeking FDA Approval

Can-Fite is submitting Piclidenoson, a novel, first-in-class, A3 adenosine receptor agonist (A3AR) small molecule, orally bioavailable drug for FDA approval. This technology was developed in the laboratory of Dr. Ken Jacobson at NIDDK.

NIEHS - National Institute of Environmental Health Sciences

The National Institute of Environmental Health Sciences (NIEHS) uses state-of-the-art science and technology to investigate the interplay between environmental exposures, human biology, genetics, and common diseases to help prevent disease and improve human health.

The Office of Technology Transfer (OTT) at NIEHS supports the development of emerging environmental health technologies. The mission of the Office of Technology Transfer is to facilitate partnerships that lead to the discovery of innovative technologies that improve human health.

NIEHS OTT successfully negotiated 454 agreements in FY2022 with 371 Material Transfer Agreements (252 of which were with Addgene), 22 Confidential Disclosure Agreements, 36 Data Transfer Agreements, 3 Copyright/Publisher Agreements, 2 Software Transfers, 9 Research Collaboration Agreements, 4 Memoranda of Understanding, and 7 Other agreements.

NIMH - National Institute of Mental Heath

The National Institute of Mental Health and Cerevel Therapeutics will collaborate to determine whether the enzyme phopshodiestewrase-4B (PDE4B) is decreased during a major depressive episode in unmedicated volunteers with major depressive disorder (MDD). The study will use positron emission tomography (PET) and the PDE4B radioligand [18F] PF-06445974.

The National Institute of Mental Health and Janssen Research & Development, LLC are collaborating to evaluate positron emission tomography (PET) radioligands for colony stimulating factor receptor-1 (CSF1R) in the brain in pre-clinical models.

Developed by researchers in the Pediatric Oncology Branch of the National Cancer Institute (NIH NCI), National Institute of Mental Health (NIMH) and technology through Patient Planning Services & Cancer Support Community. Checking IN is an evidence-informed digital distress screening solution for pediatrics.

The evidence-informed tool is a web-based distress screening measure for patients (age 8-21) with chronic medical conditions and their parents created to capture child and parents' perception of child's distress. It is designed to provide real-time results/feedback to clinical providers and allows clinical team to take immediate actionable interventions.

NINDS - National Institute of Neurological Disorders and Stroke

Under a Materials Cooperative Research and Development Agreement (M-CRADA), the National Institute of Neurological Disorders and Stroke of the National Institutes of Health will conduct research using Ionis Pharmaceuticals Inc.'s proprietary antisense technology designed to target mouse metastasis associated lung adenocarcinoma transcript 1 (MALAT1) to determine if it is possible to target cell populations using the well-characterized antisense oligonucleotides as a tool targeting the ubiquitously-expressed lncRNA MALAT1. The principal goal of this M-CRADA is to use Collaborator Research Material, to determine if it is possible to target cell populations using the well-characterized tool, ASOs targeting the ubiquitously-expressed lncRNA MALAT1. The scope of this MCRADA is limited to research using proprietary Collaborator Research Material to determine if it is possible to target cell populations using the well-characterized ASOs as a tool targeting the ubiquitously-expressed lncRNA MALAT1.

NINDS executed a Material Cooperative Research and Development Agreement with Gibson Oncology in 2022. The goal of this M-CRADA is to evaluate the cellular identity of the c-Myc expressing cells in glioblastoma (GBM) cell lines, evaluate how c-Myc blockade affect markers of stemness, proliferation, migration, and therapeutic resistance in glioblastomas, determine how the transcriptomic profile of the GBM cells change in response to c-Myc inhibition, elucidate further upstream and downstream regulatory targets of the pathway which may be distinct in GBM and perform in vivo studies to evaluate response to therapy in recurrent glioblastoma for which there is no standard of care.

The Center for Alzheimer's and Related Dementias (CARD) is a collaborative initiative between the National Institute of Aging (NIA) and the National Institute of Neurological Disorders and Stroke (NINDS). Several CARD projects are based on induced pluripotent stem cells (iPSCs). A goal of CARD is to utilize iPSCs that can make broadly available to the research community, ideally through a repository. NINDS and NIA technology transfer worked collaboratively to identify iPSCs that would help CARD achieve the desired outcome. Through this effort, KOLF2.1 was identified. The CARD researchers considered guidance received from NIA and NINDS tech transfer and, in view of scientific needs, wanted to move forward with a partnership with The Jackson Laboratory (JAX; wwwjax.org). NINDS and NIA collaboratively negotiated an agreement with JAX comprising the terms and conditions that would achieve the above-referenced goal. The process was complex because of the multi-party nature of the agreement as well as non-CARD related research involving the same iPSCs at NINDS. In August 2022, JAX announced this arrangement as part of their iPSC catalog (https://www.jax.org/jax-mice-and-services/ipsc) and highlighted the involvement of NIH and CARD in a press release. Through the persistent and coordinated efforts of the NIA and NINDS tech transfer offices, this important and valuable research partnership was established.

Credit: iStock/Rudzhan Nagiev

National Cancer Institute and Frederick National Laboratory Technology Showcase

In September approximately 300 attended the 2022 Technology Showcase in person at the Frederick National Laboratory (FNL) or online. The annual event is co-chaired and organized by the NCI Technology Transfer Center's Technology Analysis and Marketing Unit (TAMU) and the Frederick National Laboratory Partnership Development Office. In addition, through a Co-Sponsorship Agreement, the Frederick County Office of Economic Development, the City of Frederick Department of Economic Development, the Federal Laboratory Consortium for Technology Transfer and TEDCO contribute to event planning and promotion.

The annual showcase is designed to highlight technology licensing and partnering opportunities with the NCI and FNL. Historically it was either an in-person or virtual event, but 2022 was the first time it was both. The hybrid conference—which had 45 speakers, two sets of concurrently running panels, 10 technology posters, a lightning pitch session, and 10 exhibit tables for both an in-person and virtual audience—was a new planning challenge. Ultimately, the collective efforts of all involved paid off for the audience with a positive, seamless event experience that allowed people to participate virtually or take advantage of face-to-face engagement by attending in-person.

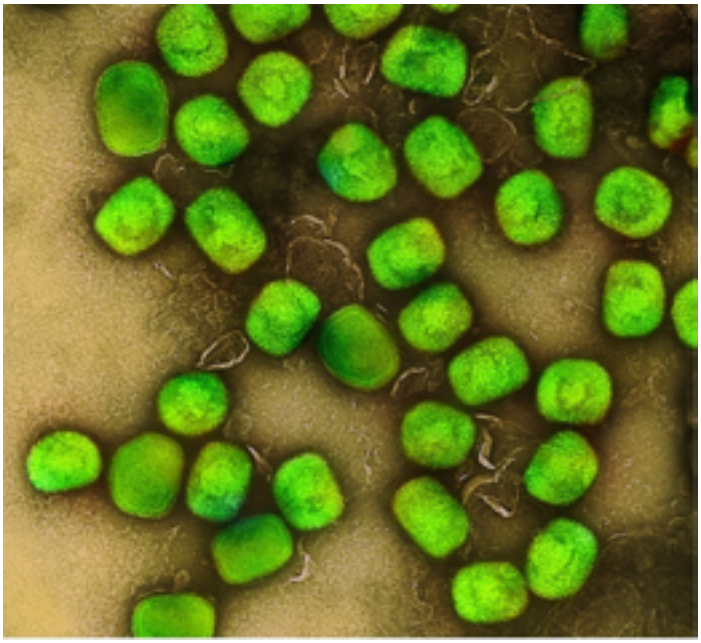
Following this year's event, TTC TAMU Supervisor, Michael Salgaller, Ph.D. and FNL Chief Innovation Officer, Vladimir Popov, Ph.D., were invited to provide an overview of the Technology Showcase to the FNL Advisory Committee. Their presentation highlighted the event's origin story, purpose, achievements, and improvements throughout its six-year history. Learn more:

- View 2022 Technology Showcase recording
- Browse the event agenda including the list of speakers and featured technologies. 2022 included the addition of programmatic presentations offering attendees a snapshot of collaboration opportunities involving departmental efforts rather than individual technologies; for example, James Gulley, M.D., Ph.D., the co-director of the NCI CCR Center for Immuno-Oncology (CIO), provided an overview and highlighted areas where the CIO is seeking collaboration partners.
- Read the Frederick News Post article, "Innovating Together Technology Showcase"
- Watch the promotional video: https://www.youtube.com/watch?v=TDVSxsPnNng
- Watch a presentation on the Technology Showcase at the October 12, 2022, Frederick National Laboratory Advisory Committee meeting, starting at 1:47:00. Presenters: Michael Salgaller, Ph.D., and Vladimir Popov, Ph.D.

