



CRITICAL PATH
INSTITUTE



Accelerating the path
to a healthier world



2019 ANNUAL REPORT

for fiscal year ending
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Accelerating the path to a healthier world



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.

Values

As an independent and trusted partner, we value leadership, innovation, integrity, transparency, efficiency and collaboration.

From the CEO



Dear Friends and Supporters,

I'm excited and honored to reflect on the past year's accomplishments for the first time as CEO of C-Path.

It's amazing how easy it is to get caught up in different projects, individual successes, the various challenges and obstacles, and the wins that come out of our efforts every day, as we push the limits of science in our move toward a healthier world. But when you take it all in together, when you look at the sum of all the parts, it impresses upon you the magnitude of accomplishment and of the progress being made toward our goal.

C-Path is making meaningful advances every day in medical innovation and regulatory science – breakthroughs that make a difference in the lives of patients in clinical trials, in the pharmacy, at the doctor's office and at home. Each and every success widens the pathway for cutting-edge science and streamlined regulatory processes to speed the development of safe and effective treatments for debilitating diseases around the globe.

In looking back on the past fiscal year, it's with a great sense of pride and accomplishment that I want to highlight some of the most exciting developments that paint a solid picture of what we do at C-Path every day. We're not just working to reach that next level, the next success, the next breakthrough or the next direction. We're engaged every day in creating a healthier world. We're committed to putting everything we've got into the all-important work that will get us to our goal. And we're motivated to do what it takes to see it through.

Some of our most noteworthy successes over the past year are the regulatory milestones we achieved. These include a qualification issued by the U.S. Food and Drug Administration (FDA) for a clinical safety biomarker for drug-induced kidney injury that will help facilitate drug development and improve early-stage clinical trials, as well as qualification of two new clinical outcome assessment tools: the *Asthma Daytime Symptom Diary* and the *Asthma Nighttime Symptom Diary*, which will help provide enhanced consistency and comparability in the evaluation of the patient-focused clinical benefit from new asthma drugs.

In addition, the FDA accepted C-Path's Type 1 Diabetes Consortium's Biomarker initiative project into the Center for Drug Evaluation and Research Biomarker Qualification Program, as well as the PRO Consortium's activity monitor-based endpoint measure for chronic heart failure into the COA Qualification Program.

Meanwhile, the European Medicines Agency issued a positive qualification opinion on an imaging test as a tool to enrich clinical trials for Parkinson's disease.

In August 2018, C-Path's Predictive Safety Testing Consortium (PSTC) and the Foundation for the National Institutes of Health (FNIH) Biomarkers Consortium (BC) received the first-ever qualification of a clinical safety biomarker awarded by the U.S. Food and Drug Administration (FDA). The qualification marks a major milestone that will improve the detection of drug-induced kidney injury in early phase drug development.

Other highlights over the 2018-2019 fiscal year include the launch of the Friedreich's Ataxia Integrated Clinical Database, a new platform that will help researchers understand disease progression and potentially help develop novel clinical trial designs in Friedreich's ataxia. We also partnered with the University of Arizona to launch a new online Graduate Certificate in Regulatory Science program that provides students with the knowledge and tools to accelerate medical product development.

Also over this past year, C-Path's first clinical consortium, previously known as the Coalition Against Major Diseases (CAMD), celebrated its 10-year anniversary by rebranding itself as Critical Path for Alzheimer's Disease (CPAD) to better reflect its more focused approach on speeding the drug development process for Alzheimer's disease.

Included among our most notable accomplishments was the opening of Critical Path Institute, Ltd., our European headquarters in Dublin, Ireland. Having this additional base of operations enables C-Path to expand upon its collaborations, increase our impact, and advance our mission of creating a healthier world.

Critically important to our work are our partners – the collaborations we build to most efficiently and effectively achieve common goals. In the last year, C-Path's PSTC and SIGNATOPE GmbH joined together to analyze and qualify promising protein safety biomarkers in mice, rats, dogs, monkeys and humans. The Huntington's Disease Regulatory Science Consortium, along with the Clinical Data Interchange Standards Consortium or CDISC, announced the open availability of a newly developed Huntington's Disease Therapeutic Area User Guide. C-Path and CDISC also announced the release of two Therapeutic Area Standards – one that specifies how to structure commonly collected data and outcome measurements in clinical trials for HIV, and another that describes how to use CDISC standards to represent data in research studies pertaining to clostridium difficile associated diarrhea.

C-Path consortia in 2018 and 2019 have launched or advanced projects with, among others, TGen North – the Pathogen and Microbiome Division of the

Translational Genomics Research Institute and Japan’s Pharmaceuticals and Medical Devices Agency.

We are grateful to have been awarded grants from the Doris Duke Charitable Foundation to advance therapies for sickle cell disease, and from the Flinn Foundation to pilot a program to respond to antimicrobial resistance. Our partnerships with these organizations are a reflection of the confidence they have in our mission and in our commitment to success.

Finally, I want to offer my sincere thanks to former C-Path President and CEO Martha Brumfield, PhD, who turned the reins over to me this past year after leading the organization since 2013. Under Martha’s leadership, C-Path’s annual revenue and staff size doubled. The organization established a Data Collaboration Center and increased the investment in unique human capital in quantitative medicine and expanded its profile in Europe. I am appreciative of Martha’s guidance and counsel as we worked through the transition in leadership, and it’s my honor to push forward and build upon the successes C-Path enjoyed under her watch.

We still have a long way to go, and it will take a lot of work to get there – but I am eager to press on. With the strength of the patient community, and with the support of our partners and donors, the backing of foundations and regulatory agency advisors, collaborations with biotech and pharmaceutical companies and other key industry stakeholders, we are moving in the right direction. We are on the right path, and we’re making strides toward a healthier tomorrow.

