National Institutes of Health
Technology Transfer

FISCAL YEAR 2024

NIH TECHNOLOGY TRANSFER

ANNUAL REPORT

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INTRODUCTION

In FY2024, the efforts of the NIH technology transfer community continued to foster significant and meaningful public health impacts. As one example, Amtagvi®, developed by Iovance Biotherapeutics, Inc., became the 48th FDA-approved product developed under a license to an NIH invention. Amtagvi® is the first and the only one-time, individualized T-cell therapy to receive FDA approval for a solid tumor cancer. In total, over 1,000 licensed products developed from NIH technologies, ranging from drugs to research reagents, were on the market in FY 2024, yielding $210.6 million in royalty income.

Tara Kirby

The Office of Technology Transfer (OTT) continued to contribute to NIH's technology transfer community in important ways. These included extensive enhancements and data integrity efforts to the NIH Enterprise Technology Transfer (ETT) data system, which launched in December 2022. In recognition of this ongoing effort, OTT was honored to receive the GOVTECH CONNECTS Digital Health Transformation Award. This award recognizes Federal and Military Health IT programs that harness the power of emerging technologies to advance their missions and spotlight the dedicated teams behind this groundbreaking work. OTT also worked collaboratively with NIAID, HHS, and other stakeholders to resolve a longstanding license dispute.

OTT continued to provide key services and support functions for all the NIH technology transfer offices 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 site and public websites. It also continued outreach activities to increase awareness of NIH as a premier technology transfer partner, including exhibiting and presenting at the 2024 Biotechnology Innovation Organization (BIO) International Convention.

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.

Sincerely,

Tara Kirby

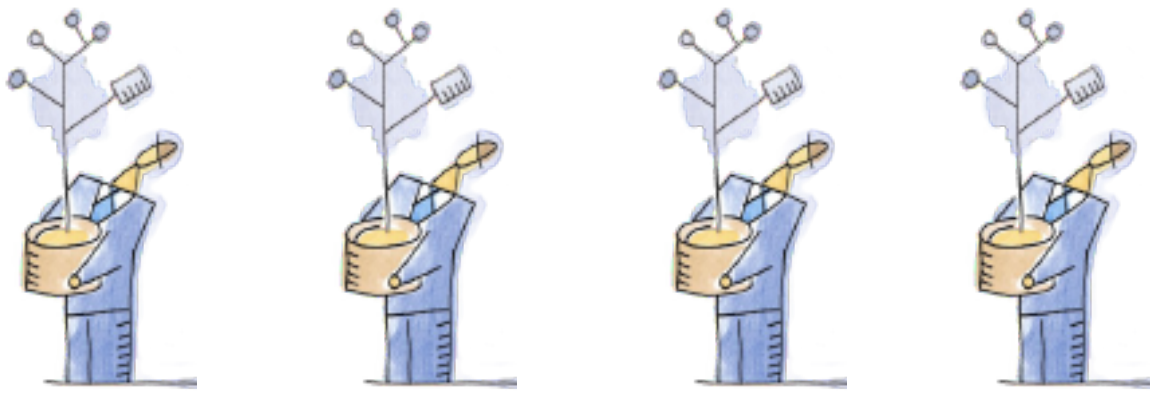
Director, Office of Technology Transfer

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.

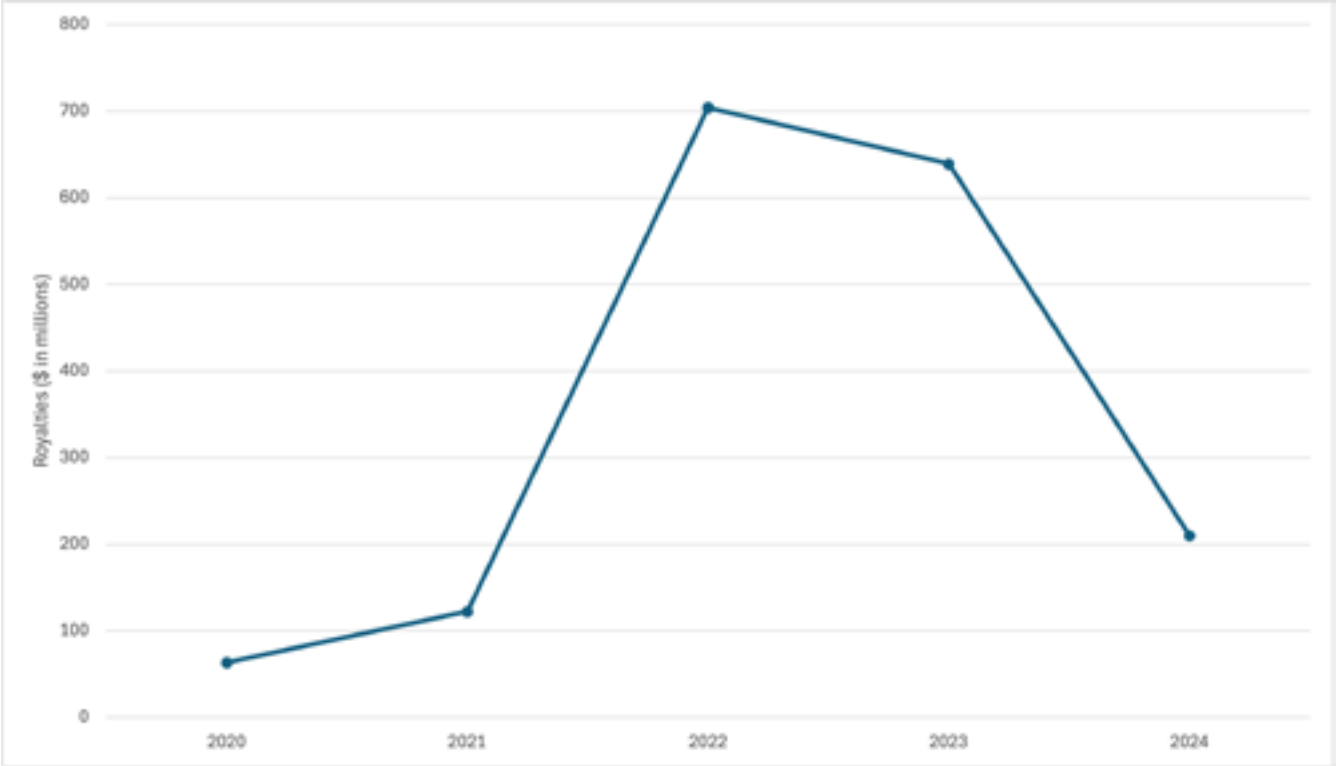
INVENTIONS AND AGREEMENTS

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.

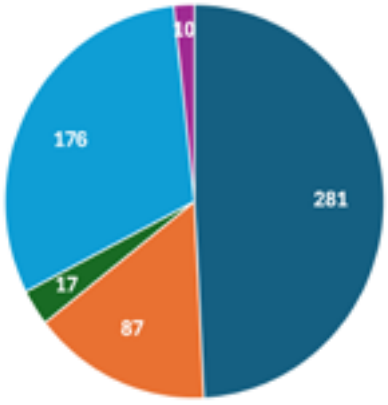
In FY2024 NIH brought in $210.6 million in royalty income. There were 251 invention disclosures, 235 patent applications filed, 131 U.S. patents issued, and 291 executed licenses. A graphical breakdown of these numbers is provided on the following pages.

Royalty Income

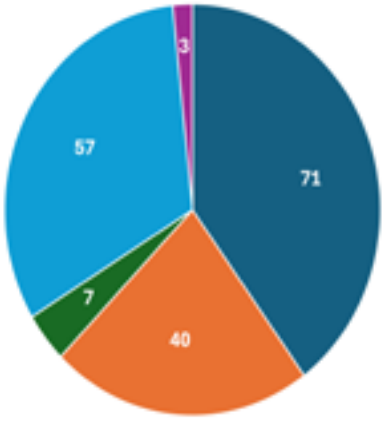


CRADA Metrics

Active CRADAs

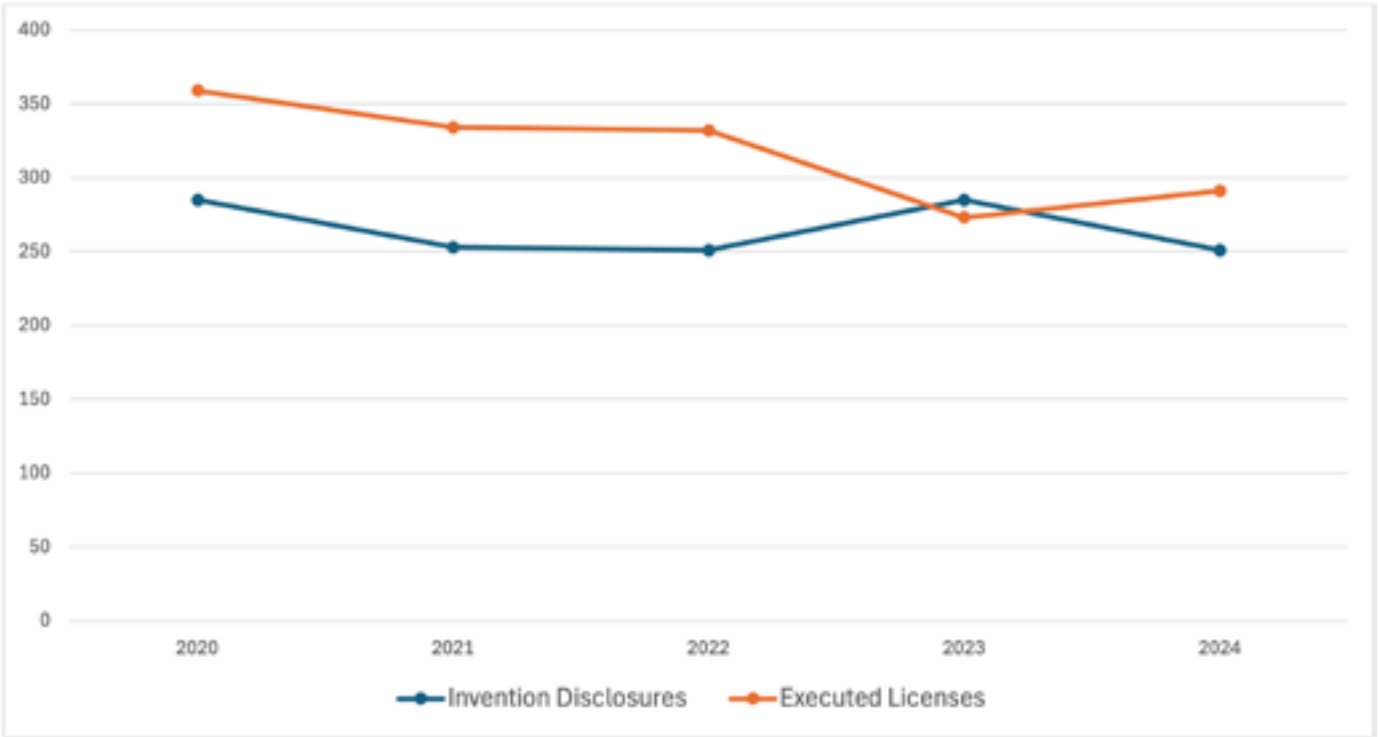


New CRADAs

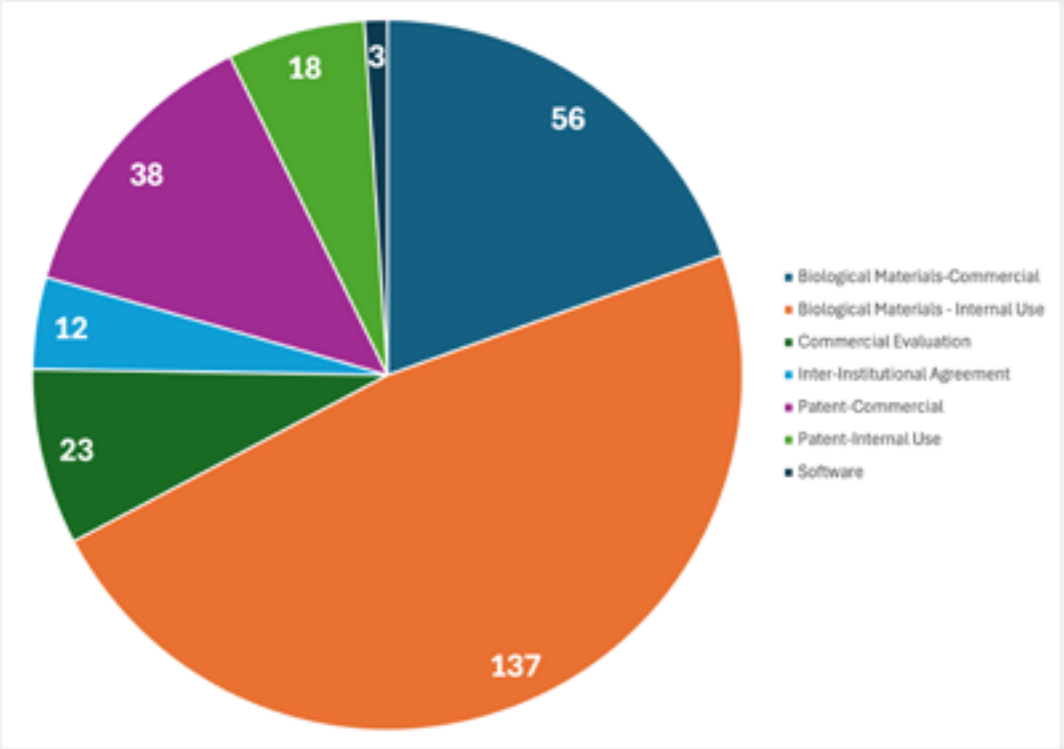


Standard Material C-RCA Clinical Other

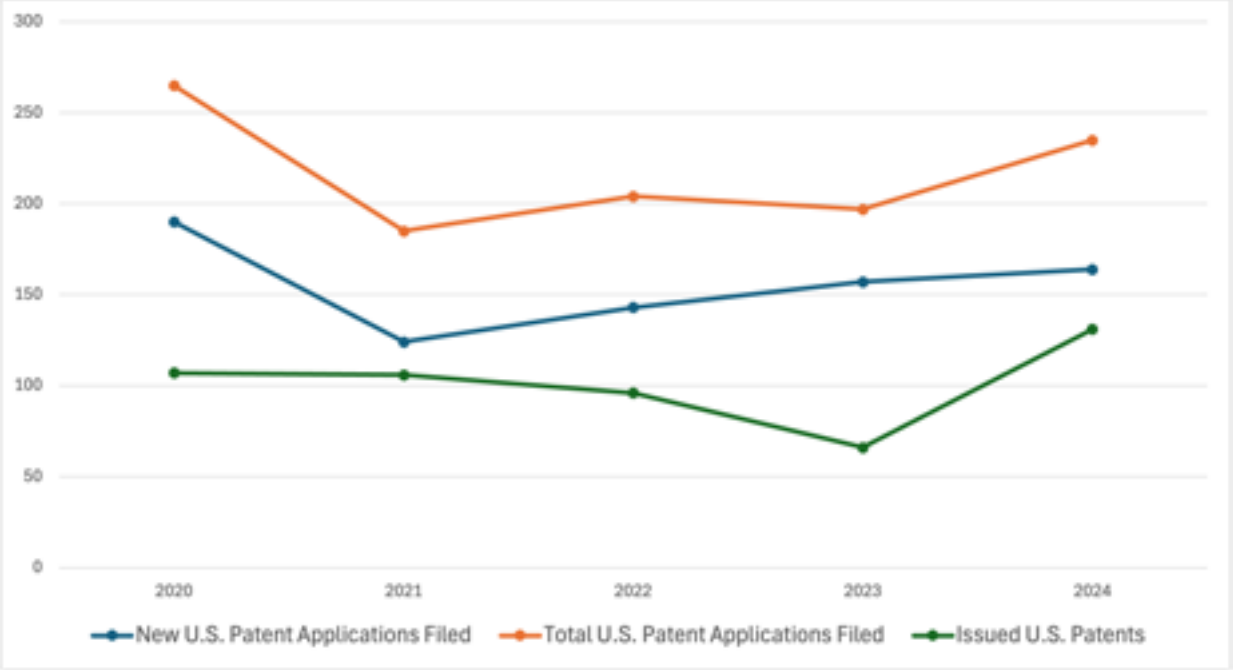
Inventions and Licenses



Licenses in a Fiscal Year by Type of Agreement



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 of 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 ensure 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., many 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 101 issued US patents, 344 issued foreign patents, and 1,386 total patent applications.