CDC Licensed Mpox Virus (Monkeypox, MPXV) Diagnostic Technology to Expand Testing Capacity in The United States (U.S.)

NIAID Technology Transfer and Intellectual Property Office (TTIPO) rapidly licensed CDC technologies to expand MPXV testing capacity in the U.S. In late June 2022, as cases of Mpox virus infection increased in the United States, the Department of Health and Human Services (DHHS) announced a program to expand access to MPXV testing in the U.S. by licensing an established CDC non-variola orthopoxvirus (the genus that Mpox is a member of) test to five commercial testing labs. The CDC had previously obtained FDA 510(k) clearance for this test and had been distributing the test kits to public health laboratories for many years. While these public health labs had the testing capacity to handle the testing demand, DHHS determined that expanded access would provide a more robust response to the Mpox outbreak. Therefore, test kits were distributed to commercial testing laboratories through a biological materials licensing agreement.

NIAID TTIPO manages the licensing and patenting of CDC technologies and efficiently negotiated these licenses to the commercial testing labs. TTIPO had to quickly integrate into the ongoing discussions and draft terms for licenses that balanced the needs of the CDC scientific programs, the FDA, and the commercial labs, while ensuring licensing standards were still met for all five licenses. Through its quick action, TTIPO completed all five licenses within three weeks of receiving the first license application from a commercial lab.

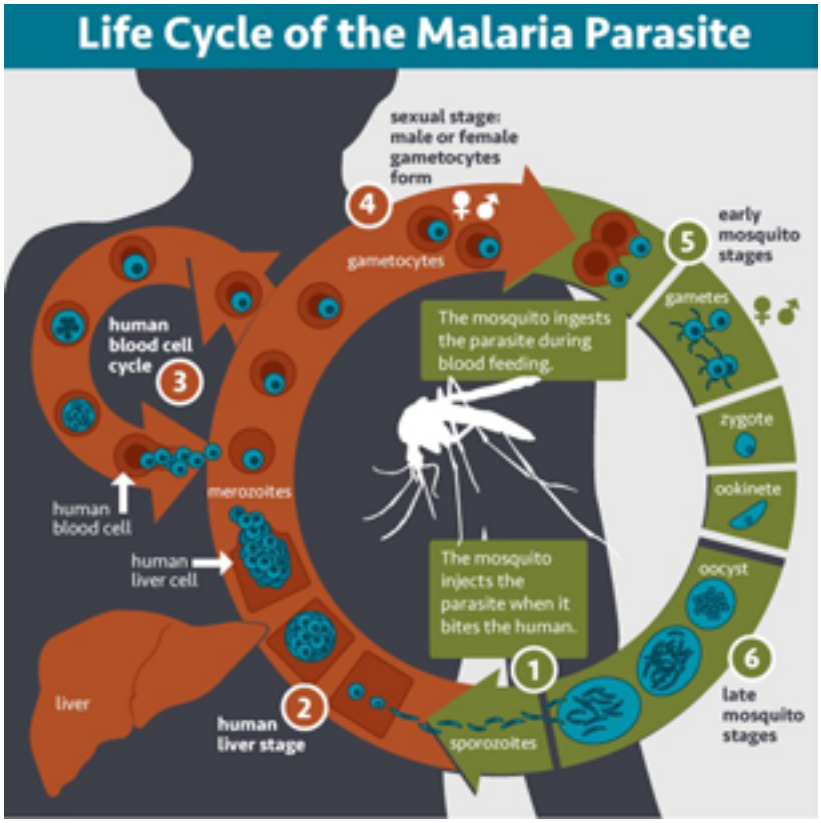


NIAID Develops Monoclonal Antibodies (mAbs) to Prevent Malaria Infection in the U.S. and in Africa

NIAID TTIPO protects NIAID intellectual property (IP) rights on malaria monoclonal antibodies (mAbs) and negotiated agreements to support their development and commercialization. Malaria, a potentially life-threatening disease caused by infection with Plasmodium parasites transmitted by an infective mosquito, is a top priority for eradication by the NIH and World Health Organization (WHO). United States residents, including military personnel, health workers, children, and older individuals with compromised immune systems traveling to one of the 96 designated malaria-endemic regions defined by the WHO are at the greatest risk of malaria infection. To prevent and target malaria infection, NIAID's Vaccine Research Center (VRC) scientists have developed several promising malaria monoclonal antibodies (mAbs).

CIS43LS and L9LS are two VRC developed recombinant human monoclonal antibodies that target the circumsporozoite protein (CSP), an immunodominant protein that coats the surface

of the sporozoite.The monoclonal antibody CIS43 prevents malaria infection in humans for up to 9 months following a single intravenous administration and has completed Phase 2 Clinical Trials. L9LS is much more potent than CIS43LS and therefore can be administered in a smaller dose as an injection under the skin (subcutaneously), rather than by intravenous infusion. An early-phase NIAID trial of L9LS in the U.S. found that the antibody was safe and prevented malaria infection for 21 days in 15 out of 17 healthy adults exposed to P. falciparum in a carefully controlled setting. Two larger, NIAID-sponsored Phase 2 trials assessing the safety and efficacy of L9LS in infants, children and adults are underway in Mali and Kenya.



As the NIAID TTIPO handles the licensing and patenting of CIS43LS and L9LS technologies, TTIPO negotiated several agreements and licenses to advance the development of the malaria antibody technology. Specifically, Clinical Material Transfer Agreements (CMTAs), a 4-party Clinical Trial Agreement (CTA), two license agreements with several biotech industry partners, non-for-profit collaborators, and academia have led to successful collaborations and the continued development of CIS43LS and L9LS for malaria prevention and public health use.

NIEHS Contributes To World Health Organization's Covid-19 Technology Access Pool

In May 2020, World Health Organization (WHO) and other partners launched the COVID-19 Technology Access Pool (C-TAP) to facilitate faster equitable and affordable access to COVID-19 health products for people in all countries. On May 12, 2022, the NIH licensed several COVID-19 technologies to the WHO's Medicines Patents Pool (MPP) for access through C-TAP. Among these technologies were NIEHS' patent-pending Leader Sequence Homology-enriched (LeaSH) RNA-Seq invention (HHS Ref # E-241-2020). LeaSH RNA-Seq is a massively paralleled multi-patient assay for pathogenic infection diagnosis and host physiology surveillance using nucleic acid sequencing. NIEHS' contribution to MPP is an important step toward facilitating wider access to lifesaving interventions for COVID-19 around the world so that quality-assured manufacturers may produce these technologies at scale.

World Health Organization

NIEHS OTT's New Website Launched

NIEHS OTT's website has been revamped to better facilitate collaboration between NIEHS and external parties. Updates to the website include educational content for NIEHS investigators on how to initiate technology transfer and collaborative research agreements with external parties in academia or industry and showcases NIEHS technologies that are available to potential partners in industry for licensing and/or co-development. The new website and our new logo were made possible through a partnership with NIEHS Office of Communications and Public Liaison.

NIH TT Community Awareness Campaign

NIH Technology Transfer Community advertising material from FY22 Awareness Campaign

INNOVATIVE COLLABORATIONS

IIA with UMB and License to UMB's start-up, Ambit Therapeutics

NCATS and University of Maryland Baltimore (UMB) jointly developed this invention entitled "Deuterated Alpha 5 Subunit Selective Negative Allosteric Modulators of Gamma-aminobutyric Acid Type A Receptors as Fast Acting Treatment for Depression and Mood Disorders". Pursuant to our IIA with UMB, it licensed this technology and their background IP to Ambit Therapeutics. The exclusive license to Ambit (Ambit License) grants worldwide rights to develop, manufacture and commercialize deuterated NAMs to treat neurologic and psychiatric disorders in all fields. Ambit will have to further specify the field of use in four years after license execution. Ambit License also includes rights to the technology solely owned by UMB and rights to future technologies (University Improvements) that might be developed by UMB in relation to the above mentioned technologies. Ambit License specifically excludes rights to future inventions made by NIH inventors outside of the scope of the licensed technology. This required significant discussion and negotiation.

NCI TTC Helps Support Growing Evidence of One-Dose HPV Vaccination Effectiveness through a Series of Agreements

Vaccines against HPV are very effective at reducing the risk of developing cervical and other types of cancer caused by persistent HPV infection. However, the current HPV vaccines employ a two- or three-dose regimen which can pose financial and logistical barriers to administration, especially in countries with limited resources, which often have high cervical cancer burden. Recently, there has been mounting evidence to suggest that a single dose of the HPV vaccine may confer sufficient protection and would be more cost effective, particularly in low and middle- income countries, and could lead to greater adoption of these vaccines.

