



2015

ANNUAL REPORT

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Mission Statement

- 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.
- Model** C-Path begins its second decade with a sound strategic foundation on which to build new collaborations. Our growth and successes in the past year, along with the work of organizations such as Europe's Innovative Medicines Initiative and undertakings such as FasterCures' Consortiapedia project, prove that the consortia model works, and, furthermore, is thriving.
- Merit** Consensus science delivers an output that is immediately applicable in developing the drug development tools that can be most useful in helping de-risk decisions in drug development and with regulatory agencies. Ultimately, a team science approach will open new doors and create new opportunities for each member. The successful collaborations that grow out of the consortia model achieve meaningful results efficiently, eliminate redundancy, and ensure sustainable progress.

10 YEARS

a decade of excellence

Letter from the CEO



MARTHA A. BRUMFIELD, PhD
President and CEO

Dear Friends and Supporters,

The past year has been a momentous one for C-Path. In 2015, C-Path celebrated its tenth anniversary, marking a decade of achievements in accelerating the path to a healthier world. With each commemoration of our first decade, we also celebrated new successes.

At an anniversary reception in London in May, we announced the opening of C-Path's London office and launched the International Neonatal Consortium (INC): the first of three new consortia to be focused on areas of unmet need in the pediatric population. Later in 2015, events in Tucson and Washington, D.C., highlighted ten years of progress made on the regulatory science opportunities identified in the FDA's Critical Path Initiative (CPI).

We were proud to launch the Data Collaboration Center (DCC) in the last fiscal year, which capitalizes on C-Path's leadership in the field of data standardization, management, and curation. The DCC serves as a trusted third party providing access to de-identified, pooled data sets for researchers.

A notable example of how C-Path applied its experience in data management to strengthen its global ties in 2015 is its selection by the World Health Organization's Special Programme for Research and Training in Tropical Diseases (TDR) to host a new TB clinical trial data-sharing platform (TB-PACTS).

C-Path is becoming a nerve center for consensus science and collaboration, and a provider of rich sources of pooled data sets. We owe our successes to the collective efforts of many: the community, donors, foundations, our board members, our collaborators (in industry, academia, and government), and patients. Thank you all. And, finally, thank you to our brilliant team for their first ten years of groundbreaking work.

Here's to the next ten years!

