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The U.S. Biotechnology Translational Research Partnership Models, Management Principles, and Best Practices

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Second Edition



Where
breakthroughs
begin

Translating the Life Sciences Research Ecosystem

MODELS, MANAGEMENT PRINCIPLES, AND BEST PRACTICES

Introduction

There has been increasing public attention and new programmatic efforts to advance translational research as the bridge between the promise of bioscience discoveries and the advancement of new biomedical innovations to improve the lives of patients. This is especially true as the post-COVID world seeks new and innovative therapies and other resources to combat the next generation of medical needs across the world.

Of particular importance to the Biotechnology Innovation Organization and its partners is a more comprehensive understanding of the evolving nature of translational research and the ability to overcome the challenging environment for bioscience innovation to advance collaborations between industry and academia as a means to both improving R&D productivity and reducing the costs of translating discoveries into new medical products.

Not only is there a greater commitment but efforts to strengthen existing close ties between industry, clinical care, and academic communities necessary to fulfill the necessary interface of “bench and bedside” required for biomedical innovation to move forward.

This first-ever best practice models review and guiding principles examines the extent of current industry-academic-non-profit collaboration considerations both for the rich environment of engagement and the contribution between industry and academic partners across the four stages that all translational research must pass through in bringing new treatments to patients—basic and applied, technology development, clinical trials, and new product launch.

By continuing to develop innovative models for collaboration that reflect the unique nature of translational bioscience innovation, these diverse relationships can continue to be accelerated to address the critical challenges facing the bioscience industry.



Translational Research in Real Time Context

Beyond just the numbers, there is a “real world” context of exciting developments taking place in industry-academic collaboration activities to advance translational research.

In basic and applied research, industry-university research collaborations are evolving. Two significant developments are taking hold in advancing collaborations at the research stage that hold promise in reaching a new level of scale beyond which individual company sponsoring and jointly conducting research with an academic institution can achieve, including:

- **Rise of multi-institutional and multi-company collaborations**
- **Rise of open innovation models that provide academic researchers broader access to research tools and even funding from industry in a more streamlined and open process**

In technology development, industry-academic collaborations are increasingly focused on advancing systematic, replicable approaches for creating value through leveraging scientific, clinical, and business know-how versus a more ad hoc project-by-project approach. Two areas where these more systematic, replicable approaches are taking hold include:

- **Advancing partnerships of clinicians with engineers and scientists**
- **Fostering new venture development approaches**

In clinical trials, industry-academic collaborations may be an area that offers opportunities for increased activities, especially in light of the complexity of clinical trials. Among the examples of emerging best practices, models include:

- **Regional clinical trials consortia**
- **CRO-CTSI partnerships**
- **Centralized patient repository**

In launching new products, industry-academic collaborations are benefiting from more applied research capacities that academic institutions are advancing, particularly in two areas:

- **Experimental therapeutics centers**
- **Advanced biomanufacturing centers**

Opportunities for BIO to Help Accelerate Industry-Academic Translational Research Collaborations

BIO, in bridging the worlds of biotechnology industry and academic research, can play a critical role in convening and disseminating emerging models. In convening, BIO can scale-up the localized efforts on collaborations to have a larger footprint as well as possibly impacting how federal and state initiatives to advance bioscience development proceed. Among opportunity areas for convening would be:

- Working with patient advocacy groups to initiate larger scale multi-institutional and multi-company translational research collaborations.

- Advancing larger scale, multi- institutional patient registries.
- Promoting more engagement between contract research organizations and NIH-funded clinical and translational research institutes.

In dissemination of emerging models and resources available, there is much to be done, including:

- Better cataloging the many available translational research activities taking place involving open innovation, applied academic research resource centers, and ongoing partnerships of industry, clinicians, engineers and scientists.
- Tracking the success and impact these industry- university translational research efforts are having and focusing increased discussion on best practices and how to achieve increased scale.

FRAMEWORK FOR MEASURING INDUSTRY-UNIVERSITY COLLABORATIONS ACROSS THE TRANSLATIONAL PARADIGM



Translating Basic Scientific Discoveries^{1, 2, 3, 4}

The translation of basic scientific discoveries into health treatments, practices and policies lags far behind the pace of discovery, and only a fraction of promising discoveries actually generates real-world applications. A number of factors contribute to the slow pace of translating discoveries from bench to bedside:

- Translational research is a complex, expensive process that requires skill sets and resources that can exceed any one entity's strengths and resources.
- Biopharmaceutical companies have reduced internal R&D efforts, and depend on alternate strategies to access innovative research and replenish their pipeline.
- Academic researchers' career advancement and recognition are tied to metrics such as publications and research output, not to translational efforts

1 Bazan, C. (2019). "From lab bench to store shelves:" A translational research & development framework for linking university science and engineering research to commercial outcomes. *Journal of Engineering and Technology Management*, 53, 1-8.

2 Freedman, S., & Mullane, K. (2017). The academic-industrial complex: navigating the translational and cultural divide. *Drug Discovery Today*, 22(7), 976-993.

3 Yu, H. W. (2016). Bridging the translational gap: collaborative drug development and dispelling the stigma of commercialization. *Drug Discovery Today*, 21(2), 299-305.

4 Libby, P. (2018). Partnerships Promote Translation in Biomedicine. *JACC: Basic to Translational Science*, 3(3), 431-433.

- Early-stage research is high-risk, so researchers face difficulty in securing funding to develop proof of concept to advance their discoveries.