To support NCI intramural researchers' efforts to investigate and disseminate findings about utilizing HPV vaccines on one-dose schedules, TTC executed a variety of agreements with multiple institutions and organizations from more than six countries since 2017, including, at least 4CDAs, 1 advisory agreement, 2 DTAs, 1 Memorandum of Understanding (MOU), and one MTA. Overall, one-dose administration for the HPV vaccine could have substantial public health impact including reducing cost and logistical difficulties inherent in vaccination with the current multiple-dose administration. NCI's Cancer Currents Blog highlights some of the most recent findings about single-dose HPV vaccines.

A new study confirms that widespread use of the HPV vaccine reduces the incidence of cervical cancer, particularly for women who are vaccinated when they are younger.
Credit: iStock

3-D Tissue Bioprinting Program C-RCA with UTMB and Texas A&M

3-D Tissue Bioprinting Program C-RCA for an NCATS collaboration with the University of Texas Medical Branch and the Texas A&M Engineering Experiment Station / Texas A&M University. The collaboration centers around the issue of spontaneous preterm birth (PTB), which is a significant contributor to neonatal mortalities and morbidities. Challenges in testing drug transport, metabolic changes, and teratogenicity have hindered PTB drug development. Current in vitro cell culture models and animal models have several limitations, prompting the development of several tissue chip models to help overcome limitations; however, the tissue chip models lacked high-throughput screening (HTS) capabilities. Therefore, the researchers submitted a proposal to the NCATS Tissue Bioprinting Program to develop a high-throughput 3-D bioprinted tissue chip to be used for HTS of large drug libraries. A research plan covering this scientific collaboration was included in the C-RCA that was executed between all three parties. Results are shared so that the knowledge derived from different improvements are available to the community.

CRADA Facilitates Collaboration Between NCI Surgery Branch and Turnstone Biologics to Develop and Evaluate Turnstone's Oncolytic Virus Technology to Improve the Generation of T-cells Reactive Against Cancer

Under a CRADA, the NCI Surgery Branch will evaluate whether tissues and peripheral blood from patients treated with Turnstone Biologics' proprietary oncolytic virus demonstrate the improved ability to generate tumor reactive tumor infiltrating lymphocytes (TILs) and peripheral blood lymphocytes (PBLs). This will be assessed using NCI proprietary methods and techniques for the isolation and characterization of tumor reactive mutations, both patient-specific (neoantigens) or patient shared (shared tumor antigens). TTC Senior Technology Transfer Manager (TTM), Aida Cremesti, Ph.D., supports NCI's Surgery Branch and negotiated this CRADA.

TTC Helps Support New Findings on Interferon Treatment Reducing Severity of COVID-19 in People with Certain Genetic Factors

Over the last two years, TTC negotiated and executed 29 MTAs and 1 DTA with institutions from 12 countries to receive DNA, other biological samples, and clinical data from patients with COVID-19 on behalf of the NCI Division of Cancer Epidemiology and Genetics (DCEG) for the COVNET study. The COVNET study employs a "phenotypic extremes" approach to rapidly determine severely and mildly affected COVID-19 patients for genomic interrogation to identify germline and somatic variants that may play a role in host susceptibility to disease and then correlate those phenotypic extremes with genetic variants. In July 2022, the NCI team and their collaborators published new findings on interferon treatment reducing severity of COVID-19 in people with certain genetic factors. In their work, the researchers uncovered a particular combination of genetic

Credit: iStock

variants in the OAS1 gene that were correlated to hospitalization and prolonged infection with SARS-CoV-2 and found that patients with COVID-19 treated with a recombinant interferon therapy had improved viral clearance, and patients with the genetic variants benefitted the most. Overall, these new findings suggest that interferon treatment may improve COVID-19 outcomes, particularly in patients with certain genetic variants who have impaired ability to clear infection. The TTC Unit led by Unit Supervisor, Lisa Finkelstein, Ph.D., negotiated the agreements to facilitate this study.

TTC Supports the Co-development of Medicines that Show Promise for Alzheimer's Disease Treatment

The National Institute on Aging (NIA) and AevisBio released promising results in March 2022 showing that NIA's licensed compound 3,6'-dithiopomalidomide (3,6'-DP or AEV103) decreases neuroinflammation and mitigates cognitive decline in animal models with progressive amyloid-beta generation and deposition. These results are part of an ongoing collaboration between Senior Investigator Nigel Greig, Ph.D., NIA, and AevisBio to evaluate a group of NIA's proprietary pomalidomide analogs for the treatment of certain symptoms relating to traumatic brain injury, Alzheimer's disease, Parkinson's disease, and multiple sclerosis. The investigators are eager to progress AEV103 to the clinic in coming years. Several TT agreements negotiated by Merissa Baxter, Ph.D., Kevin Chang, Ph.D., Nicole Guyton, Ph.D., and Nathan Whitman, Ph.D., TTMs at NCI TTC, helped facilitate this co-development effort.

For more details on this work, see the recent publication and news release.

Clinical Collaboration with Nouscom Will Test One of the First Ever Neoantigen-based Vaccines to Prevent Hereditary Cancers

The NCI Division of Cancer Prevention (DCP) is collaborating with Nouscom, a Switzerland-based cancer vaccine start-up, to evaluate the safety and efficacy of their proprietary neoantigen-based vaccine against Lynch Syndrome (LS), also known as hereditary non-polyposis colorectal cancer (HNPCC). LS is an inherited disorder in which affected individuals have a higher-than-normal chance of developing colorectal cancer (20-80%) and certain other types of cancer such as cancers of the uterus, ovaries, small bowel, stomach, urinary tract and skin, often before the age of 50. LS results from germline mutations in one of four DNA mismatch repair genes. Due to the inability to repair DNA errors in affected cells, LS carriers express unique neoantigens that stimulate the immune response. Because these neoantigens are expressed only in LS-associated neoplasms, neoantigen-based vaccination has the potential to offer a precisely targeted prophylactic strategy to prevent cancers in individuals with LS.

Scientists from NCI DCP are using Nouscom's vaccine to prime the immune system to elicit a powerful immune response in individuals with LS long before a cancer develops, while sparing non-malignant and healthy cells. This is the first clinical study of a neoantigen-based vaccine in LS carriers for the prevention of multiple cancer types. If successful, a similar strategy may work

in other related cancer syndromes, where either inherited, or random, genetic errors hinder the body's natural ability to repair mistakes in DNA. The Phase Ib/II clinical trial started in November 2022 and has broad eligibility. It is being conducted via DCP's Cancer Prevention-Clinical Trials Network at MD Anderson Cancer Center under a funding agreement with NCI and is sponsored and overseen by NCI DCP investigators. Sidra Ahsan, Ph.D., a TTM at NCI TTC, negotiated the Clinical Trial Agreement with Nouscom to establish the terms of this extramural clinical collaboration.

CRADA Supports NCI Surgery Branch Tumor Infiltrating Lymphocyte (TIL) Research

An ongoing CRADA with Iovance Therapeutics to develop Adoptive Cell Therapy (ACT) treatments using TIL provides support that is instrumental to the NCI Surgery Branch's mission and immunotherapy research. ACT utilizes a patient's immune cells after extracting them, expanding them outside of the patient to increase their numbers, and then reintroducing them back into the patient. NCI Surgery Branch is a leader in ACT research and is continuing to develop this approach to treat solid tumors, which account for over 90% of all cancers.

In 2011 NCI entered into a CRADA with the company that ultimately became Iovance, an industry partner strongly committed to moving a new class of ACT treatment using TIL technology through FDA approval and commercialization. This ongoing collaboration can point to notable milestones:

* FDA Breakthrough Therapy Designation for LN-145 for patients with recurrent, metastatic, or persistent cervical cancer with disease progression on or after chemotherapy (May 2019)
- Iovance's construction of a state-of-the art cell manufacturing facility in Philadelphia (May 2019)
- Recruitment of patients for a Phase 2 clinical trial to treat patients with recurrent, metastatic, or persistent cervical cancer (current).

Importantly, the CRADA also supports the Surgery Branch's broader, ongoing TIL research which is yielding notable results as highlighted in this NCI press release, February 2022: NCI study advances personalized immunotherapy for metastatic breast cancer

> "In a clinical trial of 42 women with metastatic breast cancer, 28 (or 67%) generated an immune reaction against their cancer. The approach was used to treat six women, half of whom experienced measurable tumor shrinkage. Results from the trial appeared Feb. 1, 2022, in the Journal of Clinical Oncology."