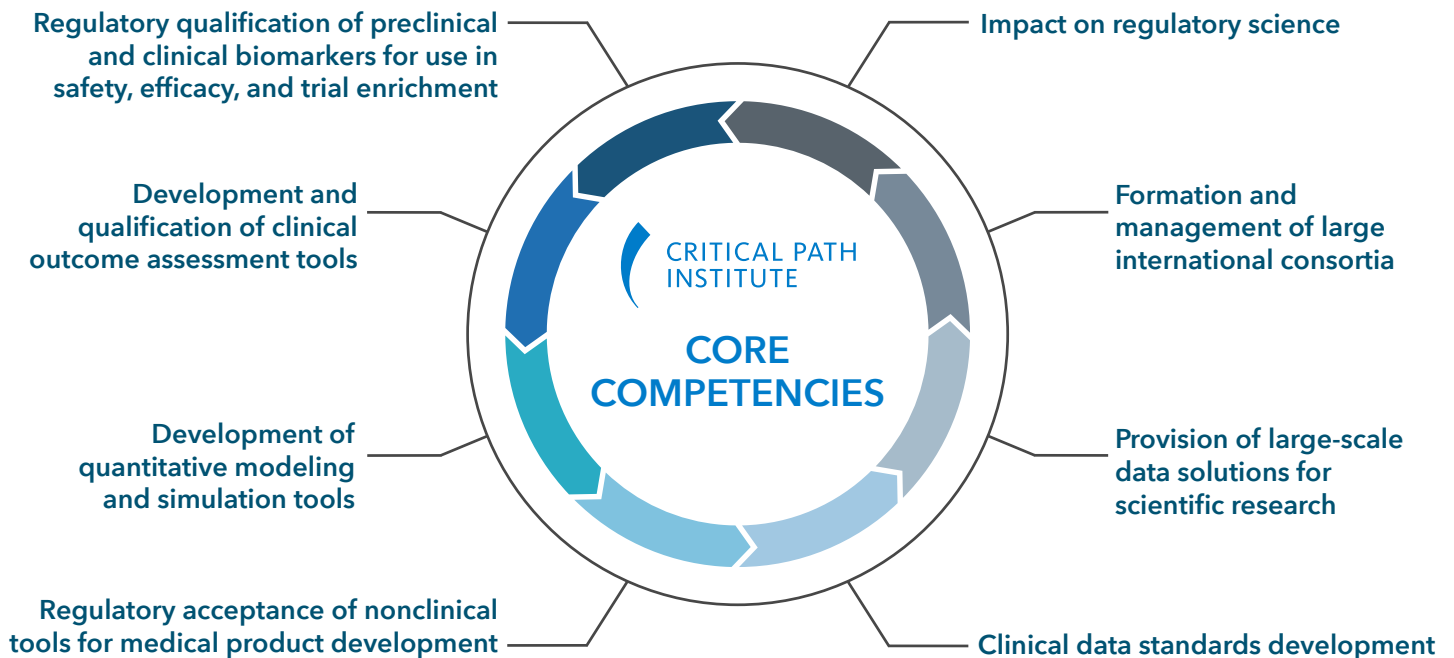
Sincerely,

Joseph Scheeren, PharmD



Core Competencies

Critical Path Institute (C-Path) serves as a neutral third party to enable multiple stakeholders across the spectrum of medical product development to work together in a pre-competitive consortium model in order to drive innovative tools and methods which help to de-risk decision making in the development and regulatory review process. This makes C-Path a unique source of expertise working to achieve many of the objectives outlined in the U.S. Food and Drug Administration's (FDA) report "Innovation / Stagnation – Challenge and Opportunity on the Critical Path to New Medical Products."



1 } Regulatory qualification of preclinical and clinical biomarkers and novel methodologies for safety, efficacy, and trial enrichment

C-Path leads the way in regulatory qualification of biomarkers. C-Path was the first organization to qualify biomarkers with the FDA, European Medicines Agency (EMA), and Japan's Pharmaceutical and Medical Devices Agency (PMDA). The following C-Path consortia have successfully qualified biomarkers with regulatory agencies:

- The Predictive Safety Testing Consortium (PSTC): FDA, EMA and PMDA qualified six nonclinical urinary biomarkers for the detection of acute drug-induced nephrotoxicity in rodents and FDA qualified a composite measure of six clinical urinary biomarkers, when used in conjunction with traditional measures of kidney function, for the detection of acute drug-induced nephrotoxicity during Phase 1 clinical trials.
- The Critical Path for Alzheimer’s Disease (CPAD): EMA qualified the baseline measurement of low hippocampal volume (atrophy) by MRI to predict whether such patients are likely to evolve to Alzheimer’s disease type dementia during the course of an Alzheimer’s disease clinical trial. More recently, EMA gave the first-ever Letter of Support (LOS) for the pre-dementia disease progression model and clinical trial simulator that incorporates hippocampal volume.
- The Polycystic Kidney Disease (PKD) Outcomes Consortium: FDA and EMA qualified Total Kidney Volume (TKV) as a prognostic biomarker for enrichment of clinical trials in Autosomal Dominant Polycystic Kidney Disease (PKD) based on a quantitative disease and biomarker progression model. Subsequently, through direct interactions with the Division of Cardiology and Nephrology, TKV was designated as a reasonably likely surrogate endpoint for PKD trials.

2 } **Development and qualification of clinical outcome assessment tools**

By working with multiple stakeholders (e.g., patients, regulators, clinical advisors, industry scientists, measurement experts) in the United States and around the globe, C-Path is a leader in the development and qualification of patient-reported outcome measures and other clinical outcome assessment (COA) tools. C-Path’s Patient-Reported Outcome (PRO) Consortium provides a collaborative framework for the qualification of COA tools that can be used to support medical product labeling claims. The Electronic Patient-Reported Outcome (ePRO) Consortium works closely with the PRO Consortium to make the COA tools emerging from its therapeutic area working groups available in multiple modes of data collection. While other COA tools are in development or other stages of qualification, the following PRO measures have obtained FDA qualification:

- *Symptoms of Major Depressive Disorder Scale (SMDDDS)*
- *Non-Small Cell Lung Cancer Symptom Assessment Questionnaire (NSCLC-SAQ)*
- *Asthma Daytime Symptom Diary (ADSD) and the Asthma Nighttime Symptom Diary (ANSD)*

3 } **Development of quantitative modeling and simulation tools**

The discipline of modeling and simulation has been a key area of expertise for C-Path since its inception. The mission of C-Path’s Quantitative Medicine (QuantMed) Program is to transform drug development through methodological innovation. The QuantMed Program also works to drive innovation in MIDD through partnerships with leading groups and organizations in the field, and through collaborations with societies such as the International Society of Pharmacometrics (ISoP) and American Society for Clinical Pharmacology and Therapeutics (ASCPT). The development of

these tools is possible thanks to the standardized integration of clinical and data analytical knowledge to solve bottlenecks in the drug development process. C-Path collaborated with scientists in industry to develop several quantitative drug development platforms, most of which have received regulatory endorsements from FDA and EMA:

- The clinical trial simulation tool for mild-to-moderate Alzheimer's disease (first-ever quantitative drug development tool to be endorsed by FDA and qualified by EMA)
- The pre-dementia disease progression model, which incorporates HV as an enrichment biomarker (first-ever quantitative drug development tool to receive a Letter of Support from EMA)
- Several quantitative drug development tools to accelerate combination drug development against TB
- Model-based qualification of total kidney volume as an enrichment biomarker for trials in polycystic kidney disease
- The clinical trial enrichment tool for early-motor Parkinson's disease utilizing dopamine transporter imaging as an enrichment biomarker

Currently, the QuantMed Program is developing clinical trial simulation tools for Parkinson's disease, Duchenne muscular dystrophy, pre-dementia in Alzheimer's disease and Huntington's disease. Additionally, model-based biomarker qualification efforts are underway for trial optimization in type 1 diabetes prevention studies and kidney transplant trials.

4 } Regulatory acceptance of nonclinical tools for medical product development

In addition to the competencies described above, C-Path also has capability in developing and providing evidence packages to regulators for *in vitro* tools and other atypical (or non-traditional) tools. *In vitro* tools are critically important in earlier phases of drug development to facilitate decision making regarding compound, dose, combination regimen selection and more.

In vitro tools can also be utilized in clinical diagnosis and pathogen detection. Surveillance of pathogen resistance is another application with which C-Path has expertise.