Collaborations between academia, industry, government, and funding agencies are critical to translational research. By pooling resources and integrating people trained across disciplines, strategic partnerships can accelerate the commercialization of biomedical research for both public benefit and commercial interest. Some of the following common mechanisms aid in catalyzing translational research:

- **Resource and expertise sharing.** Partnerships bring together the right combination of discovery and development, clinical, product design, regulatory, marketing, and business expertise to move a discovery forward. Partner organizations may also contribute valuable assets such as laboratory space and equipment, compound libraries, and data. The sharing of knowledge and resources makes the R&D process less expensive, less risky and more efficient for any single partner.
- **Funding.** Academic researchers face a dwindling supply of NIH grant funding, and federal grants are not always optimized to fund translational projects. Partnerships open up alternative funding mechanisms through industry support, public or non-profit initiatives, or dedicated funds from an academic translational center.
- **Research prioritization.** Only a small subset of basic discoveries merit serious consideration as viable translatable opportunities. Translational research programs establish guidelines to aid in that selection.

For example, a pharmaceutical company may seek academic partners to collaborate on research that complements its internal pipeline, but that is too high-risk to develop internally. Public-private partnerships may incentivize translational research that is not as financially rewarding, but that benefits underserved populations or that is in line with public health initiatives.

- **Startup creation and workforce development.** Translational programs can incorporate elements of economic development and workforce training by coaching academic researchers in commercialization and startup creation principles, funding workforce or postdoc programs, or establishing incubator and accelerator programs.

Model Frameworks for Translational Research Partnerships.

There are many existing partnerships taking place in industry-academic collaboration activities to advance translational research. While not complete, the following case studies are a beginning step in offering a sense of the range of effort beyond the one-company to one academic center partnership to a more elaborate and dynamic multi companies/multi institutions structures to gain scale and impact.

The following section describes four models of translational research partnerships, with case studies that illustrate the growing dynamic developments taking place in collaborations. The broad models include:

- University-based programs
- Industry-driven programs
- Non-profit organizations
- Public-private consortia

MODEL 1: UNIVERSITY-BASED PROGRAMS

Academic translational initiatives have been created to support entrepreneurial faculty with guidance on development and commercialization of inventions.

University-based initiatives take many forms, including tech transfer offices, independent translational centers, partnerships with specific companies, or university-affiliated incubators or investment funds that support spin-out companies. Initiatives may include elements like independent space and laboratory equipment; funding for project proposals (which may be funded or matched by a partner); collaborative engagement from industry interested in accessing technology developed at the university; and business, fundraising and IP advising to guide the inventor through the translational process.

SPARK AT THE STANFORD UNIVERSITY SCHOOL OF MEDICINE^{5, 6, 7}

sparkmed.stanford.edu

Established in 2006, SPARK is a partnership between academics and industry experts dedicated in overcoming the hurdles of translating academic discoveries into drugs and diagnostics. SPARK was started by Stanford University School of Medicine and provides financial support, infrastructure, and access to clinicians to assist researchers better understand the clinical implications of their research.

The program provides education and funding to assist academic investigator translate their research into therapies.

They accept 10-15 academic researchers (SPARK scholars) annually to pursue basic research and receive \$50,000 annually for two years, distributed as project milestones are met and mentoring from industry experts.

Their volunteer advisor network, made up of 100+ experts from startups and industry, play a critical role in the program. They evaluate projects and provide weekly mentoring and seminars in scientific and business topics.

External agencies, including government and nonprofit agencies, provide additional funding and may set research priorities tied to that funding.

UNIVERSITY OF MARYLAND: MARYLAND INDUSTRIAL PARTNERSHIPS PROGRAM (MIPS)

<http://www.mips.umd.edu/>

Maryland Industrial Partnerships (MIPS) provides funding, matched by participating companies, for university-based research projects that help the companies develop new products. In these academic-industrial, public-private partnerships, MIPS connects the resources of the Maryland public universities to Maryland businesses.

MIPS projects are focused on translational work that leads to new or improved products. The company's products may be, but do not have to be based on the universities' intellectual property.

MIPS provides matching funds to help Maryland companies pay for the university research. Projects are initiated by the companies to meet their own research and development goals. MIPS matching funds are awarded on a competitive basis for projects based on proposals submitted jointly by Maryland companies and researchers from any of the 13 University System institutions.

5 Kim, E. S., Omura, P. M., & Lo, A. W. (2017). Accelerating biomedical innovation: a case study of the SPARK program at Stanford University, School of Medicine. *Drug Discovery Today*, 22(7), 1064-1068.

6 SPARK at Stanford. www.sparkmed.stanford.edu. Accessed August 1, 2019.

7 Ouellette, Jennifer. "From Bench to Bedside." *Alta*. April 22, 2019. altaonline.com/from-bench-to-bedside/

DUKE UNIVERSITY: MEDBLUE INCUBATOR

<http://medblueincubator.com/>

MedBlue Incubator is a for-profit incubator designed to foster translation of research primarily from (but not limited to) the Duke School of Medicine. They are focused on pharmaceuticals, med devices and diagnostics, but projects from other technology areas are welcome to apply. The program offers initial seed funding, advising and early-stage business planning, strategies for deal negotiation and intellectual property protection, and opportunities for corporate partnerships. The MedBlue Incubator has an agreement with Alexandria Real Estate Equities to provide participating companies with affordable office and lab facilities in Research Triangle Park.