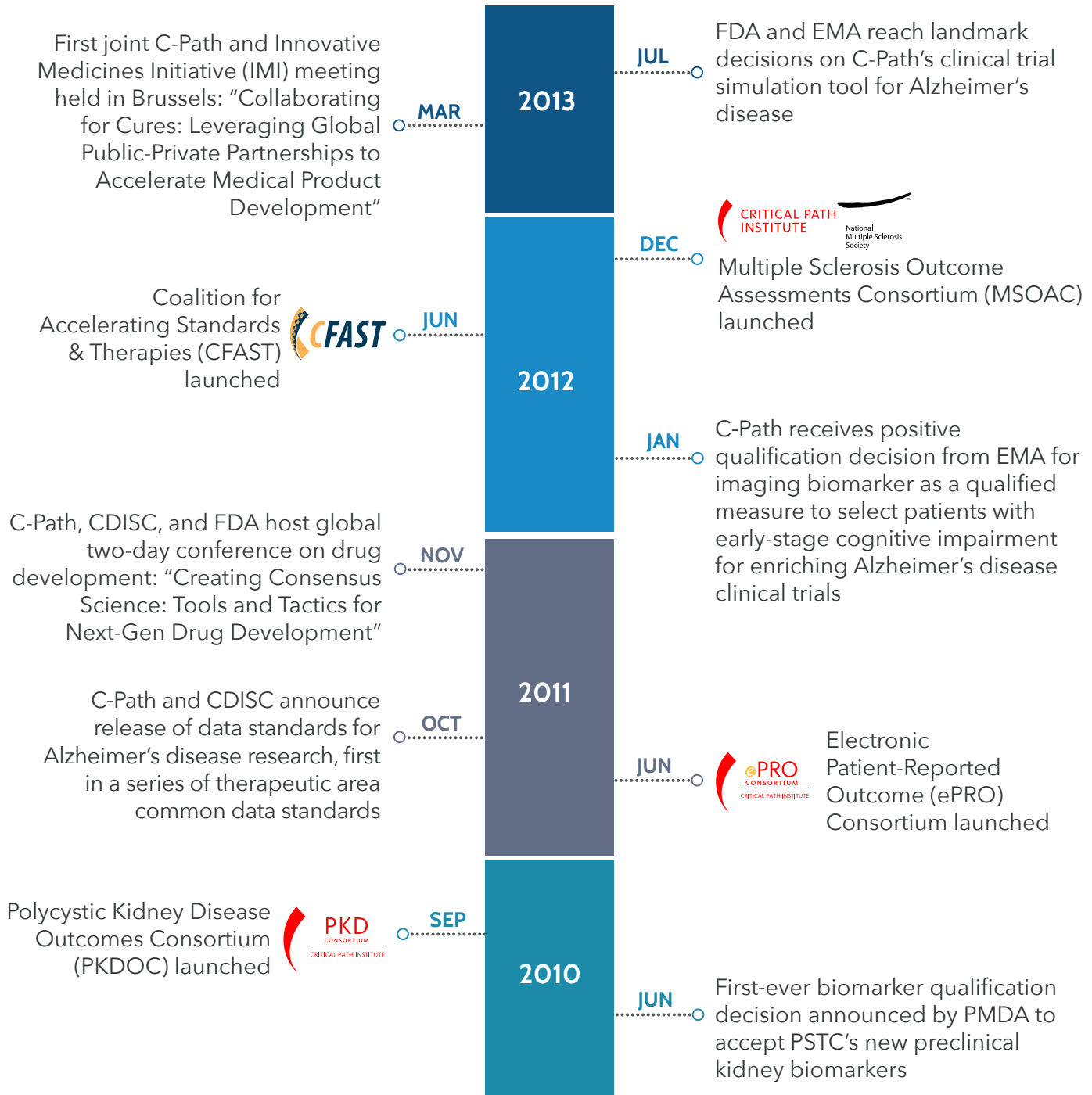
Warm regards,

Martha A. Brumfield, PhD
President and CEO

Highlights from Fiscal Year 2014-2015

MAY 19, 2015	●	C-Path launches International Neonatal Consortium (INC)
MAY 4, 2105	●	WHO selects C-Path to host TB clinical trials data
APRIL 27, 2015	●	FDA issues Letters of Support to CAMD for Alzheimer's and Parkinson's disease biomarkers
FEBRUARY 6, 2015	●	EMA qualifies "Hollow Fiber System" for anti-tuberculosis drug development
JANUARY 12, 2015	●	EMA issues first-of-its-kind Biomarker Letter of Support for two kidney safety biomarkers identified and evaluated by PSTC's nephrotoxicity working group
DECEMBER 3, 2014	●	Second Annual Meeting with Innovative Medicines Initiative
NOVEMBER 3, 2014	●	Flinn Foundation Awards C-Path \$1 Million Grant
OCTOBER 21, 2014	●	FDA issues first-of-its-kind Biomarker Letter of Support for two kidney safety biomarkers identified and evaluated by PSTC's nephrotoxicity working group

A Decade of Accomplishments





Disease progression in 75yo males, by APOE4 and baseline severity



Critical Path to TB Drug Regimens (CPTR) launched



MAR

2010

JUN

C-Path announces database of 11 industry-sponsored Alzheimer's disease clinical trials including data from more than 4,000 patients: the first effort of its kind to aggregate clinical trial data from multiple companies in a common CDISC data standard

Coalition Against Major Diseases (CAMD) launched



DEC

2009

MAR

Patient-Reported Outcome (PRO) Consortium

C-Path's preclinical kidney safety biomarkers are the first biomarkers ever qualified by FDA and EMA

JUN

2008

NOV

Reader's Digest lists C-Path as No. 7 on list of "18 Big Ideas to Fix Healthcare Now!"

Predictive Safety Testing Consortium (PSTC) launched



MAR

2007

JUN

C-Path hosts ribbon-cutting ceremony at Library of Congress to announce opening of Rockville, Maryland office

2006

2005

JUL

C-Path begins first fiscal year with six full-time employees



Trailblazing a Critical Path

In 2005, Dr. Raymond Woosley accepted the call to action laid out in the FDA's seminal report "Innovation/Stagnation: Challenge and Opportunity on the Critical Path to New Medical Products": He founded the Critical Path Institute, a first-of-its-kind social experiment in collaborative regulatory science, which could convene the regulated industry, FDA, academia, and patient advocates.



Dr. JANET WOODCOCK, Director of the U.S. Food and Drug Administration's Center for Drug Evaluation and Research

Dr. RAYMOND L. WOOSLEY, MD, PhD; Founder, Critical Path Institute; President & CEO, AZCERT, Inc.