- C-Path's Critical Path to TB Drug Regimens Initiative (CPTR): EMA qualified the *in vitro* Hollow Fiber System of Tuberculosis to inform selection of dose and treatment regimen, including combination of two or more anti-Mycobacterium tuberculosis drugs, to maximize bactericidal effects and minimize emergence of drug resistance.
- C-Path's Critical Path to TB Drug Regimens (CPTR) has developed a Relational Sequencing TB Knowledgebase (ReSeqTB) as a knowledge platform integrating genotypic, phenotypic and clinical data to aid in surveillance of TB drug resistance, support clinical decision making for TB patient management, inform development of new diagnostics and treatment regimens. The inherent value of this platform was further developed with a new instance of this platform being developed for the World Health Organization to support its Global TB Surveillance program. The delivery of the tool occurred in April 2019.

5 } **Clinical data standards development**

C-Path's core competency in clinical data standards development enables the effective aggregation of large datasets and helps expedite the regulatory review process. C-Path has a close and long-standing relationship with the Clinical Data Interchange Standards Consortium (CDISC). CDISC is a recognized standards-setting organization, and CDISC standards are required for new NDA and IND submissions to the FDA. C-Path worked with CDISC to develop the first therapeutic area data standards and has led the efforts in many others since then including Alzheimer's disease, Parkinson's disease, polycystic kidney disease, tuberculosis, multiple sclerosis, Duchenne muscular dystrophy and others. Approved data standards are published for use by the scientific research community on CDISC's website.

6 } **Provision of large-scale data solutions for scientific research**

C-Path's Data Collaboration Center (DCC) was instituted to provide large-scale data solutions for scientific research. The DCC team has more than a decade of experience in data standards development, platform development, platform hosting, data curation, stewardship of patient-level data privacy, data security and access control methodologies. The DCC's work takes place in a neutral, non-competitive environment and utilizes appropriate data standards. C-Path has developed and continues to maintain databases for Alzheimer's disease, Parkinson's disease, polycystic kidney disease, tuberculosis, Duchenne muscular dystrophy, Friedreich's ataxia kidney safety biomarkers, multiple sclerosis and other disease areas. C-Path also maintains the Biomarker Data Repository to advance and accelerate understanding about the utility of clinical and nonclinical biomarkers as drug development tools. Information about three different types of databases C-Path developed are listed below. For a complete list, visit the DCC Projects site.

1. Genetic sequencing of pathogens
2. Collection of nonclinical and clinical biomarker data
3. Integrating patient level data

7 } **Forming and managing large international consortia**

C-Path is an expert in pre-competitive collaboration and is a trusted, neutral entity in regulatory science that excels in forming, managing, and facilitating large international consortia. Stakeholders include industry, regulatory and other governmental agencies, non-governmental organizations, patient groups and academia world-wide.

C-Path's ability to drive toward focused goals is key to its extraordinary competence in overseeing large consortia through technical, scientific, legal, regulatory and project management expertise, enabling the achievement of regulatory acceptance of drug development tools or novel methodologies. Each consortium collaborator is encouraged to share data that promotes the advancement of a wide

variety of cross-cutting and disease-specific drug development tools and innovative methodologies.

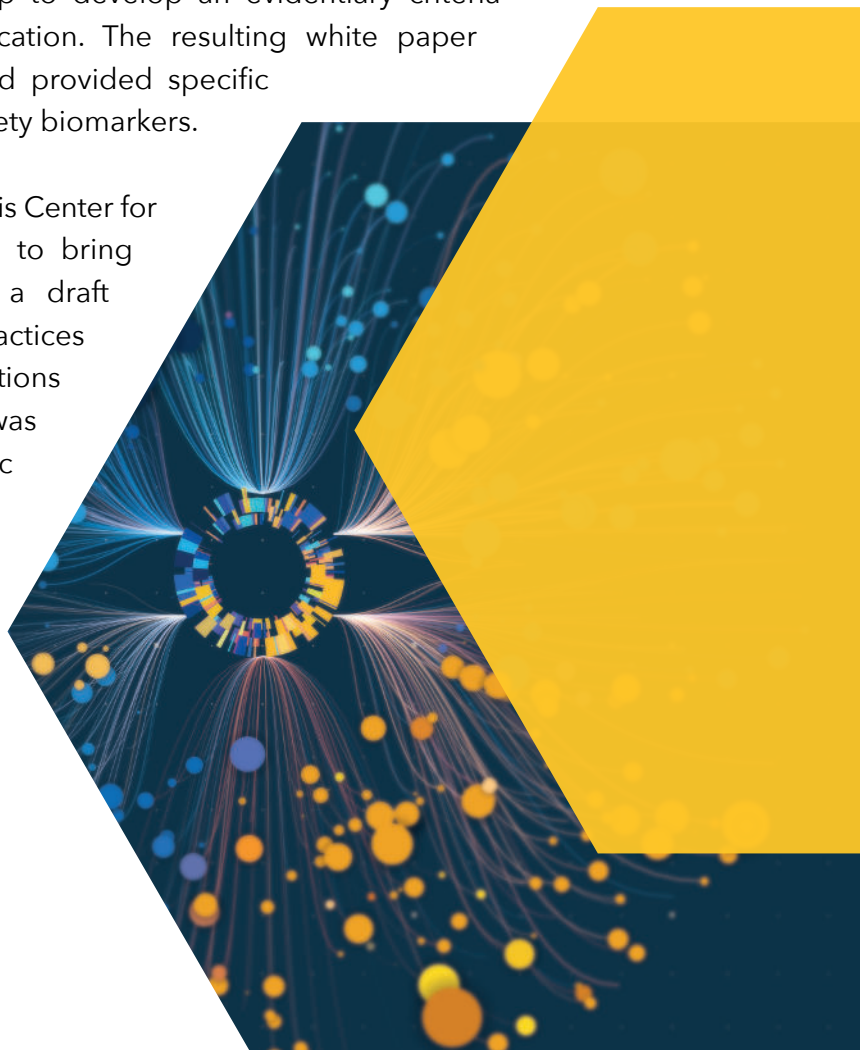
8 } Impact on Regulatory Science

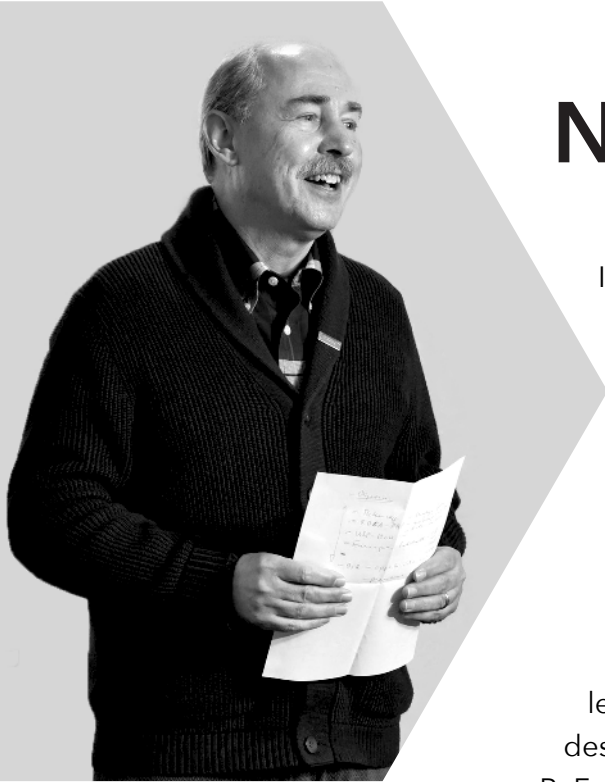
C-Path focuses on the advancement of regulatory science through multiple avenues beyond qualification. This includes the development of points to consider white papers representing consensus among experts and stakeholders within a consortium. These papers are informative to regulatory authorities as they work to develop regulatory guidance documents. Furthermore, C-Path has provided public feedback on numerous draft guidance documents released for comment by both FDA and EMA.

