ANNUAL REPORT
2017
for fiscal year ending June 30, 2017
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Mission

Critical Path Institute is a catalyst in the development of new approaches to advance medical innovation and regulatory science. We achieve this by leading teams that share data, knowledge, and expertise, resulting in sound, consensus-based science.

Vision

Accelerating the path to a healthier world

Values

As an independent and trusted partner, we value integrity, innovation, and teamwork.
C-Path’s Impact

Accepted Drug Development Tools/Novel Methodologies

Shared Knowledge

Collaborative Research

C-Path: Pre-competitive Neutral Ground

C-Path global consortia – more than 1,400 scientists

- Biomarkers
- Clinical Outcome Assessment Tools
- Quantitative Medicine Tools
- Data Standards
- In Vitro Tools
- Improved Regulatory Processes

Regulatory Submissions
C-Path works with governmental regulators, biopharmaceutical companies, and key academic institutions worldwide to improve the drug development process and help deliver new therapies to patients.

HOW C-PATH MAKES A DIFFERENCE

Developing biomarkers

- to better determine if potential new therapies are causing injury to a patient’s organs
- to select the appropriate patients to enroll in clinical trials, to increase the probability of success and minimize the number of patients needed for enrollment

Developing data standards, data platforms, and data-sharing capabilities

- to facilitate regulatory review of new therapies
- to facilitate the aggregation of data into large datasets to uncover new insights and trends
- to enable researchers to share and learn from results of trials
- to help scientists understand why different patients progress differently, and which ones will most likely benefit from a new treatment
Improving the clinical trial process

- by applying model-informed drug discovery and development (MIDDD) and simulation tools to clinical trial design, which helps prevent expensive failures and allows pharmaceutical companies to optimize their resources to develop drugs to help patients

- by enabling researchers to run clinical trials with the right participants, which can expedite drug development and get medicines faster to those who need them

- through developing master protocols to make clinical trials more efficient

- through updating informed consent forms, so in addition to knowing the benefits and risks of participating in clinical trials, participants can allow their de-identified data to be used in many research projects

Understanding complex issues surrounding newborns, such as gauging normal blood pressure and how drugs should be administered to treat complications in premature babies

Developing better ways to measure the treatment benefit of new therapies by incorporating the voice of the patient in assessments

Making it easier for companies to invest in new therapies for rare diseases
Dear friends and supporters,

As I look back on the past year, I am filled with pride and gratitude at the great strides and substantial growth C-Path has achieved. In the spirit of collaboration and innovation, C-Path has forged new partnerships that have led to opportunities for groundbreaking successes.

Our 2016 fiscal year began with a new Chair of C-Path’s Board of Directors: Dr. Timothy R. Franson brings years of experience in both clinical and regulatory pharmaceutical development to C-Path, and we look forward to achieving new and greater heights under his direction.

I’m proud to announce that in the past fiscal year we have established two new consortia to aid in the development of groundbreaking therapies and drug development tools: the Transplant Therapeutics Consortium (TTC) and Type 1 Diabetes (T1D) Consortium. Together with the CHDI Foundation, C-Path also began planning the formation of the Huntington’s Disease Regulatory Science Consortium (HD-RSC).

In May 2016, C-Path’s Critical Path to TB Drug Regimens (CPTR) partnered with the Translational Genomics Research Institute (TGen), and, with a $1.1 million grant from the Bill & Melinda Gates Foundation, will bolster their Relational Sequencing TB Data Platform (ReSeqTB) with data from at least 12,000 tuberculosis (TB) bacteria isolates from around the world, leading to a better understanding of the genetic basis for TB drug resistance. In October, in a collaborative effort called TB-ReFLECT, CPTR partnered with the World Health Organization’s Global TB Programme and researchers from the University of California, San Francisco, to develop new methods for quantitative data analyses.
This fiscal year brought many important regulatory milestones. In October, the Critical Path for Parkinson’s Consortium (CPP) announced that the European Medicines Agency (EMA) issued a Letter of Support for the use of an imaging biomarker in Parkinson’s disease clinical trials, an important step toward qualification. Also in October, the US Food and Drug Administration (FDA) and EMA each issued a Biomarker Letter of Support for drug-induced liver injury safety biomarkers based on data submitted by C-Path’s Predictive Safety Testing Consortium (PSTC) and the Innovative Medicine Initiative’s (IMI) Safer and Faster Evidence-based Translation (SAFE-T) Consortium.