Senior TTM, Aida Cremesti, NCI TTC negotiated and manages the CRADA between the NCI Surgery Branch and Iovance.

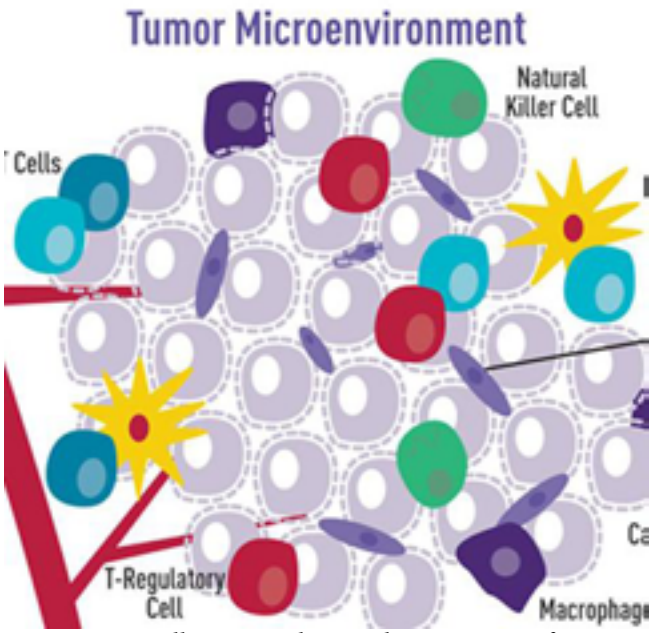
Exclusive License with Neogene Therapeutics to Develop CRISPR-Engineered T Cell Therapy Products

In January 2022, NCI executed an exclusive license with Neogene Therapeutics for the development of CRISPR-engineered T cell therapy products. The "*portfolio of TCRs [T cell receptors] developed in the NCI Surgery Branch laboratory of immunotherapy pioneer Steven Rosenberg, M.D., Ph.D., combined with Neogene's proprietary TCR isolation platform, provides expanded opportunities to target multiple neoantigens in individual patients. Neogene plans to evaluate both autologous and allogeneic T cell therapies targeting neoantigens in a broad spectrum of solid cancer*." (January 2022 Neogene press release). Among several potential advantages, the use of CRISPR-mediated gene engineering offers the prospect of a precisely edited cell therapy product with site-directed integration of the neoantigen-reactive TCR(s).

The Federal Register Notice reflects the scale of the patent estate. TTM Andrew Burke, Ph.D., NCI TTC, negotiated the license; he manages all licensing activity on behalf of the NCI Surgery Branch.

CODEFACS and LIRICS Software Transfer Agreements to Help Classify Cancer Patients and Match Them to Effective Treatments

Senior Investigator, Eytan Ruppin, M.D., Ph.D. and his team in the NCI Center for Cancer Research (CCR) Cancer Data Science Laboratory developed new software called CODEFACS (COnfident DEconvolution For All Cell Subsets) and LIRICS (LIgand-Receptor Interaction between Cell Subsets). The CODEFACS software allows researchers to predict cell-type-specific gene expression in a tumor sample from its bulk expression and LIRICS takes the output from CODEFACS to find clinically relevant ligand-receptor interactions between cell types. The researchers hope to use this software as a basis for classifying cancer patients to best match them to effective treatment strategies. This new software is already proving to be a sought-after tool. TTM Kevin Chang, Ph.D., NCI TTC, developed a software transfer agreement for NCI to provide the CODEFACS/LIRICS software to academic institutions worldwide and has executed more than 14 of these agreements. Furthermore, the software has also been provided to several other laboratories at NCI and is a part of several research collaborations between Dr. Ruppin and industry. An NCI article - "Researchers develop new method to estimate cell-type-specific information from bulk tumor data" – highlights this work.



Cartoon illustration showing how a variety of cell types surround and interact with cancer cells. This tumor microenvironment can impact tumor cell behavior.
Image source: NCI Visuals Online

Clinical Trial Agreement and Data Transfer Agreement Support NCI's New Diffuse Large B-cell Lymphoma Findings

New evidence from the NCI CCR Lymphoid Malignancies Branch (LMB) suggests that adding the targeted therapy ibrutinib (Imbruvica) to a standard chemotherapy regimen can improve how long some younger patients with a specific form of diffuse large B-cell lymphoma (DLBCL) live. NCI participated in a phase III clinical trial, called the PHOENIX trial, to evaluate the impact of adding ibrutinib to R-CHOP (standard of care chemotherapy regimen) in patients with newly diagnosed non-germinal center B-cell-like (GCB) DLBCL. Initial results from the trial showed that combining ibrutinib with the standard chemotherapy regimen did not help patients with non-GCB DLBCL live longer overall. However, by analyzing tumor biopsy samples from patients across the trial sites, Louis Staudt, M.D., Ph.D., chief, NCI LMB and this team, and their collaborators have shown that younger patients with specific genetic subtypes of non-GCB DLBCL, called MCD and N1, had an exceptional response to the treatment combination, with all such patients alive without disease three years after diagnosis.

NCI's participation in the PHOENIX trial was supported by a CTA and the analysis and interpretation of DNA/RNA sequencing data from the clinical trial was supported by a DTA. Both agreements were negotiated by TTM Ramona Bhattacharya, Ph.D., NCI TTC.

NHGRI Collaborations

NHGRI continued developing, negotiating, and administering a wide variety of technology transfer and alliance relationships and agreements in FY2022. NHGRI signed amendment #1 in 2022 and continues to administer a Data Sharing and Use Agreement (DUA) for the sharing of data between FUSION (Finland-United States Investigation of Non-insulin-dependent Diabetes Mellitus Genetics) members. One of the main goals of the project is identifying genetic risk factors that predispose to type 2 diabetes (T2D) and related intermediate traits. The FUSION Investigators at various international sites have collected biological samples from many diabetes families, as well as from type 2 diabetic, prediabetic, and normal glucose tolerant people. Various members of FUSION have sequenced and analyzed these samples. FUSION members would like to continue sharing the results and analyses of these sequencing efforts to further advance knowledge of causes of T2D and facilitate the development of potential treatments for T2D. The 8 current international FUSION members and parties to the DUA are: the National Human Genome Research Institute ("NHGRI"), Finnish Institute for Health and Welfare (Terveyden ja hyvinvoinnin laitos, THL, previously National Institute for Health and Welfare), University of Eastern Finland ("UEF"), The Regents of the University of Michigan ("UM"), Cedars-Sinai Medical Center ("CSMC"), University of North Carolina at Chapel Hill ("UNC"), the Jackson Laboratory for Genomic Medicine ("JAX-GM") and the University of California Los Angeles ("UCLA").

NHGRI continues to administer a Research Collaboration Agreement (RCA), entitled: "Preclinical development of potential gene editing therapy for Free Sialic Acid Storage Disorder" among NHGRI, Children's Hospital of Orange County (CHOC), Einstein School of Medicine, Greenwood Genetic Center (GGC), National Center for Advancing National Sciences (NCATS), and Centre

National de la Recherche Scientifique (CNRS in France) which in FY2022 included executing two Amendments and negotiating three related MTAs for transfer of human materials.

NHGRI was one of the first ICs to successfully work with the Office for Human Research Protections (OHRP) conflict of interest process involving the review of: 1) Complex intellectual property royalty issues; 2) The clinical protocol; 3) The role of the NHGRI PI in the clinical trial, particularly involving informed consent disclosures; 4) Data safety monitoring; and 5) Adverse Events. This resulted in a positive Conflicts of Interest Committee (COIC) Review Outcome. NHGRI established a Research Collaboration Agreement with the company, 23&Me. The focus of this collaboration is to study the health outcomes and covariates in data sets provided by 23&Me, define phenotypes based on this information, and develop a statistical analysis plan including univariate statistics.