C-Path has been instrumental in leading the movement within the scientific community to more clearly articulate the level of evidence necessary to achieve qualification, one of the most challenging issues in the drug development tool qualification process.

In April 2016, key stakeholders, including FDA Center for Drug Evaluation and Research (CDER), C-Path, and the Foundation for the National Institutes of Health Biomarkers Consortium (FNIH BC), held a workshop to develop an evidentiary criteria framework for safety biomarker qualification. The resulting white paper delineated the proposed framework and provided specific examples of its applicability to clinical safety biomarkers.

In June 2017, C-Path and the Duke Margolis Center for Health Policy held a public conference to bring together key stakeholders to discuss a draft framework outlining criteria and best practices for biomarker assay performance expectations and validation. A draft white paper was prepared in advance of the public forum, and input was solicited after the conference. Currently, the framework is being utilized by biomarker qualification stakeholders to evaluate assay acceptability in ongoing and planned biomarker qualification projects.





Joseph Scheeren,
PharmD

New Leadership

In March, C-Path appointed Joseph Scheeren as its new president and chief executive officer.

Scheeren previously served as Senior Advisor Research and Development for Bayer AG and brings with him a distinguished 36-year career in the pharmaceutical industry – having held positions domestically and internationally in drug development and regulatory approval on three continents.

“Dr. Scheeren exemplifies the in-depth knowledge and inspiring leadership that C-Path, and every organization we collaborate with, deserve in a chief executive officer,” said C-Path Board Chairman Timothy R. Franson, MD. “His global experience with drug development and the regulatory process is evidenced by a long list of first cycle approvals for new therapies and we couldn’t be more thrilled to welcome him to C-Path.”

Scheeren joined Bayer Pharmaceuticals in 2004 as Senior Vice President, Head of Global Regulatory Affairs, responsible for development in the U.S. In addition, in 2009 he became Site Head U.S. in Montville, New Jersey. Expanding on his role in Regulatory Affairs, in 2012, he assumed the position of Head of Global Development Asia in Beijing, and in 2015 was appointed Head of Global Regulatory Affairs Pharma and Consumer Care of Bayer Healthcare, Basel, Switzerland. In January 2018, he was appointed Senior Advisor R&D, Bayer AG. Scheeren’s experience with global regulatory affairs and development will help C-Path expand its mission worldwide.

“C-Path and its partners define the type of innovation and solutions that healthcare needs now and into the future to provide patients hope and access to new treatments and cures by accelerating innovation in the development and approval pathway,” Scheeren said. “I am committed to continuing C-Path’s success by making new science happen through collaborations and continuing to be a change agent in healthcare.”

Scheeren has an established network of connections across the globe and holds many memberships and designations. He serves on the advisory boards at the Center for Innovation in Regulatory Science and the Center of Regulatory Excellence in Singapore. He served as the Chair of the Board of Directors of the Drug Information Association (DIA) in 2018 and 2019 and has served previously as Chairman of the DIA Regional Advisory Council for Europe, the Middle East and Africa; he is also a foreign member of the Academie Nationale de Pharmacie, France, a lecturer at Yale University and an adjunct professor at Peking University for regulatory science.

Expanded Operations in EU and Continued Global Reach



C-Path in January launched its new European headquarters, Critical Path Institute, Ltd. (C-Path, Ltd.), in Ireland. This fully-owned subsidiary has enabled C-Path to increase its activity in the European Union and broaden its global operations as it works to accelerate the development of therapies in a wide range of diseases and medical conditions.

“We look forward to bringing our expertise to bear on European Research Infrastructure programmes sponsored by the European Commission, such as those led by the Innovative Medicines Initiative,” said C-Path, Ltd. Board Member, Graham Higson, M.Sc.

As its U.S. counterpart does, C-Path, Ltd. forms consortia of scientists and clinicians from the biopharmaceutical industry, government regulatory agencies, academic institutions and patient groups. These consortia collaborate to develop novel methodologies – such as biomarkers, clinical outcome assessment tools, models and clinical trial simulation tools and databases – and will submit evidence packages to European regulatory agencies (as well as agencies in the U.S. and Japan) for review.

Another example of C-Path’s global reach occurred in April when its Predictive Safety Testing

Consortium (PSTC) convened its biennial Safety Biomarker Conference in Yokohama, Japan. With cooperation and support of the RIKEN research institute, Japan’s National Institute of Health

Sciences, the Japanese Society of Toxicology and Japan Pharmaceutical Manufacturers Association, this international conference explored existing and emerging biomarkers as drug development tools, including their application in preclinical and clinical research.

In March, PSTC also announced that a formal consultation with Japan’s Pharmaceuticals and Medical Devices Agency (PMDA) resulted in an agreement on a first-of-its-kind approach to compare levels of eight novel urinary kidney safety biomarkers in healthy Japanese volunteers to data collected on these biomarkers in healthy Western volunteers in a bridging study. Results from the project, which begins in 2020, are expected to provide clinical evidence for PMDA evaluation to support utilization of these biomarkers to help guide decisions in early-stage clinical trials to ensure the safety of study participants. “Drug-induced kidney injury is a serious issue that can occur during drug development,” said John-Michael Sauer, PhD, C-Path Biomarkers Program Officer and Executive Director of PSTC. “Results from this and other PSTC clinical safety biomarker projects are designed to generate critical data to support safety decisions about whether to pause or discontinue early phase clinical trials at the tested dose and duration.”

New Data Program

Friedreich's Ataxia Integrated Clinical Database (FA-ICD)

"C-Path has a long history of expertise in data standards development, curation, and oversight of multiple data sharing initiatives. We are proud to be part of the effort to coordinate collaborative contributions from data owners and integrate that data into a single database for this rare, degenerative and life-shortening neuromuscular disorder."

Richard Liwski, Director of the DCC and C-Path's Chief Technology Officer

C-Path's Data Collaboration Center (DCC) and the Friedreich's Ataxia Research Alliance (FARA) announced on Feb. 27, 2019, the launch of the Friedreich's Ataxia Integrated Clinical Database (FA-ICD). The new platform enables collaborative research and data sharing to support the understanding of natural history, potential biomarkers and clinical endpoints, and to promote research into novel clinical trial design in Friedreich's ataxia (FA). By making this data available to researchers, the organizations hope to enable the development of tools that will help design and interpret efficient clinical trials, leading to effective treatments for FA as soon as possible.