Further, as a means for accelerating innovation and commercial development, NCATS has licensed many of its technologies (over 50 commercial licenses and nearly 100 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.

NCI - National Cancer Institute

NCI Selects Two Assays for the Vanguard Study on Multi-Cancer Detection Tests

NCI selected two assays to be included in its Vanguard Study on multi-cancer detection (MCD) tests. The two chosen assays are the Shield™ Multi-Cancer Detection Test by Guardant Health, based in Palo Alto, California, and the Avantect® Multi-Cancer Detection Test by ClearNote Health, based in San Diego, California. NCI Technology Transfer Center's (TTC) Sidra Ahsan, Ph.D. negotiated two clinical trial agreements for NCI's Vanguard Study on MCD tests. Dr. Ahsan also negotiated several agreements for the performance verification studies for companies interested in applying to have their assays considered for the Vanguard Study.

Credit: iStock/Nuttapong Punna

The Vanguard Study is a pilot study conducted by the NCI's Cancer Screening Research Network (CSRN), which is a clinical trials network established to assess new cancer screening technology. The study aims to evaluate the feasibility of using multi-cancer detection (MCD) tests in future randomized controlled trials. The Vanguard Study will enroll up to 24,000 participants to help shape the design of a much bigger randomized controlled study. This larger experiment will assess whether MCD tests can detect cancer early enough to cut fatalities and whether the advantages of using them to screen for cancer outweigh the risks.

Credit: Business Wire

NHGRI - National Human Genome Research Institute

The mission of the National Human Genome Research Institute (NHGRI) is to accelerate scientific and medical breakthroughs that improve human health. NHGRI is a leading authority in the field of genomics and pursues its mission by driving cutting-edge research, developing new technologies, and studying the impact of genomics on society.

The NHGRI Technology Transfer Office (TTO) is an integral part of the research life and administrative structure of the Division of Intramural Research (DIR) and provides vital services and support to NHGRI intramural investigators who conduct a broad program of laboratory and clinical research to translate genomics into a greater understanding of human biology and develop better method for detection, prevention, and treatment of heritable and genetic disorders.

In FY 2024, NHGRI TTO continued to manage its robust portfolio and coordinate its activities with many teams at the Institute (including Bioethics, Ethics, Procurement, and Financial Management). NHGRI TTO executed 257 standard agreements (transfer agreements, confidentiality agreements, and research collaborations), as well as 13 CRADAs, Gifts, and Licenses in FY24.

NHGRI continues to administer an All of Us Data Use and Registration Master Agreement with Vanderbilt University Medical Center to allow NHGRI investigators to access and use the All of US Research Hub database (upon individual registration), including registering new users and off-boarding those who leave NHGRI.

NHGRI continues to administer the model Reverse Phenotyping Core (RPC)-specific Data Contribution Agreement (DCA) for patient derived data contributed by various parties as part of the RPC in the Center for Precision Health Research (CPHR) in the Division of Intramural Research.

NHLBI - National Heart, Lung, and Blood Institute

The NHLBI Office of Technology Transfer and Development (OTTAD) supports the NHLBI mission to better understand, treat, and ultimately prevent infectious, immunologic, and allergic diseases through three main areas of activities:

- Negotiating CRADAs pursuant to 15 U.S.C. §3710a, and transactional agreements to support research,
- Reviewing inventions and managing patent portfolios to secure IP rights for Government technologies, and
- Negotiating licenses for technologies (including materials and IP rights) to develop and/or to commercialize vaccines, therapeutics, diagnostics, and research tools.

OTTAD manages the technology transfer efforts of five other ICs: the National Institute on Alcohol Abuse and Alcoholism (NIAAA), 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). OTTAD negotiated and executed 13 new CRADAs and 527 new transactional agreements in FY 2024 to facilitate material and information exchange between NHLBI or their service center client scientists and the research community, to support collaborative projects with governmental, academic, and industrial researchers, and to support NHLBI and their service center's research programs. New transactional agreements include 303 Material Transfer Agreements (MTAs), 172 Confidential Disclosure Agreements or Data Transfer Agreements (CDAs or DTAs), 35 Research Collaboration Agreements (RCAs), 3 Clinical Trial Agreements (CTAs), and 1 conditional gift agreement.

OTTAD staff also worked skillfully to protect IP and champion the development and commercialization of NHLBI and their service center client's discoveries. OTTAD staff evaluated 34 new inventions submitted for review, filed 75 patent applications, successfully managed patent prosecution resulting in 52 issued patents in 19 countries.

OTTAD staff worked expertly to negotiate licenses for NHLBI research materials and patented technologies. OTTAD negotiated and executed 5 new license agreements to commercialize NHLBI and NIEHS technologies.

Credit: iStock/VectorMine

NIAID - National Institute of Allergy and Infectious Diseases

Technology Transfer at NIAID: FY 2024 Snapshot

For more than 60 years, research at the National Institute of Allergy and Infectious Diseases (NIAID) has led to the development of novel therapies, vaccines, diagnostic tests, and other technologies that have improved the health of millions of people across the United States and around the world. The talented team of diverse professionals at NIAID's Technology Transfer and Intellectual Property Office (TTIPO) plays a key role in advancing the mission of the institute to better understand, treat, and ultimately prevent infectious, immunologic, and allergic diseases.

TTIPO experts work with NIAID researchers to move their innovations from the benchtop into additional research and development, testing, regulatory approval, manufacturing, distribution, and collaborative partnerships that can support continued progress and innovation. Leveraging the vast network of resources at NIAID, TTIPO fosters collaboration with high-profile stakeholders in academia, industry, and the non-profit sector by negotiating CRADAs, facilitating transactional agreements to support continued research, and managing patent portfolios to secure IP rights for NIAID technologies. TTIPO experts also negotiate licenses (including materials and IP rights) to develop and/or commercialize vaccines, therapeutics, diagnostics, and research tools for the betterment of public health.

Overview of the Technology Transfer Process and the role of multidisciplinary experts at NIAID TTIPO

In FY 2024, TTIPO's team of approximately 40 professionals negotiated and executed 15 new CRADAs and 815 new transactional agreements to facilitate material and information ex-change between NIAID scientists and the research community; promote collaborative projects be-tween NIAID and other intramural/extramural entities; and support NIAID research programs. Among the new transactional agreements were 412 MTAs, 249 CDAs and DTAs, 76 RCAs, 15 CTAs, and 7 Conditional Gift Agreements.

Working diligently to protect NIAID IP and champion the commercial development of NIAID discoveries, TTIPO experts evaluated 54 new NIAID inventions submitted for review, filed 98 patent applications, and successfully managed patent prosecution, resulting in 83 issued patents across 47 countries. In addition, TTIPO negotiated and executed 52 new license agreements, comprising 45 licenses to use, develop, and commercialize NIAID technologies and 7 IIAs to consolidate licensing and patenting of co-owned rights.

TTIPO also continued to provide patenting and licensing service to the CDC. TTIPO staff received and reviewed 8 new CDC inventions, filed 10 patent applications, obtained 26 patents issued across 12 countries, and negotiated through execution 23 new CDC license agreements, including 21 licenses to use or develop CDC technologies and 2 IIAs.

NIAID Public Health Highlights in FY 2024

Pictured from left to right: Aedes mosquito; creative artwork featuring colorized 3D prints of influenza virus; HIV awareness day stock image with globe and vial; scanning electronic microscope image showing SARS-CoV-2 (yellow; captured and colorized at NIAID's Rocky Mountain Laboratories in Hamilton, Montana); colorized transmission electron micrograph of mpox virus particles (blue) cultivated and purified from cell culture (captured at NIAID Integrated Research Facility in Fort Detrick, Maryland). Credit: NIAID.

Among the 27 institutes and centers that comprise the NIH, NIAID has a unique mandate to respond to emerging public health threats. Toward this end, TTIPO's team of experts played a critical role in managing a complex research portfolio spanning multiple areas of public health concern in FY 2024:

Coronaviruses

- In addition to facilitating transactional agreements related to the study of Long COVID through NIAID's RECOVER-TLC Initiative, TTIPO diligently managed a coronavirus monoclonal antibody (mAb) patent application covering 66 mAbs, which resulted in an exclusive license with Leyden Laboratories.
- In February 2024, TTIPO also executed an exclusive license with ModeX Therapeutics for *multispecific COVID mAbs.*

Dengue Virus

- TTIPO negotiated a CRADA with Merck and Co., Inc. to investigate the longitudinal impact of prior dengue virus and Zika virus infections on the severity of dengue disease in a pediatric Cambodian cohort. In addition, TTIPO negotiated several transactional agreements focused on mAbs, including hexameric Zika mAbs.
- Using a unique strategy combining exclusive and non-exclusive licenses, TTIPO experts were critically involved in translating decades of NIAID research into a safe, effective tetravalent vaccine against dengue (based on data from a meticulously designed and executed Phase III trial that included over 16,000 participants), ANVISA (Brazilian FDA/Regulatory Authority) approval of which is expected in 2025.

Influenza

- TTIPO negotiated several non-exclusive licenses for the development of primers and probes to detect and discriminate types and subtypes of influenza viruses, in support of the relevant CDC patent families.

Mpox

- TTIPO professionals negotiated CTAs with SIGA Therapeutics and the Institut National de Recherche Biomédicale for a clinical trial investigating the efficacy of SIGA's smallpox antiviral agent (TPOXX) in mpox infections in the Democratic Republic of the Congo.
- TTIPO experts have also taken the lead on championing an initiative for royalty-free transfer of starting material to develop a viable mpox vaccine for primary distribution in countries with the greatest immediate need.

HIV

- TTIPO has negotiated numerous clinical material transfer agreements for HIV-1 broadly neutralizing antibodies in support of clinical trials sponsored by the NIAID Division of AIDS.

Ebola

- NIAID researchers have developed a vaccine candidate for Sudan ebolavirus with a vesicular stomatitis virus (VSV) backbone that has shown promise in efficacy studies conducted in nonhuman primates. TTIPO's diligent patent prosecution efforts made this technology available for licensing in January 2024.

RSV

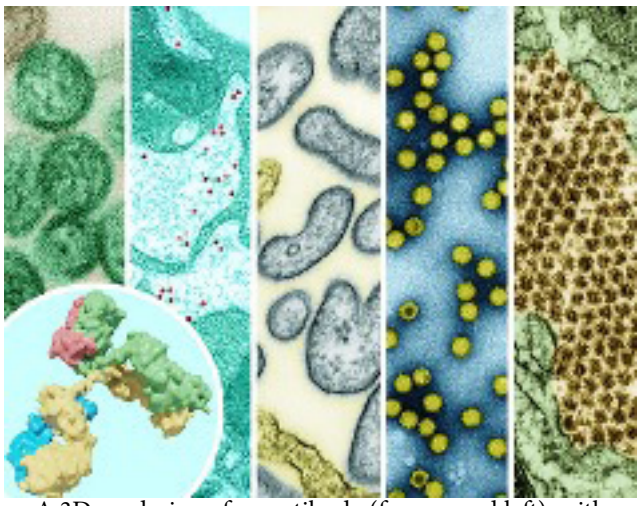
- In 2024, TTIPO executed 9 licenses for an RSV vaccine antigen (prefusion RSV F proteins), with another 14 currently in the negotiation stage.

Credit: iStock/lemono

Prototype Pathogens: Looking Ahead at NIAID

The novel solutions engineered by TTIPO's multidisciplinary experts during the COVID-19 pandemic—along with their diligence and precision in executing them—have been pivotal in discussions of pandemic preparedness and prevention.

NIAID's earlier work on the Middle East respiratory syndrome coronavirus (MERS-CoV) played a crucial role in understanding and developing safe and effective treatments and vaccines for SARS-CoV-2, with TTIPO professionals at the helm of devising innovative technology transfer solutions to expedite the development of a unified, global approach to an unprecedented threat.



A 3D rendering of an antibody (foreground left), with examples of high-priority, "prototype" pathogens (left to right): hantavirus, yellow fever virus, Nipah virus, picornavirus, and chikungunya. Credit: NAID.

As reported in a September 2024 news release, the Research and Development of Vaccines and Monoclonal Antibodies for Pandemic Preparedness (ReVAMPP) network will focus its research efforts on "prototype pathogens," representative pathogens from virus families known to infect humans, and high-priority pathogens that have the potential to cause deadly diseases. By focusing on research to develop vaccine candidates and monoclonal antibodies, the ReVAMPP network will lay the groundwork for a faster and more effective pandemic response should a virus from one of the targeted families emerge as a pandemic threat.

In addition to the breadth of work outlined above, TTIPO has already played essential roles in negotiating agreements and coordinating efforts in crucial disease areas targeted by ReVAMPP, including an outgoing MTA for dengue and Zika mAbs isolated from Rhesus macaques to the University of North Carolina and an RCA between NIAID (IRF-Frederick) and Chan Zuckerberg Biohub to facilitate studies on the replication of the chikungunya virus.

NIAID Welcomes Dr. Surekha Vathyam as TTIPO Director

After serving as Acting Director following the retirement of Michael Mowatt in 2023, Surekha Vathyam, Ph.D., began her official appointment as Director of TTIPO at NIAID on August 11, 2024. Dr. Vathyam, with over 30 years of experience in various aspects of technology transfer, has played pivotal roles at NCI, where she was part of the leadership team overseeing technology transfer operations at the TTC, and at the NIH OTT, where she managed a wide range of NIH and FDA inventions and intellectual property. Her expertise spans patent law, license negotiation, biomedical research, product development, and FDA regulations.

