CRITICAL PATH INSTITUTE

ANNUAL
REPORT 2016

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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.

Benefits of the Consortium Model

The most effective tools for evaluating medical product safety and efficacy incorporate input from the sponsors who conduct the trials, the patients who participate in the trials, and the regulators who ultimately apply the tools. The consortium model engages each of these stakeholders to arrive at consensus on how to design trials, what data to collect by what method, and what amount of change is meaningful to patients. The successful collaborations that grow out of the consortium model serve to de-risk decisions in drug development and ensure sustainable progress.

LETTER FROM THE CEO

Dear Friends and Supporters,

The past year has been another one of growth for us at C-Path, which is a testament to the value that our pre-competitive consensus science model brings to advancing innovative tools and methods to help de-risk decision making in drug development and regulatory review.

Over the past fiscal year, C-Path has widened its scope in the pediatric research space, taken on new disease areas, and enriched its expertise in data collaboration and quantitative medicine. This growth has afforded opportunities to expand our staff and forge new partnerships.

MARTHA A. BRUMFIELD, PhD
President and CEO

Launched in May 2015, the International Neonatal Consortium (INC) was the first in a series of initiatives aimed at improving clinical trials and drug development for the pediatric population. In August 2015, C-Path partnered with Parent Project Muscular Dystrophy (PPMD) to launch the Duchenne Regulatory Science Consortium (D-RSC), which aims to accelerate and improve trials for new therapies for Duchenne, a rare and life-threatening disease. C-Path's Pediatric Trials Consortium (PTC), launched in October 2015, was created to oversee and support the formation of a new, freestanding nonprofit organization. This organization would facilitate the development of innovative pediatric drugs, biologics, and devices, and would provide the global infrastructure needed to plan, conduct, and close out pediatric clinical studies. We are happy to report that such an organization is now underway: the Institute for Advanced Clinical Trials for Children (I-ACT for Children).

The same month we announced PTC to further address the needs for new innovative therapies for children, C-Path also increased its commitment to improving clinical trial design for neurodegenerative disorders. In partnership with, and with support from, Parkinson's UK, C-Path launched the Critical Path for Parkinson's Consortium (CPP), which will develop tools and information to aid in establishing best practices and more efficient protocols for clinical trials in early Parkinson's. This augments the work of our Coalition Against Major Diseases (CAMD), which focuses on Alzheimer's disease (AD) and related dementias.

Not only do we continue to widen our scope in the disease areas and populations we serve, but we at C-Path also continue to deepen our understanding of the best methods to advance research output toward regulatory qualified drug development tools. Our work in the field of quantitative medicine has led to past innovations such as the first regulatory endorsed clinical trial simulation tool for AD, as well as the Hollow Fiber System model for Tuberculosis (HFS-TB); with such successes behind us and new programs being undertaken, we have increased our staff and efforts in quantitative medicine.

Similarly, C-Path's Data Collaboration Center (DCC), launched in the past fiscal year, was founded on early successful C-Path database implementations such as the Coalition Against Major Diseases Consortium Database (CAMD AD/MCI) and the Polycystic Kidney Disease (PKD) Consortium Database. The DCC's achievements so far have included the TB-Platform for Aggregation of Clinical TB Studies (TB-PACTS), the Relational Sequencing TB Data Platform (ReSeqTB), and the Multiple Sclerosis Outcome Assessments Consortium (MSOAC) Database. These resources have been used to examine disease progression, identify potential biomarkers, and evaluate response to treatment, as well as to answer other scientific questions. The DCC is positioned to become a one-stop shop for large-scale data solutions for scientific research.

Due to years of experience, especially working with biomarker development, we have been able to contribute to efforts that span the biomedical research community. C-Path has been an active participant with leaders from FDA, NIH and FNIH, and the pharmaceutical industry in shaping a draft framework of evidentiary considerations for biomarker qualification.

Going forward, we at C-Path want to put an emphasis on sharing our knowledge and ensuring we are doing our part to educate and to facilitate the use of new tools and methodologies. Through webinars, training events, tutorials, publications, and presentations, we can disseminate these innovative tools and methods to a broad cross-section of healthcare product-development scientists.

I am proud of what we—patients, donors, foundations, the community, board members, pharmaceutical and academic members, and regulatory agency advisors—have been able to accomplish by working collaboratively. I am confident that C-Path's continued efforts in our pre-competitive public-private partnership model—which blends the expertise of translational science, drug development and regulatory review needs, and shared data collaboration—will lead to more successes that ultimately will help those living with disease. Thank you all for being on this journey with us.

Sincerely,
Martha A. Brumfield, PhD

C-PATH'S HISTORY OF SUCCESS

CAMD
CRITICAL PATH INSTITUTE

AD clinical trial database

AD clinical trial simulation tool

EMA qualified AD biomarker

FDA letters of support - AD biomarkers

CFAST

22 therapeutic area user guides, CDISC

CPP
PARKINSON'S CONSORTIUM
CRITICAL PATH INSTITUTE

FDA letter of support - PD biomarker

CPTR

EMA qualified Hollow Fiber System for Tuberculosis

ReSeqTB data platform

PKD
CONSORTIUM
CRITICAL PATH INSTITUTE

Total Kidney Volume (TKV) Imaging biomarker qualified with EMA & FDA

FDA letter of support (TKV)

PKD clinical database

PSTC
CRITICAL PATH INSTITUTE

EMA/FDA/PMDA qualified nonclinical kidney safety biomarkers

FDA & EMA letters of support:
- kidney biomarkers
- skeletal muscle biomarkers

CATALYZING COLLABORATIVE SCIENCE IN 2015-16

C-Path continues to be a leader in the field of translational and regulatory science. Our consortia innovatively address the challenges of taking what is usually considered a research finish line—the output from an academic publication, for example—and using it as the starting line for incorporating that important scientific finding into a tool or methodology that informs the drug development pathway and the route to regulatory acceptance.

CRITICAL PATH FOR PARKINSON'S CONSORTIUM: ENGAGING WITH PATIENT ORGANIZATIONS

C-Path's consortia count as their partners and members not only pharmaceutical companies and academic institutions, but also patient organizations. Our Critical Path for Parkinson's Consortium (CPP), launched in October 2015, exemplifies the importance we place on patient involvement and the patient perspective in the drug development process.