It was a productive year for CFAST, a joint initiative with the Clinical Data Interchange Standards Consortium (CDISC) to develop standards for the collection, sharing, and analysis of data. Together, the partnership announced new and updated therapeutic area data standards for TB, kidney transplants, ebola, malaria, and major depressive disorder, as well as the review period for a Duchenne muscular dystrophy therapeutic area user guide.

In last year’s report, I announced that a new organization, one that would facilitate the development of innovative pediatric therapies, was in its early stages of development. We are pleased to announce that the Institute for Advanced Clinical Trials for Children (I-ACT for Children) was officially founded in April 2017. It is gratifying to see how quickly the work of C-Path’s Pediatric Trials Consortium (PTC) translated into the formation of I-ACT for Children: an organization working with others to improve the efficiency, feasibility, timeliness, and impact of clinical trials of innovative drugs and devices in children.

As I look back on a year filled with positive change, growth, and continuing success, I see a bright future for C-Path. We have a lot to look forward to in the coming fiscal year, including some significant changes to our online presence. I am certain that we, in cooperation with our existing and newfound partners, will continue to accelerate the development of medical products, achieving regulatory endorsement for new drug development tools, and the creation of new data standards. I am so very grateful for all the hard work and dedication of our many team members around the globe. Thank you for being a part of this collaborative endeavor that is improving the lives of those living with diseases every single day. Here’s to another wonderful year.

Sincerely,

Martha A. Brumfield, PhD
C-Path’s New Consortia: Expanding Innovative Drug Development

C-Path continues to grow and thrive with each passing year. The addition of two new consortia in the past fiscal year, and the planning phase to launch a third, is an affirmation of the success, both past and present, that C-Path has achieved in bringing organizations together in a collaborative, pre-competitive environment.

In October 2016, C-Path announced plans to form the Huntington’s Disease Regulatory Science Consortium (HD-RSC), in a joint collaboration with CHDI Foundation, Inc. In response to a growing need for accelerating the drug development and approval for Huntington’s Disease (HD), the consortium would enlist participants from within the HD community to advance drug development tools (DDTs), including biomarkers and clinical outcome assessments, for regulatory endorsement. HD-RSC aims to more clearly define regulatory pathways leading to the approval of HD therapeutics and de-risk the drug development pathway.

March 2017 brought the launch of C-Path’s Type 1 Diabetes (T1D) Consortium. Funded by both The Leona M. and Harry B. Helmsley Charitable Trust and JDRF International, the T1D Consortium is working to qualify new biomarkers to be utilized in the development of new therapies and treatments for type 1 diabetes (T1D), with the ultimate goal of facilitating widespread prevention of T1D. The T1D Consortium is also collaborating with INNODIA, a consortium under the Innovative Medicines Initiative (IMI).
Cofounded with the American Society of Transplantation (AST) and the American Society of Transplant Surgeons (ASTS), C-Path’s Transplant Therapeutics Consortium (TTC) was launched in April 2017. Together with members of the transplant community, including clinicians, surgeons, industry scientists, and others, TTC is working to achieve regulatory endorsement of drug development tools for kidney transplantation, with the goal of expanding its efforts to serve multiple areas of organ transplantation in the future.

“My work in the chemistry and biological aspects of drug development has regularly included collaborative, interdisciplinary projects, so I am happy to work for an organization centered on collaboration. Through the combined efforts of academia, industry, and professional and patient groups, the Type 1 Diabetes and Transplant Therapeutics consortia can quickly and efficiently meet their goals: to qualify prognostic biomarkers in the development of therapies for type 1 diabetes, and to streamline the regulatory process to get new, qualified immunosuppressive therapies to transplant patients.”

