

ANNUAL REPORT

2012



*collaborate.*



*innovate.*



*accelerate.*



CRITICAL PATH  
INSTITUTE

## DEAR FRIENDS AND SUPPORTERS:



Martha A. Brumfield, PhD

Over the past year, Critical Path Institute (C-Path) has made continuous strides to increase the reach of our organization as a trusted leader in orchestrating successful collaborations across many sectors where disease burden is still high due to the need for new methods and tools which have the potential to accelerate the development of safe, effective medical products. As an example, we established an entity in the United Kingdom, Critical Path Global, Ltd., and have received Small/Medium Enterprise designation with the European Medicines Agency (EMA). This allows us to submit our work to the European Medicines Agency at a 90% discount, making it affordable to provide the same regulatory advancements in Europe that we are progressing within the United States.

In our seventh full year of operation, C-Path is now leading seven very active scientific consortia across multiple disease areas and attaining recognition as a global leader in leading scientific consortia to highly meaningful deliverables. In addition to the Alzheimer's Disease Standard we finalized in 2011, this past year we completed the work for a new Tuberculosis Disease Data Standard and a new Parkinson's Disease Data Standard. These new standards position C-Path well to develop new drug development tools in these disease areas.

In my first few months as President and CEO of C-Path, I am energized by the talented and dedicated staff at C-Path and their track record for accomplishment. Having recently returned from C-Path's first-ever public meeting in Europe, which was jointly sponsored with the Innovative Medicines Initiative, I am even more inspired to lead C-Path into the future as we succeed in achieving global progress towards advancing regulatory science. This meeting enabled C-Path to gain recognition with a broader audience of scientists, government policy makers, civil society organizations, and media.

As an independent non-profit organization, the financial and in-kind support we receive allows C-Path to continue to lead global efforts to develop new methods and tools to accelerate the process of getting new medicines to patients who need them. We encourage our supporters and collaborators to take pride in what you have helped to create at C-Path, and to join with us in our continued journey.

Sincerely,

A handwritten signature in blue ink that reads "Martha A. Brumfield". The signature is fluid and cursive, with a large, stylized initial 'M'.

Martha A. Brumfield, PhD  
President and CEO

# C-PATH CONSORTIA

## Predictive Safety Testing Consortium (PSTC)

PSTC brings together pharmaceutical companies to share and validate innovative safety testing methods under advisement of the FDA, EMA, and PMDA.

## Coalition Against Major Diseases (CAMD)

CAMD was created to develop innovative approaches and processes to accelerate drug development tools for the treatment of chronic neurodegenerative diseases.

## Patient-Reported Outcome (PRO) Consortium

PRO Consortium was created to develop and qualify PRO Instruments for use as primary or key secondary endpoints in clinical trials.

## Electronic Patient-Reported Outcome (ePRO) Consortium

The ePRO Consortium was created to facilitate the implementation of patient-reported outcome instruments onto electronic platforms such as smartphones, tablet computers, the web, or interactive voice response systems.

## Polycystic Kidney Disease (PKD) Consortium

The PKD Outcomes Consortium is a collaboration between the PKD Foundation, the FDA, C-Path, and clinician scientists to address the significant unmet need for effective drug therapies for Polycystic Kidney Disease.

## Critical Path to TB Drug Regimens (CPTR)

The CPTR initiative is a public-private partnership led by the Critical Path Institute, the Bill & Melinda Gates Foundation, and the Global TB Alliance focused on enabling the accelerated development of new treatment regimens for TB. Related work being conducted includes advancing novel biomarkers and data standards, developing a TB disease progression models and clinical trial simulation tools as well as enhancing global regulatory pathways for new regimens.

## Multiple Sclerosis Outcome Assessments Consortium (MSOAC)

The primary purpose of the MSOAC is to achieve qualification at the FDA and EMA of a clinician-reported outcome (ClinRO) instrument as a primary or co-primary endpoint in clinical trials of MS therapies.

## FINANCIAL STATUS

FISCAL YEAR ENDING JUNE 30, 2012

This fiscal year was one of transition for Critical Path Institute. On February 1, 2012, Carolyn Compton, M.D., Ph.D., took the reins as the new President and CEO as the founder, Dr. Ray Woosley, moved on to be the President and Chairman of the Board of AZCERT. In March of 2012, Steven Broadbent became the new Chief Operating Officer, taking over for Rick Myers, who was appointed as the chairman of the Arizona Board of Regents. Shortly after that, the leadership at C-Path identified new strategies that focused on expanding our core competencies into new therapeutic areas, diversifying our funding sources, and enhancing our methods of communicating with our stakeholders. These efforts are underway and are bearing fruit.

In February of 2012, the C-Path Board of Directors approved the use of fees collected from our consortia members specifically for C-Path staff. This was done after careful consultation with both the FDA and our industry partners to ensure it would not damage our reputation as a neutral third party, which is critical to our success. Together with the grants from the government and private foundations, these fees have enabled C-Path to continue to be financially strong in a challenging economy.