CPP is the result of C-Path's partnership with Parkinson's UK, which provided support and funding for the initiative. Since CPP's inception, the Michael J. Fox Foundation has also become an active member. CPP will develop tools and information to aid in establishing best practices and more efficient protocols for clinical trials in early Parkinson's.
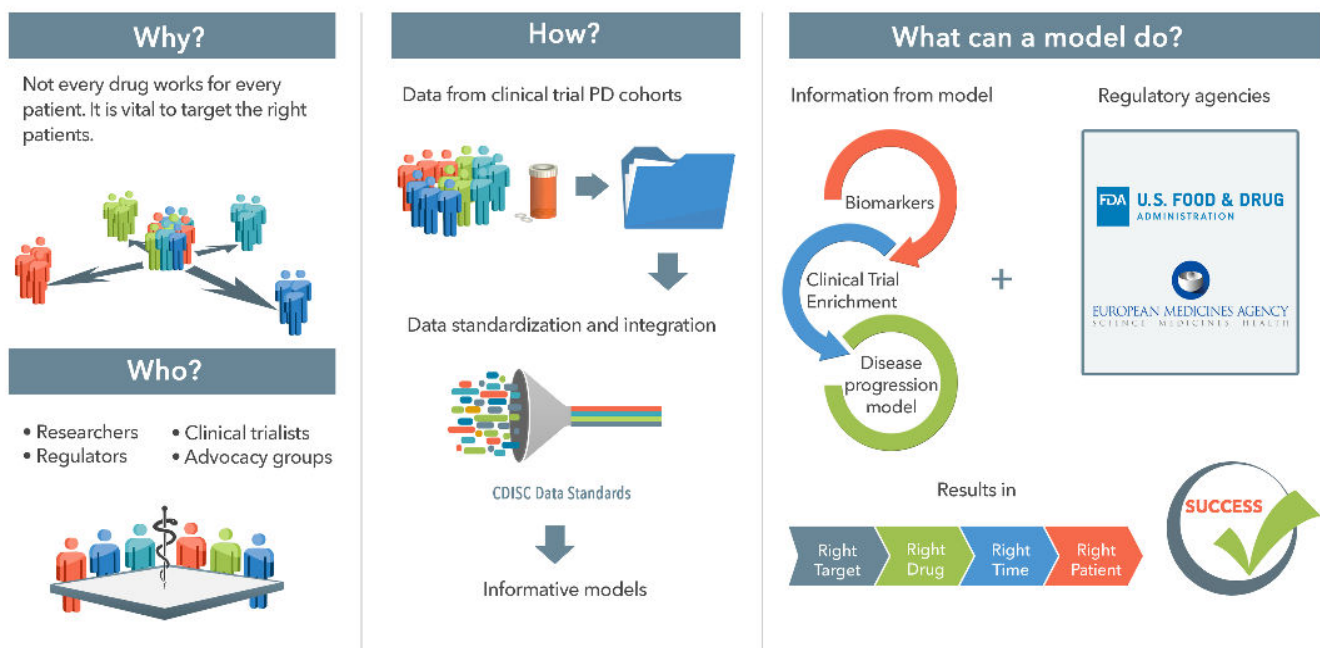
C-PATH MILESTONES

2005 — July
C-Path begins first fiscal year with six full-time employees

2006 — March
Predictive Safety Testing Consortium (PSTC) launched

D-RSC, INC, PTC, AND I-ACT FOR CHILDREN: FILLING GAPS IN PEDIATRIC DRUG DEVELOPMENT

Duchenne muscular dystrophy is a rare fatal genetic disorder that is diagnosed in childhood and primarily affects boys. There are treatments for Duchenne, but currently no cure. In August 2015, in partnership with, and with funding from, Parent Project Muscular Dystrophy (PPMD), C-Path established the Duchenne Regulatory Sciences Consortium (D-RSC). This consortium will develop innovative tools for improving trial design, to help address the challenge to developing new therapies posed by the limited number of patients with Duchenne. A number of small clinical trials are underway for Duchenne treatments, but further work is needed to optimize trial protocols and ensure trials are as effective and informative as possible.

C-Path also established the Pediatric Trials Consortium (PTC), committed to enabling the creation of a sustainable solution that assures the timely and efficient evaluation of innovative drugs, biologics, and devices for children by delivering the regulatory-quality data needed for product labeling. PTC has successfully established a new legal entity: Institute for Advanced Clinial Trials for Children (I-ACT for Children). This independent, global, collaborative organization is building the sustainable international infrastructure needed to plan, initiate, conduct, and complete pediatric studies and clinical trials. Additionally, the standardized protocols, methods, and drug development tools that are the focus of the International Neonatal Consortium (INC), launched in May 2015, will all be employed in the I-ACT for Children network. By optimizing and accelerating biomedical innovation through collaboration with like-minded institutions, I-ACT for Children will advance the development of new medicines and devices for pediatric patients. Caregivers and healthcare providers will have better prescribing information for an increasing number of therapies, helping to extend and enhance the lives of children around the world.

2007   June

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

2008   June

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

2008   November

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

D-RSC, INC, and PTC/I-ACT for Children exemplify the power and potential of the consortium approach to benefit an entire underserved patient population, namely, children and neonates. Through collaboration, consortia can tackle seemingly insurmountable challenges that no one organization could take on alone.