Surekha Vathyam, Ph.D., Director, TTIPO, NIAID

Prior to joining NIH, Dr. Vathyam served as a Patent Examiner at the US Patent and Trademark Office and held senior roles in the biotech industry, including Director of Research & Development and Director of Manufacturing. Her academic credentials include a

Ph.D.in Biochemistry from The Johns Hopkins University, an M.Sc.in Chemistry from the Indian Institute of Technology in Madras, and a B.Sc.in Chemistry from Stella Maris College.

> *Streamlining the conversion of science to products without sacrifices in quality or effectiveness relies not just on scientific knowledge but also on a team's ability to leverage the most promising partnerships in a way that is mutually beneficial and—above all—places human health at the forefront.*
> ***–Dr. Surekha Vathyam, Director, TTIPO, NIAID, NIH***

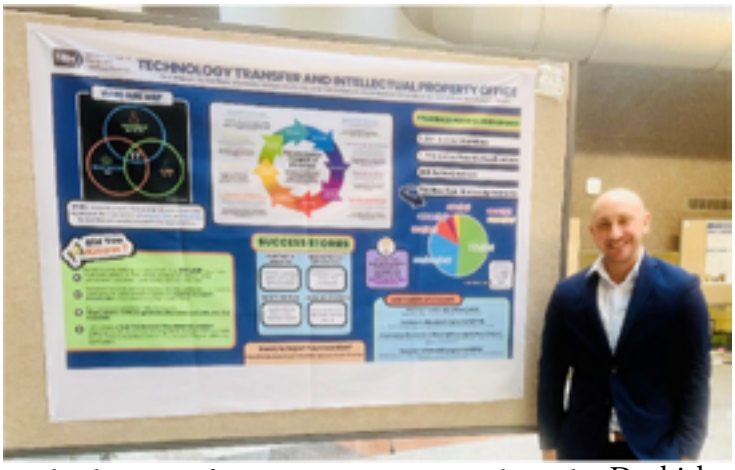
Leveraging extensive experience spanning both the public and private sectors, Dr. Vathyam has outlined several key objectives for TTIPO that directly align with the NIH-Wide Strategic Plan for Fiscal Years 2021-2025. "Addressing increasingly complex global health challenges and achieving excellence in technology transfer requires a workforce that is both collaborative and adaptable. With over half of TTIPO staff joining since the COVID-19 pandemic, I have prioritized building a culture that transcends virtual borders and empowers grassroots efforts to improve how we operate. This culture enables us to meet global health needs more effectively and deliver innovative, efficient, and impactful solutions to both internal and external stakeholders."

Within weeks of her official appointment date, Dr. Vathyam set the tone for her directorship at TTIPO's first in-person full-staff retreat since before the pandemic, held at NIAID's Fishers Lane facility—a monumental undertaking given both the logistics of coordination and the breadth of topics covered. For two days in September 2024, TTIPO personnel engaged in intensive discussions with leadership on key themes to promote productivity and success, including improvements in operational efficiencies and the optimization of knowledge management resources. Beyond these internal themes, the full team of TTIPO professionals collaborated to discuss modern, multifaceted approaches to strengthening stakeholder engagement.

The success of the meeting, as envisioned by Dr. Vathyam, cannot be understated. Follow-up conversations have not only aided in establishing working groups for optimizing intraoffice functions but have also helped to raise awareness of TTIPO's role in championing the development and commercialization of NIAID discoveries among researchers themselves. In October 2024, TTIPO professionals attended the annual Division of Intramural Research retreat to shed light on TTIPO's pivotal role in the translation of high-profile NIAID research from bench to bedside.

TTIPO staff with Dr. Jill Harper, Deputy Director for Science Management at NIAID, at the on-site all-staff meeting in September 2024.

In an invigorating internal discussion related to her high-level experience in the industrial sector, Dr. Vathyam emphasized how the insights she gained during this phase of her career will inform her approach to leadership at TTIPO. “During my tenure in industrial R&D, I learned just how critical it is to understand both your strengths and weaknesses. Streamlining the conversion of science to products without sacrifices in quality or effectiveness relies not just on scientific knowledge but also on a team’s ability to leverage the most promising partnerships in a way that is mutually beneficial and—above all—places human health at the forefront.”



Technology Transfer Communications Specialist Dylan Drobish, M.S., at Division of Intramural Research Retreat in October 2024.

NIDDK - National Institute of Diabetes and Digestive and Kidney Diseases

Laboratory mice born to wild mice have natural microbiota and model human immune responses are of increasing interest as a research tool for Pharmaceutical and Biotechnology companies.

Inbred laboratory mouse strains are used extensively in basic and translational immunology research. However, the commensal and pathogenic repertoire of resident microbes encountered in the wild is not replicated in a lab setting. This can substantially distort how the immune system develops and functions, leading to false assumptions of how our own “wild” immune system works. The Rehermann Lab at NIDDK circumvented this dilemma by implanting lab-strain embryos into wild mice. The resultant “wildlings” had a systemic immune phenotype and a bacterial, viral, and fungal microbiome much closer to those of their wild counterparts. In preclinical experiments, where lab mice had previously failed to predict the human response to drug treatments, wildlings accurately phenocopied patient outcomes. This field has blossomed with new opportunities for studying disease and therapies in animals with immune systems that more accurately match that of humans. NIDDK has entered into research collaborations with drug developers to study how this wildling mouse model can be used to identify and evaluate new approaches to improve therapeutic design and treatment regimens in humans.

NIEHS - National Institute of Environmental Health Sciences

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

health. The studies conducted at NIEHS are focused on preventing diseases that emerge from exposure to hazardous environments. Research in NIEHS laboratories lead to discoveries and innovations that aim to prevent, treat, and diagnose diseases and disorders caused by environmental exposures. Such disorders and disease include inflammation, cardiovascular disease, metabolic disorders, reproductive disorders, neurological defects, and cancer. In addition, there are several ongoing clinical studies at the NIEHS that seeking to elucidate potential health effects of exposures to environmental hazards on the U.S. population.

NIEHS OTT originates, negotiates, and executes various types of technology transfer agreements to provide or receive research materials and data, and/or collaborate on research projects with non-profit or for-profit entities. NIEHS OTT successfully negotiated 479 agreements in FY2024 with 412 MTA, 297 of which were with Addgene, 1 Memorandum of Understanding (MOU), 6 CDAs, 50 DTAs, and 10 RCAs.

NIEHS investigators disclosed 7 innovations to NIEHS OTT and had 1 patent issued; both were an increase from fiscal year 2023.

NIEHS OTT Executed Agreements for Data from the Virtual Pooled Registry

The Virtual Pooled Registry – Cancer Linkage System (VPR-CLS or VPR for short) is a web-based tool that researchers can use to link their study cohorts to data in participating state cancer registries. Dr. Dale Sandler, Chief of the Epidemiology Branch, is leading a project to link the data from the Sister Study and the Gulf Long-Term Follow-up (GuLF) Study to state cancer registries by requesting data through the VPR. The Sister Study is a prospective cohort study of environmental and genetic risk factors for breast cancer and other diseases among 50,884 sisters of women who have had breast cancer. The GuLF Study is a prospective cohort study of the potential health effects associated with response and clean-up related to the 2010 Deepwater Horizon oil spill.

Thus far, NIEHS OTT has negotiated the execution of 18 Data Transfer Agreements with various state cancer registries to obtain data on cancer incidence in participants in the Sister Study and the GuLF Study who reside in those states.

NIEHS OTT Executed Agreements to Share Data from Personalized Environment and Genes Study

The Personalized Environment and Genes Study (PEGS) is a North Carolina-based cohort from whom data was collected to understand disease etiology, identify disease risk factors, and improve disease prevention. PEGS has extensive data including both questionnaire-based and geospatial estimates of exposome-wide environmental exposures, whole genome sequencing, and genome-wide epigenetic data that can be leveraged by external collaborators to pursue new questions about public health. To this end, NIEHS OTT has executed 17 Data Transfer Agreements to share data with a variety of collaborators who submitted project proposals to PEGS.

NIMH - National Institute of Mental Health

The National Institute of Mental Health (NIMH) is the lead federal agency for research on mental disorders. Its mission is to transform the understanding and treatment of mental illnesses through basic and clinical research, paving the way for prevention, recovery, and cure.

NINDS - National Institute of Neurological Disorders and Stroke

The National Institute of Neurological Disorders and Stroke (NINDS) will use Sherpa.ai´s Artificial Intelligence (AI) Privacy-Preserving platform, provided as part of collaboration between the parties, to improve the accuracy of diagnosis of rare childhood neurological disorders, such as collagen VI-related muscular dystrophies. NINDS researchers have broad experience in the field of rare childhood neurological disorders. The proposed collaboration will combine the expertise of both parties and will contribute to improving the use of AI solutions for the benefit of such patients while respecting patients' privacy.

Sherpa.ai´s AI Privacy-Preserving solution, enables collaborative AI model training between various organizations without sharing data, and will allow the prediction algorithm to improve the diagnosis without the need to share any patient data, therefore helping to preserve patients' privacy.

One of the major challenges in developing AI algorithms for diagnosis and treatment of diseases is the limited availability of data for AI model training. Organizations often do not have a sufficient volume of quality data to develop accurate AI models, therefore collaboration with multiple organizations is required.

The Federated Learning approach of Sherpa.ai's AI Privacy-Preserving platform allows to leverage private data from different organizations in different countries without exposing or sharing any private patient data., Sherpa.ai´s AI Privacy-Preserving platform enables collaboration across organizations to develop more accurate diagnosis and targeted treatments to improve patients' life, while preserving patient privacy.

Credit: iStock/SvetaZi

Federal Laboratory Consortium Chair from NCI TTC Participates in Outreach Events

In her role as Federal Laboratory Consortium (FLC) Chair, TTC's Whitney Hastings, Ph.D. represented NIH technology transfer at several events throughout the year.

In February 2024, she highlighted NCI's technology transfer opportunities at the FLC's Partnering for Success, Meet the Federal Labs event in San Juan, Puerto Rico. This event provided an overview of federal laboratory technologies and resources to Puerto Rico's businesses and start-ups.

Dr. Hastings was a big part of the FLC National Meeting held in Dallas Texas. She joined FLC's Executive Director Paul Zielenski and Bayh-Dole Coalition's Executive Director Joe Allen for the 50th Anniversary Town Hall session to discuss the past, present and future of federal technology transfer.

In July 2024, Dr. Hastings joined America's Seed Fund Road Tour in the Great Lakes Region (MI, IL, IN, WI, MN) for FLC. This road tour connects innovators and entrepreneurs to non-dilutive, technology funding opportunities provided through the SBIR and STTR programs. Joining her NIH SEED colleagues, she attended a series of events that brought together local and national innovation ecosystems, pitching technology transfer opportunities available to small businesses had dozens of one-on-one.

In August 2024, Dr. Hastings attended the Rally Innovation conference in Indianapolis Indiana – a global cross-sector innovation conference to facilitates connections among companies, entrepreneurs, investors, and universities worldwide. At the conference, she networked with potential collaborators and licensees in the FLC booth and also participated on a panel entitled "Federal Laboratory Consortium – Funding Opportunities and Case Studies" with her technology transfer colleagues from the Navy, the Veteran's Administration, NASA and DOE's Argonne National Laboratory. During the panel she talked about the many ways entrepreneurs and businesses can work the NIH's technology transfer offices.

Whitney Hastings participating in a panel about how federal innovation translates to national progress and drives economic growth.

In addition, Dr. Hastings and TTC colleague, Lauren Nguyen-Antczak, Ph.D., J.D., participated on a panel at the AUTM Annual Meeting entitled "Finding Federal Tech Development Resources." The opportunity allowed them to highlight several FLC and NCI programs and resources such as NCI's NExT program and the Frederick National Lab's numerous applied research programs, repositories, and computational tools.
In December 2024, she presented at an event with the Congressional R&D Caucus,

co-chaired by Rep. Bill Foster (D-IL) and Rep. Jim Baird (R-IN). Along with Paul Zielinski, FLC Executive Director, and panelists from industry and government, the panel spoke about how federal innovation translates to national progress and drives economic growth.

FLC Supports NCI Outreach Efforts to Facilitate NIH Partnerships

The FLC partnered with the NCI TTC to support efforts to get the word out about opportunities to work with NIH:

- For its December 16, 2024 *Tech Transfer Files* podcast, the FLC interviewed TTC's Michael Salgaller, Ph.D. for an episode entitled "Marketing from All Angles With Michael Salgaller." The podcast "provides an insider's look at how government laboratories bring groundbreaking technologies to public life- through partnerships, collaboration and determination.
- The FLC served as one of several co-sponsors for the 2024 Technology Showcase. In particular, the FLC made available to Technology Showcase attendees its FLC Connect meeting app. Meeting partnering apps are commonly used at many professional conferences and meetings to facilitate networking and engagement, and to make it easy for attendees to have relevant information about the event in one, convenient, easily accessible online place. As a co-sponsor, the FLC was able to offer this platform at no cost.

FLC Interviews OTT on The Transfer Files Podcast

The FLC podcast *The Transfer Files* interviewed OTT's director, Tara Kirby, Ph.D., for an episode titled How NIH is Fast-Tracking Innovation. They discussed how technology transfer works at the NIH and the award-winning NIH Enterprise Technology Transfer system. They touched on the creation and transition to this system, as well as how this system aids the NIH Technology Transfer Community in moving innovations from the lab to market.

The Transfer Files also interviewed OTT Special Advisor, Steven Ferguson, discussing one of the many ways the FLC provides value for federal labs and their business partners and collaborators.

This episode discussed the FLC hosting a pavilion at the Biotechnology Innovation Organization's 2024 International Convention and how they invited member labs to have booths within it for discussions and meetings with biotech and pharma companies.This is the second year that the NIH Technology Transfer program has attended BIO with the FLC. Ferguson discussed the benefit that NIH has gotten from attending BIO in conjunction with the FLC.

2024 NCI/FNL Technology Showcase

The 2024 NCI/Frederick National Laboratory (FNL)Technology Showcase in an annual event held at the Frederick National Laboratory for Cancer Research (FNLCR). The purpose is to highlight technologies and capabilities from both the NCI and the FNLCR to encourage collaboration and licensing. The event features technology presentations, poster sessions and expert discussions on topics including financing, leveraging incubators and working with patient advocacy groups. The target audience is potential collaborators and licensees. It is organized under a co-sponsorship agreement between the NCI, FNLCR, the City of Frederick, the County of Frederick, TEDCO and the Federal Laboratory Consortium for technology transfer.

Featured technologies and programs: The event provided a platform for three NCI and two FNLCR inventors to highlight technologies and partnering opportunities. Four FNL program leaders provided overviews of unique programs offered at the FNLCR that biotech community can access. Ambassadors from the Technology Transfer Ambassadors Program (TTAP) who analyzed select technologies added value by exposing attendees to eight more technologies.

Format: The 2024 Tech Showcase Planning Committee decided on an in-person only event. Feedback from 2023 indicated that attendees valued the opportunity to network and engage in one:on:one conversations. Furthermore, the Committee felt an obligation to ensure a full in-person audience for the slate of invited expert speakers, panelists and ambassadors. The strategy paid off with an increase in final registration and in-person attendance over 2023 (150 vs. 209).