"FA is a rare, progressive and fatal disease that affects multiple organ systems, and those living with the disease are in urgent need of effective treatments," explained FARA Executive Director Jennifer Farmer. "FA-ICD addresses this need by providing a platform to share data and making it available to qualified researchers to expedite the drug development process. We believe this resource will inform and give future clinical trials of potential therapies the best chance of success."

FA-ICD contains data contributed by collaborating companies that have carried out clinical trials in FA, as well as natural history and clinical outcome measure data from the Friedreich's Ataxia Clinical Outcomes Measures Study (FACOMS) collected by the Collaborative Clinical Research Network in FA and funded by FARA.

All data contributed to the project is de-identified, mapped to standards defined by the Clinical Data Interchange Standards Consortium (CDISC) and curated by C-Path's DCC before inclusion in FA-ICD.

Broadening Horizons

The University of Arizona (UA) and C-Path in September 2018 launched a new online graduate certificate program designed to equip students and working professionals across multiple disciplines with core competencies in regulatory science.

"There is a large and growing need for current and future pharmaceutical and medical device professionals, regulators, business leaders and scientists to have specialized training in translating research into new interventions for safe and effective medical products," said Tara Sklar, JD, MPH, director of the Graduate Certificate in Regulatory Science and professor with the University of Arizona College of Law.

The Graduate Certificate in Regulatory Science program is offered through University of Arizona College of Law in collaboration with the UA College of Pharmacy and C-Path. Multiple members of C-Path's leadership team serve as instructors and guest lecturers sharing their vast expertise in the field.

"This program provides a background for graduate students and professionals to influence and directly participate in the field of regulatory science," said John-Michael Sauer, PhD, executive director of the Predictive Safety Testing Consortium at C-Path and pharmacology professor at the UA College of Medicine - Tucson. "In connection with our work at the Critical Path Institute, we provide students the knowledge and tools to accelerate medical product development."

The unique coursework reflects a partnership with faculty experts and industry leaders to provide students with an understanding of what goes into creating drugs, biologics or devices and the legal practices governing them to make an impact in the biotech and pharmaceutical industry.





CAMD 10-Year Anniversary Rebrand

In 2018, C-Path's first clinical consortium, previously known as the Coalition Against Major Diseases (CAMD), was rebranded to Critical Path for Alzheimer's Disease (CPAD), a name that better reflects the consortium's more focused approach to its work, which centers on speeding the drug development process for Alzheimer's disease (AD) and mechanistically-related dementias. CPAD subsequently refined and refocused its mission in celebration of its 10-year anniversary.

CPAD is a public-private partnership established to create new tools and methods that can be applied to increase the efficiency of the development processes leading to treatments for AD, the most common and devastating form of dementia globally, as well as other neurodegenerative diseases that share similar characteristics with AD and progress to

dementia. To further grow and strengthen the initiative, CPAD forged a partnership in 2016 with the Global Alzheimer's Association Interactive Network (GAAIN), an open-access data resource portal that provides scientists with rapid access to AD research data. CPAD works with industry, regulatory authorities, and patient advocacy organizations to advance Drug Development Tools (DDTs) for evaluating drug efficacy and safety, to optimize novel clinical trial designs, and aggregating anonymized patient-level data using CDISC consensus standards to facilitate the regulatory review process.

CPAD is actively expanding its patient-level clinical studies database, to include data sources earlier in the disease continuum. This will allow CPAD and the Quantitative Medicine Program to develop a comprehensive disease progression model across the disease continuum, from as early to as late in the disease as the data allow.

Data sharing is the cornerstone for enabling advances in regulatory sciences that provide a gateway to new innovative treatments for patients with dementias related to AD. In collaboration with its members and regulators, the CPAD consortium is set to pave the way for early diagnosis, new treatments, and better quality of life for individuals affected by AD.

Regulatory Milestones



PARKINSON'S DISEASE

C-Path's Critical Path for Parkinson's Consortium (CPP), in partnership with Parkinson's UK, announced in July 2018 that the European Medicines Agency (EMA) issued a positive model-based qualification opinion on an imaging test (biomarker) as a tool to enrich Parkinson's clinical trials. The purpose of this enrichment biomarker is to serve as a measurement that can be used to select people with Parkinson's who are most suitable to take part in clinical trials.

The biomarker is used to determine the presence of dopamine transport deficiency in the brain and has been qualified as an enrichment biomarker for clinical trials targeting early stages of Parkinson's soon after diagnosis. The qualified biomarker involves the intravenous injection of a small amount of a radioactive tracer before the brain images are acquired and can be done at any one of many specialist imaging centers. The imaging agent binds very specifically to dopamine transporter sites on the neurons that are lost in Parkinson's disease. The use of this biomarker can help better identify patients that are more likely to exhibit significant progression in their motor signs and symptoms, thus helping select patients for clinical trials.

"This endorsement from the European Medicines Agency represents many years of hard work and incredible collaboration among companies, universities, and charities facilitated by the Critical Path Institute," said Diane Stephenson, PhD, Executive Director of CPP, who led the work. "These brain scans in themselves are not new, but until now there has not been a clear consensus that they can and should be used to select participants for clinical trials. Through our global project, we've been able to bring all the data and expertise together to make a powerful case, so we're delighted that this endorsement from the EMA will improve the quality and chances of success for future trials of Parkinson's treatments. This success is just the first in a suite of new tools that we hope to deliver for Parkinson's."



ASTHMA

C-Path's Patient-Reported Outcome (PRO) Consortium announced in April 2019 the FDA qualification of two new clinical outcome assessment tools: the *Asthma Daytime Symptom Diary (ADSD)* and the *Asthma Nighttime Symptom Diary (ANSD)*. The qualification of the *ADSD* and the *ANSD* represents a major milestone for the PRO Consortium and specifically for the Asthma Working Group. It is the culmination of a multi-year collaboration between FDA's Center for Drug Evaluation and Research (CDER) and the PRO Consortium.

The *ADSD* and the *ANSD* are each 6-item, PRO measures developed to assess the severity of the core symptoms of asthma in adults and adolescents with a clinical diagnosis of mild to severe persistent asthma.

"Asthma symptoms have a substantial impact on patients, including limiting the ability to participate in daily activities and disrupting sleep. The qualification of the *ADSD* and the *ANSD* is a significant advance in our ability to document the patient's experience of asthma symptoms in clinical trials," stated Linda Nelsen, MHS, Senior Director and Head, Patient Centered Outcomes, Value Evidence and Outcomes, at GlaxoSmithKline and co-chair of the PRO Consortium's Asthma Working Group.



DRUG-INDUCED KIDNEY INJURY

C-Path's Predictive Safety Testing Consortium (PSTC) and the Foundation for the National Institutes of Health (FNIH) Biomarkers Consortium (BC) in August 2018 received the first-ever qualification of a clinical safety biomarker awarded by the U.S. Food and Drug Administration (FDA). The qualification marks a major milestone that will improve the detection of drug-induced kidney injury in early phase drug development.