2008   December
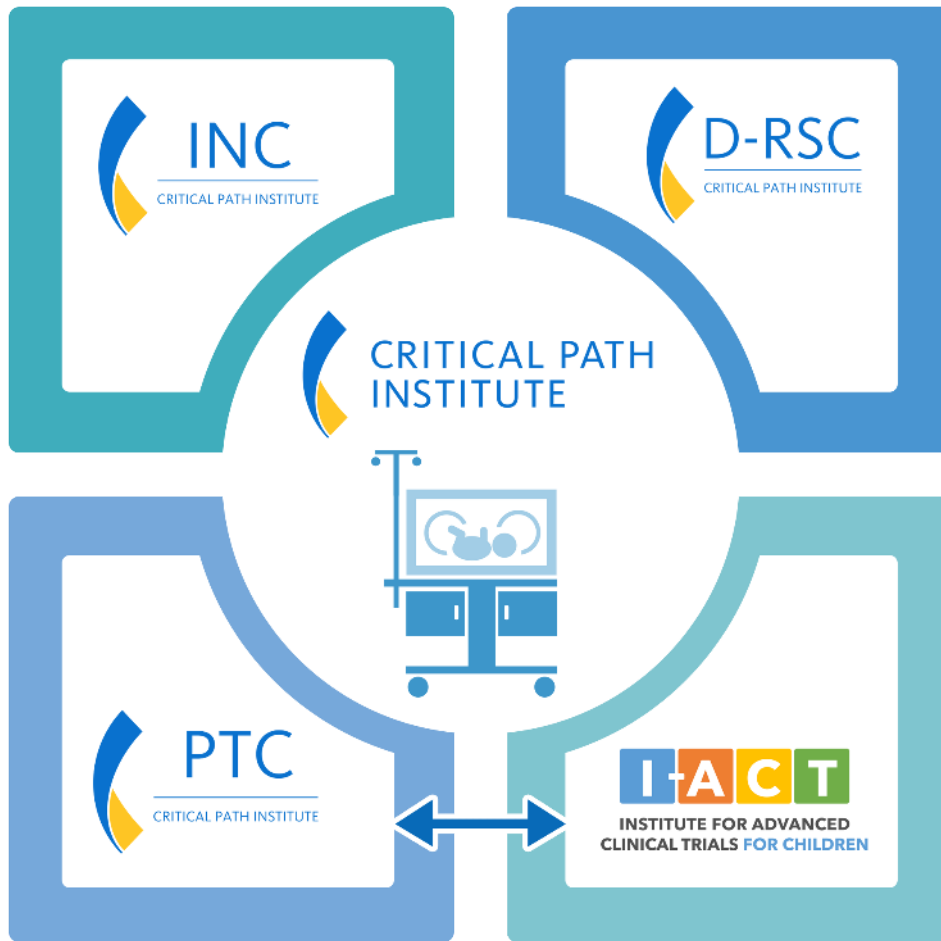
CAMD
CRITICAL PATH INSTITUTE

Coalition Against Major
Diseases (CAMD) launched

2009   March

PRO
CONSORTIUM
CRITICAL PATH INSTITUTE

Patient-Reported Outcome
Consortium (PROC) launched

2010   March

Critical Path to
TB Drug Regimens

Critical Path to TB Drug
Regimens (CPTR) launched

C-PATH'S DATA COLLABORATION CENTER: THE EVOLUTION OF DATA

C-Path's Data Collaboration Center (DCC) was founded in fiscal year 2014-15 to provide large-scale data solutions for scientific research, and fiscal year 2015-16 has seen the center flourish with new projects. DCC's data-sharing platform securely hosts contributed data from 86 clinical trials and 118 nonclinical studies, representing over 50,000 subjects, over 100 million data points, and seven different therapeutic areas, including Alzheimer's, polycystic kidney disease, multiple sclerosis, Parkinson's, and tuberculosis. Information has been shared with the research community and used to develop biomarkers, clinical outcome assessments, and clinical trial simulation tools.

• Data to fight TB

C-Path's TB-Platform for Aggregation of Clinical TB Studies (TB-PACTS), launched in April 2016, was designed to catalyze and accelerate tuberculosis (TB) research by curating and standardizing Phase III TB clinical trial patient-level data from the REMoxTB, RIFAQUIN, and OFLOTUB clinical trials, and making these data available to the research community. C-Path's partners in this program include the Special Programme for Research and Training in Tropical Diseases (TDR), hosted by the World Health Organization (WHO), TB Alliance, and St. George's, University of London. Types of data available include demographic information, concomitant medications information, dose/concentration information, outcomes data, and relevant covariates of interest. Having these data curated, standardized, and easily accessible on one platform helps inform recommendations for policymaking as well as informing the development of novel drug and drug regimens, which would ultimately benefit TB patients.

2010   June

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

2010   June

First-ever biomarker qualification decision announced by PMDA to accept PSTC's new preclinical kidney biomarkers

2010   September

PKD
CONSORTIUM
CRITICAL PATH INSTITUTE

Polycystic Kidney Disease Outcomes Consortium (PKDOC) launched

TB-PACTS is one of three DCC projects focused on informing research and new treatments for tuberculosis. The others are the Relational Sequencing TB Data Platform (ReSeqTB) and Critical Path to TB Drug Regimens (CPTR): CDC Study Data. Part of the Rapid Drug Susceptibility Testing (RDST) working group of the CPTR Initiative, ReSeqTB collects and standardizes global TB patient data from multiple private and public databases, to help identify correlations between Mycobacterium tuberculosis (M.tb) mutations and clinically relevant resistance, aid in the development of new rapid drug susceptibility tests, facilitate international research and collaboration, and, eventually, directly enable sequencing data interpretation for personalized patient care. CPTR's CDC Study Data is an aggregated collection of data from three TB trial datasets contributed by the Centers for Disease Control (CDC) to CPTR for use by qualified TB researchers. The database contains, but is not limited to, data on drug susceptibility, demographics, M.tb diagnostic testing, concomitant medications, adverse events, and TB symptoms and relapse.

• Data for increased understanding of MS

The DCC launched the Multiple Sclerosis Outcome Assessments Consortium (MSOAC) Placebo Database in April 2016. It contains nearly 2,500 patient records from the placebo arms of nine multiple sclerosis (MS) clinical trials and includes records from relapsing-remitting, secondary progressive, and primary progressive forms of MS. The database contains, but is not limited to, data on demographics, medical history, performance outcome measures, clinician-reported outcome measures, patient-reported outcome measures, relapse information, and MS type.

• Greater access to Alzheimer's data

In May 2016, C-Path's Coalition Against Major Diseases (CAMD) provided access to its CAMD Alzheimer's Disease Database through the Global Alzheimer's Association Interactive Network (GAAIN) data portal. GAAIN is an open-access, big data resource including information on more than 320,000 research participants from 21 data partners.

2011   June

Electronic Patient-Reported Outcome Consortium (ePROC) launched

2011   October

C-Path and CDISC announce release of data standards for Alzheimer's disease research, first in a series of therapeutic area common data standards

2011   November

C-Path, CDISC, and FDA host global two-day conference on drug development: "Creating Consensus Science: Tools and Tactics for Next-Gen Drug Development"

With the Alzheimer's Disease Database, DCC and CAMD enrich the GAAIN portal with patient-level, placebo-arm data from 6,500 patients across 24 clinical trials of Alzheimer's disease (AD) and mild cognitive impairment (MCI), including demographic information, APOE4 genotype, concomitant medications, and cognitive scales. The de-identified data, which have been remapped to a common data standard, are openly available to CAMD members, as well as to external qualified researchers who submit, and are approved for, a request for access.