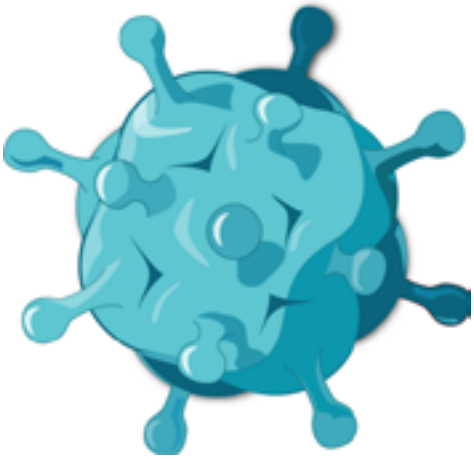
NHGRI continues to administer the model Reverse Phenotyping Core (RPC)-specific Data Contribution Agreement (DCA) we developed for the RPC with various data contributors including Inova Health Care Services, for patient derived data contributed as part of the RPC in the Center for Precision Health Research (CPHR) in the DIR.

NHGRI continues to collaborate with the NCATS-led Platform Vector Gene Therapy (PaVe-GT) pilot project. PaVe-GT is a pilot project that will test whether we can significantly increase the efficiency of gene therapy trial startup by using a standardized process, with the same capsid and manufacturing methods for four different rare diseases. NHGRI PI has been selected to collaborate on treatment for Propionic Acidemia (PA), which is one of four NIH diseases targeted for this program.

NHGRI established a multi-party Research Collaboration Agreement with the National Institute of Environmental Health Sciences (NIEHS) and University of California, San Francisco (UCSF) to characterize the metabolome of Systemic Lupus Erythematosus at different stages and compare with DNA Methylome using patient derived samples provided by UCSF.

NHGRI negotiated a Research Collaboration Agreement with the National Center for Advancing Translational Sciences (NCATS) at NIH and Simon Fraser University to develop new methods to properly evaluate the activity of non-inhibitory small molecule chaperones of glucocerebrosidase (GCase). The parties also aim to find and optimize novel series of noninhibitory GCase chaperones as possible disease modifying therapeutics for Gaucher and Parkinson's diseases.

NIH Initiates Antiviral Program for Pandemics (APP)

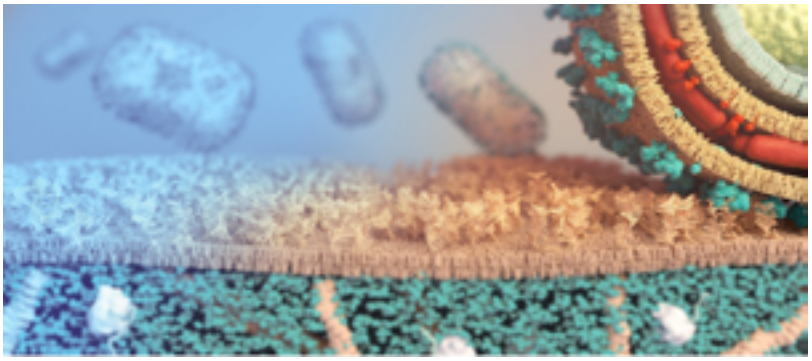


The NIAID Technology Transfer & Intellectual Property Office (TTIPO) developed templates and negotiated non-disclosure agreements to support the Antiviral Program for Pandemics (APP). The National Institutes of Health (NIH) established the multi-agency program to develop safe and effective antivirals. This initiative leverages the capabilities and resources of NIAID, NCATS, and BARDA to help speed the development

of antiviral compounds developed by commercial and academic organizations. APP focuses on antivirals for the treatment of RNA viruses of pandemic potential (Coronaviridae, including SARS-CoV-2, Paramyxoviridae, Bunyavirales, Picornaviridae, Filoviridae, Togaviridae, and Flaviviridae). Antivirals of interest are new chemical entities, which include small molecules and biotherapeutics, that directly block viral targets. Of particular interest is discovery and development of drug candidates with suitable safety profiles for broad use in an outpatient setting (e.g., oral, intranasal) to reduce viral burden in early stages of infection.

The NIAID TTIPO in collaboration with the NCATS Office of Strategic Partnerships (OSP) and the Biomedical Advanced Research and Development Authority (BARDA) developed multiparty template agreements to support interactions with outside organizations with antiviral compounds potentially suitable for inclusion in APP and regularly consulted with APP staff on potential intellectual property issues. Additionally, TIPPO, NCATS, and BARDA negotiated nearly three dozen four-party non-disclosure agreements with outside organizations to allow the exchange of confidential information in support of the evaluation of potential antiviral compounds for inclusion in APP.

NIAID Supports Mpox Treatment Clinical Trials in the U.S. and in Democratic Republic of The Congo (DRC)



The NIAID TTIPO negotiated Clinical Trial Agreements (CTAs) to support Mpox treatment clinical trials. The NIAID Division of Clinical Research (DCR) in partnership with the National Institute for Biomedical Research (INRB) of the Democratic Republic of the Congo (DRC) initiated a clinical trial to evaluate the antiviral drug tecovirimat, also known as TPOXX, in adults and children with Mpox. The trial is evaluating the drug's safety and its ability to mitigate Mpox symptoms and prevent serious outcomes, including death. TPOXX, made by the pharmaceutical company SIGA Technologies, Inc. (New York), is approved by the U.S. Food and Drug Administration for the treatment of smallpox. The drug impedes the spread of virus in the body by preventing virus particles from exiting human cells, and targets a protein that is found on both the virus that causes smallpox and the Mpox virus. NIAID and INRB also initiated a concurrent clinical trial evaluating the use of the JYNNEOS Mpox vaccine in health care staff in the DRC supporting the TPOXX clinical trial.

The NIAID TTIPO supported both clinical trials by negotiating CTAs for the supply of TPOXX for the clinical trial and for the conduct of both clinical trials by INRB sites in the DRC. TTIPO also played an integral role in negotiations with the DHHS Administration for Strategic Preparedness and Response (ASPR) and Bavarian Nordic AS, the manufacturer for the JYNNEOS vaccine, to allow doses of the JYNNEOS vaccine from the Strategic National Stockpile (SNS) to be used for the vaccination of health care workers in the DRC.

NIEHS Partners with ChromaDex to Research and Develop Therapies for Parkinson's Disease

NIEHS has entered a materials CRADA (M-CRADA) with ChromaDex, Inc. to study the effects of nicotinamide riboside on the motor function of mice that model Parkinson's Disease (PD). PD is a neurodegenerative disorder that becomes more prevalent in the population with age, and results in a gradual loss of motor function in the patient. PD's increased prevalence with age makes it burdensome for public healthcare for populations like the U.S. that has a substantial proportion of its population over the age of 60. This research partnership between NIEHS and ChromaDex will provide mechanistic insight for the role of nicotinamide riboside (a precursor for NAD+ used by mitochondria to produce energy in cells) in ameliorating PD.

Credit: iStock/Nadezhda Buravieva

NIEHS Performs a Clinical Study of Idiopathic Pulmonary Fibrosis with Halo Biosciences

Idiopathic Pulmonary Fibrosis (IPF) is a progressive, fatal lung disease that has high mortality and a paucity of therapeutic options. Hyaluronan is an extracellular matrix component that is implicated in lung diseases like IPF. NIEHS will collaborate with Halo Biosciences, Inc. on a clinical study that will evaluate the safety and efficacy of hymecromone (4-methylumbelliferone [4-MU]), reducing hyaluronan in IPF patients. This collaboration facilitates the development of a therapeutic for a lethal disease for which there is a need for better treat options and outcomes.

NIEHS Partners with the University of Birmingham (United Kingdom) to Create Toxicology Tools

NIEHS and the University of Birmingham (UK) are collaborating on a research project that will develop and test new protocols to obtain information about toxic exposures and the mechanism of action of potentially toxic substances from a single experiment, by using novel metabolomics methodologies and measurements of ADME (Absorption, Distribution, Metabolism, and Excretion) and TK (Toxicokinetic) properties.

NIEHS Co-Develops COVID-19 Diagnostic with Emory University

NIEHS and Emory University have entered into a Research Collaboration Agreement (RCA) to develop a method to analyze nasopharyngeal, oropharyngeal, saliva, and/or blood samples from COVID-19 patients collected very early in the COVID-19 disease course (e.g. at diagnosis of infection) and prospectively determine or predict COVID-19 disease severity (e.g. asymptomatic, mild, severe, long COVID, etc). The method seeks to both quantify the viral content in the biological sample and to also obtain an informative gene expression profile from patient cells. These profiles will be compared with patient symptoms and clinical data using machine learning to identify biomarkers that are prognostic for COVID-19 disease severity.

Credit: iStock/nuttapong punna

NIEHS Collaborates with AbbVie to Seek Treatment for Precocious Puberty

Puberty that happens early is called precocious puberty and can be caused by premature activation of the hypothalamus or pituitary gland. Precocious puberty warrants immediate clinical attention and treatment to prevent short stature in adulthood and to mitigate the distress that secondary sexual characteristics and menstrual periods may create for a young child and her family. In a Cooperative Research and Development Agreement, the NIEHS and AbbVie will conduct a dose-finding study of an oral GnRH antagonist (elagolix) in the treatment of central precocious puberty in girls.