–Inish O’Doherty, PhD, Executive Director, T1D and TTC
Also in October, FDA and EMA each issued a Biomarker Letter of Support for new liver safety biomarkers investigated through a cross-organization collaboration between C-Path’s Predictive Safety Testing Consortium’s (PSTC) Hepatotoxicity Working Group and the Drug-Induced Liver Injury Work Package of IMI’s SAFE-T (Safer and Faster Evidence-based Translation) Consortium. Both FDA and EMA acknowledged that higher levels of these biomarkers in patients could indicate a risk for progression toward liver failure.

With leadership of C-Path, the Biomarker Assay Collaborative Evidentiary Considerations Writing Group is developing a draft framework outlining key criteria and best practices for biomarker assay performance expectations and validation. In June, a two-day public workshop convened as a forum for broader input and feedback on this framework, with the goal of creating alignment on the evidentiary considerations.

“C-Path leads the writing group whose goal is to develop the foundation of best practices for validation of biomarker assays used in the qualification of biomarkers. On June 14 and 15, we advanced a step closer to that goal by co-organizing a public workshop with the Duke-Margolis Center for Health Policy and FDA’s CDER to collect broader input on this evidentiary consideration framework. This work has the potential to clarify regulatory expectations, and accelerate the qualification processes.”

–John Michael Sauer, PhD, Biomarker Program Officer and Executive Director, PSTC
As organizations such as C-Path continue to bring together drug companies, academics, patient groups, and regulators to accelerate the drug development timeline, the importance of standardized data has never been greater. The Coalition for Accelerating Standards and Therapies (CFAST), C-Path’s collaboration with the Clinical Data Interchange Standards Consortium (CDISC), announced several new and updated standards over the year, from an updated therapeutic area standard for TB (v2.0), to a standard for kidney transplant (v1.0), to a therapeutic area user guide for Duchenne muscular dystrophy, whose review period began in May.

In the upcoming year, the Data Collaboration Center (DCC), a C-Path initiative that enables multiple organizations to work together and share data in a neutral setting, will continue to branch out beyond C-Path’s own consortia and offer its expertise to outside research organizations.

“I cannot stress enough the importance of data collaboration and standardization in the work of all C-Path consortia. For the Critical Path for Parkinson’s Consortium specifically, development and utilization of consensus CDISC data standards enable us to collect patient-level data from around the world and develop a global, integrated, and unified database. This not only makes a wealth of data accessible to PD researchers across countries and disciplines, but it also provides a valuable, rich resource from which we can learn to model and develop better, more effective clinical trials.”

–Diane Stephenson, PhD,
Executive Director, CPP

Clinical Data Contributed to C-Path
Advancements in the Global Fight to End TB

Tuberculosis (TB) is the leading cause of death by infectious disease globally. According to the World Health Organization (WHO), 1.8 million people die every year as a result of TB. With more drug-resistant strains appearing, there is an urgent need for more effective and more accessible treatments, particularly in developing countries. In the past fiscal year, C-Path’s Critical Path to TB Drug Regimens (CPTR) initiative has dedicated considerable resources to answering the global call for better TB research and treatments.

On World TB Day (March 24), C-Path raised awareness for TB and Arizona’s role in addressing the disease by telling the story of the nearby Tucson Medical Center. With roots extending back nearly a century, to a time when experimental TB treatment was shaping the future of the American Southwest, the Tucson Medical Center’s history in the treatment of TB serves as both striking contrast and thread of continuity to C-Path’s modern efforts to combat TB worldwide.

