



CRITICAL PATH
INSTITUTE

ACCELERATING THE PATH TO A HEALTHIER WORLD

2014

ANNUAL REPORT

Dear Friends and Supporters,

As I look back at all of C-Path's achievements over the past year, I feel enormous gratitude towards all of C-Path's supporters, consortia participants, collaborating agencies, sponsors, stakeholders, and team members. It is overwhelming to behold the continuous engagement, interest, support, and hard work that individuals and groups contribute within C-Path's eight global consortia. Whether it be through the development of robust mathematical models (such as disease progression or clinical trial simulation), development of clinical trial data standards, demonstrating the value of biomarkers as trial enrichment tools, or evaluating new clinical outcome assessment instruments, I am extremely proud of the progress this organization has made over the past year through a variety of collaborations born from the hearts and minds of our enthusiastic partners and our dedicated staff.



MARTHA A. BRUMFIELD, PHD
President and CEO

By being part of a collaborative community that truly delivers innovative tools and methods that are the start of a paradigm change, we are collectively making a difference towards improving the lives of those who suffer with disease. We recognize that collaboration must occur across borders and oceans, across institutions, and across initiatives that are working toward similar goals either within a defined disease or methodology. We have advanced new ways to collaborate with the FNIH Biomarkers Consortium to evaluate kidney safety biomarkers in a clinical setting and with the Innovative Medicines Initiative's Safer and Faster Evidence-based Translation Consortium (SAFE-T) in identifying opportunities for data sharing with C-Path's Predictive Safety Testing Consortium. We will continue our quest to seek collaborations where they are needed to expedite the knowledge sharing, data sharing, and workload sharing to help find pathways to deliver safe and effective treatments to those who need them.

C-Path remains strongly committed to our partners and contributors, and to the delivery and development of even more efficient and effective drug development tools and methodologies that help yield critical and life-saving interventions throughout the globe. We are genuinely humbled to be at the nexus of so many collaborative partnerships as we move forward in FY 2014-15.

Warm regards,

A handwritten signature in blue ink that reads "Martha A. Brumfield". The signature is fluid and cursive.

Martha A. Brumfield, PhD
President and CEO



Accelerating the Path to a Healthier World



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.



As an independent and trusted partner, we value integrity, innovation, and teamwork.

WE COLLABORATE, INNOVATE, AND ACCELERATE ON A GLOBAL SCALE

who we are

The Critical Path Institute is an independent, non-profit organization that brings together pharmaceutical, academic, government, and non-profit organizations to work on important drug and medical product development challenges.

C-Path's teams advance regulatory science by developing tools and scientifically valid processes that decrease the length of time, cost, and risk for developing safe, effective medical products.

what we do

how we do it

C-Path forms and manages global consortia that share knowledge and data to develop clinical outcome assessment instruments, biomarkers, and other drug development tools that provide solutions to limitations in regulatory science.



NEWS AND ACCOMPLISHMENTS

Jul 10, 2013 The U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) independently reach favorable decisions on the value of C-Path's new Alzheimer's disease clinical trial simulation tool – the first such instrument to receive these regulatory designations.

Sep 11, 2013 The Coalition for Accelerating Standards and Therapies (CFAST), a collaboration between C-Path and the Clinical Data Interchange Standards Consortium (CDISC), receives a \$2 million grant from the FDA to continue to develop data standards.

Sep 18, 2013 C-Path's Critical Path to TB Drug Regimens (CPTR) consortium expands its scope to include the development of vital drug susceptibility tests.

Dec 2, 2013 C-Path's Alzheimer's disease simulator featured in the Wall Street Journal.

Jan 28, 2014 C-Path's Coalition for Accelerating Standards and Therapies (CFAST), in collaboration with CDISC and TransCelerate BioPharma Inc., launch version 1.0 of the Asthma Therapeutic Area (TA) User Guide (UG) and version 2.0 of the Alzheimer's TAUG.

Feb 4, 2014 C-Path receives a three-year grant from the Bill & Melinda Gates Foundation to enable the development of novel regimens for tuberculosis.

May 19, 2014 C-Path, the FDA, and PhRMA co-sponsor the 14th Drug-Induced Liver Injury Conference.

Apr 29, 2014 C-Path and the FDA co-sponsor the fifth annual Patient-Reported Outcome (PRO) Consortium Workshop.

Jun 4, 2014 New multiple sclerosis data standard developed by C-Path's Multiple Sclerosis Outcome Assessments Consortium (MSOAC) released by CDISC.