• From standardized data to regulatory success

Autosomal Dominant Polycystic Kidney Disease (ADPKD) is a debilitating genetic disorder for which there is currently no known cure and no available treatments approved for use in the United States. There is critical need for a biomarker that will assess disease progression at an earlier stage, when patients may be more likely to respond to new therapies. This fiscal year, C-Path's Polycystic Kidney Disease Outcomes Consortium (PKDOC) received FDA and EMA qualification for the enrichment biomarker total kidney volume (TKV) in ADPKD. Using standardized, aggregated data from the PKDOC database, the consortium developed a joint model linking baseline TKV with clinical outcomes, thus demonstrating TKV's utility as a prognostic biomarker to select patients for clinical trials of new therapies for ADPKD. The PKDOC database consists of de-identified data from three longitudinal observational patient registries and two NIH-sponsored observational studies, and contains data from a total of 2,498 subjects with ages ranging from 0 to 84 years at study entry.

2012 — January

C-Path receives positive qualification decision from EMA for an imaging biomarker as a qualified measure to select patients with early-stage cognitive impairment for enriching Alzheimer's disease clinical trials

2012 — June

CFAST

Coalition For Accelerating Standards & Therapies (CFAST) launched

2012 — December

CRITICAL PATH INSTITUTE National Multiple Sclerosis Society

Multiple Sclerosis Outcome Assessments Consortium (MSOAC) launched

OUR MILESTONES ARE THE FOUNDATION FOR NEW GROWTH

Learning and innovation go hand in hand. For C-Path, our past accomplishments are not just milestones, they are markers by which to achieve and measure future progress. From its inception over a decade ago, C-Path has shaped the evolution of public-private partnerships and expanded the boundaries of precompetitive space in which disparate organizations—regulators, patient groups, drug companies, universities—can collaborate and share data to accelerate the pace of drug development.

• Expanding possibilities

Not long ago, a public-private partnership in the field of drug development was a novel but bewildering proposition. Public and private institutions often viewed each other as competitors only, while regulatory authorities, desiring to engage in such a collaboration, were uncertain how to do so. Currently, dozens of public-private partnerships, covering a variety of subjects and objectives, are thriving. What's next? Potentially, an exponential increase in collaboration that leads to a pipeline of compounds hitting the right target, biomarkers and other tools to inform proof of concept (POC), dose selection, and trial enrichment, and, ultimately, new treatments that are safe and effective.

In order for such partnerships to flourish, stakeholders need a safe, neutral, and precompetitive arena in which to collaborate. The successes of C-Path's consortia have demonstrated that sharing information and data once considered proprietary or confidential is a path to greater accomplishment for all members. Companies are increasingly willing to share information, lessons learned, and even patient-level data; more academic institutions are participating in this neutral space, and all parties are learning from one another's past missteps.

2013　March

First joint C-Path and Innovative Medicines Initiative (IMI) meeting held in Brussels: "Collaborating for Cures: Leveraging Global Public-Private Partnerships to Accelerate Medical Product Development"

2014　October

FDA issues first-of-its-kind Biomarker Letter of Support for two kidney safety biomarkers identified and evaluated by PSTC's nephrotoxicity working group

2014　December

Second Annual Meeting with IMI

By continuing to expand the boundaries of precompetitive space, trial developers can avoid repeating failed trial designs, and as a result the number of failed trials could be substantially reduced. FDA and other regulatory agencies could extract lessons learned across multiple programs and appropriately share this information. Taken to the next level, this neutral space enables consensus science organizations, such as C-Path, to collaborate with each other.

• Next-level collaboration

The Predictive Safety Testing Consortium (PSTC) and the Critical Path to TB Drug Regimens (CPTR) Initiative have provided successful examples of such cross-organization collaboration.

"The IMI SAFE-T collaboration has resulted in several Letters of Support from both FDA and EMA focused on the implementation of exploratory safety biomarkers in clinical trials supporting drug development. PSTC played a key strategic and operational role in each of the regulatory interactions. We have also significantly advanced qualification of kidney safety biomarkers, which is the primary focus of the FNIH BC collaboration. The prospective studies are nearly complete and we are aligning the final details of the qualification with both FDA and EMA."

John Michael Sauer, PSTC's executive director

Over the past year, PSTC has continued key collaborations with both the Foundation for the National Institutes of Health's (FNIH's) Biomarkers Consortium (BC) to study drug-induced kidney injury, and with Innovative Medicines Initiative (IMI) SAFE-T (Safer and Faster Evidence-based Translation) Consortium to research biomarkers related to drug-induced injury to the liver, kidney, and vascular system.

2015 January

EMA issues first-of-its-kind Biomarker Letter of Support for two kidney safety biomarkers identified and evaluated by PSTC's nephrotoxicity working group

2015 February

EMA qualifies "Hollow Fiber System" for anti-tuberculosis drug development
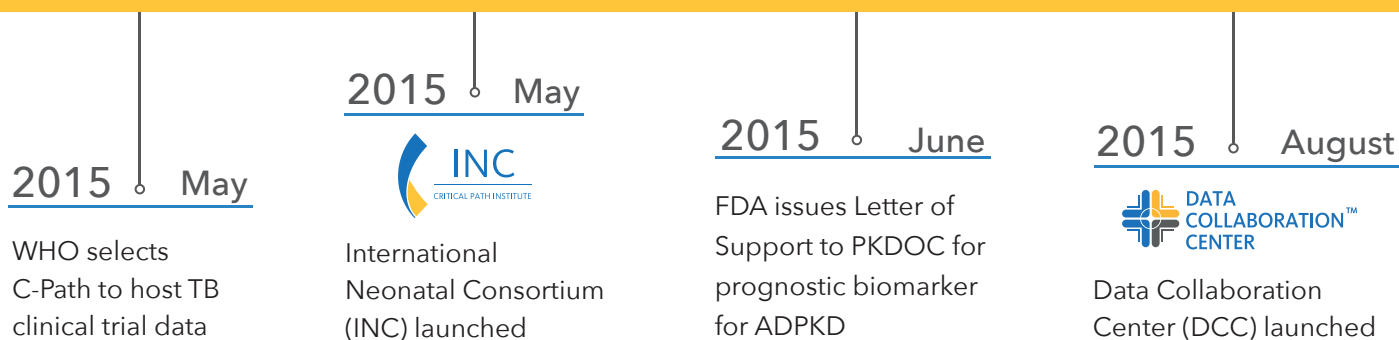
2015 April

FDA issues Letters of Support to CAMD for Alzheimer's and Parkinson's diseases

> "The development of novel treatment regimens and diagnostics needed to end TB requires the sharing of data and knowledge from across the globe. C-Path is proud to partner with these global organizations to advance this mission and accelerate the end of TB."
>
> *Debra Hanna, CPTR's executive director*

C-Path's CPTR, FIND, WHO, New Diagnostics Working Group, CDC, and NIAID worked collaboratively to launch ReSeqTB as a "one-stop" source for curated genetic data to infer drug resistance. Similarly, TB-PACTS, made available to researchers across the globe in April 2016, is hosted by C-Path, in partnership with the WHO Special Programme for Research and Training in Tropical Diseases (TDR), the TB Alliance, and St. George's, University of London.