Data Transfer Agreements (DTAs) for Pooled Phthalate Project

Phthalates are a group of chemicals that make plastics more durable and are found in everyday products, such as personal care products, detergents, and food packaging. OTT negotiated 16 separate DTAs to receive data from 16 pregnancy and birth cohorts to explore phthalate exposure and preterm birth. After analyzing data from more than 6,000 pregnant women in the United States, NIEHS researchers found that women with higher concentrations of several phthalate metabolites in their urine were more likely to deliver their babies preterm. Learn more: NIEHS press release.

NIH National Institute of Environmental Health Sciences

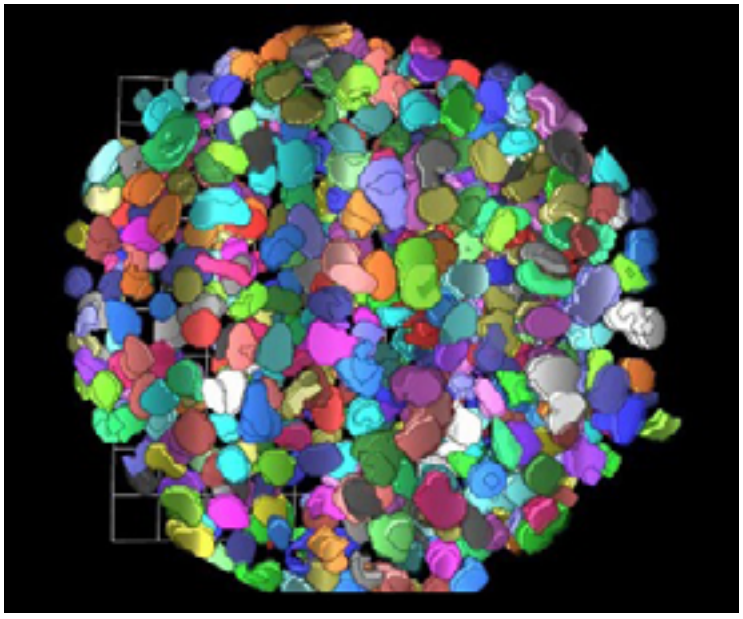
AWARDS, PRESENTATIONS, AND PUBLICATIONS

Sury Vepa wins NCATS Mentorship Award

Dr. Suryanarayana (Sury) Vepa was honored with the 2022 NCATS Mentorship Award which recognizes employees who have made significant contributions to the lives of fellow colleagues through mentoring activities. Sury distills his 20-plus years of experience into bite-sized wisdom allowing new employees to excel in the world of patenting and licensing. Sury is highly valued by his colleagues at NCATS, NIH, and the wider technology transfer community for his depth of knowledge, experience, and clarity of thought. His experience in licensing is impressive not only in terms of the number of agreements, but also their complexity. He shares his knowledge and personal know-how most freely and thoroughly in two-way conversations that leave the recipient enriched with new ideas and tools. Sury believes that the greatest service he can provide NIH in addition to his own work is by shaping future generations of IP professionals. He embodies the “open door policy” making himself available to share his wealth of knowledge freely. Closing with a quote from a current mentee, “Sury is an encyclopedia of government technology transfer and the resident mentor for patenting and licensing related matters.”

IP, Licensing, Tech Transfer Presentations

NCATS Office of Strategic Alliances (OSA) presented both internally and externally on various patenting, licensing, and technology transfer topics. Internally (or within NCATS), we presented our entire NCATS-led patent portfolio to our new NCATS Director and developed a licensing basics seminar and presented it to all NCATS staff. This expanded on the software licensing presentation that we developed and presented to our Bioinformatics groups. Also, we played a key role in the Translational Science Training Program - NCATS Preclinical Development Workshop, where we presented on, “Innovation and Translation Through Collaborations: NCATS Models and Success Stories”. This enabled scientists to understand a roadmap for success in terms of establishing the right IP and tech transfer framework. Externally (outside of NCATS/NIH) we presented on the following topics: “Contemporary Topics in Open Source & Dual Licensing” at the AUTM Software Course”; and “Inter-institutional Inspiration: IIAs and How to Make Them Work for Your Office” at the AUTM Operations and Compliance Course, Agreements & Finance Track.



NCATS Publishes on Technology Transfer Tactics

The October 2022 issue of Technology Transfer Tactics featured the article, "*NCATS breaks the mold: Case studies of unique tech transfer mechanisms*" authored by Ami D. Gadhia, JD, LLM, CLP, Rebecca A. Erwin-Cohen, Ph.D., Krishna (Balki) Balakrishnan, PhD, MBA, and Lili M. Portilla, MPA.

The article provided an excellent overview of the nature of technology transfer within the OSA. Using three case studies, the authors highlighted innovative approaches to complex, collaborative, multi-party ventures, made possible due to the unique nature of NCATS acting both as a granting institution administered by our extramural program and a research enterprise fueled by the Division of Pre-Clinical Innovation (DPI).

Specifically, the use of Cooperative Agreement (CA) grant mechanism has been leveraged to afford NCATS remarkable flexibility in assembling diverse teams. The three case studies demonstrating the versatility of CA grants spanned across drug development life cycles, commercialization pipelines, and institution types. The first case study highlighted an academic institution and their Start-up, collaborating with NCATS Intramural labs. Here NCATS supported Cincinnati Children's Innovation Ventures through the "Valley of Death" to a successful spin-off, Kurome Therapeutics, along the way deploying tools such as an IIA and CRADA.

The second and third case studies explored the NCATS 3-D Tissue Bioprinting and the New Therapeutic Uses (NTU) programs, respectively. Bioprinting and allied methods, unique scaffolds and growth conditions, as well as new testing and imaging techniques will be developed in a Consortium brought together under a CA grant mechanism wherein the funding opportunity announcement (FOA) contained language to ensure that technology transfer and IP provisions would be agreed upon before the grant work could begin. A cooperative research collaboration agreement (C-RCA) was used with all the grantees/collaborators to enable execution of the agreements in a timely fashion and to work within their granting/budgeting constraints.

Finally, the NCATS NTU exists to repurpose existing molecules for new indications and supports development through Phase II clinical trials. The technology transfer tools employed for NTU range from clear IP roadmap set up for participating partners to incorporation of innovative template agreements designed to streamline the process and reduce prolonged negotiations. These template agreements included tailored confidential disclosure agreement (CDAs), memoranda of understanding (MOU), and C-RCAs. These examples demonstrate the power of non-linear, innovative, and tailored approaches in tackling outstanding public health challenges. The succinct and well-written article can be found on page 145, volume 16, issue no. 10 of Technology Transfer Tactics (TechTransferCentral.com/TTT).

"Hiding in Plain Sight: Surprising Pharma and Biotech Connections to NIH's National Cancer Institute"

A manuscript published in the Journal of Commercial Biotechnology (JCB, Volume 27, Number 2, 2022) explores and analyzes collaborations and licensing partnerships between NCI and NCI TTC client Institutes and life science and pharmaceutical companies. The findings highlight the importance of collaborations and licensing for both early-stage and established life science and pharmaceutical companies. It is the first study to detail the substantial, but not well-recognized, relationship between collaborating and licensing with the NCI and other NCI TTC client Institutes and successful outcomes in early-stage life science companies. The paper was authored by Berna Uygur, Ph.D., a former Cancer Research Training Award (CRTA) fellow with TTC, now a Full-time equivalent employee (FTE) at NCI Cancer Therapy Evaluation Program (CTEP); Unit Supervisor Michael Pollack, Ph.D., TTC; and Special Advisor, Steven Ferguson, CLP, NIH Office of Technology Transfer (OTT). The primary discovery of their analysis is that early-stage life science companies with "Successful Outcomes" had a nearly three times greater likelihood of having collaborative interactions with the NIH, mediated by NCI TTC.

Some notable findings uncovered by the authors include:

- While 36.3% of early-stage life science companies with "Successful Outcomes," had established collaborations mediated by TTC, only 12.9% with "Unsuccessful Outcomes" had established collaborations. Thus, companies with "Successful Outcomes" were three times more likely to have collaborations mediated by NCI TTC than companies with an "Unsuccessful Outcome."
- All of the Top 20 pharmaceutical companies have some sort of TTC-mediated collaborations and/or licensing partnerships - Licensing Agreements, Material Transfer Agreements (MTA), Clinical Trial Agreements (CTA), and Cooperative Research and Development Agreements (CRADA). Most strikingly, TTC mediated at least two different collaborative and licensing agreements with all Top 20 pharmaceutical companies.