Strategic investors are invited to invest and become MedBlue shareholders. Investors receive equity and dividends from MedBlue, priority access to technology and IP, and the opportunity to get more involved in the development of new companies around these technologies.

THE BIOMARKER FACTORY AT DUKE UNIVERSITY

<http://www.biomarkerfactory.com/>

The Biomarker Factory is a for-profit limited liability company jointly founded by Duke University's Duke Medical Strategies, and Laboratory Corporation of America (LabCorp). The goal of this effort is the identification and development of biomarkers for use in clinical practice and commercialization of diagnostic tests.

There is not a fixed level of funding for projects, and proposals are accepted from academic institutions, non-profit organizations and for-profit companies. Typically, funding covers all project costs for a period of one to two years. The Biomarker

Factory has the option to license the inventions made from funded projects, as well as pre-existing intellectual property related to the funded project. LabCorp then has the first option to license these inventions from the Biomarker Factory.

UNIVERSITY OF MASSACHUSETTS AMHERST: INSTITUTE FOR APPLIED LIFE SCIENCES

<https://www.umass.edu/ials/>

The Umass Institute for Applied Life Sciences (IALS) is focused into three centers: The Models to Medicine Center, the Center for Bioactive Delivery, and the Center for Personalized Health Monitoring. More than 30 core facilities are offered to internal and external researchers, including government and industry partners.



MODEL 2 INDUSTRY-DRIVEN PROGRAMS

Many multi-national pharmaceutical corporations have established some kind of activity or program towards open innovation and collaborative research. This includes funding translational research at academic institutions, providing accelerator spaces for entrepreneurial activities, screening of novel compounds, and making their own technologies and compound libraries accessible to academic and start-up collaborators.

The increasing costs and complexity of developing new medicines have pushed pharmaceutical companies to pursue partnerships that trade competitive secrecy for collaborative development and access to early research. Such partnerships can provide new opportunities and knowledge to the company at a lower up-front cost and lower risk to the company.

The shift to a networked, partner model has been driven in part by the concept of open innovation, through which companies make available their assets and data (often a company's compound libraries) to testing and development by external researchers. Industry-led translational efforts may also include sponsoring postdoctoral programs that support the company's research interests; creating life science incubators to encourage entrepreneurial innovation; crowd-sourcing initiatives to resolve discrete R&D problems; and funding venture arms to invest in research related to the company's interests.

ASTRAZENECA OPEN INNOVATION^{8, 9}

openinnovation.astrazeneca.com

AstraZeneca launched its Open Innovation initiative in 2014 to help it forge better research links with academia, industry, NGOs and governments through collaboration on complimentary research projects. This is part of its effort to reinvigorate the company's R&D operations by generating new insights and potential research programs from AZ's existing data and compounds.

- **AstraZeneca** provides access to employees' expertise, company resources, data and technology, and in some cases, grant funding and lab space. AZ may also partner with external funding providers to provide additional funding for collaborative projects.
- **Researchers at universities, nonprofits or government** bring disease knowledge and technology to solve R&D challenges and test hypotheses that has the value potential for AZ.
- **External funding agencies**, including national and international funding bodies and academic institutions may provide additional funding for collaborative studies that use AZ compounds.

AMAZON CATALYST

<https://catalyst.amazon.com/>

Amazon has partnered with the University of Washington and Washington State University to provide funding for innovative research projects meeting real-world needs, through a program called Amazon Catalyst. Additional universities may be added in the future. Current faculty, staff and students at these universities may submit projects for consideration on a quarterly cycle. Approved projects receive grant awards from \$10,000 to \$100,000 for projects lasting up to 18 months. Though the projects across all disciplines are

8 Open Innovation. [www.openinnovation.astrazeneca.com](https://openinnovation.astrazeneca.com). Accessed August 1, 2019.

9 Roland, Denise. "AstraZeneca reinforces pipeline with 'open innovation.'" The Telegraph. April 28, 2014. www.telegraph.co.uk/finance/newsbysector/pharmaceuticalsandchemicals/10775160/AstraZeneca-reinforces-pipeline-with-open-innovation.html

considered, many of the funded projects have been in medical and life sciences. Intellectual property developed as a part of the funded projects is held by the sponsoring university.

ASTRAZENECA: OPEN INNOVATION

<https://openinnovation.astrazeneca.com/>

Through their Open Innovation platform, AstraZeneca seeks clinical and basic researchers to partner on the identification of new applications for their identified compounds. Both preclinical, and 'patient-ready' compounds are available to external partners for translational research. A selection of later-stage compounds are also offered through their Externally Sponsored Scientific Research portal. Researchers wishing to investigate these compounds submit an initial research proposal to AstraZeneca which is reviewed within 8 weeks. If a full project proposal is approved for execution of the project, AstraZeneca meets regularly with the principle investigator of the work and a final report is submitted with findings.

The investigators have the rights to publish their results and use data to support additional studies and grant applications. They also may receive royalties if the IP is licensed and commercially successful.

AstraZeneca also accepts proposals for the use of their screening libraries, oncology combinations and preclinical data set. They solicit submissions of new molecules for assessment within their scientific areas of focus, and share some of their own internal R&D challenges in a crowd-sourcing model to find solutions. All of these pieces are included under the Open Innovation umbrella.