Tech Showcase Firsts:

- **Fireside Chat** - Troy LeMaile-Stovall, CEO, TEDCO, interviewed Dr. Helen Sabzevari, President and CEO of Precigen, Inc., an NCI CRADA collaborator.
- **Poster competition** – Ambassadors answered questions about a selected technology and its market potential. Judges awarded the best poster presentation.
- **"FLC Connect"** meeting partnering app. Tech Showcase organizers were able to offer use of the FLC Connect partnering app to registrants through the in-kind support of the FLC, a Tech Showcase co-sponsor. Meeting apps are expected at comparable professional conferences. The Tech Showcase is unique in that it is executed without a budget through the in-kind work and support of co-sponsors.

Outreach at BIO International Convention

The Biotechnology Innovation Organization's (BIO) International Convention is the world's largest conference representing the biotechnology and life science industries. Presenting, exhibiting and participating in one-on-one partnering creates awareness of, and information regarding, licensing and collaborative opportunities across the NIH. TTC's Technology Marketing and Analysis Unit (TAMU) helped staff NIH's section in a federal labs booth organized by the Federal Laboratory Consortium. Alongside NIH Office of Technology Transfer leadership—Tara Kirby, Ph.D. (Director) and Steve Ferguson (Special Advisor)—TAMU engaged booth visitors and conducted more than 30 one:one meetings. Companies, investors, entrepreneurs and other stakeholders gained insight regarding technology transfer opportunities at NIH.

BIO attendees pictured from left to right: Jennifer Dyer (OTT), Tara Kirby (OTT), Steve Ferguson (OTT), Joseph Conrad (NCI), Michael Salgaller (NCI), Annie Morgan (NCI), and Richelle Holnick (OTT)

Michael Salgaller, Ph.D. moderated a panel on an Impact Report commissioned by NIH detailing the significant effect of licensing on global health and economic development. TAMU was also invited to provide brief overviews at booths for the State of Oregon, the European Union and others. Months of planning by Joe Conrad, Ph.D., J.D., Michele Newton and Michael Salgaller, Ph.D. resulted in numerous leads for potential licensees and collaborators—not just for ICs covered by TTC, but across NIH ICs as well.

Steve Ferguson was a part of the faculty for the BIO Professional Development Course - Become a Biotech or MedTech Entrepreneur. He also spoke on *The Priority Review Voucher Program: Incentivizing Neglected Disease Research and Promoting Biotech Investment and Development for More than a Decade* panel.

Tara Kirby participated in the In and Out of This World: Partnering with Federal Labs panel. This panel, moderated by the Frederick National lab's Vladimir Popov showcased the versatility of federal research and benefits of partnering with federal labs.

Technology Transfer Ambassadors Program

The NCI Technology Transfer Ambassadors Program (TTAP) is a unique, free intramural training program for NIH scientists (including post-doctoral fellows, Staff Scientists/Staff Clinicians, lab technicians, etc.) seeking hands-on training in technology transfer. TTC Fellows can also benefit by attending Boot Camp courses.

Structure:

Part I - Boot Camp (January – March): Ambassadors attend weekly classes and complete homework assignments. In a final Boot Camp project, ambassadors assess a technology for patentability and commercialization potential, and then present their assessment to tech transfer staff. Ambassadors who pass Part I Boot Camp may go on to Part II.

Part II - Assignment to TTC Unit (April – December): Ambassadors work with a TTC Technology Transfer Manager (TTM) who serves as a mentor. Ambassadors receive hands-on training in various activities from that TTM's docket and participate in monthly workshops on in-depth tech transfer topics. At the annual NCI / FNL Technology Showcase, ambassadors apply their training by presenting a one-minute technology pitch and poster highlighting licensing/development opportunities for their assigned technology.

Credit: iStock/Color_Life

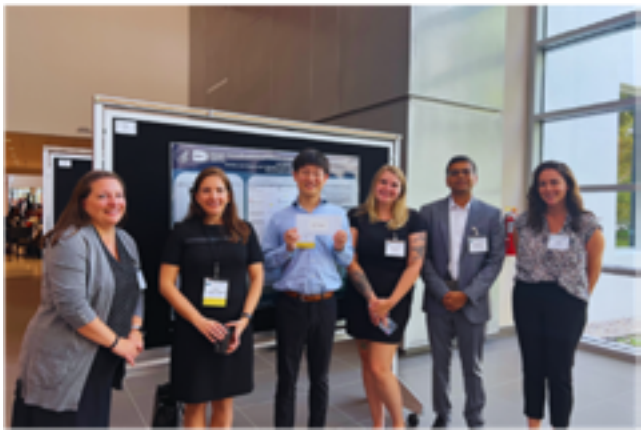
Who supports TTAP?:

- **TTAP Leads** are TTMs who volunteer and dedicate significant time to lead the program. The TTAP Leads for 2023 – 2025 are Drs. Rose Freel, Suna Gulay French, Whitney Hastings, Taryn Dick, and Lidia Beka.
- **TTC Staff teach select Boot Camp classes:**
  - Dr. Lauren Nguyen-Antczak teaches two introductory classes on patents and the patent system.
  - Dr. Jaime Greene teaches one of the monthly workshops on international patent search reports.
  - Dr. Geoffrey Ravilious teaches a workshop on FDA regulations as they relate to patenting and inventions.
- **External experts** also contribute time training ambassadors in the heavier legal and marketing aspects of the program.

TTAP involves most of TTC. Most TTMs with ambassadors assigned to their unit spend time and effort to train. Beyond TTMs, TTC staff including Heidi Bowman, Chris Sappington, Michele Newton, and TTC fellows support as well.

History: In 2016, Drs. Laura Prestia, Alan Alfano and Rob Sons (three former members of TTC) launched TTAP. The program was initially geared towards training post-doctoral level scientists to be ambassadors of technology transfer in their laboratories to help TTC spread the word to the NCI scientific community about the importance of tech transfer. Based on TTC's experience running the program, in 2019, Laura Prestia, Rose Freel, Abritee Dhal and Taryn Dick significantly restructured the program including expanding it beyond NCI to other NIH ICs and turning it into the streamlined version that is offered today. Since 2023, TTAP is available across the entire NIH intramural program; all ICs agreed to participate and allow their scientists to apply for this valuable training opportunity.

Impact: TTAP ambassadors provide direct assistance to TTMs in technology transfer matters, contributing directly to the mission of TTC. Ambassador projects include invention assessment, preparing slides for Technology Review Group (TRG) presentations, drafting Invention Summary Living Documents (ISLDs) and the ISLD-equivalent for client ICs, transactional agreement negotiating, license application review and drafting licenses, and marketing campaigns. Most recently, in 2024, eight TTAP ambassadors completed a reported 471 hours of technology transfer projects under the guidance of TTM mentors and TTAP leads. Through these training and program efforts, TTAP supported the dockets of 15 TTMs and Unit Supervisors across TTC, serving NCI and several other NIH ICs including NEI and the NIH Clinical Center.



A few TTAP team leads from the 2024 program stand with the winner of the poster competition at the 2024 Technology Showcase. Ambassadors prepared a poster and a one-minute pitch. The event provides ambassadors the opportunity to apply their training.

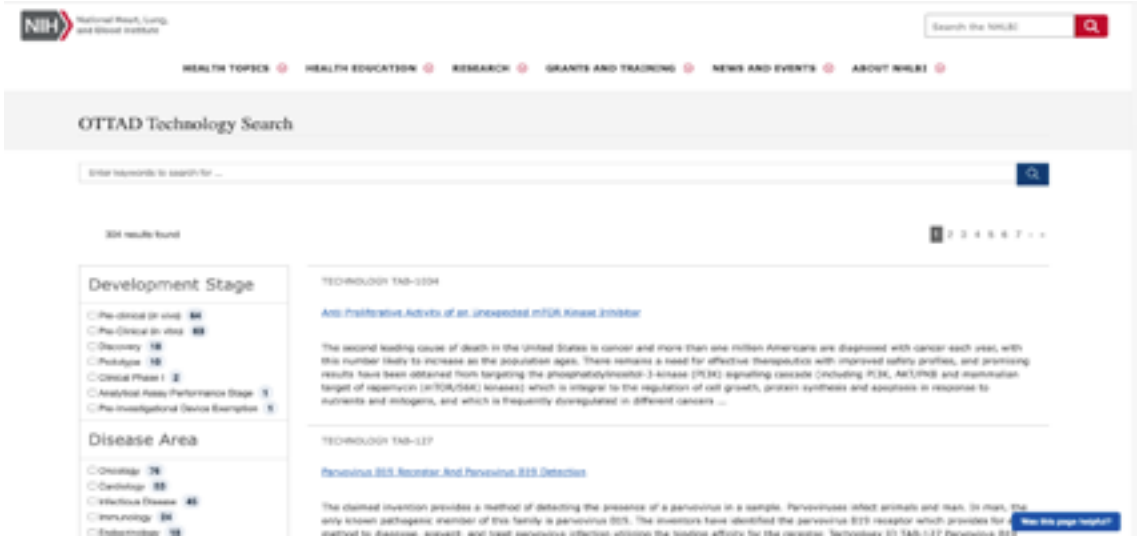
Furthermore, ambassadors make important contributions to the annual Technology Showcase by increasing the number of NIH technologies showcased to the attendees comprised of potential collaborators and licensees. Since 2018, Ambassadors exposed Tech Showcase attendees to the commercial potential of 76 technologies.

Besides the direct contributions of the TTAP ambassadors to the TTC, the experience the ambassadors gain in TTAP is valuable toward a wide variety of non-traditional PhD career paths. Scientists who completed TTAP have pursued careers federal and private sectors, such as: federal Technology Transfer Managers, Patent Agents, Technical Specialists at law firms, Senior Associates working in Clinical and Corporate Contract Resource Management at universities, federal Health Science Analysts, FDA Drug Reviewers, and staff or senior scientists in federal government or the private sector.

Marketing NHLBI Technologies

NHLBI added a feature to their website that NIH OTT has provided to all ICs: the **Embedded Search of technology marketing abstracts**. This feature gives potential licensees and collaborators a way to quickly search and filter NHLBI technology abstracts using the powerful search engine from the NIH Technology Transfer website. This feature allows po-tential licensees visiting the NHLBI website to have a live feed of the technologies available and the ability to drill down by keywords and categories including disease area, development stage, type of collaboration sought, inventor, and more. This is a great addition to the NHLBI website, allowing the site to stay up to date with available abstracts and increase the reach of these abstracts.

NHLBI is one of six ICs that is utilizing the Embedded Search functionality offered by OTT.



NIH Hosts First Industry Day

On January 18, 2024, NIH held its first conference designed to foster greater collaboration between public and private entities, offering external stakeholders an inside look at the cutting-edge research conducted across NIH's 27 ICs.

NIH Industry Day was a virtual conference that boasted **1,200 registrants**, 50% of whom were non-NIH, including about 30% from the industrial sector.

Organizers Dr. Bibi Bielekova, Chief of NIAID's Neuroimmunological Diseases Section; Dr. Amy Klion, Chief of NIAID's Human Eosinophil Section; and Dr. Mukul Ranjan, Senior Advisor for Innovation and Technology Transfer at NIAID, said the event aimed to help junior scientists better understand the benefits and challenges of government–industry collaboration. Dr. Ranjan of TTIPO, who was among the committee members who received an NIH Director's Award for their exceptional efforts in the planning and execution of NIH Industry Day, played a particularly crucial role in the conceptualization and development of the inaugural event.

At the heart of the conference were panels of scientific leaders from NIH, academia, and industry who showcased how synergistic partnerships are tackling problems at the forefront of biomedical research. Six focal areas for NIH Industry Day were selected for Industry Day in 2024, including vaccines, therapeutics (including gene therapy), novel target identification/validation, drug repurposing, diagnostics, and biomarkers.

NIH Director Dr. Monica Bertagnolli spoke about the promise of successful collaboration between NIH scientists and private industry:

> **"When we think about delivering on NIH's mission, we know that includes making sure that insights and discoveries that emerge from our research get out into the world and improve people's lives," she said, adding, "NIH does not bring new products and services to market—the agency and nation rely on productive industry partnerships to achieve this."**

Steering Committee Members included:

- Bibi Bielekova, NIAID
- Melissa Brotman, NIMH
- Hasni Sarfaraz, NIAMS
- Amy Klion, NIAID
- John Tisdale, NHLBI
- John Schiller, NCI

with two members representing technology transfer:

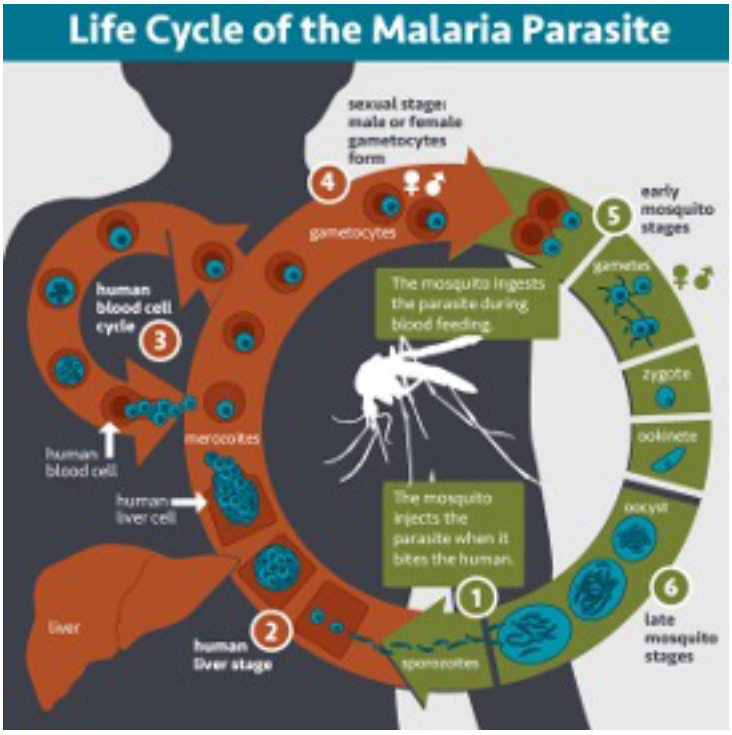
- Ranjan Mukul, NIAID
- Michael Salgaller, NCI

Credit: NIH Medical Arts, NIAID

NIAID Researchers Target P. falciparum Sporozoites for Malaria Prevention

In recent years, new interventions have been developed against malaria, including vaccines that are currently being rolled out for young children in regions where the disease is prevalent. Anti-malarial mAbs are another promising new tool that have been shown to be safe and efficacious against infection with Plasmodium falciparum in adults and children in early clinical trials. The anti-malarial mAbs evaluated in trials in malaria-endemic regions target the P. falciparum sporozoite—the life stage of the parasite that is transmitted from mosquitoes to people.