Dr. MARTHA A. BRUMFIELD, PhD; President and CEO, Critical Path Institute

Within this collaborative sphere, data from disparate sources can be standardized, then applied to modeling and simulation technology, and other drug development tools, leading to drug development innovation, safer and more efficacious drugs reaching patients more quickly, improved health outcomes, and lives saved.

"The leaders in these jobs have to be diplomats—I would call them 'health diplomats,' because it's not only understanding the science, it's understanding the culture, and how to bring these disparate groups together and see a common vision."

—Richard Carmona, MD, MPH, FACS, former Surgeon General of the United States

"C-Path specializes in daunting challenges.... C-Path has taken on massively complex problems, and is solving them."

—Jack Jewett, President and CEO, Flinn Foundation

Broadening the Path



“With the launch of the International Neonatal Consortium, I think C-Path was very pioneering, because it is an area where few public-private partnerships have been established. This was new ground, to bring together stakeholders from all of these different arenas: from industry, from academia, from parent and patient organizations, from professional societies, and from the regulators and other government entities to facilitate the development of safe and effective therapies for neonates.”

–Susan McCune, MD, Deputy Director,
Office of Translational Sciences, Center for
Drug Evaluation and Research, FDA

New Initiatives with a Global Reach

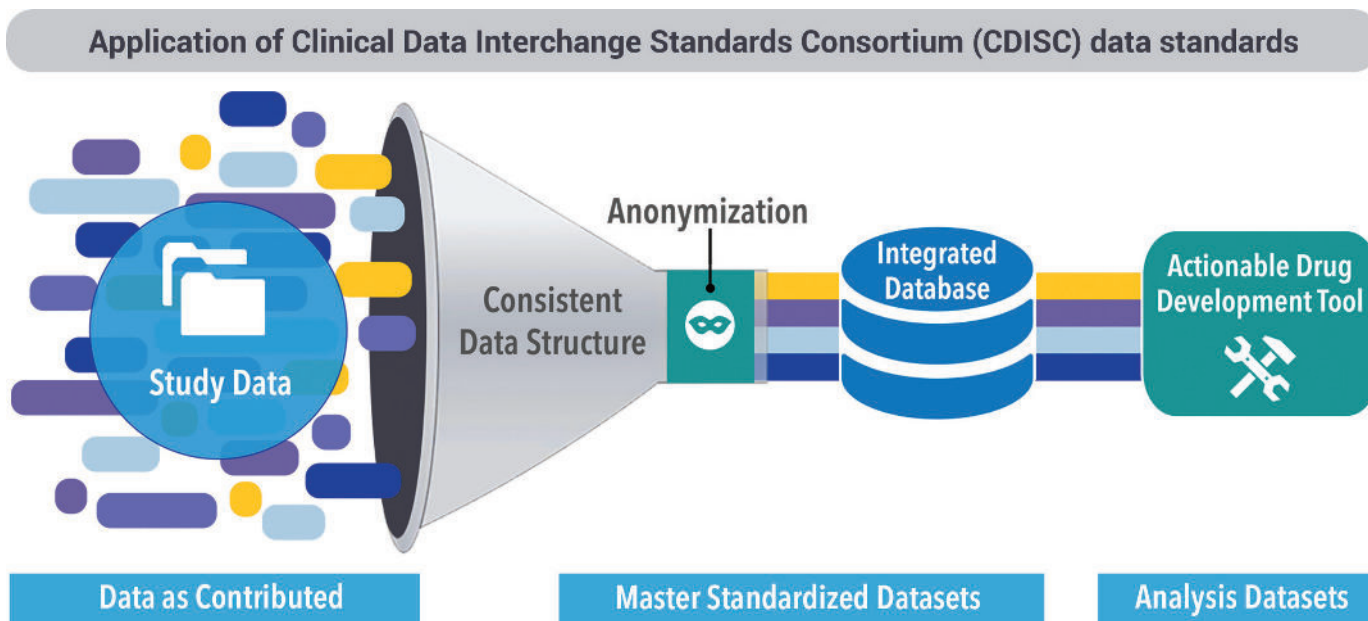
In 2015, C-Path launched new consortia and strengthened its presence in Europe. C-Path made plans to join forces with Parkinson’s UK to launch the Critical Path for Parkinson’s Consortium (CPP). The International Neonatal Consortium (INC), launched in May, comprises hospitals, academic institutions, regulatory agencies, and patient advocacy groups from North America, Europe, Asia, and Australia/New Zealand. INC, which will forge a more predictable regulatory path for evaluating neonatal therapies, is an example of how C-Path is growing its international presence as well as making substantial advances in the pediatric research space. Plans have been in the works to create the Pediatric Trials Consortium (PTC)—an entity enabling C-Path to establish a new, independent nonprofit organization. The Duchenne Regulatory Science Consortium (D-RSC), to be launched in partnership with Parent Project Muscular Dystrophy (PPMD), will work to aggregate clinical data and establish drug development tools to facilitate the development of new treatments for this genetic disorder.