An important ongoing initiative in which C-Path is a key player builds on these successes and illustrates potential future directions of consensus science. This particular project is a broad-based effort to create alignment among government agencies, pharmaceutical companies, patient advocacy groups, and nonprofit organizations in determining the levels of evidence required to qualify biomarkers for use in drug development. On August 21, 2015, C-Path, the University of Maryland CERSI, and FDA co-sponsored a symposium titled "Evidentiary Considerations for Integration of Biomarkers in Drug Development" at the University of Maryland School of Pharmacy. The objective of the symposium was to begin to define, and ultimately solidify, the scientific and regulatory expectations for the qualification of biomarkers. In April 2016, FDA's Center for Drug Evaluation and Research (CDER), in co-sponsorship with the FNIH Biomarkers Consortium, developed the idea further with the workshop "Developing an Evidentiary Criteria Framework for Safety Biomarkers Qualification," the output of which was a proposed framework that members of C-Path were instrumental in drafting.

2015 May

WHO selects
C-Path to host TB
clinical trial data

2015 May

INC
CRITICAL PATH INSTITUTE

International
Neonatal Consortium
(INC) launched

2015 June

FDA issues Letter of
Support to PKDOC for
prognostic biomarker
for ADPKD

2015 August

DATA
COLLABORATION™
CENTER

Data Collaboration
Center (DCC) launched

FINANCIAL STATEMENT

In 2015, C-Path celebrated its 10th anniversary, marking a decade of achievements in accelerating the path to a healthier world. Since C-Path is not an organization that rests on past achievements, however, our first decade has motivated us to set the bar even higher for our second decade of leading world-class teams to share data, knowledge, and expertise in the drug development community. Because of our accomplishments, more organizations in varied disease areas are choosing to work with C-Path to help expedite the medical product development process.

Since our last annual report, C-Path launched three new consortia, going from nine to twelve major programs in operation. C-Path established the Pediatric Trials Consortium (PTC) with the goal of forming a new, independent nonprofit organization that would develop a network of clinical trial sites to conduct trials for new medical products for children.

Another pediatric-focused initiative C-Path launched was the Duchenne Regulatory Sciences Consortium (D-RSC). In partnership with Parent Project Muscular Dystrophy (PPMD) the D-RSC will apply C-Path's proven consensus science model to the battle against Duchenne Muscular Dystrophy.

In October 2015, C-Path launched the Critical Path for Parkinson's Consortium (CPP), formed with Parkinson's UK, one of the world's largest charity funders of Parkinson's research. Data from past Parkinson's trials will inform the design of new clinical trials focused on early-stage Parkinson's. The Michael J. Fox Foundation for Parkinson's research (MJFF), the Parkinson's Disease Foundation, the Davis Phinney Foundation, Cure Parkinson's Trust, and seven of the world's largest pharmaceutical companies have also signed on to the initiative.

Last fiscal year, Arizona's Flinn Foundation awarded C-Path a $1 million dollar grant over three years to build the Data Collaboration Center (DCC). This fiscal year, the DCC has securely hosted data from 86 clinical trials and 118 nonclinical studies, and is poised to become a valuable service to provide to other organizations looking for secure data curation and management. C-Path was also awarded a three-year grant renewal from the Bill & Melinda Gates Foundation to continue to advance the science and regulatory pathways that lead to more effective and shorter-duration drug regimens for TB through the

2015 — August

D-RSC
CRITICAL PATH INSTITUTE

Duchenne Regulatory Science Consortium (D-RSC) launched

2015 — August

FDA issues Qualification Decision to PKDOC for TKV as a prognostic biomarker for ADPKD

2015 — October

PTC
CRITICAL PATH INSTITUTE

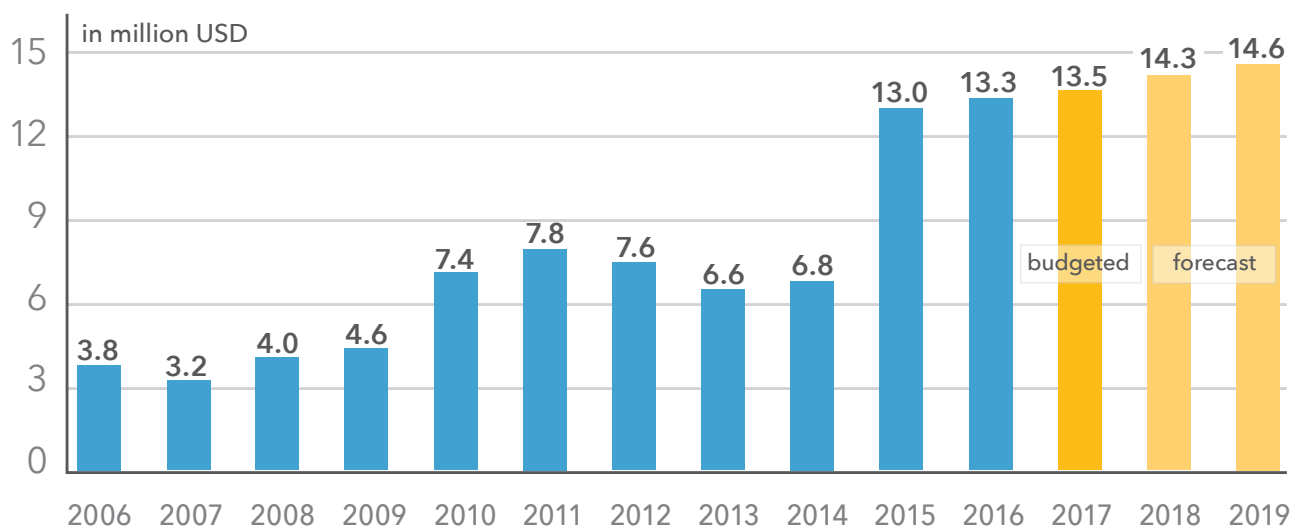Pediatric Trials Consortium (PTC) launched

Critical Path to TB Drug Regimens initiative.