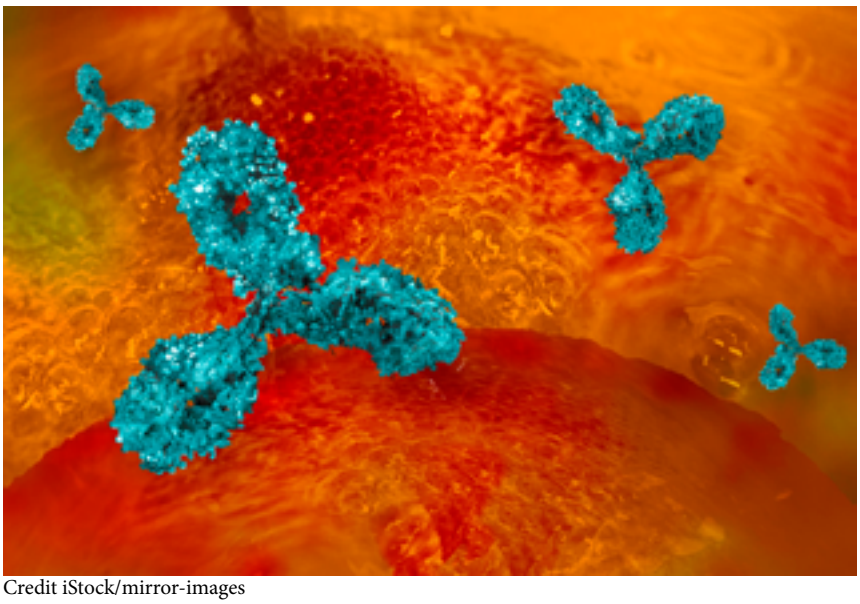
The most promising anti-malarial mAbs tested in humans to date bind to a protein on the sporozoite surface called the circumsporozoite protein (PfCSP) at locations near to or containing amino acid repeats in a region called the central repeat region. This portion of PfCSP also is included in the two available malaria vaccines.



An illustration of the life cycle of the malaria parasite.
Credit: NIAID

NIAID researchers have developed 11 human monoclonal antibodies that bind to a unique site on the circumsporozoite protein (CSP) in P. falciparum sporozoites.

Across the portfolio of technologies managed by TTIPO, several of these antibodies have substantially reduced liver parasite burden in a mouse model of malaria, with some conferring advanced protection when combined with an established protocol of mAbs, suggesting that together they may form an effective cocktail for malaria prevention.



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INNOVATIVE COLLABORATIONS

Partners Across NIH: Translational Science Is a Team Sport

The NCATS-led Platform Vector Gene Therapy (PaVe-GT) pilot project seeks to increase the efficiency of clinical trial startup by using the same gene delivery system and manufacturing methods for multiple rare disease gene therapies. The goal is to share project results and lessons learned with the public in such a way that the information is useful to any party interested in efficiently developing a gene therapy, and especially useful for parties interested in developing gene therapies for diseases with very small populations.

NCATS is applying its collaborative translational science model to transform understanding, diagnosis and treatment of rare diseases. The NCATS Division of Preclinical Innovation (DPI), Division of Rare Diseases Research Innovation (DRDRI), and Office of Strategic Alliances (OSA), the National Human Genome Research Institute (NHGRI), the Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD) and the National Institute of Neurological Disorders and Stroke (NINDS) work together with multiple outside stakeholders and partners, to achieve PaVe-GT objectives.

OSA is playing a critical role in guiding, establishing, and managing diverse collaboration agreements and other transactional agreements to enable the Pa-Ve GT projects. For example, OSA has negotiated and executed complex MOUs, confidentiality agreements, RCAs, and MTAs among many others to enable smooth progress of Pave-GT projects. OSA advises the cross functional Pa-Ve GT teams on complex IP related matters.

Together, the PaVe-GT team will answer a pressing translational science question in AAV gene therapy:

Can researchers significantly increase the efficiency of gene therapy trial startup by using the same capsid and manufacturing methods for different AAV gene therapies, thereby minimizing redundancies in the preclinical development of gene therapies for extremely rare diseases?

Bespoke Gene Therapy Consortium (BGTC)

BGTC is part of the Accelerating Medicines Partnership® (AMP®) program, a public–private partnership among NIH, the U.S. Food and Drug Administration (FDA), multiple pharmaceutical and life sciences companies, and nonprofit and other organizations.

NCATS is partnering with the FDA, the Foundation of the National Institutes of Health (FNIH), 10 NIH ICs, and several pharmaceutical companies and nonprofit organizations to form the BGTC. NCATS will serve as the lead NIH IC for BGTC, and we expect to contribute approximately $8 million out of a total $39.5 million provided by participating NIH ICs. Together, NIH and private partners will contribute approximately $104 million over 5 years to support BGTC-funded projects.

The BGTC mission is to re-engineer the translational research process so new treatments and cures for diseases can reach patients faster. Notably, the focus is on the critical and unmet needs of people with rare diseases. The BGTC is one of several NCATS-led programs that apply the "many-diseases-at-a-time" approach to gene therapy development for rare diseases, including the Platform Vector Gene Therapy (PaVe-GT) pilot project.

A critical factor for the success of BGTC is establishing and navigating partnerships and collaborations across and among multiple stakeholders and with complex scientific, technical, legal, ethical, and financial issues. OSA is playing a central role working with all stakeholders in enabling the smooth progress of BGTC projects and towards crafting novel and unique agreements to meet the expectations of the parties. As an example of this, recently OSA coordinated and played a central role in establishing a novel Data warehousing agreement with a private partner to enable the partner to deposit the clinical data into a central database for easy access and use. This agreement will be used as a template for several other BGTC related programs and projects.

Recently, OSA facilitated a MOU among several NIH Institutes to collaborate and move the methylmalonic acidemia (MMA) gene therapy project forward. The clinical trial is expected to begin in fall 2025. In addition to giving new hope to people with the disease, the project could lay the groundwork for other similar trials at NIH.

NCATS/NIH-FDA RDD 2025 LOA

NCATS OSA worked closely with the NCATS Division of Rare Diseases Research Innovation to put in place a Letter of Agreement (LOA) with the FDA for a joint Rare Diseases Day to be held at NIH on Feb. 27-28, 2025. The LOA outlines how both HHS agencies will work together to host RDD 2025, which is an important HHS initiative. RDD takes place worldwide to raise awareness among policymakers and the public about rare diseases and their impact on patient's lives. FDA-NIH Rare Disease Day aims to provide the rare disease community — patients, caregivers, families, patient advocate groups, researchers, clinicians, health care providers, and trainees/students —with information relevant to their lived experiences, including activities by FDA and NIH that promote research and product development for this underserved population. The LOA itself outlines the detailed roles and responsibilities of both agencies for event planning and execution, including logistics, to ensure a smooth transition from singly held agency events to a shared joint event in 2025.

Marengo Therapeutics Received U.S. FDA Fast Track Designation for STAR0602

STAR0602 is a novel T cell receptor (TCR) variable beta chain (Vβ) directed antibody-fusion molecule being co-developed by the NCI, Center for Cancer Research, Center for Immuno-Oncology (CIO) and Marengo Therapeutics. This TCR agonist selectively targets a common Vβ T cell subset present in all cancers and, by combining a novel mode of TCR activation with a T cell co-stimulator in the same molecule, promotes expansion of a new population of clonally enriched, effector memory Vβ T cells that promote durable tumor immune responses and clearance of tumors.

Marengo Therapeutics announced in January 2025 the U.S. FDA's Fast Track Designation of STAR0602 for the treatment of unresectable, locally advanced, or metastatic colorectal cancers with high tumor mutational burden (TMB-H). The FDA's decision is supported by the positive outcomes of Marengo's first-in-human Phase 1 clinical trial of STAR0602 in patients with heavily pretreated cancer. It is currently being studied in the Marengo-sponsored START-001 (NCT05592626) Phase 1/2 clinical trial. This study has 10 clinical sites including one at the NCI with the Center for Immuno-Oncology (CIO) Co-Director, James Gulley, M.D., Ph.D., as the NCI lead investigator. This novel immunotherapy is being co-developed under a clinical CRADA between CIO and Marengo. Laura Henmueller, Ph.D. was the lead TTM negotiating this CRADA with Supervisory TTM, Michael Pollack, Ph.D. providing support. Importantly, the CRADA allows the CIO to study this therapeutic in combination with other proprietary molecules which may result in novel combination therapies.

Groundbreaking Pivotal Study Conducted at NCI for PRGN-2012 in Patients with Recurrent Respiratory Papillomatosis (RRP)

Recurrent respiratory papillomatosis (RRP) is a rare, devastating HPV-mediated chronic disease that causes wart-like (benign) growths in the airways. These growths can be removed by surgery but typically grow back. In 2023, the FDA granted the first-in-class investigational PRGN-2012 (a gorilla adenovirus–based gene therapy from Precigen Inc.) Breakthrough Therapy Designation. In 2021, the FDA granted PRGN-2012 Orphan Drug Designation for the treatment of RRP.

The first-in-human, phase 1/2 pivotal study (NCT04724980) of PRGN-2012 in adult patients with RRP, was conducted at the NCI and led by Scott M. Norberg, D.O., Associate Research Physician in the Center for Immuno-Oncology, Center for Cancer Research, NCI. This trial demonstrated the overall safety and clinically meaningful benefit observed with PRGN-2012, with a 51% complete response rate in patients treated at the highest dose.

PRGN-2012 is being developed under two NCI CRADAs with Precigen:

1. A clinical CRADA where the NCI, CCR, and the Center for Immuno-Oncology (CIO) are lead.
2. A standard CRADA where the NCI, CCR, and the Surgical Oncology Program (SOP) are lead.

Championing these collaborations are James Gulley, M.D., Ph.D., Jeffrey Schlom, Ph.D., Scott Norberg, D.O., NCI CIO, and Clint Allen, M.D., NCI SOP. Dr. Allen is an expert in the field of RRP. TTC played a crucial role in managing the related agreements. TTC's Dr. Michael Pollack negotiated the CIO's clinical CRADA with Precigen. TTC TTM's, Suna Gulay French, Ph.D. and Zehra Sherwani, Ph.D. manage the SOP CRADA.

FDA Grants Accelerated Approval to Lifileucel-the first TIL Therapy for Advanced Melanoma

On February 16, 2024, the FDA approved lifileucel (Amtagvi, Iovance Biotherapeutics, Inc.), the first cancer treatment that harnesses immune cells known as tumor-infiltrating lymphocytes, or TILs, for unresectable or metastatic melanoma. The development of this therapy took more than thirty years to complete. Steven Rosenberg, M.D., and his colleagues in the Surgery Branch at NCI developed the TIL treatment. Dr. Rosenberg led the first-ever clinical trials of TIL therapy in the 1980s which showed that TIL therapy could reduce the size of the tumors of patients with very advanced melanoma. The NCI researchers improved the method for producing and administering TIL treatment during the following years.

FDA's decision also makes lifileucel the first cellular therapy to be authorized for melanoma, a solid tumor. Data from 73 study participants whose cancer had worsened after receiving treatment with a BRAF inhibitor or a PD-1/PD-L1-targeted immune checkpoint inhibitor and whose lifileucel dose was at least 7.5 billion cells (the dose specified in the FDA approval) served as the basis for the FDA's approval. These results are substantially consistent with longer-term results from a larger group of 153 study participants, which were presented at the European Society of Medical Oncology's (ESMO) 2023 Immuno-Oncology Congress in December 2023.

This partnership with Iovance took place under a CRADA handled by Senior Technology Transfer Manager, Aida Cremesti, Ph.D. and associated licenses were handled by Technology Transfer Manager Andrew Burke, Ph.D..

NHGRI Collaborations

NHGRI continued developing, negotiating, and administering a variety of technology transfer an alliance relationships and agreements in FY2024. The following are some examples.
NHGRI TTO negotiated and executed a Research Collaboration Agreement with Oregon Health and Science University to collaborate on a project entitled: "*An examination of whole genome sequence data in monozygotic twins discordant for attention-deficit/hyperactivity disorder (ADHD)*."

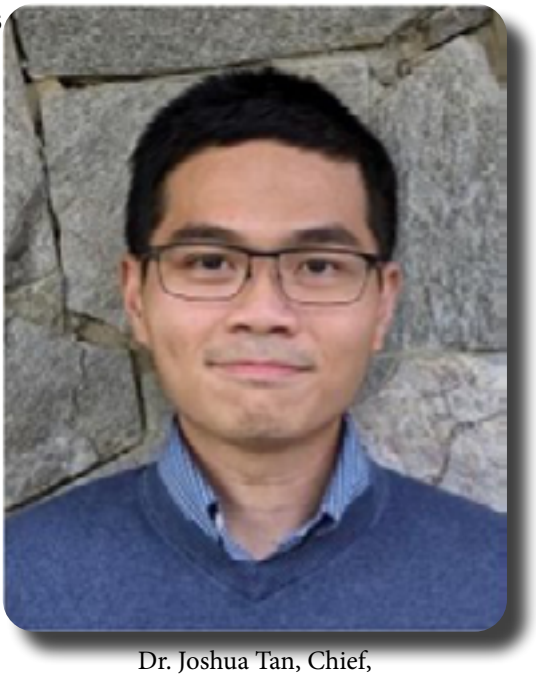
NHGRI TTO negotiated and executed a Research Collaboration Agreement with Zvitambo Institute for Maternal and Child Health Research in Harare, Zimbabwe, and the Tropical Gastroenterology and Nutrition Group, Zambia to collaborate on a project entitled: "*An international investigation into the Genetic Etiology of Severe Acute Malnutrition*."

NHGRI TTO negotiated and executed a Material Transfer Agreement (MTA) and a related Information Transfer Agreement (ITA) with the Federal University of Minas Gerais (UFMG) in Brazil related to the transfer and analysis of samples and data from the North Amazonian region.

NIAID Researchers Invent Human Monoclonal Antibodies That Broadly Target Coronaviruses

First discovered in the 1960s, human coronaviruses entered the international public discourse in 2019 when severe acute respiratory syndrome-coronavirus-2 (SARS-CoV-2) began spreading throughout the world.

Extensive sequencing of the SARS-CoV-2 genome has resulted in the identification of thousands of distinct variants, many of which include mutations that alter the characteristic spike protein of the virus. Because the spike protein mediates entry of the virus into the host cell and is essential for replication, spike protein mutations have been a major roadblock in the development of vaccines and antibodies that can neutralize the virus. In addition to altering patterns of infectivity and trans-mission, spike protein mutations can even "shield" some important binding sites, allowing the virus to escape detection by the immune system. Neutralizing antibodies that broadly target conserved sites of the coronavirus spike protein may be vital for developing treatments that will not lose their efficacy as the virus mutates and more variants enter the landscape.