Maximizing Our Data Expertise



In 2014, C-Path planned and launched the Data Collaboration Center (DCC) as a means of leveraging data-sharing expertise. The DCC serves as a trusted third party providing access to anonymized, pooled data sets for researchers. Increasing our technology capabilities remains a long-term goal and, to this end, we are implementing significant improvements in functionality for our data management platform.

C-Path will continue to be at the forefront of delivering best practices and policy thought leadership surrounding data curation, data standards, and data sharing. The C-Path Data Standards, Management and Technology (DSMT) team has been responsible for the implementation of the DCC, as well as C-Path's Online Data Repository (CODR), and C-Path's overall Information Technology infrastructure and operations.

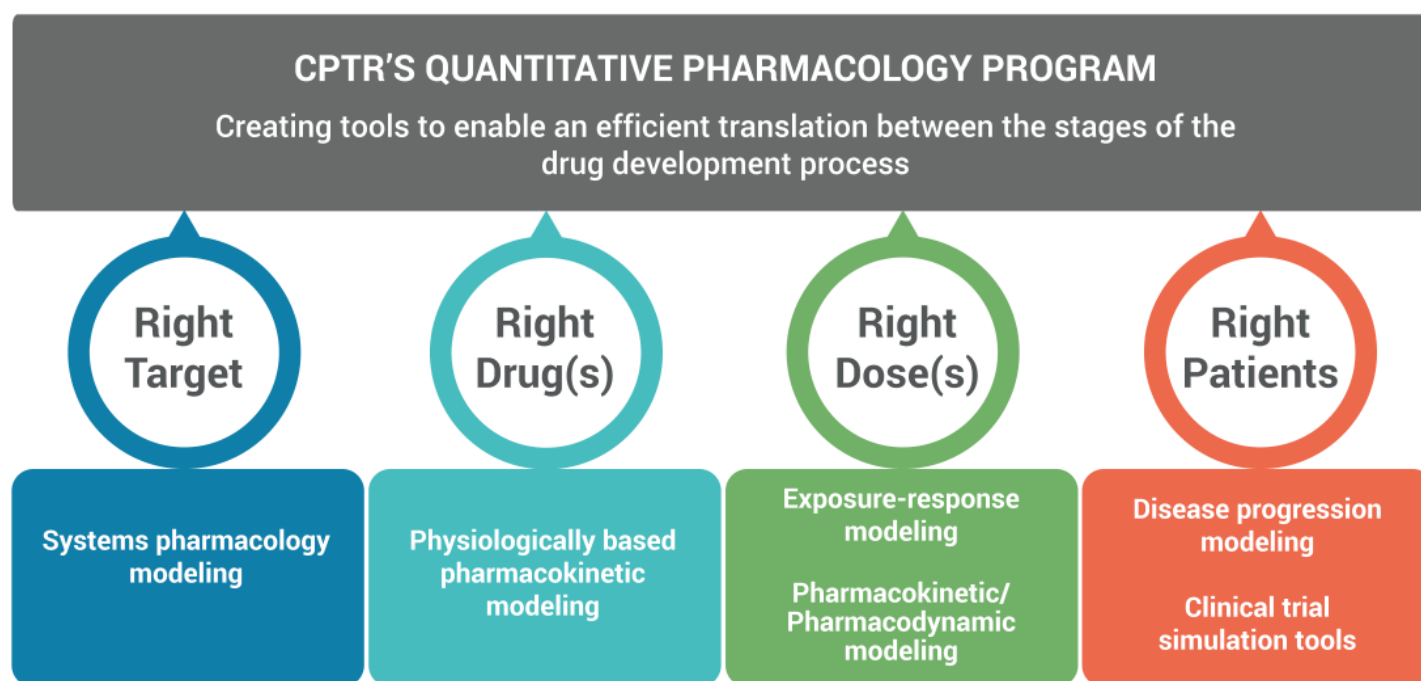


This illustrates the process of taking non-standardized data from individual studies, applying CDISC standards so all the data can be aggregated, and utilizing that fully integrated database to support the delivery of drug development tools.