The qualification applies to a single composite measure (CM) of six urine biomarkers, to be used in conjunction with traditional measures of kidney function; the six safety biomarkers include clusterin (CLU), cystatin-C (CysC), kidney injury molecule-1 (KIM-1), N-acetyl-beta-D-glucosaminidase (NAG), neutrophil gelatinase-associated lipocalin (NGAL), and osteopontin (OPN).

The newly qualified biomarker can now be used in Phase I clinical trials to aid in the detection of acute kidney tubular injury in healthy volunteers. This will help improve the development of safe and effective medicines where concern has been raised that an investigational drug may cause kidney injury. In achieving this milestone, PSTC, C-Path's longest running consortium, celebrates the successful translation of its preclinical biomarkers, previously qualified for use in animal

studies, for use now in Phase 1 clinical trials.

“The acceptance of these clinical safety biomarkers marks our first successful translation of biomarkers qualified for preclinical use to use in clinical drug development. This is a major step forward in our efforts to accelerate the development of safer medicines for patients in need,” said John-Michael Sauer, PhD, Executive Director, PSTC.

These accomplishments were followed by the publication of the User’s Guide: Kidney Safety Composite Measure Biomarker for Use in Clinical Development in 2019. Included in the User’s Guide is information about why the biomarker is important, its context of use, how it should be used, how results should be interpreted, mode of measurement and methodologies drug developers can use to calculate and apply the biomarker to detect kidney injury during Phase 1 clinical trials.



TYPE 1 DIABETES

In December 2018 C-Path’s Type 1 Diabetes (T1D) Consortium received a positive response to its Letter of Intent (LOI) from the FDA, detailing the FDA’s decision to accept the consortium’s Biomarker Initiative project into the Center for Drug Evaluation and Research (CDER) Biomarker Qualification Program (BQP).

In its LOI, the T1D Consortium provided information to support its proposed qualification of islet autoantibodies (AAs) as susceptibility/ risk biomarkers that could identify individuals who are likely to develop a clinical diagnosis of T1D. The FDA indicated in its LOI decision letter that it supports the consortium’s plan to pursue biomarker qualification aimed at providing clinical validation of the islet AAs.

“The encouraging statements that FDA incorporated in the LOI decision letter add weight to the recognition of this significant unmet medical need as well as the critical importance of identifying factors for those at risk of developing T1D,” said then C-Path President and CEO Martha Brumfield, PhD. “This early support can serve to encourage those with data in this space to participate with C-Path to positively impact the lives of individuals affected by the disease, and it also represents a meaningful advance in the prevention of T1D.”

In May 2019, the T1D Consortium proceeded with the EMA regulatory acceptance process by submitting a final Letter of Intent (LOI) and Briefing Package (BP) to EMA for the autoantibody qualification effort. This submission included information to support the qualification of islet AAs as enrichment biomarkers for clinical trials focusing on the delay or prevention of the clinical diagnosis of T1D.



Looking Ahead

At the close of the 2019 fiscal year, C-Path's position in the global drug development arena remains strong. It has expanded its reach with the opening of a European headquarters in Ireland, and with its unique business model and a growing list of successes in its core competencies it continues to gain traction within the industry and bolster a reputation for being the go-to authority on putting together dynamic, responsive and effective collaborations to work on finding cutting-edge solutions to the world's urgent health challenges.

Demand for C-Path participation and collaboration on projects with biotechnology companies, pharma companies and patient groups continues to rise, and these organizations' commitment to working with C-Path demonstrate the confidence and credibility that have been built into the bedrock of C-Path's mission and their reflection in the broader community of key stakeholders in the rare disease landscape.

New grants from the Doris Duke Charitable Foundation (therapies for sickle cell disease) and the Flinn Foundation (pilot program to respond to antimicrobial resistance) are indicative of C-Path's reputation for establishing solid methodologies and finding new pathways to success, and the award of a contract by the U.S. Food and Drug Administration (FDA) (establishment of a kidney transplant database) demonstrates the positive outlook and confidence in C-Path strategies from visionaries in the field.

As a leader in aggregating and curating data, C-Path is on a trajectory to change the face of drug development and catalyze a paradigm shift in the achievement, management and maintenance of good health. Its accomplishments over the last year are evidence of the strength and soundness of the Institute's operational principles and its dedication to creating solutions for a better life.

With continued support from the broader global health community – including patients, donors, foundations, academics, pharmaceutical companies and regulatory agency advisors – C-Path will continue to forge a path of success, leaving in its wake the stepping stones that mark the journey to a healthier world.

Financial Update

With a growing list of achievements in the development of innovative tools and methods to speed drug development and streamline regulatory review processes, C-Path continues to demonstrate extraordinary value in the drug development space and maintains a strong financial position at the close of this fiscal year.

C-Path's unique business model, which fosters collaboration among leaders from the pharmaceutical and biotech industries, universities, patient organizations and regulatory agencies from around the world, is accelerating drug development and regulatory review processes and, ultimately, improving public health. Organizations that typically fund basic research increasingly are looking to C-Path to lead the way in areas of translational and regulatory science and, as a result, it continues to experience steady growth and expansion into new disease areas and geographical regions.

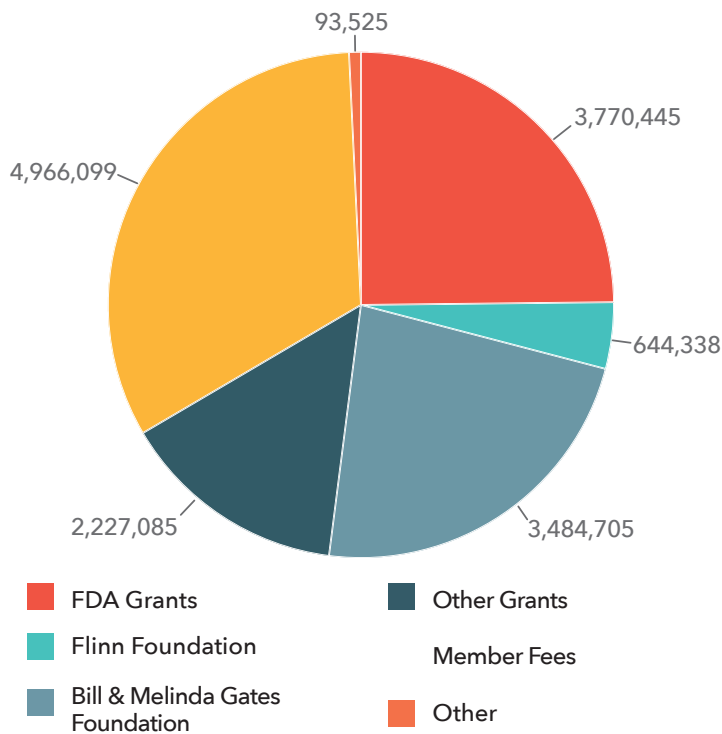
With its new entity, C-Path, Ltd. now operating in Ireland, C-Path has broadened its global operations and begun to ramp up activities in the European Union.