In October 2015, The FDA awarded several grants to C-Path, to work with the Clinical Data Interchange Standards Consortium (CDISC), to fund development of additional Therapeutic Area (TA) standards through their joint initiative, the Coalition for Accelerating Standards and Therapies (CFAST). CDISC and C-Path have partnered to date on the development and implementation of numerous CDISC therapeutic area standards, including those for Alzheimer's disease, Parkinson's disease,

tuberculosis, polycystic kidney disease, multiple sclerosis, influenza, virology, traumatic brain injury, and more. The recent grants will provide funding necessary to develop new standards.

At the close of the 2015-16 fiscal year, C-Path has several additional new programs in development that will launch in the coming year. C-Path remains committed to continued innovation in leading efforts to improve the development and regulatory review of new therapies to improve the lives of people around the world.

TOTAL REVENUE BY FISCAL YEAR, 2006-2019

in million USD

Year	Revenue
2006	3.8
2007	3.2
2008	4.0
2009	4.6
2010	7.4
2011	7.8
2012	7.6
2013	6.6
2014	6.8
2015	13.0
2016	13.3
2017	13.5 (budgeted)
2018	14.3 (forecast)
2019	14.6 (forecast)

2015 — October
CPP
CRITICAL PATH INSTITUTE

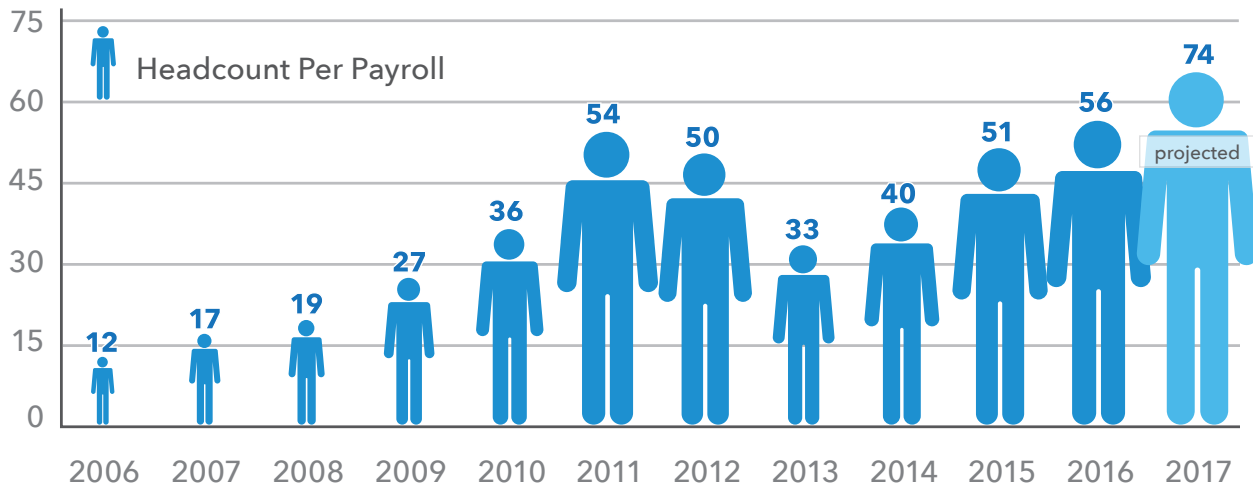Critical Path for Parkinson's Consortium (CPP) launched

2015 — November

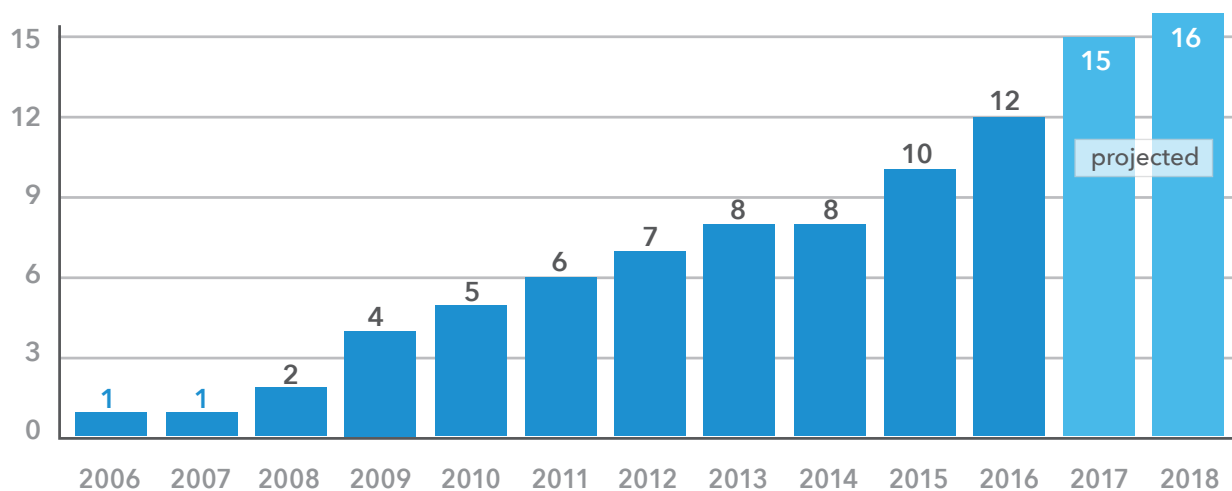FDA awards grants to CDISC and C-Path to continue CFAST initiative

2015 — November

EMA issues positive qualification opinion to PKDOC for TKV as a prognostic biomarker for ADPKD

HUMAN RESOURCES EVOLUTION, 2006-2017

Headcount Per Payroll

Year	2006	2007	2008	2009	2010	2011	2012	2013	2014	2015	2016	2017
Headcount	12	17	19	27	36	54	50	33	40	51	56	74 (projected)

CONSORTIA GROWTH BY FISCAL YEAR, 2006-2018

Year	2006	2007	2008	2009	2010	2011	2012	2013	2014	2015	2016	2017	2018
Consortia	1	1	2	4	5	6	7	8	8	10	12	15 (projected)	16 (projected)

2016 — April

MSOAC makes available new database for sharing MS clinical trial data

2016 — April

TB-PACTS
PLATFORM FOR AGGREGATION OF CLINICAL TB STUDIES
CRITICAL PATH INSTITUTE

TB-Platform for Aggregation of Clinical TB Studies (TB-PACTS) launched

2016 — May

CAMD becomes partner in Global Alzheimer's Association Interactive Network (GAAIN) data portal

FISCAL YEAR 2016

ASSETS

Cash and Cash Equivalents	$	12,490,901
Certificates of Deposit	$	4,248,265
Accounts Receivable	$	1,350,930
Property and Equipment, Net	$	86,511
Other	$	58,013
TOTAL ASSETS	**$**	**18,234,620**