The Path Ahead

Quantitative Pharmacology

The accurate, highly rigorous standardized data that C-Path curates are the foundation for quantitative model-based drug development tools, which can facilitate the design of optimal clinical trials, increase the potential for successful trial results, and allow for the continual refinement of the designs as more relevant data become available. Quantitative pharmacology facilitates the creation of tools to enable efficient transitions between stages of the drug development process.



The knowledge that comes from these new tools can provide pathways to precision medicine. Research endeavors involving disease-biomarker modeling are being applied to support development of new combination drug regimens for tuberculosis.

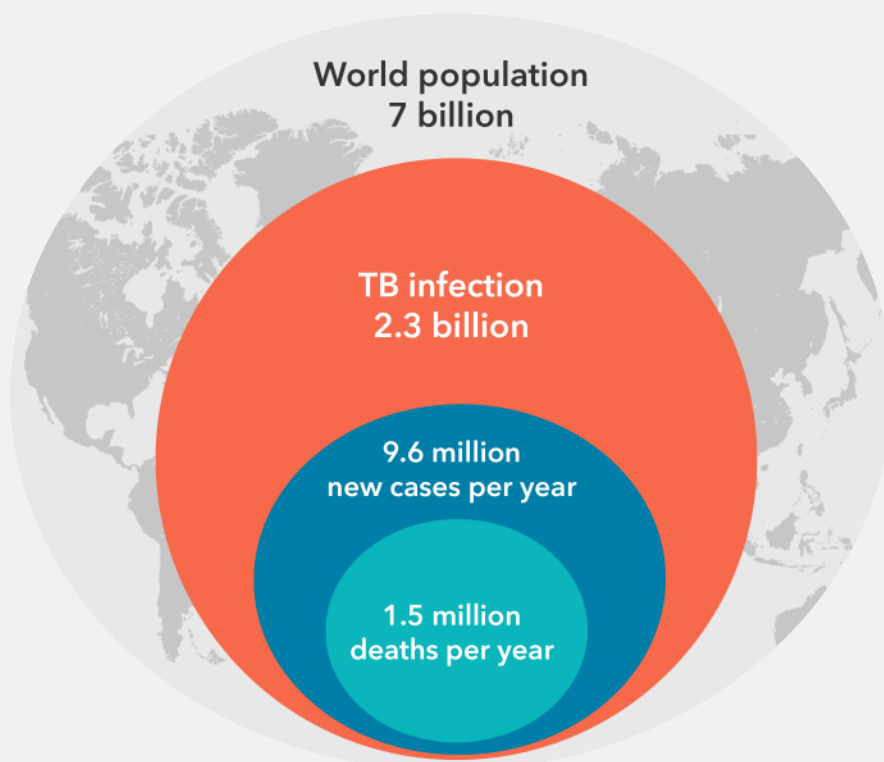
Data as a Weapon to Fight Infectious Disease

C-Path's TB-Platform for Aggregation of Clinical TB Studies (TB-PACTS) database is a testament to both our data-curation expertise and our global reach. Through a competitive proposal submission process, C-Path was selected to host the database, which is scheduled to go live in March 2016. It is designed as a resource to catalyze and accelerate innovative tuberculosis (TB) research by making aggregated Phase III TB clinical trial data publicly available. This initiative is a collaborative partnership between the Special Programme for Research and Training in Tropical Diseases (which is hosted by the World Health Organization), the TB Alliance, St. George's University of London, and C-Path. The TB-PACTS database, and the collaborative effort and data aggregation expertise that has gone into its creation, compose a valuable prototype for data-sharing in the global health arena.

"About five years ago, we started working with the Critical Path Institute on tuberculosis.... to accelerate the development of TB drug regimens, and to develop new tools that could be used in the evaluation of TB drugs and TB drug candidates.... Many people thought this could not be done, that it was not feasible. Instead, the program began to impact the way TB drugs are developed, and this is largely due to the expertise, the impartiality, and the scientific integrity which the Critical Path Institute brings to the table."

—Jan Gheuens, Deputy Director,
Bill & Melinda Gates Foundation

2015 Global TB Report Statistics



Financial Status

Fiscal Year Ending June 30, 2015

Thanks to the growing list of partners who see the value that Critical Path Institute (C-Path) provides in advancing regulatory science to help expedite the medical product development process, the state of C-Path is strong.