C-Path is committed to expanding the arsenal of approved biomarkers, clinical outcome assessments, quantitative models and other tools available to help bring safe and effective therapies to market for rare and devastating diseases. The organization is positioned for success and will continue to bring to fruition its vision to accelerate the path to a healthier world.

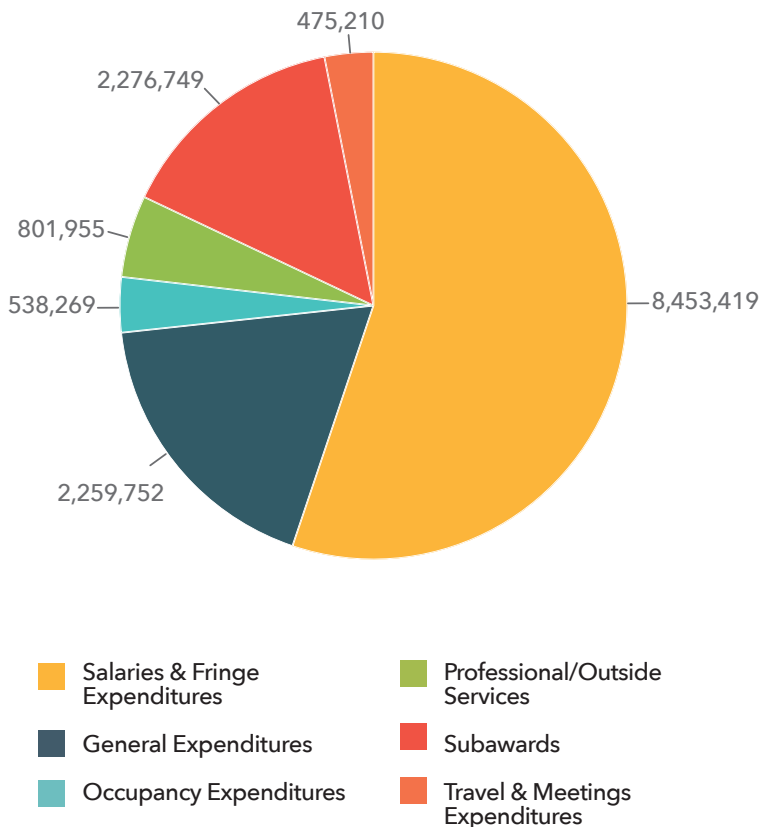
Significant financial news this year includes:

- The Doris Duke Charitable Foundation awarded a grant to C-Path to lay the groundwork for forming a collaboration of multiple stakeholder organizations to accelerate the development of therapies for sickle cell anemia.
- The U.S. Food and Drug Administration (FDA) awarded a contract to C-Path in support of a project to create a new integrated database of patient-level clinical trial data that will serve as a foundational resource to inform the design of drug development tools (DDTs) that support the development of novel immunosuppressive drugs for the treatment of individuals who receive kidney transplants.
- C-Path and Flagstaff, Arizona-based TGen North received a Phase 1 Concept Validation grant from the Flinn Foundation to develop and pilot a framework enabling the state institutions to be more responsive to antimicrobial resistance.
- C-Path, Ltd. launched in Ireland, enabling C-Path to increase its activity in the European Union and broaden its global operations as it works to accelerate the development of therapies in a wide range of diseases and medical conditions.

C-PATH 2019 FISCAL YEAR REVENUE: \$ 15,186,200



C-PATH 2019 FISCAL YEAR EXPENSES: \$ 14,805,355



Fiscal Year 2019

ASSETS

Cash and Cash Equivalents	\$	9,984,942
Certificates of Deposit	\$	6,402,760
Accounts Receivable	\$	904,294
Property and Equipment, Net	\$	76,758
Other	\$	112,549

TOTAL ASSETS \$ 17,481,303

LIABILITIES AND NET ASSETS

Liabilities

Accounts Payable	\$	560,921
Accrued Expenses	\$	345,375
Deferred Revenue*	\$	8,404,570
Deferred Rent	\$	55,040

Total Liabilities \$ 9,365,906

Net Assets

Undesignated	\$	3,949,712
Board Designated**	\$	3,688,927
Property and Equipment	\$	76,758
Donor Restricted	\$	400,000

Total Net Assets \$ 8,115,397

TOTAL LIABILITIES AND NET ASSETS \$ 17,481,303

* Pre-awarded funds received for grants

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

C-Path Initiatives

ACTIVE CONSORTIA



The **Critical Path for Alzheimer's Disease (CPAD)** was founded to create new tools and methods that can be applied to increase the efficiency of the development process of new treatments for Alzheimer's disease (AD). Alzheimer's disease is recognized as a major public health issue because the burden is large, the impact is major, and there is growing interest in intervening earlier in the disease process. This requires a robust understanding of disease progression across all stages of disease. CPAD focuses on pre-competitive sharing of patient-level data from clinical trials and observational studies. These data support the development of novel tools and comprehensive disease progression models across the AD continuum, to be submitted to the regulatory agencies, and developing consensus data standards all with the goal of de-risking drug development process and accelerating development of therapeutic options for patients with AD.



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



The **Critical Path to TB Drug Regimens (CPTR)** initiative was created through a partnership between the Bill & Melinda Gates Foundation, TB Alliance and C-Path to promote innovation in regulatory science needs to accelerate development of novel drug regimens for tuberculosis (TB). Through the development of new or better validated tools and methodologies, sponsors are better able to de-risk and expedite decisions related to their drug development programs.

Tuberculosis is a disease that still impacts one-third of the world's population, which is in desperate need of a safer, shorter-duration and more effective drug regimen. Much of this critical work is enabled by a global data-sharing initiative, funded by the Bill & Melinda Gates Foundation and led by C-Path and partner organizations, which include the World Health Organization, TB Alliance, and multiple data contributors representing industry, academia and government agencies.



The **Duchenne Regulatory Science Consortium (D-RSC)** was formed in partnership with Parent Project Muscular Dystrophy (PPMD) to aggregate data and develop a disease progression model to accelerate the development of new therapies for Duchenne muscular dystrophy, which is an urgent unmet medical need. Duchenne is a genetic disease that causes progressive loss of muscle, cardiac issues, the inability to walk and breathe, and ultimately results in premature death. Through the integration of patient-level data from observational studies and clinical trials, D-RSC aims to generate a set of models that describe disease progression across the disease continuum, as a basis for a comprehensive clinical trial simulation tool. This tool will improve trial protocol development and optimize the number of patients needed to demonstrate the effect of new therapies, thereby accelerating the development of the therapies for this disease.



The **Electronic Patient-Reported Outcome (ePRO) Consortium** was established to advance the science surrounding electronic collection of clinical outcome assessment data in clinical trials. The ePRO Consortium provides a pre-competitive environment in which a critical mass of experts collaborates to support and conduct research, design and deliver educational opportunities, and develop and disseminate best practice recommendations for electronic collection of clinical outcome data.