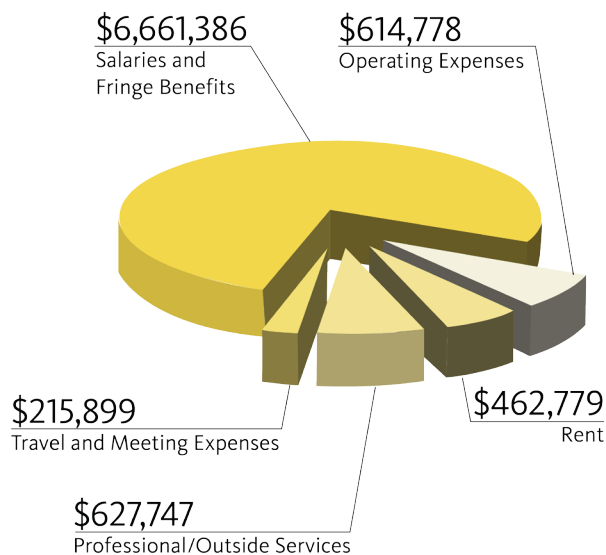
C-Path is continuing its evolution from a startup organization to a small, stable and mature organization. The \$12 million in initial funding from the local community and the \$14 million from Science Foundation Arizona have enabled C-Path to become firmly established and recognized in the nation and around the world as an organization with a track record of successful accomplishments. Several organizations have approached us with a desire to partner or fund efforts that are important to them and that align with our mission and core competencies. C-Path is now receiving grants from the U. S. Food and Drug Administration, the Bill & Melinda Gates Foundation, the Polycystic Kidney Disease Foundation, and the National Multiple Sclerosis Society for specified projects. As part of an overall effort to become more efficient, the decision was made to reduce the non-scientific staff. With the potential projects in the pipeline, we anticipate adding scientific staff next year.

Philanthropy remains an important source of funding for C-Path. We're grateful to those who continue to support us because they share our vision of accelerating the development of new therapies by reducing the time, cost, and risk of bringing these medicines to those who need them.

The steadfast support of our community, the passion and dedication of our scientists and staff, and the trust of our stakeholders continue to enable C-Path to fulfill its mission.

### FY12 BUDGET

**\$8,582,589**



\*Supported by federal grant

#### ASSETS

Cash and cash equivalents	\$4,687,188
Restricted funds	\$3,217,568
Accounts receivable	\$753,893
Property and equipment, net	\$328,372
Other Assets	\$81,672
<b>TOTAL ASSETS</b>	<b>\$9,068,693</b>

#### LIABILITIES & NET ASSETS

Accounts payable	\$247,940
Deferred revenue**	\$4,791,650
Deferred rent	\$64,221
<b>TOTAL LIABILITIES</b>	<b>\$5,103,811</b>
Unrestricted:	
Undesignated funds	\$1,000,855
Consortia funds	\$2,478,357
Property and equipment	\$328,372
Temporarily Restricted	\$157,298
<b>TOTAL NET ASSETS</b>	<b>\$ 3,964,882</b>
<b>TOTAL LIABILITIES &amp; NET ASSETS</b>	<b>\$9,068,693</b>

\*\*Pre-awarded funds received for grants

# OUR SUCCESSES

**Seven** consortia, **1,000+** scientists, and **41** member companies

**Seven** safety biomarkers qualified by the FDA, EMA, and PMDA (the Japanese counterpart) and in use by industry

**First imaging biomarker qualification by the EMA** to enrich Alzheimer's disease clinical trials

**Data standards published for Alzheimer's disease**  
developed with Clinical Data Interchange Standards Consortium

**Data standards published for Tuberculosis**  
developed with Clinical Data Interchange Standards Consortium

**Data standards published for Parkinson's disease**  
developed with Clinical Data Interchange Standards Consortium

**Data standards published for Polycystic Kidney disease**  
developed with Clinical Data Interchange Standards Consortium

**Database of aggregated Alzheimer's Disease clinical trial data**  
(6,500 patients and 24 clinical trials) - publicly available to qualified researchers

**First Alzheimer's disease progression model and simulation platform** in review by the FDA

Launched the **Coalition For Accelerating Standards and Therapies**. (CFAST) in partnership with CDISC

Established a **European subsidiary**, Critical Path Global, Ltd.

Won Arizona Bio-industry (AZBIO) "**Fast Lane Award**" for outstanding achievement

# CONSORTIA MEMBERS



# PARTNERS



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

## OUR OFFICE

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## MISSION

To improve health and save lives by accelerating the development of safe, effective medicines.

## VISION

To be the global leader in creating collaborations that advance scientific innovations to improve human health.