In October, C-Path collaborated with researchers from the University of California, San Francisco, to develop new methods for data analyses to be used with the TB-Platform for Aggregation of Clinical TB Studies (TB-PACTS) database, which integrates data from the REMox, OFLOTUB, and RIFAQUIN trials. The cooperative effort, titled TB-ReFLECT, maximizes the learnings from these failed Phase III trials by applying a model-based meta-analysis that can inform and optimize future trial designs. The TB-ReFLECT collaboration could potentially provide crucial guidance for the development of faster, more optimized treatments.
Another milestone in CPTR’s fight against TB was the public availability of its Relational Sequencing TB Data Platform (ReSeqTB), announced in January 2017. The data-sharing platform and analytic visualization tool can be used to discover, grade, and track key bacterial drug resistance mutations. A few months later, in May, after receiving a $1.1 million grant from the Bill & Melinda Gates Foundation, C-Path partnered with the Translational Genomics Research Institute (TGen) to sequence at least 12,000 TB bacteria isolates from around the world. The results will be included in the ReSeqTB platform. C-Path will use that sequencing data to provide newfound knowledge that is essential for the identification of TB resistance patterns, which could one day lead to treatments that are more effective.

1. Pharmacokinetic/pharmacodynamic modeling
2. Corrected QT interval
3. Physiologically based pharmacokinetic modeling
4. Hollow fiber system model of tuberculosis
The next fiscal year is shaping up to be an exciting one, as C-Path’s expertise in regulatory science continues to be recognized and valued. C-Path’s research into neurodegenerative disorders will broaden with the launch of HD-RSC, with Huntington’s disease included as a focus of C-Path’s consortia alongside Parkinson’s disease, Alzheimer’s disease and related dementias, Duchenne muscular dystrophy, and multiple sclerosis. C-Path’s Patient-Reported Outcome (PRO) Consortium anticipates several of their clinical outcome assessment instruments will be qualified by FDA.
Financial Update

C-PATH 2017 FISCAL YEAR REVENUE

- Industry Fees: $3,755,546
- FDA: $4,501,041
- CHDI: $2,824,994
- Parkinson's UK: $628,369
- Bill & Melinda Gates Foundation: $547,959
- Other: $1,647,522

Total Revenue: $13,905,431

C-PATH 2017 FISCAL YEAR EXPENSES

- Salary & Fringe Benefits: $7,187,320
- General Expenses: $3,899,71
- Occupancy Expenses: $958,791
- Subawards/Subcontracts: $2,019,142
- Professional/Outside Services: $514,007
- Travel & Meeting Expenses: $2,272,006

Total Expenses: $13,341,237
In fiscal 2017, C-Path continued to grow in revenue, scope, and personnel, and broaden its impact geographically and across therapeutic areas. We accomplished this through the evolving work of our existing initiatives and the addition of new ones.

In October 2016, C-Path announced a collaboration with CHDI, Inc., a privately funded, nonprofit biomedical research and science management organization dedicated to developing new treatments for Huntington’s disease.

In November, FDA awarded C-Path three new grants to develop data standards. With this funding, C-Path is collaborating with CDISC to develop data standards for HIV treatment, Clostridium difficile-associated diarrhea, and Animal Efficacy Studies and Natural History Studies for Animal Rule.

C-Path’s PSTC was awarded an FDA contract to continue its work with the Foundation for the National Institutes of Health (FNIH) to obtain regulatory qualification of several kidney clinical safety biomarkers for use in drug development.

In spring 2017, C-Path launched two consortia. C-Path collaborated with the American Society of Transplantation (AST) and the American Society of Transplant Surgeons (ASTS) to launch the Transplant Therapeutics Consortium, which has brought key stakeholders from the biopharmaceutical industry, academia, and federal agencies. The Type 1 Diabetes (T1D) Consortium is funded by the Leona M. and Harry B. Helmsley Charitable Trust, JDRF, and the pharmaceutical industry. Both consortia are working to deliver novel drug development tools that may enable the development and approval of new therapies in their respective therapeutic areas.

The vision of C-Path’s Pediatric Trials Consortium was realized in April, with the launch of the Institute for Advanced Clinical Trials for Children (I-ACT for Children). I-ACT for Children is an independent nonprofit organization that works with public and private stakeholders to improve the timeliness, quality, and medical impact of clinical trials of innovative therapeutics on child health.