STRATEGIC DIRECTION

C-Path's mission to catalyze the development of new approaches that spark medical innovation and advance regulatory science is grounded in our fundamental values of integrity, innovation, and teamwork. These values remain crucial factors in our ability to serve as a neutral third party and to foster an environment conducive to sharing information and delivering results. Successful collaborations depend upon clarity of purpose and objectives, transparency, and trust. We pride ourselves on building such collaborations.

While many of our collaborations focus on specific disease areas (including Alzheimer's disease, multiple sclerosis, Parkinson's disease, and tuberculosis), we have also established formal partnerships with other public-private initiatives working toward similar objectives. Examples include our collaborations with The Biomarkers Consortium managed by the Foundation for the National Institutes of Health, and the Innovative Medicines Initiative's Safer and Faster Evidence-based Translation (SAFE-T) consortium, both of which are helping to advance kidney safety biomarkers towards regulatory qualification in a clinical setting.

These kinds of partnerships enhance our ability to more efficiently achieve meaningful results, eliminate redundancy, and ensure sustainable

progress for all. We will continue to seek out and evaluate additional collaborative opportunities that advance medical product innovation and to establish strategic alliances that are either compatible with or complementary to our mission and core competencies.

C-Path will also continue to be at the forefront of delivering best practices and policy development surrounding data curation, data standards, data access, and data sharing in the pre-competitive phases of scientific, clinical, and biomedical research. Increasing our technology capabilities is a long-term goal and, to this end, we anticipate significant growth within our data management platform.

Our future looks bright. In the coming year, we plan to launch a new data collaboration center which will allow C-Path to better leverage our clinical and non-clinical data sharing expertise and serve as a unique resource for researchers, foundations, and other programs needing sustainable data solutions from a trusted third party. We will also augment our scientific and technical teams to lead new consortia, increase our presence in Europe through new partnerships, and expand our Board of Directors to support our wider scope of activities.

FINANCIAL STATUS

FISCAL YEAR ENDING JUNE 30, 2014

Critical Path Institute (C-Path) shares a common goal with its supporters and friends: that of helping transform drug and medical product development around the world. C-Path is a non-profit entity headquartered in Tucson, Arizona, and is grateful for its public and private support. Contributors and collaborators include the U.S. Food and Drug Administration (FDA), the National Multiple Sclerosis Society, the Bill & Melinda Gates Foundation, the Polycystic Kidney Disease Foundation, many pharmaceutical companies, as well as several other organizations and private philanthropists.

C-Path is a financially strong institution and operates in a fiscally sound manner. In this fiscal year, C-Path received two new opportunities funded by the FDA. In November, 2013, C-Path received a grant of \$2 million over two years to support the development of CDISC data standards designed to streamline the evaluation and approval of new medical therapies and allow easy aggregation of clinical data. A separate contract was also awarded to C-Path to work on an influenza data standard. This funding furthers the work of the Coalition for Accelerating Standards and Therapies (CFAST), a joint initiative C-Path has undertaken with the Clinical Data Interchange Standards Consortium (CDISC) to support the goals of the FDA's Therapeutic Area Standards Initiative Project Plan. As of the end of this fiscal year, 12 therapeutic area data standards have been developed, and nine more are scheduled to be completed in the next year.

Through funding from the National Multiple Sclerosis Society, C-Path continues to lead an effort to develop a new clinical outcome assessment instrument to improve the method of efficacy evaluation for new MS therapies. The work on innovative tools and methods to guide the development of novel regimens for tuberculosis, funded by the Bill & Melinda Gates Foundation, is going extremely well and is expanding into the diagnostic space. The Polycystic Kidney Disease Outcome Consortium, funded by the PKD Foundation, is waiting on a final qualification decision for its clinical imaging biomarker from both the FDA and the European Medicines Agency (EMA). Through these initiatives and others, C-Path has developed a strong reputation with the biopharmaceutical industry, the scientific and research community, and others.

After the close of this fiscal year, C-Path received significant additional funding from the Bill & Melinda Gates Foundation, the FDA, and the Flinn Foundation. This funding enables C-Path to expand the work to develop new diagnostics for tuberculosis, continue the work on Alzheimer's and Parkinson's diseases, carry on the development of six new Patient-Reported Outcome instruments, develop biomarkers of drug-

induced toxicity, and to support the development and growth of C-Path's Data Collaboration Center.

Each year C-Path is approached by several organizations eager to partner with it. There is a healthy pipeline of opportunities that will enable C-Path to grow and expand its work into other therapeutic areas with unmet needs. We look forward to another exciting year that promises to be filled with great accomplishments.