ELI LILLY: OPEN INNOVATION DRUG DISCOVERY

<https://openinnovation.lilly.com>

Eli Lilly's Open Innovation Drug Discovery (OIDD) program is open to any researchers at eligible research universities, institutes and small biotechnology companies. Institutions simply complete Lilly's universal program agreement to participate, and are then given access to Lilly's chemical libraries, and tools for computational design and in silico analysis.

Compounds which meet the strategic focus of Lilly and Elanco can then be sent for biological evaluation and screening through the company's target-based assays. Participating institutions retain control of their intellectual property rights and confidentiality, and publication of results is encouraged. If the compound is of interest, Lilly may decide to negotiate a collaboration, license or acquisition of the compound. The program also includes the option to access Lilly's Automated Synthesis Laboratory, and resulting synthesized samples are split between the investigator and Lilly for subsequent experimentation. So far, more than 30,000 compounds have been evaluated from more than 200,000 individual structures created in the OIDD platform.

ILLUMINA ACCELERATOR

<https://www.illumina.com/science/accelerator.html>

Illumina's Accelerator program aims to support genomics startups launching in the San Francisco Bay Area. Start-ups apply for a six-month funding cycle which offers access to Illumina sequencing technologies, coaching, and fully-equipped laboratory space. Illumina's angel/seed investors provide up to \$100,000 in convertible notes to participating startups, and up to 20% of a staff scientist's time, with the goal of producing the data needed for additional funding efforts.

Participating companies can also tap into the \$40 million Illumina Accelerator Boost Capital, and match funding of up to \$5 million from Viking Global Investors. During the 6-month cycle, the company (or at least 2 company co-founders) must be located in San Francisco.

JOHNSON & JOHNSON INNOVATION: JLABS

<https://jlabs.jnjinnovation.com/>

JLABS is described as an ecosystem to help “bridge the gap between idea and patient solution”. The accelerator offers research space, equipment and training, an operations team, business services, and regulatory support so that early-stage companies can remain focused on their science. Participants are also provided access to J&J’s experts and their industry partners. In February 2018, Johnson & Johnson Innovation announced their tenth JLABS location in Beerse, Belgium, this will be the first European location.

LEO PHARMA OPEN INNOVATION

<http://openinnovation.leo-pharma.com/>

LEO Pharma is focused on dermatology and skin inflammation diseases. Through their open innovation program, they encourage research scientists to test their own compounds using LEO Pharma’s in vitro assays and tools. Scientists retain their intellectual property and confidentiality, and the ability to walk away at any time.

MERCK KGAA: BIOPHARMA OPEN INNOVATION PORTAL

<https://www.emdgroup.com/en/research/open-innovation/biopharma-open-innovation-portal.html>

Academic and corporate researchers can apply to be guest researchers and bring their own sponsored research with them to work in Merck’s Open Lab in Darmstadt, Germany. This gives researchers access to Merck’s screening tools and protein engineering technologies, expert advice, and industry experience. Merck also offers “fast” grant funding of up to €30,000 for preclinical research through their Biopharma Speed Grant Initiative, with funds made available within three weeks of application. Applicants retain all rights to their intellectual property and results, and Merck can even assist with submission of a patent application.

Merck’s Open Compound Sourcing invites partners to submit their compounds for consideration to be included in screening for potential therapeutic activity in Merck’s strategic therapeutic areas. Merck will also supply collaborating investigators with their own free Mini Library of former research compounds and their derivatives (delivered in a 96-well plate) which can be tested in their assays systems. They only ask that all data generated is shared back to Merck. Both of these activities can potentially lead to a follow-up collaboration or licensing agreement.¹⁰

A related Accelerator program offers start-up companies both workspace and financial support of up to €30,000 as a project-based agreement, or up to €50,000 as a silent partnership requiring a limited profit sharing agreement.

¹⁰ <https://accelerator.emdgroup.com/faqs/> accessed 9 February 2018.

NOVARTIS: INSTITUTES FOR BIOMEDICAL RESEARCH

<https://www.novartis.com/our-science/novartis-institutes-biomedical-research>

Six research campuses and 6,000 researchers around the world make up the Novartis Institutes for Biomedical Research (NIBR). The NIBR's stated goal is to be the innovation engine of Novartis, maintaining "a highly collaborative culture that encourages teams and individuals to work across institutional and geographic boundaries".

Within the NIBR, business development and alliance management teams work to form open innovation collaborations and licensing, currently citing more than 300 academic and 100 industry alliances around their strategic areas of focus.

PFIZER: CENTERS FOR THERAPEUTIC INNOVATION

<https://www.pfizercti.com/>

Pfizer's approach to the open innovation model is through the Centers for Therapeutic Innovation (CTI), first established in 2010 and currently located in Massachusetts, New York and California. Each location provides space for collaboration with local academic institutions and start-up activities, with more than 80 colleagues working to speed the drug discovery process. Three core principles of the CTI model include: results-driven research, entrepreneurial environment, and collaboration. Collaborating partners receive the backing of Pfizer resources, including funding for investigators and post-docs, and access to Pfizer's extensive small-molecule and antibody libraries.

Academic medical centers can apply through a regular call for proposals, and resulting projects have joint intellectual property and ownership, rights to publication, and milestone payments/royalties built into the agreements. To date, Pfizer has partnered with more than 20 leading academic medical centers and reviewed more than 500 project proposals.

Foundation partnerships are also a part of these efforts, including the first partnership with the Alliance for Lupus Research (ALR) announced in 2012. A joint steering committee made up of equal members from Pfizer and ALR managed a call for proposals, evaluated and selected four projects to receive lupus research funding in the CTI pipeline.