In May, CPTR announced a $1.1 million grant from the Bill & Melinda Gates Foundation to significantly enrich ReSeqTB. As a sub-award from C-Path, and working with multiple collaborators, the Translational Genomics Research Institute (TGen) will sequence thousands of TB bacteria isolates, derived from as many as 35 countries around the world.

At the close of the fiscal year, C-Path has new programs and initiatives in development for the year to come. C-Path remains committed to innovation in the drug development and regulatory review processes, for the accelerated availability of safe, new therapies to improve lives globally. C-Path remains in sound financial condition, and anticipates continued interest in the expertise that it brings to the drug development arena.
C-Path Initiatives

The Coalition Against Major Diseases (CAMD) brings together diverse stakeholders to accelerate the development of treatments for those living with Alzheimer’s disease (AD) and those in the presymptomatic stages who will likely progress to Alzheimer’s. These experts use their combined knowledge to develop new tools to evaluate clinical trial designs and to improve decision-making around enrolling patients. CAMD’s focus on AD requires a collaborative approach due to the complexity of this disease and the need to learn across multiple drug development programs. Our aim is to advance regulatory science goals by sharing data, especially from clinical trials and longitudinal studies, so that new methods can be applied to reduce the risk, time, and cost for therapeutic trials now and in the future.
The Coalition For Accelerating Standards and Therapies (CFAST), a joint initiative of C-Path and CDISC, was founded to advance clinical research and medical product development by creating and maintaining data standards, tools, and methods for conducting research in therapeutic areas that are important to public health. C-Path led the development of the first CDISC Therapeutic Area (TA) Data Standards, in order to advance the data aggregation needs of specific C-Path consortia. This was done in collaboration with CDISC. To date, in partnership with CDISC and the US FDA, the National Cancer Institute Enterprise Vocabulary Services (NCI EVS), TransCelerate BioPharma, the European Medicines Agency (EMA), the Innovative Medicines Initiative (IMI), and the Association of Clinical Research Organizations (ACRO), 28 CDISC therapeutic area standards have been published, and C-Path has led or supported the work on 14 of these projects.

The Critical Path for Parkinson's Consortium (CPP) was created in partnership with Parkinson's UK, one of the world's largest charity funders of Parkinson's research. Parkinson's has traditionally been viewed as a disorder in which individuals don't have enough dopamine, because specific nerve cells inside the brain have died. Current research, however, indicates that the processes leading to dopamine deficit start much earlier (decades), increasing the need to understand the early stages of Parkinson's progression. CPP brings together pharmaceutical companies and academic partners working toward a common goal of establishing best practices and more efficient protocols for planning and designing clinical trials in early Parkinson's, which will improve the clinical trial process and deliver better treatments faster.

The Critical Path to TB Drug Regimens (CPTR) facilitates the accelerated development of novel drug regimens and rapid drug susceptibility diagnostics for TB. Tuberculosis is a disease that still impacts one-third of the world's population, which is in desperate need of a safer, shorter-duration, and more effective drug regimen. Much of this critical work is enabled by a global data-sharing initiative, funded by the Bill & Melinda Gates Foundation, and led by C-Path and partner organizations, which include WHO, TB Alliance, and multiple data contributors representing industry, academia, and government agencies.
The Data Collaboration Center (DCC) built C-Path’s Online Data Repository (CODR), and it has become the home of the CFAST initiative. The goal of the DCC is to enable multiple organizations to work together to share clinical data, in order to optimize its value in creating new insights and tools that accelerate drug development in areas with unmet medical needs. The DCC supports data-sharing projects aligned with specific C-Path consortia, as well as data-sharing initiatives that are independent of C-Path consortia. All of DCC’s work takes place in a neutral, pre-competitive environment, utilizing appropriate CDISC standards. The DCC possesses the technical and scientific subject matter and project management expertise necessary to support advanced research efforts.