LIABILITIES AND NET ASSETS

Liabilities

Accounts Payable	$	420,508
Accrued Expenses	$	175,938
Deferred Revenue*	$	12,149,997
Deferred Rent	$	36,186
Total Liabilities	**$**	**12,782,629**

Net Assets

|		
Undesignated	$	2,644,740
Board Designated**	$	2,720,740
Property and Equipment	$	86,511
Total Net Assets	**$**	**5,451,991**
TOTAL LIABILITIES AND NET ASSETS	**$**	**18,234,620**

 * Pre-awarded funds received for grants
** Consortia fees managed by C-Path to support consortia activities

C-Path 2016 Fiscal Year Revenue

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

$1,359,860

$5,204,621

$4,912,824

$669,488

$3,200,284
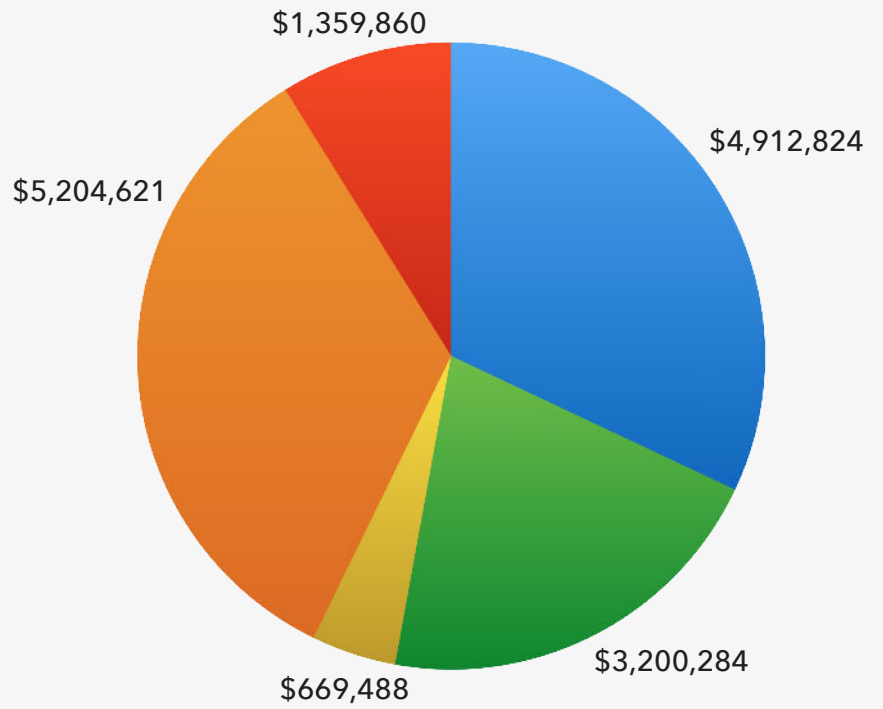
C-Path 2016 Fiscal Year Expenses

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

$471,435

$1,392,380

$3,419,983

$6,010,065

$448,272

$2,937,382

C-PATH INITIATIVES

Patient Perspective — PRO CONSORTIUM · CRITICAL PATH INSTITUTE, ePRO CONSORTIUM · CRITICAL PATH INSTITUTE

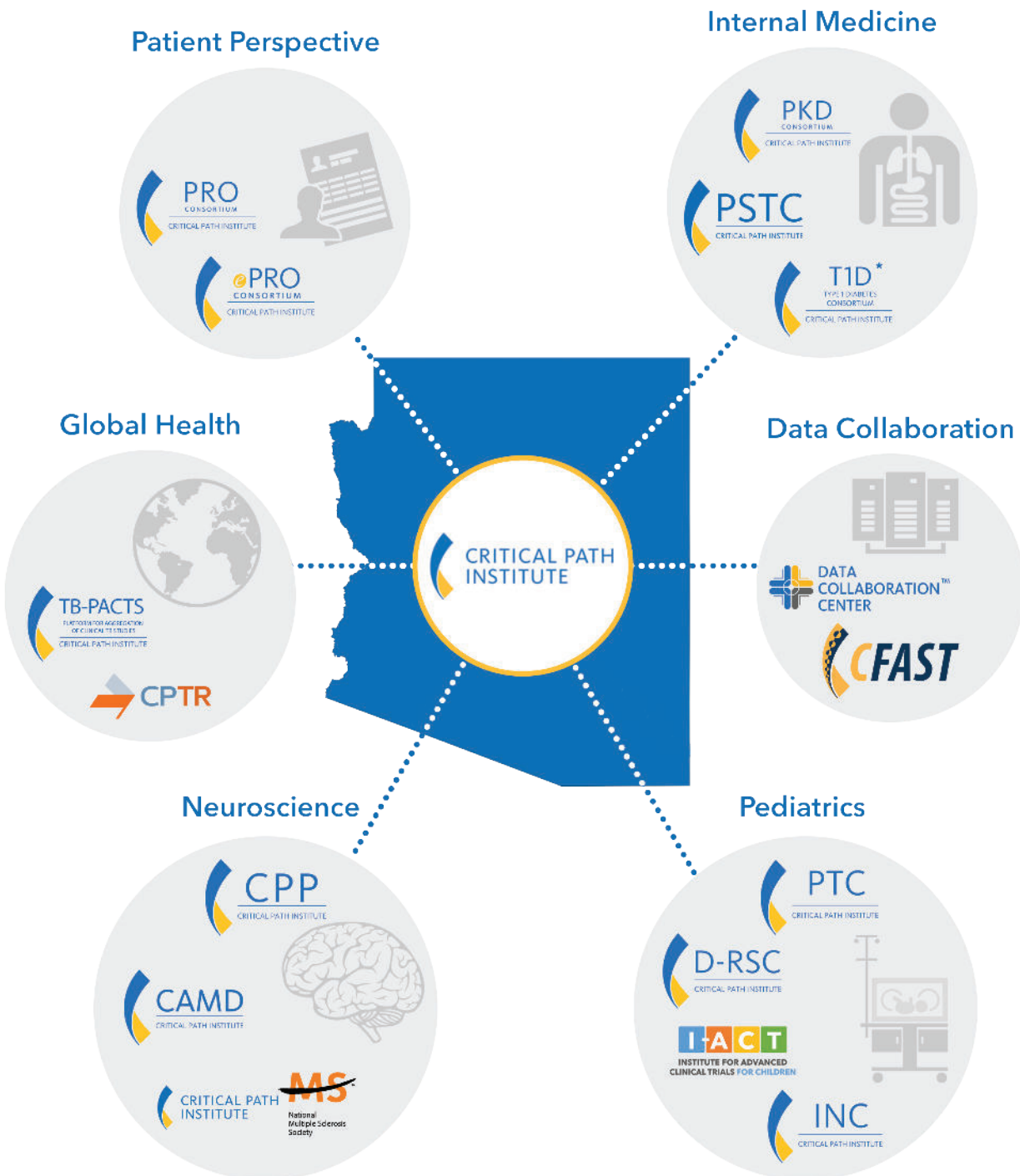Internal Medicine — PKD CONSORTIUM · CRITICAL PATH INSTITUTE, PSTC · CRITICAL PATH INSTITUTE, T1D* TYPE 1 DIABETES CONSORTIUM · CRITICAL PATH INSTITUTE