Model 3 Non-Profit Organizations

BILL & MELINDA GATES FOUNDATION: DISCOVERY & TRANSLATIONAL SCIENCES

<https://www.gatesfoundation.org/What-We-Do/Global-Health/Discovery-and-Translational-Sciences>

The Discovery & Translational Sciences program at the Gates Foundation attempts to identify and fill in gaps where scientific knowledge is needed to move ideas and technology forward. They invest through a number of mechanisms, including the Grant Challenges grant programs, which have been awarded to over 1,000 innovators for early-stage research. They work with partners at government agencies and other donor organizations to define areas of need and build collaborative networks of researchers and funders. Main areas of focus are vaccine discovery, drug discovery, maternal and child health, and disease-transmitting mosquitoes.

INNOVATION FUND AMERICA

<http://www.innovationfundamerica.org>

Innovation Fund America is a non-profit organization that partners with community colleges to accelerate technology development and entrepreneurship. The focus is on high-growth technology-based companies, to facilitate economic development in the regions where the fund is active. Community colleges pay a membership fee to access consulting support and other guidelines for implementation on their campus. Companies who receive funding of \$25,000 (A award) and \$100,000 (B award) are expected to 'give back' by providing an internship for a student and participating in educational activities. The Innovation Fund does not take equity in the companies.

Since their founding in Northeast Ohio in 2007, the Innovation Fund has made 191 awards to 158 companies totaling nearly \$11 million. These companies have gone on to raise \$211 in follow-on funding and created 555 jobs.

When the gap between the work of basic scientists and the work of clinical researchers is bridged, avenues of research will open up. Translational research will flourish, bringing better treatments and cures for many diseases to those who so desperately need them. To maximize the nation's investment... better mechanisms are needed to quickly translate research into clinical practice.

-Parkinson's Action Network 2022

LIFEARC

<https://www.lifearc.org/>

LifeArc is a UK-based non-profit entity affiliated with the government-funded Medical Research Council (formerly known as MRC Technology). Two new funds will be launched in early 2018, the Philanthropic Fund providing grants for academic research, and the Seed Fund investing in early stage therapeutics. Their Centre for Therapeutics Discovery consists of 80 scientists working on various aspects of drug discovery and four business development managers. A London location focuses on licensing and technology transfer, and a Centre for Diagnostics development employs 15 people in Edinburg, Scotland.

LifeArc's "Call for Diagnostics" campaign recruits academic scientists and biopharmaceutical companies on projects to develop and validate diagnostic assays. Through this program, they invest time and resources in proof-of-concept research for promising opportunities, at no cost to the partner.

LifeArc provides support to other charitable organizations in the UK, prioritizing patient impact over financial returns. One initiative, the Dementia Consortium, links non-profit and industry partners to help accelerate development of therapies for dementia and neurodegenerative diseases.

A related consortium of charities and foundations founded the Neurodegeneration Medicines Acceleration Programme in 2014 to work with the pharmaceutical industry to identify shelved technologies and invest in them. Finally, LifeArc's Act New network seeks to develop solutions in areas of childhood cancer, rare diseases, and antibiotic resistance.

Model 4: Public-private Partnerships and Consortia

Public-private partnerships bring together the public sector, nonprofit research institutes, industry, academia, and philanthropic and patient advocacy organizations to address major unmet medical needs, including in areas that may have a lower financial payoff. Involving multiple stakeholders can be more effective than a bipartisan agreement between industry and academia for these reasons:

- Initiatives established by consortia can create technologies or solve common problems that affect multiple parties in the precompetitive space, such as validated biomarkers, superior procedures to assess clinical outcomes and predictive safety testing methods.**
- Public-private partnerships can harness federal or foundation funding, which has a primary purpose of economic development and public good, not direct return on investment. This funding is appropriate for risk-heavy early-stage research, as well as for research in areas that are less financially rewarding.**



CALIFORNIA INSTITUTE FOR BIOMEDICAL RESEARCH (CALIBR) AT SCRIPPS RESEARCH ^{11,12,13}

www.scripps.edu/science-and-medicine/calibr/

Calibr is a nonprofit translational research institute founded to accelerate the creation of new medicines by integrating biomedical research with drug discovery and development capabilities. Calibr works largely through the creation and proliferation of partnerships.

Calibr employs more than 100 interdisciplinary scientists with drug discovery, clinical and industry experience. The primary purpose of the program is to identify and advance new therapeutic strategies through preclinical and clinical development by working closely with scientists at Scripps Research. Calibr also trains scientists in translational research through its postdoctoral associate program, and develops strategic alliances with foundations, government agencies, and pharmaceutical industry partners to accelerate discovery of medicines.

Calibr has created a broad therapeutic pipeline extending from early stage discovery through clinic-ready programs, with at least ten drug candidates based on Scripps technologies currently in early clinical development. Moreover, Calibr is engaged at least ten alliances and partnerships with external entities.

¹¹ Calibr. www.scripps.edu/science-and-medicine/calibr/. Accessed 1 August 2019.

¹² Freedman, 2017.

¹³ Service, Robert F. "Two major California research institutes will merge." *Science*. October 21, 2016. www.sciencemag.org/news/2016/10/two-major-california-research-institutes-will-merge

ASCENION

<https://www.ascenion.de/index.php?id=1&L=1>

Ascenion was founded in 2001 as a wholly-owned subsidiary of the LifeScience Foundation for the Promotion of Science and Research. They provide a technology transfer function for 23 research organizations across Germany, including hospitals and research centers, and involving about 5000 scientists.