The Duchenne Regulatory Science Consortium (D-RSC) was formed in partnership with Parent Project Muscular Dystrophy (PPMD) to aggregate data and develop a disease progression model to accelerate the development of new therapies for Duchenne muscular dystrophy, which is an urgent unmet medical need. Duchenne is a genetic disease that causes progressive loss of muscle, resulting in the inability to walk, breathing and cardiac issues, and premature death. D-RSC aims to improve trial protocol development and reduce the number of patients needed to demonstrate the effect of new therapies, thereby accelerating the development of the therapies themselves.

The Electronic Patient-Reported Outcome (ePRO) Consortium was established to advance the science surrounding electronic collection of PRO endpoints in clinical trials. The movement from “paper and pencil” to electronic data collection has profoundly enhanced the quality of clinical trial data. Handheld, touchscreen-based devices and web-based programs have become the mainstay for remote (i.e., off-site, unsupervised) PRO data collection in clinical trials.
The International Neonatal Consortium (INC) is a global collaboration forging a more predictable regulatory path to evaluating the safety and effectiveness of therapies for neonates. The consortium engages the global neonatal community—families, neonatal nurses, academic scientists, regulators, pharmaceutical investigators, advocacy organizations, clinicians, and funders—to focus on the needs of the neonate. Through teams that share data, knowledge, and expertise, INC advances medical innovation and regulatory science for this underserved population.

The Multiple Sclerosis Outcome Assessments Consortium (MSOAC) collects, standardizes, and analyzes data about MS that have been generated over several decades, with the goal of qualifying a new measure of disability as a primary or secondary endpoint for future trials of MS therapies. MSOAC has brought together members from academia and industry, regulatory authorities, patient advocacy groups, and persons living with multiple sclerosis. MSOAC is working to speed the development of new therapeutic options by developing better measures of outcomes.

The Polycystic Kidney Disease Outcomes Consortium (PKDOC) brings together leading nephrologists and other scientists from academia, industry, and government to spur the development of new therapies for patients with this debilitating disease. PKDOC’s mission is to develop drug development tools and methods to promote research that will lead to the discovery of treatments for PKD and improve the lives of all it affects. PKDOC has developed CDISC data standards for PKD and aggregated clinical data from Autosomal Dominant Polycystic Kidney Disease (ADPKD) patients collected over many years in patient registries and observational studies. These data enabled the development of a disease-biomarker model that provided the support necessary for FDA and EMA to qualify an imaging biomarker, Total Kidney Volume (TKV), for use as an enrichment strategy in drug development trials. PKDOC continues to work on alternative endpoints and clinical trial designs.
Despite considerable advances in medicine and technology, many of the tests used to evaluate drug safety have not changed in decades. The mission of the Predictive Safety Testing Consortium (PSTC) is to bring together pharmaceutical companies to share and validate innovative safety-testing methods to accelerate drug development under advisement of the FDA, EMA, and PMDA. PSTC does this by developing and implementing scientific research strategies in a neutral, pre-competitive environment, thereby allowing members to share expertise, resources, data, and internally developed approaches, which improves both the speed and precision of the drug development process. PSTC’s efforts are intended to develop drug development tools that assist pharmaceutical companies and regulatory agencies in making better-informed decisions, all of which ultimately benefits patients. Currently, PSTC is engaged in the qualification of novel nonclinical and clinical safety biomarkers across several organ systems for application in the development of drugs.

The aim of the Pediatric Trials Consortium (PTC) was to develop a business model and the infrastructure required to launch a new, independent nonprofit organization whose mission is to facilitate clinical trials in children. PTC launched a new, independent legal entity (the Institute for Advanced Clinical Trials for Children [I-ACT for Children]) that provides the sustainable global infrastructure needed to plan, start up, conduct, and complete pediatric studies. I-ACT for Children spans subspecialties, study types, phases, and sponsor types (such as industry, academia, and nonprofits). I-ACT for Children will accelerate the availability of innovative, safe, and effective medicines for children, improving health and wellness globally.