Global Health — TB-PACTS · CRITICAL PATH INSTITUTE, CPTR

Data Collaboration — DATA COLLABORATION CENTER, CFAST

Neuroscience — CPP · CRITICAL PATH INSTITUTE, CAMD · CRITICAL PATH INSTITUTE, CRITICAL PATH INSTITUTE · MS National Multiple Sclerosis Society

Pediatrics — PTC · CRITICAL PATH INSTITUTE, D-RSC · CRITICAL PATH INSTITUTE, I-ACT INSTITUTE FOR ADVANCED CLINICAL TRIALS FOR CHILDREN, INC · CRITICAL PATH INSTITUTE

CRITICAL PATH INSTITUTE

* This initiative was launched after the close of the fiscal year.

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), 22 CDISC therapeutic area standards have been published, and C-Path has led or supported the work on 13 of these projects.

Launched in FY 2015-16

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 their brain have died. Current research, however, indicates that the processes leading to dopamine deficit start much earlier (decades), making it a pressing need to increase the understanding 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.

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

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.

D-RSC
CRITICAL PATH INSTITUTE

Launched in FY 2015-16

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 evolution of 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, progressive 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.

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.

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 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 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.
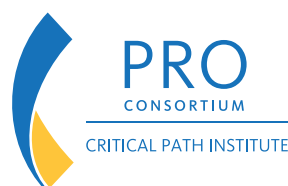
The Patient-Reported Outcome (PRO) Consortium brings together drug developers, measurement scientists, patients, clinicians, and regulators to collaborate on effectively incorporating the voice of the patient into the drug development process. Its primary goal is to obtain regulatory qualification of patient-completed questionnaires for use in clinical trials where PRO endpoints can, and should, be used to evaluate patient-focused treatment benefit.

PSTC
CRITICAL PATH INSTITUTE

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.

PTC
CRITICAL PATH INSTITUTE

Launched in FY 2015-16

The aim of the Pediatric Trials Consortium (PTC) is to facilitate the development of innovative and quality medicines according to the highest ethical and scientific standards, to help extend and enhance the lives of infants, children, and adolescents. The PTC has enabled C-Path to launch a new, independent legal entity (the Institute for Advanced Clinical Trials for Children [I-ACT for Children]) that can provide 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, industry, and nonprofits). Working together, PTC and I-ACT for Children will accelerate the availability of innovative, safe, and effective medicines for children, improving health and wellness globally.

TB-PACTS
PLATFORM FOR AGGREGATION OF CLINICAL TB STUDIES
CRITICAL PATH INSTITUTE
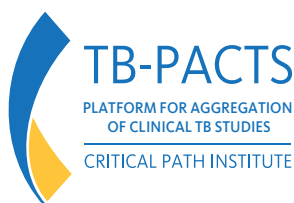
Launched in FY 2015-16

The TB-Platform for Aggregation of Clinical TB Studies (TB-PACTS) is designed to catalyze and accelerate tuberculosis (TB) research by curating and standardizing Phase III TB clinical trial data and making this data publicly available to qualified researchers. Researches can access and analyze data in aggregate, or filter and view individual patient-level data. The TB–PACTS data platform will catalogue contemporary TB clinical trial data sets. Approved researchers can access patient-level data from the REMoxTB, RIFAQUIN, and OFLOTUB clinical trials. Additional trial data is expected to be made available in the future. This initiative represents a collaborative partnership among the Special Programme for Research and Training in Tropical Diseases (TDR), the TB Alliance, St. George's University of London, and C-Path.

CONSORTIA MEMBERS

CONSORTIA PARTNERS

alzheimer's association

Alzheimer's Drug Discovery Foundation

Alzheimer's Research UK

BILL & MELINDA GATES foundation

Bracket

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CDISC

CoINN
Council of International Neonatal Nurses

C-R-F HEALTH
SIMPLY RELIABLE

EMORY UNIVERSITY
Center for Translational Social Neuroscience

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FIND

FLINN FOUNDATION

FNIH
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FOUNDATION
Fighting for Premature Babies

Health Canada   Santé Canada

imi
innovative medicines initiative

march of dimes

MAYO CLINIC

National Association of Neonatal Nurses

National Institute of Allergy and Infectious Diseases

Eunice Kennedy Shriver
NICHD
National Institute of Child Health & Human Development

NIDDK

NIH
National Institute of Neurological Disorders and Stroke

NIH
National Institute on Aging

NIH
National Institutes of Health

MS
National Multiple Sclerosis Society

Parent Project Muscular Dystrophy
LEADING THE FIGHT TO END DUCHENNE

PARKINSON'S UK
CHANGE ATTITUDES. FIND A CURE. JOIN US.

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SCIENCE foundation ARIZONA
Innovation at Work

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

BOARD OF DIRECTORS

In July 2016, C-Path announced the appointment of Dr. Timothy R. Franson as Chair of C-Path's Board of Directors. Dr. Franson replaced Peter B. Corr, MD, PhD, who continues to serve as Senior Advisor to the Board. Dr. Franson brings extensive clinical and regulatory expertise in all pre- and post-approval phases of pharmaceutical development.

"This is a very exciting time at the Critical Path Institute. I look forward to building on Dr. Corr's outstanding work guiding one of the most progressive nonprofit organizations leading collaborations in regulatory science. I'm honored the Board has entrusted me with this responsibility."

—Timothy R. Franson, MD

D. Craig Brater, MD

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

Peter B. Corr, MD, PhD, Senior Advisor

Co-founder and General Partner of Auven Therapeutics Management LLLP

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Timothy R. Franson, MD, Chairman

Chief Medical Officer, YourEncore

The Honorable James C. Greenwood

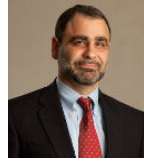President and CEO of the Biotechnology Industry Organization (BIO)

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