ASSETS

Cash and Cash Equivalents	\$	10,428,253
Certificates of Deposit	\$	3,374,261
Accounts Receivable	\$	1,233,637
Property and Equipment, Net	\$	163,400
Other	\$	53,545
TOTAL ASSETS	\$	15,253,096

LIABILITIES AND NET ASSETS

Liabilities

Accounts Payable	\$	396,935
Accrued Expenses	\$	132,284
Deferred Revenue*	\$	10,787,286
Deferred Rent	\$	43,015
Total Liabilities	\$	11,359,520

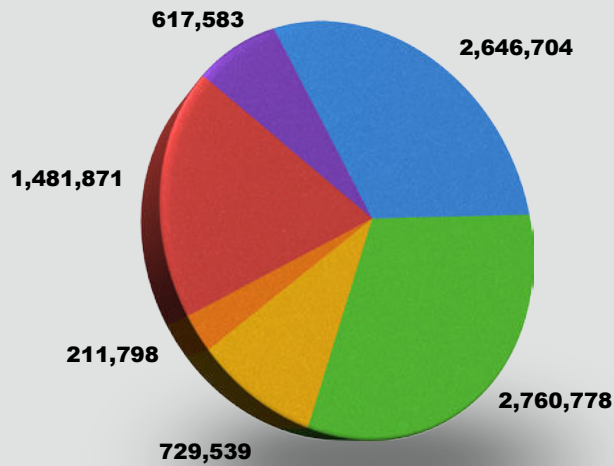
Net Assets

Undesignated	\$	1,382,797
Board Designated**	\$	2,331,717
Property and Equipment	\$	163,400
Temporarily Restricted	\$	15,662
Total Net Assets	\$	3,893,576
TOTAL LIABILITIES AND NET ASSETS	\$	15,253,096

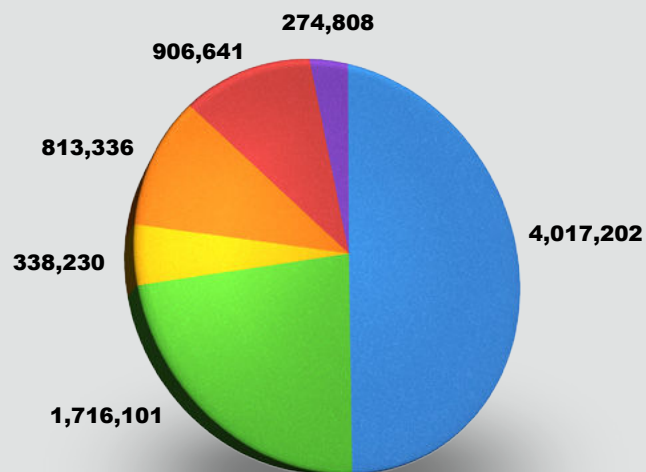
* Pre-awarded funds received for grants

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

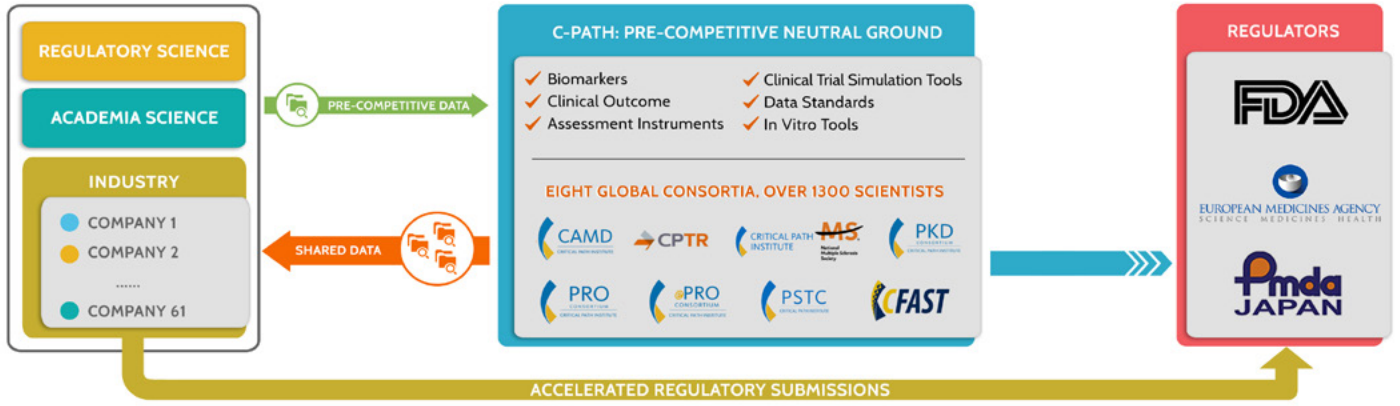
C-Path 2014 Fiscal Year Revenue



C-Path 2014 Fiscal Year Expenses



COLLABORATIVE DEVELOPMENT



C-Path’s Innovative Consortia Model Breaks New Ground to Accelerate Pathways to Novel Treatments