Since 2001, partner institutes have received €65 million return from agreements mediated by Ascenion, and an additional €12.1 million has been received from exit proceeds and given back to the LifeScience Foundation to support additional publicly-funded research. They have actively assisted in the coaching of more than 100 spin-off projects, which provided over 420 new jobs.

COALITION AGAINST MAJOR DISEASES / CRITICAL PATH FOR PARKINSON'S CONSORTIUM

<https://c-path.org/programs/camd/>, <https://c-path.org/programs/cpp/>

The Coalition Against Major Diseases (CAMD) is a public-private partnership focused on developing new tools and methods to accelerate development of therapies for Alzheimer's disease and related neurodegenerative disorders. This includes development of biomarkers, common data standards, integrated databases, and other model-based tools. The Coalition is comprised of pharmaceutical companies, foundations, patient advocacy groups, government agencies, academic researchers and regulators.

The Critical Path for Parkinson's Consortium (CPP) was formed in 2015 as a spinout of CAMD, which had previously managed both Alzheimer's and Parkinson's Disease projects. CPP brings together the same types of partners towards the development of

new drug development tools for Parkinson's Disease, with a priority for early stages and early intervention. An initial project has been the creation of a global unified database of standardized patient data.

Both groups were formed by the Critical Path Institute (C-Path), a non-profit organization founded in 2005 to facilitate the partnerships between FDA, industry and academia for improving the drug development and regulatory process. Drug development tools (DDTs) are developed and tested within these partnerships, building a consensus before official application to the FDA for approval as valid tools used in drug development.

CQDM

<http://www.cqdm.org/en/>

CQDM is a drug discovery and development consortium made up of nine top pharmaceutical organizations, Quebec and Canadian governments, and public and private researchers. Their model is collaborative, all partners share the costs of research. While intellectual property remains with the originating institutions, industry partners are usually provided with a non-exclusive license option for R&D purposes.

Current programs offer \$500,000 to \$2 million per project, and are selected based on commercial viability of the technology, as well as strong interest from at least one industrial partner or member. Pharmaceutical company partners also provide mentorship

Since its founding in 2008, CQDM has funded 63 projects at a total of \$44 million in funding, and these projects have gone on to raise \$34 million in additional investments. Ten startups and nearly 700 jobs have been created.

LONG ISLAND HIGH TECH INCUBATOR

<https://www.lihti.net/>

The Long Island High Tech Incubator (LIHTI) was founded in 1992 as a non-profit partnership of two university-associated member corporations: The Research Foundation of the State University of New York and the Stony Brook Foundation Inc. Their stated mission is to support and stabilize early-stage, high-risk, high-tech businesses, to create jobs and expand the tax base in New York State. Over 60 companies (many in the life sciences) have graduated from the LIHTI program, and have created over 500 jobs and contributed over \$2.5 billion to the national economy.

The incubator is located on the Stony Brook University campus, and consists of 60 suites, both office and laboratory space, as well as access to the Stony Brook University libraries and research facilities. Business development assistance is provided through mentoring and training programs, and research assistance may be provided with interns and students from the associated universities. The group also supports companies through an “Incubator Without Walls” program.

MARS INNOVATION (TORONTO, CANADA)

<http://marsinnovation.com/>

MaRS Innovation (MI) is a non-profit organization consisting of 15 member organizations, including universities, teaching hospitals and research institutes. MI was created in 2008 to help inventors and researchers at these organizations bring their discoveries to market. Services including business and strategy development, IP management, marketing, financial and human resources support. In the first 8 years, MI assessed 1500 disclosures, created 60 companies and managed an additional 100 technologies./ The companies obtained \$160 million in external investment and created over 400 jobs.

The MI Innovation Industry Access Program provides selected researchers with a grant of \$75,000 to \$150,000 to gauge commercial interest in their technology. Industry partners (Baxter, GSK, Johnson & Johnson, Merck, Pfizer, and LifeLabs) commit strategic partnership funds in their therapeutic areas of interest, and participants are able to present their technologies to these industry partners.

NIHR TRANSLATIONAL RESEARCH COLLABORATIONS

<https://www.nihr.ac.uk/life-sciences-industry/access-to-expertise-and-collaborations/collaborations-for-early-phase-translational-research/>

The UK’s National Institute for Health Research (NIHR) now supports four collaborative networks of leading universities and hospitals to work together, and with industry partners in translational research. Current areas of focus are joint and related inflammatory diseases, inflammatory respiratory diseases, dementia, and rare diseases.

<https://www.nihr.ac.uk/life-sciences-industry/access-to-expertise-and-collaborations/industry-alliance-initiative.htm>

The NIHR also facilitates official alliances with pharmaceutical companies and the NIHR Clinical Research Network. The alliances are formed with a Memorandum of Understanding between the company and clinical researchers, allowing clinicians to access compounds or technologies still in development, and encouraging companies to help design academically-sponsored studies in areas that are not currently part of their direct development plan. Companies already participating include Pfizer, AstraZeneca/ MedImmune, GlaxoSmithKline and Verastem – all focused in areas of cancer research.