The Patient-Reported Outcome (PRO) Consortium brings together drug developers, measurement scientists, patients, clinicians, and regulators to collaborate on effectively incorporating the voice of the patient into the drug development process. Its primary goal is to obtain regulatory qualification of patient-reported outcome measures and other clinical outcome assessments (COAs) for use in clinical trials where COAs can, and should, be used to evaluate patient-focused treatment benefit.
The Transplant Therapeutics Consortium (TTC) convenes diverse stakeholders (industry, academia, and government agencies) to support collaborative development and regulatory endorsement of new drug development tools for solid organ transplantation, which, in turn, may help to shorten the time needed to deliver safe, effective therapies for transplantation patients. There are a number of barriers to developing new therapies for transplantation, and there is a need for experts in the field, and other interested parties, to work collaboratively to prioritize and resolve them. The TTC brings together key stakeholders in the transplant community with the primary goal of identifying and addressing the regulatory challenges that impact the development and approval of new therapies in transplantation.

The Type 1 Diabetes (T1D) Consortium is a public-private partnership initiated by C-Path, JDRF, the Leona M. and Harry B. Helmsley Charitable Trust, Sanofi, and Janssen Research & Development, LLC. Membership also comprises pharmaceutical companies, academic partners, and advisors from the National Institutes of Health (NIH). The goal of the T1D Consortium is to initiate a qualification program for biomarkers of islet autoimmunity for use in the development of therapies for the treatment of T1D. The objective of this consortium is to understand the scientific basis for the use of these biomarkers as well as the regulatory expectations associated with their use as prognostic and potentially pharmacodynamic markers. The islet autoantibodies of interest include insulin autoantibodies (IAA), glutamic acid decarboxylase 65 (GADA), insulinoma antigen-2 (IA-2), and zinc transporter 8 (ZnT8) autoantibodies, and will be evaluated per data availability.
C-Path Collaborators

ACADEMIC INSTITUTIONS

• Binghampton University
• Boston University
• Brigham and Women’s Hospital
• Case Western Reserve University
• Catholic University of Leuven
• Cleveland Clinic
• Colorado State University
• Duke University
• Emory University
• Fraunhofer Institute for Algorithms and Scientific Computing
• Helmholtz Centre Munich
• Imperial College London
• Indiana University School of Medicine
• Johns Hopkins University
• Leiden University
• Lund University
• Mayo Clinic
• McGill University
• Mount Sinai Hospital
• Newcastle University
• New York University
• Oregon Health & Science University
• Stanford University
• State University of New York at Buffalo
• Stony Brook Medicine
• Tufts University
• University College London Institute of Neurology
• University of Alabama at Birmingham
• University of Arizona
• University of Bristol
• University of California, Davis
• University of California, Irvine
• University of California, San Francisco
• University of Cambridge
• University of Cape Town
• University of Chicago
• University of Colorado Denver
• University of Florida
• University of Genoa
• University of Glasgow
• University of Hasselt
• University of Helsinki
• University of Illinois
• University of Kansas
• University of Maryland
• University of Munich
• University of OULO
• University of Oxford
• University of Pennsylvania
• University of Rochester
• University of Sheffield
• University of St Andrews
• University of Texas
• University of Turku
• University of Tampere
• University of Virginia
• University of Washington
• VU University Medical Center
• Washington University in St. Louis
• assisTek
• Biomedical Systems Corporation
• Bracket
• CRF Health
• ERT
• FIND

• ICON
• MedAvante-ProPhase
• Medidata Solutions
• PATH
• YPrime

• Bambino Gesù Children’s Hospital
• Children’s National Health System
• Cincinnati Children’s Hospital Medical Center
• Paris Descartes University, Necker-Enfants Malades Hospital

• American Society of Transplantation
• American Society of Transplant Surgeons
• Council of International Neonatal Nurses
• Consortium of Multiple Sclerosis Centers
• International Society of Pharmacometrics
• National Association of Neonatal Nurses
• NEC Society
• Preemie Parent Alliance
• Tuberculosis Clinical Diagnostics Research Consortium
• The Transplantation Society
• Working Group on New TB Drugs (Stop TB Partnership)

• The Leona M. and Harry B. Helmsley Charitable Trust
• PhRMA
• TB Alliance
• Treatment Action Group
PHARMACEUTICAL COMPANIES