Credit: iStock/lemono

- More than 1,700 former fellows or employees who received training at NCI and the NIH Institutes served by TTC are current employees of the Top 20 pharmaceutical companies. Thus, NCI and the NIH Institutes served by TTC also contribute toward educating and preparing trainees to join the Top 20 pharmaceutical companies as a possible career step.

TTC Staff Honored with 2021 NIH Clinical Center CEO Award

TTC's Ken Rose, J.D. and Tedd Fenn, J.D. manage the technology transfer and patent activities for the NIH Clinical Center. When the COVID-19 pandemic broke out in 2020 and as it continued in 2021, their "fast and competent action" [as stated by the Principal Investigator] coordinating, drafting, and negotiating complex agreements with universities and companies enabled the Clinical Center to partner with many institutions across the globe to help diagnose COVID-19

patients and predict clinical outcomes quickly and accurately. For this work, the Clinical Center recognized Mr. Rose and Mr. Fenn as part of a team for outstanding achievements with a 2021 NIH Clinical Center CEO Award.

"RADx® Tech & ATP" Team Win 2021 NIH Director's Award

The NIH launched the Rapid Acceleration of Diagnostics (RADx®) initiative to speed up innovation in the development, commercialization, and implementation of technologies for COVID-19 testing. One aspect of the RADx® initiative is to provide government financial and other support to companies developing new technologies to combat the COVID-19 crisis. In support of this effort, Richard Girards, Jr., Esq., MBA, a TTM with NCI TTC, volunteered as a Contracting Officer Representative to oversee aspects of a $6.2M contract under the RADx® program to Xtrava Health (Santa Clara, California) to accelerate the development and launch of its 15-minute, lateral-flow rapid antigen test. Girards along with the RADx® and Advanced Technology Platforms (ATP) team was recognized for their support of this project with a 2021 NIH Director's Award.

Credit: iStock/PCH-Vector

TTC Associate Director Selected for NIH Office of the Director Honor Award

The NIH Office of the Director selected TTC Associate Director, Kathleen Carroll, Ph.D. for its OD Honor Award "in recognition for notable resourcefulness and outstanding creative efforts in developing a process to mitigate risk related to removal of copies of Federal records by departing staff." Carroll assisted the NIH Information Management Branch in creating a workflow for staff leaving NIH employment who also request to take copies of information they developed while working at NIH.

TTC Technology Transfer Manager Honored with Hubert H. Humphrey Award for Service for Supporting the HHS Response to an Unprecedented Humanitarian Crisis in 2021

HHS recognized Lauren Nguyen-Antczak, Ph.D., J.D., a TTM with NCI TTC, for her volunteer efforts and contributions to the health, safety, and well-being of our nation's most vulnerable children and families during Operation Artemis. Nguyen-Antczak answered the call to serve as volunteer to assist with the HHS mission to reunify Unaccompanied Children at the San Diego Emergency Intake Site (SD EIS) at the personal request of NCI Executive Officer, Donna Siegle. In total, she volunteered for six weeks at the SD EIS as part of Operation Artemis, an HHS joint operation with the Department of Homeland Security and FEMA and its successor Operation Apollo following the departure of FEMA.

NHGRI Awards, Presentations, and Publications

NHGRI has continued its tradition of volunteering and community service activities in FY2022, with all Office members contributing to various technology transfer activities and receiving numerous awards.

Claire Driscoll, Director, served as a member of the AUTM (formerly known as the Association of University Technology Managers) Annual Meeting Planning Committee, organized a career development session at the AUTM 2022 conference, and is helping to organize 2023 annual conference to be held in Austin, TX in February 2023.

Claire Driscoll served on the NeuroNext (NN) 109 study team (GNE myopathy Phase 2/3 clinical trial which is being carried under a NN extramural cooperative agreement in conjunction with a 4-party CRADA which involves NHGRI, NINDS, NIAMS and a company, Leadiant Biosciences, and the trial started enrolling patients in 2022).

Claire Driscoll served on the NHGRI extramural division's Third-Party Engagement workgroup and previously helped to develop an internal as well as external publicly available guidance documents to be used by NHGRI extramural program directors and program officers as well as used by NHGRI grantees.

Ms. Driscoll and Eggerton Campbell volunteered for the USPTO's Patents for Humanity project and served as a second round Judges (February 2022).

Credit: iStock/Roman Yaroshchuk

Claire gave several presentations during FY2022 including a presentation to senior NHGRI faculty, a presentation to the NIH Gene Therapy Task Force entitled "Clinical Development of Gene Therapies: What Do IP, Licenses and CRADAs Have to Do with it?" and a presentation to the NHGRI Division of Intramural Research Board of Scientific Counselors entitled "Technology Transfer at NHGRI & NIH".

In December 2021, Anna Solowiej, Associate Director, received the 2021 Genome Recognition of Employee Accomplishments and Talents (GREAT) Award "For providing exceptional leadership and advice as a technology transfer and intellectual property expert to advance NHGRI's scientific and biomedical initiatives." She was nominated by an NHGRI investigator.

Anna Solowiej served as the Program Chair of AUTM (formerly known as the Association of University Technology Managers) Annual Meeting Planning Committee, helping to organize the 2022 and 2023 annual meetings and coordinating work of about 25 Committee members.
Dr. Solowiej completed Senior Leadership Training in November 2021.

Anna Solowiej and Eggerton Campbell completed the Contracting Officer's Representative (COR) Level I and became COR Level I certified in July 2022.

Anna Solowiej and Eggerton Campbell have volunteered on an NIH-wide Technology Transfer User Group (TTUG), helping to coordinate technology transfer transition to a new database system and its related services.

Anna Solowiej and Eggerton Campbell have volunteered on an NIH-wide Patent Legal Services (PLS) group to coordinate implementation of the new patent services contract.

Dr. Campbell organized an AUTM panel for the 2022 AUTM annual conference (which became a virtual meeting and some panels, like this, had to be cancelled) on the topic of licensing terms for priority review vouchers for rare diseases (this session is now scheduled to take place at the 2023 AUTM annual conference).

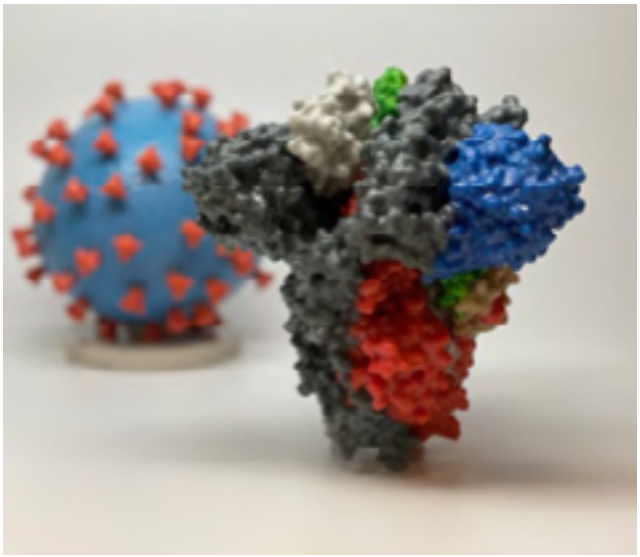
Dr. Campbell took the lead in successfully interacting with Office for Human Research Protections (OHSRP) conflict of interest process involving the review of 1) Complex intellectual property royalty issues; 2) The clinical protocol; 3) The role of the NHGRI PI in the clinical trial, particularly involving informed consent disclosures; 4) Data safety monitoring; and 5) Adverse Events. This led to a positive review outcome.

Eggerton Campbell served as a member of the NIH Platform Vector Gene Therapies (PaVe-GT) Project team.

Sangeetha Raghavan volunteered as a member of the NIH-wide Enterprise Technology Transfer (ETT) group for purpose of evaluating the process for tagging and classifying technologies to be listed within the soon-to-be established Inteum Minuet database.

NIAID Won 2022 FLC Award for Excellence in TT for Antibody-Focused Collaboration with AbCellera

Originally established to study influenza, a partnership between NIAID and biotechnology company AbCellera quickly pivoted to become a major player in the development of antibody-based COVID-19 therapies. One of those therapies, bamlanivimab, has been authorized to treat mild to moderate COVID-19 symptoms in patients who are at risk for more severe disease, when combined with another antibody.