The initial five-year grant received from the U.S. Food and Drug Administration (FDA) as part of its Critical Path Initiative program expired in the fall of 2014. C-Path applied for a new competitive funding opportunity from the FDA in this fiscal year and was awarded \$2.1 million in first-year funding of a new five-year grant having the potential of \$10.5 million over five years. This award is financially significant, but is also indicative of continued support for C-Path's collaborative model.

This fiscal year, the Critical Path to TB Drug Regimens (CPTR) marked a five-year anniversary in its fight against tuberculosis (TB), a disease that claims one million lives each year. CPTR received a three-year grant renewal from the Bill & Melinda Gates Foundation (BMGF) to continue to advance the science and regulatory pathways that lead to more effective and shorter-duration drug regimens for TB.

In addition, BMGF awarded a new multi-year grant to fund the creation and implementation of the Rapid Drug Susceptibility Test initiative (RDST), which included developing a relational sequencing TB data platform to catalog a vast amount of TB genomic data of worldwide

TB strains. This database will advance the development of rapid drug susceptibility tests for TB, which will enable a faster selection of an effective treatment regimen of multidrug-resistant TB.

Due to C-Path's vast experience in designing and implementing global data platforms in numerous major disease areas, C-Path competed for and was selected by the World Health Organization (WHO) to host a new TB clinical trial data sharing platform. In this role, C-Path will host, curate, and make key TB clinical trial data sets available to qualified researchers.

Arizona-based financial support was provided to C-Path from a leader in developing a bioscience roadmap for Arizona, the Flinn Foundation. The foundation awarded a \$1 million grant over three years to build C-Path's Data Collaboration Center (DCC), which underscores C-Path's capacity in technical data governance, standards-based data aggregation, and secure sharing of clinical data that aids medical product development and decision-making.

At the request of the FDA, C-Path launched a new consortium in May of 2015, the International Neonatal Consortium (INC), dedicated to helping meet the challenges in developing new therapies for newborn infants. This effort is fully funded by the participating pharmaceutical companies.

Fiscal Year 2015

ASSETS

Cash and Cash Equivalents	\$	9,973,263
Certificates of Deposit	\$	4,727,502
Accounts Receivable	\$	841,762
Property and Equipment, Net	\$	121,311
Other	\$	52,866

TOTAL ASSETS \$ 15,716,704

LIABILITIES AND NET ASSETS

Liabilities

Accounts Payable	\$	985,077
Accrued Expenses	\$	145,737
Deferred Revenue*	\$	9,764,627
Deferred Rent	\$	36,832

Total Liabilities \$ 10,932,273

Net Assets

Undesignated	\$	2,116,359
Board Designated**	\$	2,546,761
Property and Equipment	\$	121,311
Temporarily Restricted	\$	-

Total Net Assets \$ 4,784,431

TOTAL LIABILITIES AND NET ASSETS \$ 15,716,704

* Pre-awarded funds received for grants

** Consortia fees managed by C-Path to support consortia activities

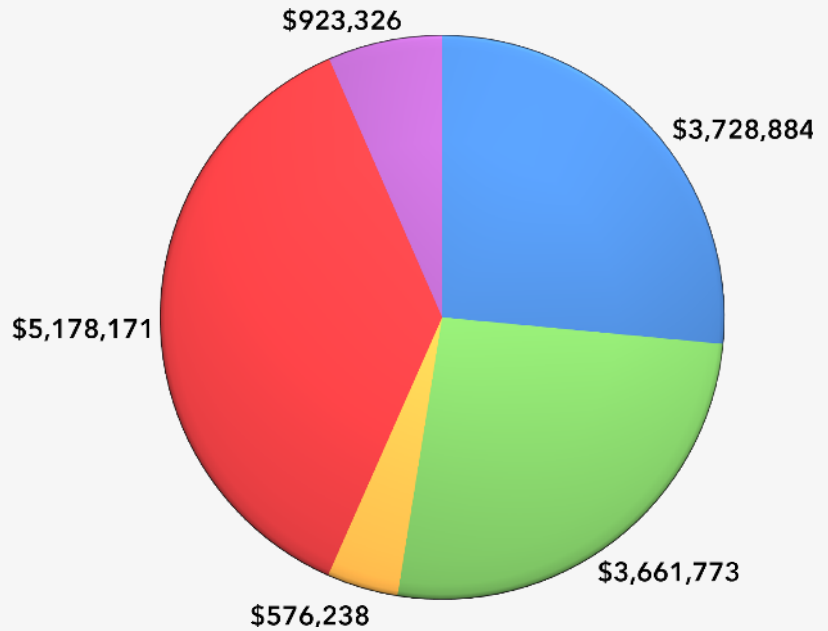
Advancing regulatory science is a global concern, and, this fiscal year, C-Path established a presence in London in May 2015 to more efficiently continue its work with European collaborators such as the European Medicines Agency (EMA) and the Innovative Medicines Initiative (IMI), as well as to develop new partnerships. Our consortia have already started utilizing this contracted office space to host some important meetings with key partners.