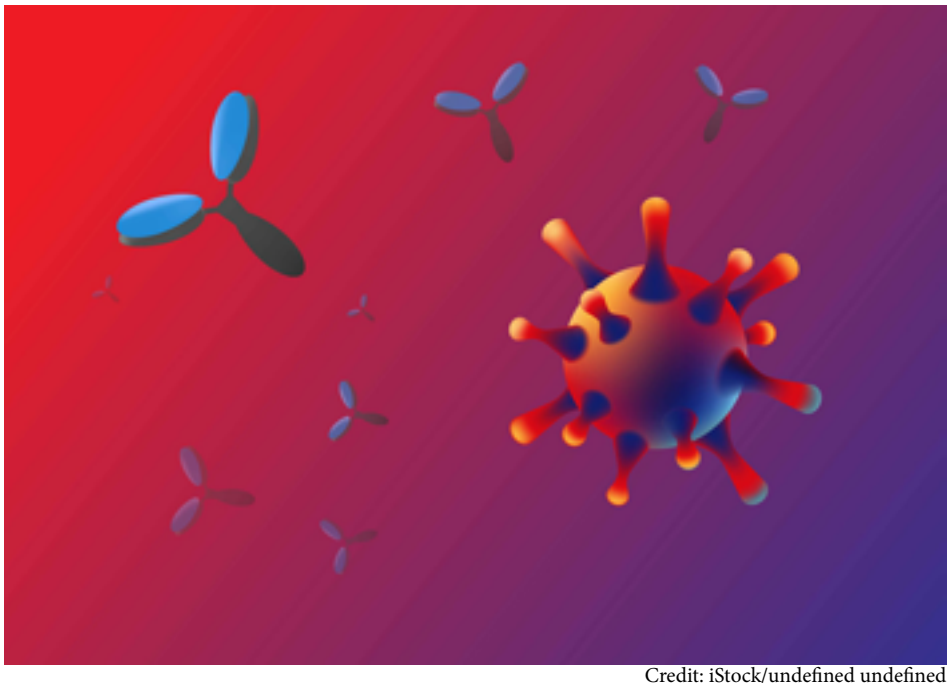


Dr. Joshua Tan, Chief,
Antibody Biology Unit, NIAID

The recombinant mAbs invented by Dr. Joshua Tan and his research team at NIAID can bind to the spike protein of SARS-CoV-2 and at least one other betacoronavirus (MERS/HKU1/OC43) or to the spike protein of at least two betacoronaviruses. The coronavirus mAb patent application managed by TTIPO covers 66 mAbs, several of which have already been shown to be highly effective in neutralizing SARS-CoV-2 in cell culture experiments and in preventing disease in an animal model.

To transfer the NIAID technology to Leyden Laboratories B.V. (Leyden Labs), specialists at TTIPO employed 1) a commercial evaluation license (CEL) and a series of extension amendments, 2) a CRADA, and 3) an exclusive commercialization license. Beyond these mechanisms, the efforts required extensive patent prosecution and invention management work, pre-license execution, and Federal Register Notice (FRN) work to facilitate the advertisement of the technology and the potential exclusive license.

The significance of these efforts at each step cannot be understated—from the extent of patent prosecution to the scale of licensing to the need to navigate background evaluations of IP and fore-ground IP development in a way that properly empowered the licensee. Further, given the manner of development, efforts related to the IP, invention, and agreements were iterative and extensive. The extreme diligence and skill with which Dr. Dawn Taylor-Mulneix (NIAID TTIPO) navigated these negotiations were critical in ensuring that the CRADA, CEL, license amendment, and eventual exclusive license were free of inter-agreement conflicts that could have jeopardized the collaboration.


Credit: iStock/undefined undefined

TTIPO's diligence has not only helped to ensure protection of NIAID IP but sets the stage for additional collaborations with stakeholders in the realm of pandemic prevention, including academic partners, non-profit entities, and government agencies worldwide. The transfer has placed four of the most compelling candidates into the hands of a commercial entity that is positioned to test, develop, and commercialize broadly neutralizing antibodies that can target a variety of coronaviruses—even those yet to be discovered. This is critical considering the setbacks associated with coronavirus antibody development and the present gap in credible therapeutics and prophylactics for coronaviruses. As such and in support of this invention, NIAID TTIPO is also pursuing rights in the United States, Australia, Bahrain, Brazil, Canada, China, Europe, India, Indonesia, Israel, Japan, Kuwait, Malaysia, Mexico, Nigeria, Oman, Philippines, Qatar, Saudi Arabia, Singapore, South Africa, South Korea, and United Arab Emirates.

In recognition of their outstanding efforts, both the research team led by Dr. Joshua Tan and the team of TTIPO professionals managing the technology portfolio (**Dawn Taylor-Mulneix, Ph.D.; Chris Kornak, J.D., M.S.;** and **Fatima Sayyid, M.H.P.M**.) have been nominated for a 2025 Excellence in Technology Transfer Award through the Federal Laboratory Consortium.

Four Decades of International Efforts at NIAID Lead to Breakthrough Dengue Vaccine

Each year, up to 390 million people contract dengue virus—a mosquito borne virus belonging to the same genus as the Zika, West Nile, and yellow fever viruses (*Flavivirus*) (World Health Organization "WHO"). Endemic to nearly 100 countries, dengue virus places incredible demands on public health systems tasked with preventing severe infection, which can be life threatening due to vascular leakage, shock, hemorrhage, severe organ involvement, and respiratory distress. Dengue virus presents unique challenges given that it exists as four serotypes, meaning that a truly effective vaccine would need to ensure sufficient protection against all four of these distinct types (a "tetravalent vaccine").

Dr. Stephen Whitehead,
Senior Investigator, Arbovirus Vaccine Research Section NIAID

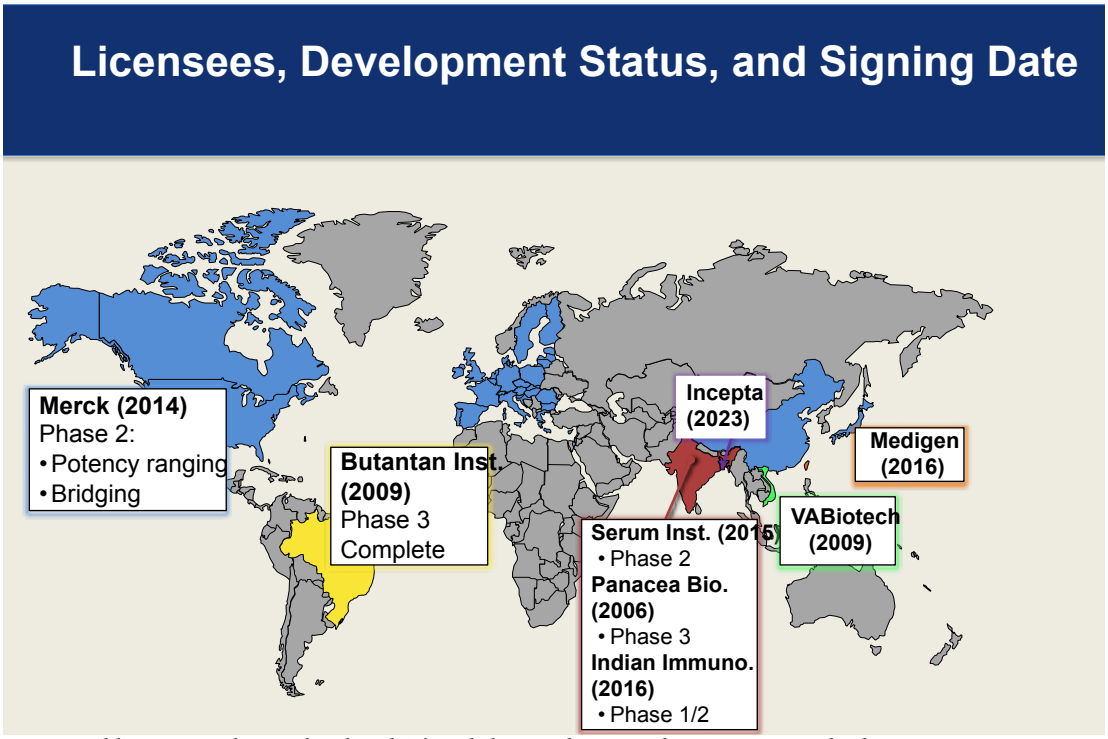
Most adults living in dengue-endemic areas gain natural immunity by "sequential exposure," or exposure to several of these different types during their lives; however, attempting to induce the same sort of immunity in "dengue-naïve" children with a sequence of vaccines is sub-optimal. In fact—and critically, as a key roadblock in the more than 40-year history of dengue vaccine development—it can make things far worse through a phenomenon known as antibody-dependent enhancement (ADE). In ADE, antibodiesfrom a prior infection are present, but they are not necessarily the type that can neutralize the virus, instead binding the incoming virus to enhance infection, resulting in more severe disease.

Beginning in the early 2000s, Dr. Stephen Whitehead and other scientists at NIAID made great progress on dengue virus research and developed a series of vaccine technologies. As the lead inventor, Dr. Whitehead recently delivered the Philip S. Chen Jr., Ph.D., Distinguished Lecture on Innovation and Technology Transfer at NIH, in recognition of his outstanding efforts leading to vaccine development.

While the Dengvaxia vaccine approved by the FDA in 2022 requires three doses, the NIH/Butantan vaccine has demonstrated equivalent protection against all four serotypes in a single dose. Moreover, published trial data for very young children who had received Dengvaxia show that the relative risk of hospitalization at the next dengue exposure was more than seven times higher for those without previous dengue exposure than for those with previous dengue exposure. The NIH/Butantan vaccine, expected to receive ANVISA (Brazilian FDA/Regulatory Authority) in 2025, addresses this major safety concern by more precisely replicating the immune profile of those who have developed sequential immunity over time in these young children.

Because the virus is found mainly in developing countries, TTIPO was especially interested in establishing relationships with regional vaccine developers/manufacturers and encouraging their involvement from the beginning. To increase the likelihood of successful vaccine development while maximizing global access after regulatory approval, TTIPO specialists adopted an innovative approach, employing a combination of both exclusive and non-exclusive licenses depending on regional needs.

Given that vaccines to diseases such as dengue are often considered low-profit, high-volume initiatives, successful transfer leading to actual vaccine development was only possible through a truly international public–private collaboration, leveraging the strengths of each for the benefit of global public health. The care taken to balance the needs of each stakeholder in a field with a 44-year history of challenges was key in securing (and maintaining) collaborative relationships. TTIPO specialists (including Mr. Peter Soukas and Dr. Maryann Puglielli) diligently negotiated agreements based on the needs of each stakeholder and each region, highlighting that it is not just about having the right tools (i.e., the agreements and policies themselves) but in knowing how to use them. Their ingenuity in developing tailored hybrid materials/patent licenses, in combination with the active involvement of NIAID researchers in the marketing and oversight stages to ensure the utmost precision of development efforts, ultimately maximized the chances of successful implementation at the global level.



Created by Dr. Stephen Whitehead, The Philip S. Chen Jr., Ph.D., Distinguished Lecture on Innovation and Technology Transfer, September 25, 2024.

In recognition of their outstanding efforts, both the research team led by Dr. Stephen Whitehead and the team of TTIPO professionals managing the technology portfolio (Maryann Puglielli, J.D., Ph.D.; Peter Soukas, J.D.; and former Branch Chief, Richard Williams, Ph.D.) have been nominated for a 2025 Excellence in Technology Transfer Award through the Federal Laboratory Consortium.

Pozelimab: First FDA-Approved Drug for an NIAID-Identified Ultra-Rare Genetic Disease

In 2016, researchers at NIAID were the first to identify a rare genetic disorder due to the deficiency in the complement protein CD55, CHAPLE (**C**D55 deficiency, **h**yperactivation of the complement, **a**ngiopathic thrombosis, and **p**rotein-**l**osing **e**nteropathy). CHAPLE is an ultra-rare disease affecting fewer than 100 known people worldwide. Patients with CHAPLE disease have two defective copies of the CD55 gene, and the deadly attack complex that ordinarily targets invaders begins to attack the body's healthy tissues, leading to a cascade of life-threatening health problems.

After observing promising results using eculizumab—an FDA-approved treatment for another genetic disease in the inflammatory bowel disease spectrum, paroxysmal nocturnal hemoglobinuria (PNH)—the NIAID team sought to develop a treatment specific to the needs of

patients with CHAPLE. In 2020, NIAID entered a CRADA with Regeneron to study the safety and efficacy of Regeneron's proprietary compound pozelimab (REGN 3918) in a Phase II/III trial in patients with CHAPLE. In August 2023, Veopoz (pozelimab-bbfg) became the first FDA-approved treatment for CHAPLE disease.

Dr. Michael Lenardo of NIAID, lead inventor on the CHAPLE technology, noted that most individuals diagnosed with CHAPLE disease are children who face severely debilitating symptoms and often life-threatening complications that begin in infancy. "*I saw first-hand the transformational clinical improvement that pozelimab achieves in those [living with] CHAPLE. The approval of pozelimab is a milestone to celebrate.*"

One of the participants, Micael, experienced dramatic recovery from the symptoms of this terrible disease shortly after beginning the drug. Shown left to right: Priscilla Quackenbush (the patient's nurse practitioner), Heather Moorman, Mrs. Meri Limachi Pantoja (mother), Ms. Tatiana Limachi (sister), Micael Guzman Limachi, Dr. Michael Lenardo.

From the clinical coordinators attending to the complex needs of the people enrolled, to the team members who fought for compassionate use abroad to obtain the data needed to promote research into novel agents, to Dr. Yogikala Prabhu at TTIPO who worked tirelessly to negotiate agreements that best served the public—efforts at each stage have been significant.

Prior to the initial CRADA draft, Dr. Michael Lenardo participated in advisory board meetings and served as co-chair for steering committee meetings related to the protocol/site feasibility. Dr. Lenardo's active involvement in these preliminary stages ensured that the protocol was both scientifically sound and practically implementable. The CRADA was extensively negotiated, requiring 13 rounds of revisions, budget negotiations, and re-negotiations prior to finalization in August 2020 and was amended three times to address study visits, budgeting, and research costs. Recognizing the need to deliver an effective treatment as quickly as possible to those with the greatest need, co-inventor on the initial CHAPLE discovery Dr. Ahmet Ozen also negotiated with the Turkish Health Ministry on the Ministry's purchase of the first drug studied, eculizumab, for compassionate use therapy in patients with CHAPLE.

In just 8 years, successful, coordinated transfer efforts between the public and private sector have allowed us to go from the initial identification of a rare disease (CHAPLE) to FDA approval of its first treatment option. This success story serves as an inspiration—and model—for future initiatives aimed at tackling rare and complex health challenges through meticulous research and strategic partnerships. The CHAPLE Disease Clinical Trial Team also received a 2024 NIH Director's Award for their exceptional work.

In recognition of their outstanding efforts, both the research/clinical team formerly led by Dr. Michael Lenardo and the team of TTIPO professionals managing the technology portfolio Yogikala Prabhu, Ph.D.; Cecilia Pazman, Ph.D. (formerly at TTIPO); Cosimo Fuda, J.D., Ph.D.; and former Branch Chief Richard Williams, Ph.D., have been nominated for a **2025 Impact Award** through the FLC.