The NIAID Technology Transfer & Intellectual Property Office (TTIPO) negotiated a Research Collaboration Agreement (RCA) when NIAID and AbCellera first joined forces in 2018, aiming at identifying and characterizing antibodies against the influenza virus. The relationship evolved to encompass methods for studying coronavirus antibodies, as well as developing coronavirus monoclonal antibodies that could be used for prevention or treatment. This partnership, along with the coronavirus research it supported, paid huge dividends when the COVID-19 pandemic emerged just a few years later.

NIAID scientists engineered the spike protein based on their previous work with other types of coronaviruses. This step was crucial, because live SARS-CoV-2 or isolated “natural” SARS-CoV-2 spike protein cannot be used effectively to identify and isolate neutralizing antibodies. AbCellera then used its proprietary high-throughput, machine-based process — developed as part of the Defense Advanced Research Projects Agency (DARPA)’s Pandemic Prevention Platform — to find the most potent antibodies among millions of antibody-producing cells from a patient who had recovered from COVID-19 infection.

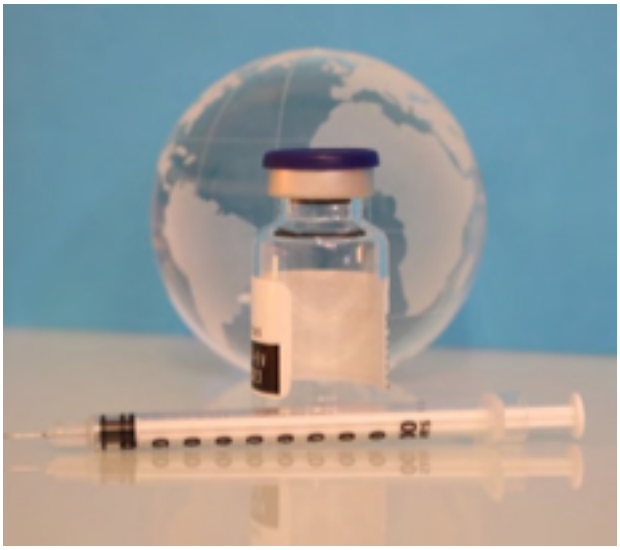
TTIPO negotiated an exclusive license with AbCellera for the intellectual property covering these antibodies to expedite development of antibodies for treatment and prevention. AbCellera further partnered with Eli Lilly, which had the capacity and resources to develop a therapeutic antibody to SARS-CoV-2.

NIAID Won 2022 FLC Impact Award for Industry Partnerships That Facilitated NIAID Trials to Support Authorization of COVID-19 Vaccine Candidates

In response to the COVID-19 outbreak, researchers and technology transfer professionals at NIAID accelerated collaborations with COVID-19 vaccine developers that enabled the rapid study, emergency authorization and public use of urgently needed vaccines.

The results of these efforts became evident between December 2020, when the COVID-19 vaccination campaign began, and April 2021, when the rate of dose administration peaked. During that period, the number of COVID-19 cases in the United States dropped precipitously. It is estimated that global vaccination against COVID-19 could prevent several trillion dollars per year in economic losses.

The technology transfer effort began shortly after the genetic sequence of Moderna’s first vaccine candidate (mRNA-1273) was finalized and appropriate preclinical data had been obtained. NIAID’s Division of Microbiology and Infectious Diseases (DMID) took the lead as the sponsor of the “first in human” clinical study of this experimental vaccine.



In parallel with development of the clinical study protocol, DMID and TTIPO rapidly negotiated a Clinical Trial Agreement (CTA) with Moderna to obtain mRNA-1273 for the study. The first Phase I trial participant was dosed on March 16, 2020 — just five days after the COVID-19 outbreak was declared a pandemic. Positive findings from this trial led to Phase II and Phase III trials that were supported by funding from the Biomedical Advanced Research and Development Authority (BARDA). The Phase III trial used NIAID’s newly formed COVID-19 Prevention Trials Network (CoVPN).

Based on the outcomes of these trials, on Dec. 18, 2020, the Food and Drug Administration (FDA)

granted mRNA-1273 Emergency Use Authorization (EUA) for the prevention of COVID-19 in individuals 18 years and older.

SARS-CoV-2 variants and pediatric populations represent two classes of indications that remain to be fully addressed. TTIPO negotiated an amendment to the CTA with Moderna that enabled evaluation of new vaccine candidates against COVID-19 variants. In addition, TTIPO negotiated a new CTA with Moderna to initiate a trial (the KidCOVE Study) of the company's original vaccine in children aged 6 months to 12 years.

DMID initiated another adaptive protocol for the "MixNMatch" vaccine trial to evaluate delayed doses (boosts) of a vaccine other than the one initially administered. TTIPO developed new agreements to cover the vaccine candidates included in this study.

If many of these clinical trials produce positive safety and efficacy results, a larger proportion of the US and global population could receive COVID-19 vaccines or booster shots to improve their collective immunity against SARS-CoV-2. These efforts, in part, could help to tackle the COVID-19 pandemic.

NIEHS OTT Director Received an NIEHS Individual Merit Award

Director of NIEHS OTT, Dr. Sharon Soucek received a NIEHS Individual Merit Award for exemplary performance in transforming and improving the Office of Technology Transfer by expanding services and outreach to the NIEHS scientific community.

NINDS Awards

Dr. Lola Olufemi received a NINDS Director's Outstanding Innovation individual award for her role in the above-referenced NINDS-NIA-JAX agreement in support of CARD.

Drs. Oksana Dukhanina and Sue Ano were recognized as part of a NINDS Director's Spotlight on Science: Advances and Collaboration group award for their role in negotiating and executing a clinical trial agreement, which included transfer of IND sponsorship, with a corporate partner for commercial development of a gene therapy.

Credit: iStock/ajijchan

APPENDIX

HHS Technology Transfer Offices

NIH OTT - NIH Office of Technology Transfer

https://www.techtransfer.nih.gov

CDC - Centers for Disease Control and Prevention

CDC Office of Technology and Innovation

https://www.cdc.gov/os/technology/techtransfer/aboutus.htm

NCATS - National Center for Advancing Translational Sciences

NCATS Office of Strategic Alliances

https://ncats.nih.gov/alliances/about

NCI - National Cancer Institute

NCI Technology Transfer Center

https://techtransfer .cancer .gov

Service Center for:

- CC - NIH Clinical Center
- CIT - Center for Information Technology
- NCCIH - National Center for Complementary and Integrative Health
- NEI - National Eye Institute
- NIA - National Institute on Aging
- NIDA - National Institute on Drug Abuse
- NICHD - *Eunice Kennedy Shriver* National Institute on Child Health and Human Development
- NIMHD - National Institute on Minority Health and Health Disparities
- NLM - National Library of Medicine

NHGRI - National Human Genome Research Institute

NHGRI Technology Transfer Office

https://www.genome.gov/techtransfer

NHLBI - National Heart, Lung, and Blood Institute

NHLBI Office of Technology Transfer and Development

https://www.nhlbi.nih.gov/research/tt

Service Center for:

- NIAAA - National Institute on Alcohol Abuse and Alcoholism
- NIAMS - National Institute of Arthritis and Musculoskeletal and Skin Diseases
- NIBIB - National Institute of Biomedical Imaging and Bioengineering
- NIDCD - National Institute on Deafness and Other Communication Disorders
- NIEHS - National Institute of Environmental Health Sciences
- NINR - National Institute of Nursing Research

NIAID - National Institute of Allergy and Infectious Diseases

NIAID Technology Transfer and Intellectual Property Office

https://www.niaid.nih.gov/research/technology-transfer-and-intellectual-property-office

Service Center for:

- CDC - Centers for Disease Control and Prevention (CDC)

NIDCR - National Institute of Dental and Craniofacial Research

NIDCR Office of Technology Transfer and Innovation Access

https://www.nidcr.nih.gov/research/NIDCRLaboratories/Intramural_Technology_Transfer_Office

NIDDK - National Institute of Diabetes and Digestive and Kidney Diseases

NIDDK Technology Advancement Office

https://www.niddk.nih.gov/about-niddk/offices-divisions/technology-advancement-office

Service Center for:

- ORS - Office of Research Services

NIMH - National Institute of Mental Health

NIMH Office of Technology Transfer

https://www.nimh.nih.gov/research/research-conducted-at-nimh/scientific-director/office-of-technology-transfer/index.shtml

NINDS - National Institute of Neurological Disorders and Stroke

NINDS Technology Transfer Office

https://tto.ninds.nih.gov/