NIH CLINICAL AND TRANSLATIONAL SCIENCE AWARDS PROGRAM

<https://ncats.nih.gov/ctsa>

Clinical and Translational Science Awards have been awarded to more than 50 institutions across the country, since inception of the NIH National Center for Advancing Translational Sciences in 2011. CTSA program funding supports the development of a national network of medical research institutions, with a goal of catalyzing innovation and tools that will bring new treatments to market. Their approach focuses on scientist training, patient and community engagement, underserved populations, innovative processes to increase quality and efficiency of translational research, and cutting-edge informatics.

In addition to support of the hub institutions, they also provide funds to encourage collaboration between three or more institutions through their Collaboration Innovation Awards. Another collaboration initiative is the Trial Innovation Network, working to identify and address roadblocks in clinical trials.

NHLBI CENTERS FOR ACCELERATED INNOVATIONS AND REACH

<https://ncai.nhlbi.nih.gov/ncai/>

The inaugural NIH Centers for Accelerated Innovations (NCAI) grants, totaling \$31.5 million, were issued from the National Heart, Lung, and Blood Institute in 2013. These grants funded the establishment of three multi-institution Centers, involving 24 high-impact research institutions in Massachusetts, Ohio, and California. A related NIH program called Research Evaluation and Commercialization Hubs (REACH) added three hubs in New York, Minnesota, and Kentucky.

Each NCAI Center offers pilot and proof-of-concept funding grants ranging from about \$50,000 to \$400,000 per award, towards the commercialization of devices, diagnostics and therapeutics in the areas of interest for the NHLBI: cardiovascular, lung disease, sleep disorders and blood diseases. Centers also provide skills development, consulting services and coaching or mentorship to assist with innovative product development and commercialization.

Through the NCAI network, early-stage companies can connect with federal partners (such as the FDA and CMS), other companies, and angel and venture capital firms. A list of funded technologies, many available for licensing, is searchable from the NCAI website.

More than 70 proposals have been funded through the NCAI program to date, and 12 start-up companies created. In a survey of participating innovators, 93% of respondents believed that the program had accelerated their technology development, on average 18 months ahead of where they estimated they would have been without the program.¹⁴ Though REACH is a younger program established in 2015, at least 400 technologies have been evaluated, resulting in more than 60 experimental therapies and technologies in the pipeline.¹⁵

QB3

<https://qb3.org>

QB3 was founded in 2000 by California's Governor Gray Davis, as a partnership between University of California Santa Cruz, University of California Berkeley, and University of California San Francisco, helping to promote commercialization and invention out of the University of California system, particularly in the Bay Area. They

¹⁴ Antman, E.M. et al. "NIH Centers for Accelerated Innovations Program: principles, practices, successes and challenges." *Nature Rev Drug Disc* 2017; 16(10): 663-4. <https://www.ncbi.nlm.nih.gov/pubmed/28775338>

¹⁵ NIH program strives to turn more lab discoveries into real-world treatments." *STAT News*, 17 April 2017. <https://www.statnews.com/2017/04/17/nih-reach-biomedicine-treatments/>

also have an initiative for medical devices called the Rosenman Institute, based at UC San Francisco. They estimate their efforts bring more than \$750 million annually into the Bay Area. Participating entrepreneurs can take advantage of five incubators and two seed-stage venture capital firms, as well as mentoring, training, and partnering services. Access to core lab facilities, outsourced R&D service providers, legal services, and launch support are also offered. Their network of current and past participants includes more than 400 life science startups.

STRUCTURAL GENOMICS CONSORTIUM

<http://www.thesgc.org>

The Structural Genomics Consortium (SGC) is a non-profit, public-private partnership that supports open access research in areas of human health and drug discovery. The SGC does this by explicitly focusing on areas of the human genome that are relatively unexplored.

The consortium has six official locations in Canada, United Kingdom, Brazil, Germany, Sweden, and North Carolina, and represents a network of hundreds of research universities, several foundations, and nine global pharmaceutical companies. For a donation of \$8 million, consortium members gain the rights to nominate targets to the target list, nominate a member to the governing boards (Board of Directors and Scientific Committee), and place scientists to work within the SGC laboratories.

All research output is made available freely to the scientific community, including the protein structures, reagents and methods discovered. The SGC recognizes that many proteins in their research programs are likely already under investigation elsewhere in academia and industry, and they hope to facilitate that research by rapid and open access to their work.

Among their many achievements since their founding in 2008, the SGC has developed more than 14 chemical probes for epigenetics research, deposited more than 1500 structures of medically relevant human and parasite proteins into public databases, and assisted in coordination of the Renewable Protein Binding Working Group which produced hundreds of antibodies.

In late 2017, a new partnership between the SGC and Montreal Neurological Institute and Hospital was announced. The newly-formed NeuroSGC will focus on the discovery of treatments for ALS and Parkinson's disease, two neurological disorders which currently lack effective therapeutics.¹⁶ The Montreal Neurological Institute also recently created their own MNI Open Research publishing platform.

Translational research, when correctly applied, can combine the rigor of academic research with the enterprise of clinical development to produce tangible health innovations. And its lessons can change not only medicine and drug development but any business for the better.

-Forbes Business Development Council 2021

¹⁶ Canada's NeuroSGC partnership seeks potential treatments for ALS, Parkinson's Disease." *ALS News Today*, 10 January 2018. <https://alsnewstoday.com/2018/01/10/als-parkinsons-treatment-sought-by-canada-new-neurosgc-partnership/>

Biotechnology Translational Research Guiding Principles^{17,18,19}

The following are nonscientific strategies that can improve success of translational research collaborations. But to make the chemistry work, each side must overcome the cultural and communication divide that can impair biotech industry-university partnerships and undercut their potential.