The Coalition Against Major Diseases (CAMD) is a public-private partnership aimed at streamlining drug development for Alzheimer’s disease (AD) and Parkinson’s disease (PD). Diverse stakeholders partner together to share data, develop consensus data standards, and create new drug development tools that can be used to increase the efficiency of the drug development and regulatory review processes. CAMD focuses on the early stages of these neurodegenerative diseases when therapies hold the most promise for treatment benefit.



The Critical Path to TB Drug Regimens (CPTR Initiative) is a public-private partnership initiated in March, 2010, by Critical Path Institute (C-Path), the Bill & Melinda Gates Foundation, and the TB Alliance. CPTR is focused on accelerating the development of an entirely new drug regimen to treat TB as well as the development of rapid drug susceptibility tests to ensure the effective deployment of new drugs and regimens. C-Path leads the CPTR Regulatory Science Consortium and the CPTR Rapid Drug Susceptibility Testing (RDST) Consortium, with participation from the pharmaceutical industry and academia, as well as national and global regulatory and other government agencies.



Launched in December, 2012, MSOAC is C-Path’s newest consortium – another dynamic partnership formed to promote consensus science. Created jointly with the National Multiple Sclerosis Society, MSOAC will collect, standardize, and analyze data about MS with the goal of qualifying a new measure of disability as a primary or secondary endpoint for future trials of MS therapies.



The Polycystic Kidney Disease (PKD) Outcomes Consortium is a successful collaboration between Critical Path Institute (C-Path), the PKD Foundation, Clinical Data Interchange Standards Consortium (CDISC), four leading academic medical centers (Tufts University, University of Colorado Denver, Emory University, and Mayo Clinic), and three pharmaceutical companies. Its mission is to develop tools and promote research that will lead to the discovery of treatments for PKD and improve the lives of all it affects.



The Patient-Reported Outcome (PRO) Consortium is based upon a collaborative framework of appropriate stakeholders that develops qualified and publicly available PRO instruments for use in clinical trials in order to support labeling claims. The PRO Consortium was formed in December of 2008 and formally launched in March of 2009. The PRO Consortium's membership is comprised of pharmaceutical companies along with representatives from the FDA, EMA, and NIH, who provide advice to the Coordinating Committee.



Critical Path Institute has established the Electronic Patient-Reported Outcome (ePRO) Consortium in cooperation with firms that provide electronic data collection technologies/services to the medical products industry for capturing patient-reported outcome (PRO) endpoints in clinical trials.



The Predictive Safety Testing Consortium (PSTC) brings together pharmaceutical companies to share and validate innovative safety testing methods under advisement of the U.S. Food and Drug Administration (FDA), its European counterpart, the European Medicines Agency (EMA), and the Japanese Pharmaceutical and Medical Devices Agency (PMDA). PSTC was formed and officially announced on March 16, 2006 by Health and Human Services Secretary Michael Leavitt, FDA Commissioner Dr. Andrew von Eschenbach, and FDA Deputy Commissioner Dr. Janet Woodcock, who identified the consortium as "unprecedented" and a "shining example" of the type of work the FDA would like to see conducted.



The Coalition for Accelerating Standards and Therapies (CFAST), a joint initiative of C-Path and the Clinical Data Interchange Standards Consortium (CDISC), was launched in June, 2012, to accelerate clinical research and medical product development by facilitating the establishment and maintenance of data standards, tools, and methods for conducting research in therapeutic areas important to public health. CFAST collaborators include the U.S. Food and Drug Administration (FDA), TransCelerate BioPharma Inc., the National Cancer Institute Enterprise Vocabulary Services (NCI-EVS), the Innovative Medicines Initiative (IMI), and the Association of Clinical Research Organizations (ACRO), with participation and input from many C-Path and CDISC members, as well as other organizations.

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

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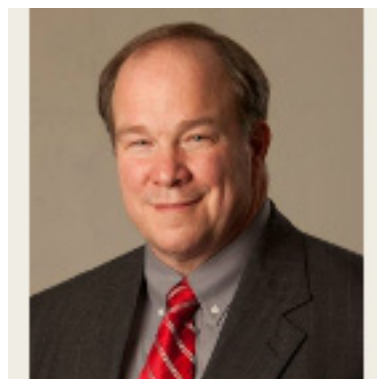
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