NIAID Spearheads Royalty-Free Initiative To Target Mpox Threat in Priority Regions

On August 14, 2024, WHO Director-General Tedros Adhanom Ghebreyesus convened with leading health authorities, declaring the current mpox outbreak concentrated around the Democratic Republic of the Congo a Public Health Emergency of International Concern (PHEIC). Among the major concerns leading to this designation are the identification of mpox cases in at least 122 locations since initial data collection in 2022 (115 of which have not historically reported mpox), representing approximately 95% of cases; a case-fatality rate (CFR) up to 30 times higher than that reported for the clade that fueled the 2022 outbreak; and further evidence of adapted transmission patterns and atypical presentations in humans first reported during the 2022 outbreak.

Modified vaccinia virus Ankara (MVA), developed more than 30 years ago as a highly attenuated candidate smallpox vaccine, was recloned at NIAID (referred to here as “MVA clone-1”) from a 1974-originating passage and evaluated for safety and immunogenicity in both normal and partially immune-deficient animals. Subsequent studies verified the protective ability of this attenuated vaccine against mpox in nonhuman primates, and clinical efforts since have resulted in FDA approval and availability of a two-dose MVA vaccine in the United States.

In support of the global humanitarian effort to achieve equitable vaccine access and in light of the PHEIC—an outbreak that has resulted in more than 1,000 deaths in the Democratic Republic of the Congo and surrounding regions since the beginning of 2024—TTIPO professionals at NIAID are seeking inquiries from parties interested in independent R&D and/or collaborative research to further develop, evaluate, and commercialize a viable mpox vaccine for distribution (particularly in developing nations/regions currently having minimal access to mpox vaccines) using NIH-provided starting material (MVA clone-1).

While traditional licensing opportunities related to mpox detection are also available (e.g., antibodies, neutralization assays), NIAID will transfer the MVA clone-1 material in question on a royalty-free basis to qualified partners in an effort to combat the current PHEIC. In the event that NIAID has limited ability to distribute material, or if supply

approaches exhaustion, priority will be given to collaborators with a proposed plan demonstrating, in NIAID's sole judgment, the ability to develop a viable vaccine. Potential collaborators considered equally competitive in terms of capacity will also be evaluated based on their plans and intent to distribute in areas with immediate need, followed by the likelihood of the proposed plan contributing to the achievement of a self-sustaining vaccine ecosystem in developing nations.

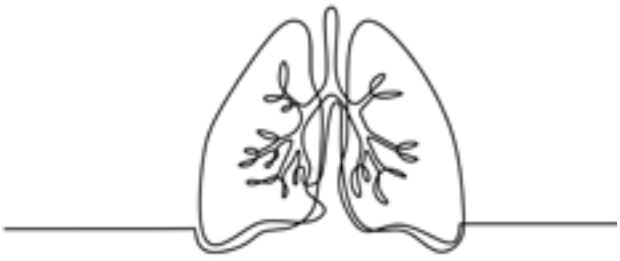
This global initiative, which attenuates access barriers in many critically affected regions and is intended to facilitate such public-private collaborations, was spearheaded by TTIPO professionals, whose experience in navigating international agreements during the height of the COVID-19 pandemic has set the stage for successful and expedient delivery of the materials to regions with the greatest need.

Commercialization of an Investigational Drug in a Cohort of Rare Disease Patients

The Technology Advancement Office at NIDDK (the Technology Transfer service center for the National Institute of Dental and Craniofacial Research (NIDCR)) has negotiated multiple agreements with Calcilytix for using their investigational drug, encaleret, to restore calcium levels in people with autosomal dominant hypocalcemia type 1 (ADH1), a rare genetic disorder marked by an imbalance of calcium in the blood and urine, as well as abnormally low levels of parathyroid hormone, which regulates blood calcium levels. Led by clinician-scientists from NIDCR at the NIH's Clinical Center, results from the successful clinical trial are published in the New England Journal of Medicine. Successful efforts to receive FDA fast track market approval to treat this rare condition continue with pivotal Phase III studies.

US Patent Issued for Invention in the Field of Lung Disease

Michael Fessler, M.D., Clinical Director of NIEHS, was issued a patent on February 6, 2024, for his technology targeting a specific protein called EMP2 on the lining of airways. It is currently the focus of a collaboration with Novartis Institutes for BioMedical Research Inc. to develop antibodies to treat lung diseases, such as asthma, chronic obstructive pulmonary disease (COPD), and cystic fibrosis.



Credit: iStock

NINDS CRADA with DopaVision

At the optic nerve head (ONH) of the mouse retina, axons of retinal ganglion cells (RGCs) converge to form the optic fiber which carries the retinal output to different brain regions. Due to high density of axonal processes around ONH, the retina is devoid of rod and cone photoreceptors in this region and therefore this is also known as the 'blind spot'. However, the blind spot contains axons of intrinsically photosensitive retinal ganglion cells (ipRGCs) which express melanopsin and thereby sense light. Our goal is to stimulate the blind spot using blue light which maximally activates melanopsin (~470nm) and generates action potentials in M1 ipRGCs. M1 ipRGCs provide excitatory inputs to downstream dopaminergic amacrine cells (DACs) which are the sole source of dopamine neurotransmitter in the retina. NINDS and DopaVision entered in to CRADA to study DAC activation elicited via blue light stimulation of blind spot and develop a thorough understanding of the stimulus parameters (intensity, contrast, modulation frequency etc. which increases M1 driven DACspiking activity. Furthermore, we want to investigate changes in dopamine release resulting from ONH stimulation. Dopamine levels are down regulated in myopia and amplifying dopamine release might prove to be a potential therapy for treating this disorder.

NINDS CRADA with Abata

Under a CRADA, NINDS and Abata Therapeutics, Inc., will work together to conduct a study to investigate the impact of Abata's ABA-101 Treg cell therapy on neuroimaging biomarkers including Paramagnetic Rim Lesions (PRLs), as determined by advanced Magnetic Resonance Imaging (MRI), that may be associated with non-relapsing progressive multiple sclerosis.

NINDS CRADA with Elpida

NINDS is collaborating with ELPIDA Therapeutics SPC as one site in a multi-site pre-Phase 1 prospective, non interventional clinical assessment study of FIG4-related Charcot-Marie Tooth Disease (CMT4J). This collaboration is connected to ELPIDA's participation in the FNIH Accelerating Medicines Partnerships (AMP ®) Bespoke Gene Therapy Consortium (BGTC), in which NCATS is the developer and administrator of the associated database. NCATS and Elpida have a separate agreement. NINDS negotiations involved coordination with NCATS and Elpida to ensure the CRADA did not conflict with the other agreements to which NINDS was not a party.

NINDS Collaboration with Sherpa.ai

NINDS will use Sherpa.ai´s Artificial Intelligence (AI) Privacy-Preserving platform, provided as part of collaboration between the parties, to improve the accuracy of diagnosis of rare childhood neurological disorders, such as collagen VI-related muscular dystrophies. NINDS researchers have broad experience in the field of rare childhood neurological disorders. The proposed collaboration will combine the expertise of both parties and will contribute to improving the use of AI solutions for the benefit of such patients while respecting patients' privacy.

Sherpa.ai´s AI Privacy-Preserving solution, enables collaborative AI model training between various organizations without sharing data, and will allow the prediction algorithm to improve the diagnosis without the need to share any patient data, therefore helping to preserve patients' privacy.

One of the major challenges in developing AI algorithms for diagnosis and treatment of diseases is the limited availability of data for AI model training. Organizations often do not have a sufficient volume of quality data to develop accurate AI models, therefore collaboration with multiple organizations is required.

The Federated Learning approach of Sherpa.ai's AI Privacy-Preserving platform allows to leverage private data from different organizations in different countries without exposing or sharing any private patient data., Sherpa.ai´s AI Privacy-Preserving platform enables collaboration across organizations to develop more accurate diagnosis and targeted treatments to improve patients' life, while preserving patient privacy.

Outcome of Agreements with Sanofi for a Multiple Sclerosis Treatment

Sanofi has developed a new treatment that shows significant benefits for people with a form of multiple sclerosis (MS) known as non-relapsing secondary progressive multiple sclerosis (nrSPMS). This is the first time a treatment has effectively slowed down the progression of disability in these patients, reducing the rate of disability progression by about 30% compared to a placebo. This reduction is similar to improvements seen in recent Alzheimer's disease treatments. In trials for another form of MS, called relapsing MS (RMS), this same treatment also delayed the onset of disability progression.

A key factor in the success of the phase 3 study was the collaborative relationship between NINDS and Sanofi. This collaboration was enabled through several important technology transfer agreements, including advisory and authorship agreements, a CRADA for a phase 2 study, and symposium presentations. These agreements enabled the sharing of expertise and resources, paving the way for this groundbreaking research. Researchers at NINDS are hopeful that this study marks the start of more effective treatments for nrSPMS.

Additionally, the FDA has designated this treatment, called Tolebrutinib, as a Breakthrough Therapy for nrSPMS. Sanofi reported that Tolebrutinib delayed the progression of disability by 31% compared to a placebo. Further analysis showed that nearly twice as many participants on Tolebrutinib (10%) experienced an improvement in their disability compared to those on placebo (5%).

This breakthrough is a significant step forward in treating multiple sclerosis, highlighting the crucial role of NINDS and technology transfer agreements and our collaborations with industry in advancing medical research and offering hope for better management of the disease.

Data Deposit for ME/CFS Study Publication – Outcome of 2023 Agreement

The 2024 publication highlighted in the press release found here references a high-profile publication that has been accessed over 90,000 times and referenced in over 1200 social media posts and news outlets. The journal required depositing associated data into a publicly-accessible database as a condition for publishing the manuscript. The study authors ran into challenges with some of the repositories they reached out to, and eventually started dialog with one who sent a data transfer and use agreement that had been developed for a NIH-funded consortium. While the existing framework addressed some common issues, several remained based on the distinction between consortium use, the involvement of a company, and the specifics of the situation. NINDS tech transfer successfully negotiated the agreement in 2023 on an expedited basis to overcome what was the last hurdle to moving the publication forward.

Credit:iStock/Jinda Noipho

AWARDS, PRESENTATIONS, AND PUBLICATIONS

NHGRI Awards and Outreach

NHGRI has continued its tradition of volunteering and community services in FY2024, with all office members contributing to various technology transfer activities across the NIH and academic communities.

The TTO team continued its educational outreach to the NHGRI community by giving talks to various groups and visiting intramural labs. In December 2023, the team presented in-person at an extramural meeting an overview titled: "T*echnology Transfer at NHGRI and NIH.*" The TTO also gave a talk "T*echnology Transfer in Intramural Research*" to various labs and branches in FY2024.

The TTO Director gave a number of educational talks within and outside the NIH, including: "*Filing Employee Invention Reports with the NHGRI Technology Transfer Office*," at a Faculty Meeting; "*Overview of Gifts and Technology Transfer at NHGRI and NIH*," for the Division of Management, and "*Material Transfer Agreements (MTAs) at the National Institutes of Health*" to the Pan American Health Organization.

Credit:iStock/PCH-Vector

Two Office members received an NIH Office of Director Honor Award "*In recognition of the outstanding commitment and collaboration shown in implementing the Enterprise Technology Transfer system as the system of record for NIH Technology Transfer.*"

Another office member is a participant in various NIH and federal working groups, including one for updating the PHS Technology Transfer Policy Manual; a Technology Transfer University Working Group; and Federal Technology Transfer wide working group/subcommittee for Awards for the FLC.

FLC Award: NIAID Research Leads to First FDA-Approved Treatment for APDS

APDS (activated PI3 kinase delta syndrome)—also known as PASLI (p110 delta-activating mutation causing senescent T cells, lymphadenopathy, and immunodeficiency) disease—is a rare disorder that severely impairs the immune system's ability to fight bacterial and viral infections, making patients susceptible to severe and recurrent infections, lymphoma, autoimmune diseases, and other health issues.

NIAID researchers discovered that people with APDS have a common variation in the PI3 kinase gene that causes the gene's pathway to become hyperactive, producing an imbalance in white

blood cells and making it difficult for the body to fight infections.

In 2015, NIAID entered into a CRADA with Novartis to assess the safety and efficacy of their proprietary compound leniolisib as a treatment for APDS in a clinical trial. After this trial produced positive results, the team launched a Phase III clinical trial in 2018 and an extension study to test the drug's long-term safety and efficacy in APDS. In 2019, the commercialization rights to the clinical development of leniolisib were transferred from Novartis to Pharming Group N.V. The CRADA was amended to establish Pharming as NIAID's new collaborator for the leniolisib/APDS trial. In July 2022, Pharming submitted a New Drug Application for leniolisib; the following March, the FDA granted full approval of leniolisib to treat APDS in adults and pediatric patients 12 years and older.

TTIPO professionals including Dr. Yogikala Prabhu played a key role in formalizing the collaboration under a CRADA with Novartis and in negotiating the amendments necessary to launch the Phase III trial and extension studies. Working in close collaboration with clinical, financial, legal, and research teams at NIAID and the partnering entities, TTIPO meticulously executed 10 separate CRADA amendments to accommodate the partners' evolving needs.
In recognition of their central roles in the conceptual and commercial development of the first and only U.S. FDA-approved treatment for APDS, the NIAID research/clinical team and TTIPO professionals received a **2024 Excellence in Technology Transfer Award** from the FLC. In recognition of these groundbreaking efforts, NIAID also received a congressional citation from Senator Chris Van Hollen (D-MD), a who serves as a member of the Rare Disease Legislative Caucus.

Awardees included Yogikala Prabhu, Ph.D.; Michael Lenardo, M.D. (formerly at NIAID); V. Koneti Rao, M.D.; Gulbu Uzel, M.D.; Sharon Webster (formerly at NIAID); Alan Orpia; Cecilia Pazman, Ph.D. (formerly at NIAID); Peter Tung, Ph.D., MBA; and Cosimo Fuda, J.D., Ph.D.

FLC Award: FDA-Approved RSV Vaccine Based on NIAID's F Protein Technology

Respiratory syncytial virus (RSV) is a common virus that typically causes mild, cold-like symptoms; however, it can be deadly to older adults, young children, and people with chronic illnesses or weakened immune systems. In the United States, 60,000 to 160,000 adults 65 years and older are hospitalized with RSV each year, and up to 10,000 people die each year from RSV infection, according to the Centers for Disease Control and Prevention.

In 1984, researchers at NIAID isolated a crucial protein, known as the fusion glycoprotein or F protein, that enables the virus to infect human cells. Building on this knowledge, NIAID's Vaccine Research Center began investigating the F protein for the development of an RSV vaccine in 2006. In 2013, NIAID scientists achieved a breakthrough by finding a new way to stabilize the F protein in its prefusion form, making it more effective in stimulating an immune response. This achievement paved the way for the creation of antibodies and vaccines, such as Arexvy, which is now the first U.S FDA-approved vaccine of its kind to prevent RSV disease in individuals aged 60 and older. By bringing together cutting-edge science and innovative research, Arexvy offers a

promising solution to help protect older individuals from the most severe effects of RSV-associated lower respiratory tract disease.

GlaxoSmithKline Biologicals SA's (GSK) RSV vaccine, Arexvy, has shown over 80% efficacy in preventing lower respiratory tract disease caused by RSV in individuals aged 60 and older. It is similarly effective for older adults with select underlying medical conditions that put them at higher risk for RSV disease.