PRINCIPLES

Guiding Principle # 1: Successful university-industry collaborations reflect an understanding of the mission and culture of each partner:

Successful sponsored research transactions require all parties – industry and academia – to identify the core mission of their organizations early in the process and defining the scope and focus of the proposed alliance.

Guiding Principle #2: Alliance management resources are essential:

Industry and university participants engaging in strong alliance management strategies devote resources, energy and attention to maintaining relationships and working together in an ongoing effort to facilitate productive, transparent outcomes to ensure sponsored research success.

Guiding Principle #3: Universities and industry participants should understand the objectives and benefits to each party that will result from collaborations:

Goals, objectives, and timelines for completion are essential for productive end results in partnerships; each party must understand and support the objectives and proposed research benefits of the other party.

Guiding Principle #4: Commitments in sponsored research agreements should ensure legal integrity and consistency:

Commitments contained in sponsored research agreements concerning future research results shall be consistent with all applicable laws and regulations and with any contractual obligations the University or biotechnology company may owe to others.

Guiding Principle #5: Parties should have a clear focus on each other's licensing strategies:

Both industry and academia must commit to engaging in open and honest discussions to develop creative and effective licensing strategies that promote global access to innovation. The mutual goal should be an authentic partnership where each party understands the collaboration goals and objective of the other party and is committed to each party achieving success.

Guiding Principle #6: Parties should focus on streamlining negotiation protocols:

Universities and biotechnology industry should focus on the benefits to each party that will result from collaborations by streamlining negotiations to ensure timely conduct of the research and the development of the research findings.

Guiding Principle #7: Negotiator training is essential:

In order to effectively navigate towards an overall success rate for the institution, all sponsored research officers, contract negotiators and licensing officers, and especially those early in their careers, must understand how each research collaboration with which they are engaged reflects forces in the larger world of biotechnology development.

17 Freedman, 2017.

18 Libby, 2018

19 Reichman, M., & Simpson, P. B. (2016). Open innovation in early drug discovery: roadmaps and roadblocks. *Drug Discovery Today*, 21(5), 779-788.

Guiding Principle #8: Partnerships should work to lower the cost of transactional efforts:

Systematically reducing transactional costs should be a major combined effort of both the university and biotechnology industry sector. This includes broader efforts to engage interpersonally through these partnerships in order to lower communication barriers among participants.

- **Establish transparency between partners.**

Partnerships should create transparency about how the program fits into each partners' strategy and goals. Agreements should set expectations about milestones and timelines, termination terms, IP ownership and commercialization rights, and financial commitments. Agreements should take into consideration concerns about industry's proprietary information and academics' right to publish.

- **Understand cultural obstacles.** Cultural obstacles between academia and industry can hinder a partnership. Tensions may arise over the prioritization of scientific understanding versus commercial viability; freedom to publish versus confidentiality; and IP valuation.

- **Form proactive management structures.** Management structures should ensure relationships are proactively managed and should be able to respond to commercial, regulatory and scientific forces. For example, a joint steering committee should be equipped to monitor progress, review data and resolve disputes between partners.

- **Encourage collaboration and interdisciplinary learning.** To mutually vest the parties as collaborators and encourage mutual respect for contributions, the partnership should provide opportunities for interdisciplinary learning. Partnerships may establish environments and activities that facilitate mingling, such as shared laboratory space or joint seminars.

- **Professional Expertise Essential.**

Engaging a facilitator or entrepreneur with background in academia and industry can be instrumental in interpreting and translate differences in language and culture.

- **Create institutional buy-in.** Universities in particular may need to consider policy changes to foster an environment that encourages translational research activity. This may include changes in their tenure and promotion policies to ensure that investigators are recognized for their research contributions beyond grants, publications, and supervision. Universities may also make funds available to support limited translational activities, or adopt policies that enable researchers to retain a sign.

- **Maintaining alignment and momentum.** In large, complex partnerships, the participating parties may come to the table with divergent interests and goals, funding may be linked to political will or be unreliable, and programs will be subject to market forces and changing strategic objectives, which can result in substantial management challenges.



CONCLUSION

As noted in publications and commentary by leading science and industry experts, the challenges in addressing new models of translational research focused on promising discoveries and the advancement of new biomedical innovations continue. Some other those challenges include:

- Pressures on industry to raise the effectiveness of R&D expenditures in light of an observed decline in R&D productivity caused likely by complex diseases and need for more sophisticated scientific approaches.
- A constrained funding environment in the US for bioscience R&D innovation, fueled by both the loss of industry revenues caused by expired patents and the challenging federal funding environment to the NIH and other related agencies.
- Strong competition for the venture capital need to foster new bioscience firm formation and growth. Competition principally stems from other technology sectors that offer high returns in the near-term with less risk, as well as a shift in bioscience venture funding from early-stage companies to those that are ready to enter clinical trials.

Finally, BIO and other bioscience-related entities can increase new models and opportunistic outcomes with a larger footprint by impacting how federal and state initiatives to advance bioscience developments. Among the opportunities for convening partners include working with patient advocacy groups for larger scale multi-institutional and multi-company collaboration; advance large scale, multi-institutional patient registries; and more engagement with the NIH and their translational research institutes.

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