After the close of the fiscal year, C-Path launched three additional new consortia with significant funding from philanthropic and industry partners. Additional opportunities are in development, and next year promises to be another one of continued successes and growth.

In summary, the collaborative, consortium-based approach to problem-solving that C-Path pioneered back in 2005 continues to gain recognition for the value it brings to help solve some of the biggest challenges facing drug development. C-Path is committed to continued innovation while ensuring delivery on current projects.

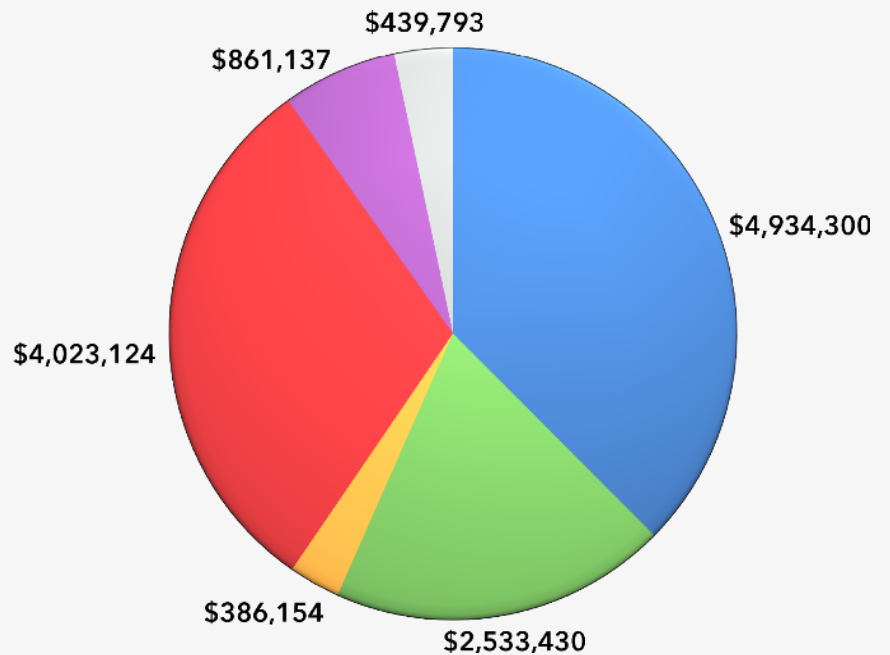
C-Path 2015 Fiscal Year Revenue

- Industry Fees
- FDA
- National MS Society
- Bill & Melinda Gates Foundation
- Other



C-Path 2015 Fiscal Year Expenses

- Salary & Fringe Benefits
- General Expenses
- Occupancy Expenses
- Subawards/Subcontracts
- Professional/Outside Services
- Travel & Meeting Expenses



C-Path's Consortia and Initiatives



The Coalition Against Major Diseases (CAMD) brings together diverse stakeholders to accelerate the development of treatments for those living with Alzheimer's and other dementias, as well as those in the presymptomatic stages who will likely progress to dementia. These experts use their combined knowledge to develop new tools to plan and design clinical trials, to improve decision-making around enrolling the right patients, and to gain confidence in accurately monitoring changes in functional and cognitive capabilities with disease progression. CAMD focuses on neurodegenerative diseases that require a collaborative approach due to the complexity of these diseases 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 accelerate clinical research and medical product development by facilitating the establishment and maintenance of CDISC therapeutic area data standards, tools, and methods for conducting research in therapeutic areas 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), 21 CDISC therapeutic area standards have been published, and C-Path has led or supported the work on 11 of these projects.



Critical Path to TB Drug Regimens

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, led by the Critical Path Institute and partner organizations, which include WHO, TB Alliance, the Bill & Melinda Gates Foundation, and multiple data contributors representing industry, academia, and government agencies.