In 2013, NIAID filed a patent and published its discovery. Shortly after, Novartis applied for and signed a nonexclusive commercial license. In 2015, when Novartis sold its vaccine business to GSK, the RSV license from NIAID was transferred to GSK. To further develop the RSV vaccine, TTIPO professionals at NIAID negotiated two RCAs with GSK. These agreements enabled fruitful collaboration between NIAID and GSK scientists. TTIPO professionals including Dr. Carol Salata also developed an innovative strategy that allowed for nonexclusive licensing to 10 other companies, encouraging these companies to develop their own RSV vaccines, diagnostic tools, and treatment products while expanding the impact of NIAID's pioneering research.

With an estimated 76.5 million people aged 60 and older in the United States, Arexvy has the potential to make a significant positive impact. By helping to reduce the number of symptomatic RSV cases among older adults, this vaccine can help improve health outcomes and decrease hospitalizations, ultimately lightening the load on healthcare systems and lowering healthcare costs.

Credit:iStock/ajijchan

In recognition of their central roles in the conceptual and commercial development of the first U.S. FDA-approved vaccine for RSV—which has been in strong demand based on sales data provided by GSK in January 2024—the NIAID research team and TTIPO professionals managing the technology portfolio received a 2024 Impact Award from the Federal Laboratory Consortium.
Awardees included Carol Salata, Ph.D.; former NIAID personnel Jason McLellan, Ph.D., Barney Graham, Ph.D., Peter Kwong, Ph.D., Vince Contreras, Ph.D., Barry Buchbinder, Ph.D., and Vincent Feliccia, Ph.D.; and Cristina Thalhammer-Reyero, Ph.D. (formerly at NIH OTT).

Research Collaboration Results in NIEHS Individual Merit Award

Dr. Stavros Garantziotis (Immunity, Inflammation and Disease Laboratory) received a NIEHS Individual Merit Award for, "excellence in scientific validation and clinical translation of a novel matrix-based therapeutic for human lung disease." On April 26, 2023, NIEHS OTT negotiated and executed a CRADA for Dr. Garantziotis to collaborate on a research and development project with TFF Pharmaceuticals, Inc. to develop respirable, dry powder formulations of high molecular weight hyaluronan for treating or preventing respiratory diseases.

Two NIH Inventors Named National Academy of Inventors Fellows

Two NIH inventors, Drs. Peter Basser and Carlos Zarate, Jr., have been selected as part of the 2024 National Academy of Inventors (NAI) Fellows. NAI Fellowship is the highest professional distinction awarded solely to inventors.

Dr. Peter Basser

The NAI is a member organization comprising U.S. and international universities, and governmental and non-profit research institutes, with over 4,000 individual inventor members and Fellows spanning more than 250 institutions. The 2024 Fellows hail from 135 research universities, governmental and non-profit research institutions worldwide and their work spans across various disciplines. They are not only phenomenal researchers holding prestigious honors and distinctions but are also incredible inventors who collectively hold over 5,000 issued U.S. patents and whose innovations are making significant tangible societal and economic impacts today and will well into the future.

Dr. Peter Basser is a Senior Investigator in the Section on Quantitative Imaging and Tissue Sciences at the NIH Eunice Kennedy Shriver National Institute of Child Health and Human Development (NICHD). Dr. Basser is widely known for the invention, development, and clinical implementation of MR diffusion tensor imaging (DTI), diffusion tensor “streamline tractography,” and other quantitative MRI methods for performing in vivo MRI histology or “microstructure imaging”. Dr. Basser’s work has transformed how neurological disorders and diseases are diagnosed and treated, and how brain architecture, organization, structure, and anatomical “connectivity” are studied and visualized. Dr. Basser was recently an NIH Distinguished Lecturer for the 15th Annual Philip S. Chen, Jr., Ph.D., Distinguished Lecture on Innovation and Technology Transfer hosted by the NIH. His talk was on using water migration to probe brain structure and architecture.

Dr. Carlos Zarate

Dr. Carlos A. Zarate, Jr., is Chief, Section on the Neurobiology and Treatment of Mood Disorders and Chief of Experimental Therapeutics and Pathophysiology Branch (ETPB) at NIMH and Clinical Professor of Psychiatry and Behavioral Sciences at The George Washington University. Dr. Zarate formed the Experimental Therapeutics and Pathophysiology Branch at the NIMH in 2009. Dr. Zarate has had a prolific career researching the treatment of mood and anxiety disorders, most recently licensing an invention that led to the development of the first FDA-approved drug for treatment-resistant depression (Spravato®). Dr. Zarate’s Branch conducts proof-of-concept studies utilizing novel compounds and biomarkers (magnetoencephalography [MEG] and polysomnography [PSG], positron emission tomography [PET], functional magnetic resonance imaging [fMRI] and magnetic resonance spectroscopy [MRS]) to identify potentially relevant drug

targets and biosignatures of treatment response. He is a member of the National Academy of Medicine and was the NIH Distinguished Lecturer for the 16th Annual Philip S. Chen, Jr., Ph.D. Distinguished Lecture on Innovation and Technology Transfer hosted by the NIH. His talk was on developing novel medications for treatment resistant depression and bipolar disorder.

NINDS Presentations

NINDS co-authored a poster entitled "*Proposed NIH Core for Advanced Genetic Therapies - Spotlight on Clinical Development of AAV Products for Neuromuscular Disorders*", presented at World Muscle Society Annual Congress.

NINDS participation in Korean Bio I-Corp meeting in September 2024.

NINDS co-presented with Lauren Nguyen-Antczak (NCI) in NINDS extramural-led Parkinson's Disease workshop in session about patents, IP, and tech transfer on April 24, 2024.

NIH OTT Wins GOVTECH CONNECT's Digital Health Transformation Award

The NIH OTT Enterprise Technology Transfer (ETT) system has been selected as a GOVTECH CONNECTS's Digital Health Transformation Award Recipient.

The Digital Health Transformation Award recognizes Federal and Military Health IT programs that are harnessing the power of emerging technologies to advance their missions and spotlight the dedicated teams behind this groundbreaking work.

NIH OTT partnered with Publicis Sapient to create the Enterprise Technology Transfer (ETT) system, a Herculean effort that involved migrating nine unique technology transfer systems into one unified platform. This ambitious project required exceptional leadership to align the 27 Institutes and Centers (ICs), each of which had grown comfortable with their legacy systems. The project team led extensive data mapping, ensuring the functionality of each IC's customized processes within the new system.

The ETT system revolutionized technology transfer at NIH by automating processes and centralizing data, eliminating duplicative work, and enhancing transparency across the ICs. Unlike most cloud-based systems, ETT operates securely behind the NIH firewall, ensuring robust data protection while maintaining compliance with security and policy guidelines—a strategic decision that required significant upfront effort but provides long-term security benefits.

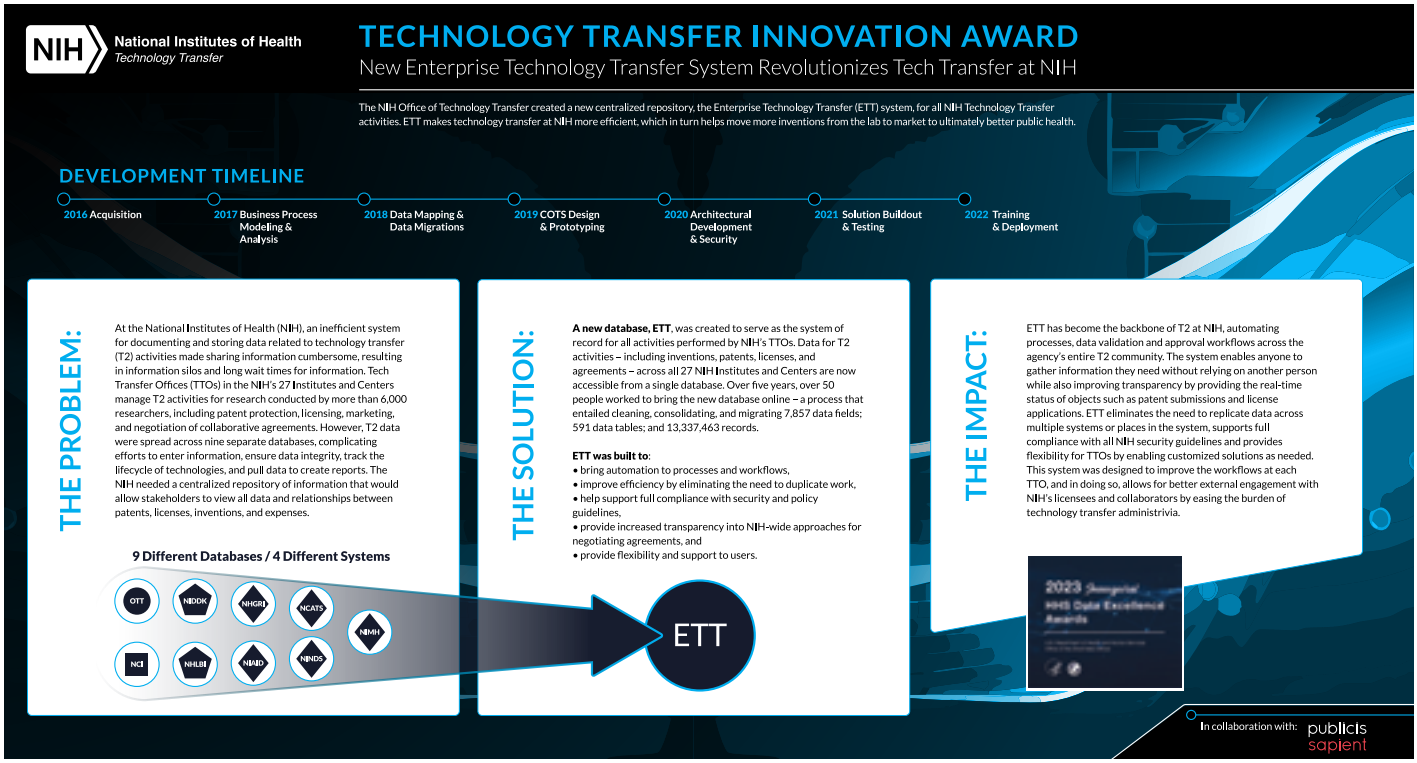
ETT's cloud-based architecture offers scalability and agility,

streamlining tasks like filing patents, marketing technologies, and managing agreements. This infrastructure ensures the system's resilience and adaptability, enabling seamless operations even during high demand or technological changes. By simplifying workflows and reducing administrative burdens, ETT enhances engagement with NIH's licensees and collaborators, allowing the TTOs to focus on fostering innovation.

ETT also provides a comprehensive database, offering detailed history and performance for each organization that has previously done business with NIH Tech Transfer. This transparency and accountability empower NIH to manage research, patents, and financial resources more effectively, promoting innovation in biomedical research. Through its forward-thinking design and strategic use of emerging technologies, ETT has transformed how NIH manages technology transfer, overcoming challenges and setting a new standard for efficiency and security.
By engaging with stakeholders at every level, the project team built trust, convinced skeptics, and ultimately delivered a system that revolutionized how NIH manages technology transfer, setting a new benchmark for efficiency, transparency, and security in biomedical research.

Honorees were formally recognized at the Digital Health Summit '24 that took place on Wednesday, October 30, 2024. This event brought together leaders and innovators from across government and industry to celebrate transformative achievements in health IT.

ETT also accepted an **FLC Technology Transfer Innovation award** at the 2024 National Meeting and was showcased on the **HHS Office of the Chief Data Officer Data Strategy** webpage.



Other Conference Presentations and Publications

Conference Presentations

MedInvest Investor Conferences

- ""NIH as Your MedTech Development and Commercialization Partner", October 4, 2023, Steven Ferguson (OTT).
- "Beyond SBIRs: Leveraging the NIH to Improve Pipelines and Bottom Lines" and "NIH as Your Technology Development and Commercialization Partner", December 5-6, 2023, Michael Salgaller (NCI) and Steven Ferguson (OTT).
- "NIH as Your Technology Development and Commercialization Partner" and "Beyond SBIRs: Leveraging the NIH to Improve Pipelines and Bottom Lines" and April 3-4, 2024, Michael Salgaller (NCI) and Steven Ferguson (OTT).
- "Beyond SBIRs: Leveraging the NIH to Improve Pipelines and Bottom Lines" and "NIH as Your Technology Development and Commercialization Partner", September 18-19, 2024, Michael Salgaller (NCI) and Steven Ferguson (OTT).

AUTM Annual Meeting

- "AUTM Technology Valuation Course", February 18, 2024, Steven Ferguson (OTT).

Licensing Executives Society Annual Meeting

- "Art of Negotiating & Monetizing Rare Pediatric Disease Priority Voucher Terms into a License Agreement", October 16, 2023, Eggerton Campbell (NHGRI), and Steven Ferguson (OTT).
- "The U.S. Manufacturing Requirement of the Bayh-Dole Act: Analysis and Practical Tips, October 16, 2023, Tara Kirby (OTT).

Licensing Executives Society Webinars

- "LES Webinar: Beyond the Contracts - Measuring the Actual Impact of a Patent Licensing Program", November 29, 2023, Steven Ferguson (OTT).
- "LES Webinar: Yescarta®: A Journey from Development to Licensing", December 7, 2023, Andrew Burke (NCI).
- "LES Webinar: Industry, Universities, and Government – How to Work Together Through Contracts", June 25, 2024, Steven Ferguson (OTT).

NIH-KWiSE Biotech Intellectual Property Forum

- "Careers for Scientists in Technology Transfer and Business Development", March 21, 2024, Steven Ferguson (OTT).

Federal Laboratory Consortium Annual Meeting

- "Training Day: Negotiations Course", April 9, 2024, Steven Ferguson (OTT).
- "Beyond Patents and Contracts: Measuring the True Impact of a Technology Transfer Program", April 11, 2024, Steven Ferguson (OTT) and Tara Kirby (OTT).
- "NCATS Advances Translational Research via Collaborations and the Synergistic Blending of Extramural and Intramural Programs", April 11, 2024, Krishna (Balki) Balakrishnan (NCATS), Meena Rajagopal (NCATS), Jasmine Kalsi (NCATS) and Ami Gadhia (NCATS).

Federal Laboratory Consortium Podcast

- “The Technology Transfer Files Podcast”, May 23, 2024, Steven Ferguson (OTT).
- Technology Transfer Society (DC Chapter) Course
- “Introduction to Technology Transfer”, September 11, 2024, Steven Ferguson (OTT).

American Society for Pharmacology and Experimental Therapeutics Annual Meeting.

- “Moving Your Discovery from Bench to Bedside: IP Considerations for Scientists”, May 19, 2024, Tara Kirby (OTT).

Publications

- “Will AI Shape the Future of Technology Transfer?” by Berna Uygur (NCI) and Steven Ferguson (OTT), les Nouvelles Vol. LIX No. 1 2024, March; 1-11.

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
- 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)

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:

- NIAMS - National Institute of Arthritis and Musculoskeletal and Skin Diseases
- NIDCR - National Institute of Dental and Craniofacial Research
- 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/