- Abbott Molecular
- AbbVie
- Actelion Pharmaceuticals Ltd
- Alere
- Allergan
- Amgen
- Astellas Pharma
- AstraZeneca Pharmaceuticals
- Avrobio
- Baxter Healthcare Corporation
- Bayer Pharma AG
- Becton Dickinson Diagnostic Systems
- Biogen
- BioMérieux
- Boehringer Ingelheim Pharmaceuticals, Inc
- Bristol-Myers Squibb Company
- Catabasis Pharmaceuticals
- Celgene
- Cepheid
- Chiesi Farmaceutici
- Commonwealth Biopharma
- CTI BioPharma
- Cydan Development
- Daiichi Sankyo
- Eisai
- Eli Lilly and Company
- EMD Serono

- Ferring Research Institute
- F. Hoffmann-La Roche
- Genentech, Inc
- GE Healthcare
- Gilead Sciences
- GlaxoSmithKline
- Goldfinch Biopharma
- Hansa Medical
- H. Lundbeck
- Horizon Pharma
- Ironwood Pharmaceuticals, Inc
- Innate Immunotherapeutics
- Kadmon Corporation
- Johnson & Johnson Pharmaceutical Services
- Mallinckrodt Pharmaceuticals
- Metabolic Solutions Development Company
- Merck Sharp & Dohme Corporation
- Millenium: The Takeda Oncology Company
- Mitsubishi Tanabe Pharma Corporation
- Novartis Pharmaceuticals Corporation
- Novo Nordisk
- Otsuka Pharmaceutical
- Palladio Biosciences
- Pfizer, Inc
- PharmaStat
- Qiagen
- Regulus Therapeutics
• Sanofi-Aventis U.S.
• Santhera Pharmaceuticals
• Sequella
• Sarepta Therapeutics, Inc
• Shire Pharmaceuticals, Inc
• Summit (Oxford) Limited
• Sunovion Pharmaceuticals
• Takeda Pharmaceuticals U.S.A.

• TEVA Pharmaceutical Industries, Ltd
• Thermo Fisher Scientific
• Transplant Genomics
• UCB Pharma
• Veloxis Pharmaceuticals
• Vertex Pharmaceuticals
• Wave Life Sciences

RESEARCH ORGANIZATIONS AND PATIENT GROUPS

• Alzheimer’s Association
• Alzheimer’s Drug Discovery Foundation
• Alzheimer’s Research UK
• Benaroya Research Institute
• Bill & Melinda Gates Foundation
• CHDI Foundation
• Cure Parkinson’s Trust
• Davis Phinney Foundation
• Graham’s Foundation
• Italian Multiple Sclerosis Society
• JDRF
• Kessler Foundation
• March of Dimes
• Mario Negri Institute for Pharmacological Research

• Michael J Fox Foundation
• Multiple Sclerosis Society of Canada
• Multiple Sclerosis Society of Great Britain and Northern Ireland
• National Multiple Sclerosis Society
• Parkinson’s Disease Foundation
• Parkinson’s UK
• Polycystic Kidney Disease Charity
• PKD Foundation
• USAgainstAlzheimer’s
• Parent Project Muscular Dystrophy
• Scientific Institute H.S. Raffaele, Italy
• Terasaki Research Institute
• Translational Genomics Research Institute
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Chief Medical Officer, YourEncore

The Honorable James C. Greenwood
President and CEO, Biotechnology Industry Organization (BIO)

Peter Barton Hutt, LLB, LLM
Senior Counsel, Covington & Burling LLP
Michael Kasser, MBA, PhD
CEO and President, Holualoa Companies

Jeffrey E. Jacob, SM
CEO, Cancer Prevention Pharmaceuticals; Principal, Tucson Pharma Ventures LLC

Shaun A. Kirkpatrick, MA
President and CEO, Research Corporation Technologies

Alan G. Levin, MS, CPA
Former Executive Vice President and CFO, Endo Health Solutions Inc

Richard T. Myers, Jr.
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