DATA COLLABORATION CENTER™

Launched in FY 2014-15

The Data Collaboration Center (DCC) evolved from the work of the Data Standards, Management and Technology (DSMT) group, which has been the force behind C-Path's Online Data Repository (CODR) and its CFAST initiative. The DCC has expanded on the DSMT's best practices and policy thought leadership concerning data curation, providing rich data resources for scientific research, including data sharing, administration, storage, multi-source aggregation and standardization, and the facilitation of analysis and interpretation by collaborative teams. All of DCC's work takes place in a neutral, pre-competitive environment, utilizing appropriate data standards (such as those of CDISC). The DCC possesses the technical and scientific subject matter and project management expertise necessary to support advanced research efforts.



ePRO CONSORTIUM

CRITICAL PATH INSTITUTE

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.



Launched in FY 2014-15

The International Neonatal Consortium (INC) is a global collaboration forging a 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, 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 has 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 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-completed questionnaires for use in clinical trials where PRO endpoints can, and should, be used to evaluate patient-focused treatment benefit.





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 an imaging tool and 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 used clinical data from 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.



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 regulatory science 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 clinical safety biomarkers across several organ systems to be applied in the development of drugs.

Consortia Members



abbvie



Consortia Partners



We want to thank the U.S. Food and Drug Administration and Science Foundation Arizona for their significant funding of our work.

Board of Directors

To meet our goal of expanding our Board to support our wider scope of activities, in 2014-2015 we welcomed Timothy R. Franson, MD, Peter Barton Hutt, LLB, LLM, Alan G. Levin, MS, CPA, and Paula J. Olsiewski, PhD, to our board. Their combined expertise covers the areas of food and drug law and legislation, international finance, capital markets, business operations in the pharmaceutical industry, biotech and biomedical communities, and fostering relationships between academia and industry.

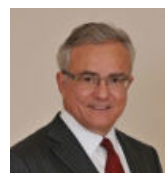
D. Craig Brater, MD

Vice President of Programs at the Walther Cancer Foundation and the Regenstrief Foundation



Peter B. Corr, MD, PhD, Chairman

Co-founder and General Partner of Auen Therapeutics Management LLLP



M. Wainwright Fishburn, Jr.

Founding partner of Cooley LLP's San Diego office, Chairman of the Sanford-Burnham Institute for Medical Research



Timothy R. Franson, MD

Chief Medical Officer, YourEncore



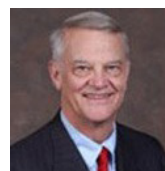
The Honorable James C. Greenwood

President and CEO of the Biotechnology Industry Organization (BIO)



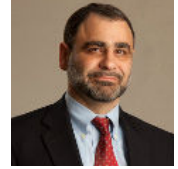
Peter Barton Hutt, LLB, LLM

Senior Counsel at Covington & Burling LLP



Jeffrey E. Jacob, SM, Deputy Chair

CEO of Cancer Prevention Pharmaceuticals,
Principal of Tucson Pharma Ventures LLC



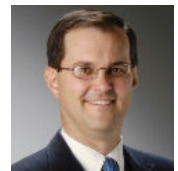
Michael Kasser, MBA, PhD

CEO and President of Holualoa Companies



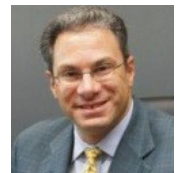
Shaun A. Kirkpatrick, MA

President and CEO of Research Corporation
Technologies (RCT)



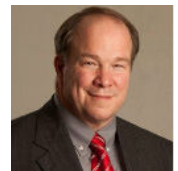
Alan G. Levin, MS, CPA

Former Executive Vice President and CFO of
Endo Health Solutions Inc.



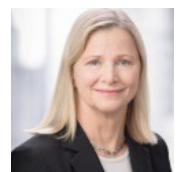
Richard T. Myers, Jr.

Member of the Arizona Board of Regents,
CEO of Tempronics, Inc.



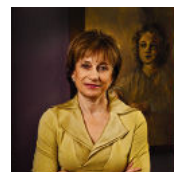
Paula J. Olsiewski, PhD

Program Director, Alfred P. Sloan Foundation



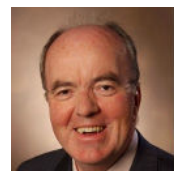
Cindy Parseghian

President and Co-founder of the Ara
Parseghian Medical Research Foundation



Alistair J.J. Wood, MB, ChB

Professor of Medicine and Professor of
Pharmacology at Weill Cornell Medical College





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