The **Huntington's Disease Regulatory Science Consortium (HD-RSC)** was created in partnership with the CHDI Foundation, a privately funded, nonprofit biomedical research organization devoted solely to Huntington's disease. This disease is a dominant genetic disorder that causes the progressive breakdown of nerve cells in the brain. It deteriorates a person's physical and mental abilities usually during their prime working years, making it imperative to optimize the understanding of disease progression. With the goal of improving the efficiency of development, review and approval of emerging therapeutics for Huntington's disease, this global initiative aims to facilitate interaction between biotech and pharmaceutical industry partners with

regulatory agencies to work towards the regulatory acceptance of drug development tools, biomarkers and better clinical outcome assessments. HD-RSC will also collect and standardize natural history and clinical trial data from HD patients around the world to develop an integrated database of patient-level data, which will be used to gain a better understanding of disease progression dynamics and work towards model-informed drug development to de-risk HD programs and accelerate the global regulatory approval of urgently needed HD therapies.



The **International Neonatal Consortium (INC)** is a global collaboration forging a more predictable regulatory path to evaluating the safety and effectiveness of therapies for neonates. The consortium engages the global neonatal community – families, neonatal nurses, academic scientists, regulators, pharmaceutical investigators, advocacy organizations, clinicians and funders – to focus on the needs of the neonate. Through teams that share data, knowledge and expertise, INC advances medical innovation and regulatory science for this underserved population.



The **Multiple Sclerosis Outcome Assessments Consortium (MSOAC)** collects, standardizes, and analyzes data about multiple sclerosis (MS) that have been generated over several decades, with the goal of qualifying a new measure of disability as a primary or secondary endpoint for future trials of MS therapies. MS is a demyelinating disease in which a specific layer of nerve cells in the brain and spinal cord are damaged. This damage disrupts the ability of parts of the nervous system to exchange information, resulting in variety of signs and symptoms, requiring the optimization of outcome measures to capture these relevant aspects of disease. MSOAC works on the development of such optimized outcome measures by bringing together members from academia and industry, regulatory authorities, patient advocacy groups and individuals living with multiple sclerosis, aiming to speed the development of new therapeutic options for this disease.



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 polycystic kidney disease (PKD). PKD is a disorder that affects the kidneys, in which collections of fluid (cysts) develop, interfering with the kidney's ability to filter waste products from the blood. The growth of cysts causes the kidneys to become enlarged and can lead to kidney failure. PKDOC's mission is to develop drug development tools and methods to

to promote research that will lead to the development of treatments for PKD and improve the lives of all it affects. PKDOC has developed CDISC data standards for PKD and aggregated clinical data from Autosomal Dominant Polycystic Kidney Disease patients collected over many years in patient registries and observational studies. These data enabled the development of a disease-biomarker progression model that provided the support necessary for the U.S. Food and Drug Administration and European Medicines Agency to qualify an imaging biomarker, Total Kidney Volume, for use as an enrichment strategy in drug development trials. PKDOC continues to work on alternative endpoints and clinical trial designs.



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



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 U.S. Food and Drug Administration, European Medicines Agency and Japan's Pharmaceutical and Medical Devices Agency. PSTC does this by developing and implementing scientific research strategies in a neutral, pre-competitive environment, thereby allowing members to share expertise, resources, data and internally developed approaches, which improves both the speed and precision of the drug development process. PSTC's efforts are intended to develop drug development tools that assist pharmaceutical companies and regulatory agencies in making better-informed decisions, all of which ultimately benefit patients. Currently, PSTC is engaged in the qualification of novel nonclinical and clinical safety biomarkers across several organ systems for application in the development of drugs.



The **Type 1 Diabetes (T1D) Consortium** is a public-private partnership initiated in March 2017. Current membership is composed of the following industry and foundation members: JDRF, the Leona M. and Harry B. Helmsley Charitable Trust, Janssen Research & Development, LLC, Novo Nordisk and Provention Bio. Membership is also comprised

of academic partners and advisors from the National Institutes of Health (NIH). Type 1 diabetes is a condition in which the insulin-producing cells of the pancreas are damaged by antibodies generated by the body's own immune system (autoantibodies). In order to optimize the drug development process for novel T1D treatments, it is crucial to understand how the presence of the autoantibodies affects the risk of reaching a T1D diagnosis. As such, the primary goal of the T1D Consortium is to qualify islet autoantibodies as enrichment biomarkers to be used in the development of therapies for the treatment, and ultimately prevention of type 1 diabetes. The islet autoantibodies of interest include insulin autoantibodies (IAA), glutamic acid decarboxylase 65 (GADA), insulinoma antigen-2 (IA-2) and zinc transporter 8 (ZnT8) autoantibodies.



The **Transplant Therapeutics Consortium (TTC)** convenes diverse stakeholders (industry, academia, and government agencies) to optimize the development of medical products for transplant patients. Solid organ transplantation can be the treatment of choice for many people with end-stage conditions. The development of novel medicines that can maximize the lifespan of a transplanted organ will improve quality of life and reduce mortality, which requires optimized clinical trial designs, based on tools that provide a robust quantitative understanding of predictors of clinically relevant endpoints. TTC supports collaborative development and regulatory endorsement of such drug development tools for solid organ transplantation, which, in turn, may help to shorten the time needed to deliver safe, effective therapies for transplantation patients. The consortium achieves this by bringing together key stakeholders in the transplant community with the primary goal of identifying and addressing the regulatory challenges that impact the development and approval of new therapies in transplantation.



MODEL-INFORMED DRUG DEVELOPMENT PROGRAMS



The mission of C-Path's **Quantitative Medicine (QuantMed)** Program is to improve population and individual health by transforming drug development through methodological innovation.

QuantMed's vision is to become a nationally and internationally recognized program for Model-Informed Drug Development (MIDD). Such actionable knowledge is then turned into specific quantitative solutions to optimize the efficiency of the drug development process. These solutions can take the form of disease progression models, clinical trial simulators, physiologically-based pharmacokinetic platforms, biomarker dynamic models, etc. In partnership with other C-Path initiatives, the QuantMed Program is transforming drug development for Alzheimer's, Huntington's and Parkinson's disease, type 1 diabetes, kidney transplantation, Duchenne muscular dystrophy, as well as polycystic kidney disease and tuberculosis; while actively working on novel quantitative approaches in pharmacometrics, artificial intelligence and digital outcome assessment tools.

DATA PROGRAMS



The **Biomarker Data Repository (BmDR)**, launched by C-Path's Predictive Safety Testing Consortium (PSTC), is a repository for data on novel translational safety biomarkers from drug development programs. Masked, de-identified data from multiple sponsors is collected and stored in a secure repository, and that data is then made available to C-Path and U.S. Food and Drug Administration staff to support research that leads to the submission of documents to worldwide regulatory agencies to qualify novel safety biomarkers for new Contexts of Use (CoU) statements as well as to modify and expand existing CoU statements and to identify appropriate exploratory biomarkers to advance drug development in the future. The initial pilot focuses on kidney safety biomarkers, and the main goal of the BmDR is to provide industry with new drug development tools. Existing biomarker data could be used to significantly advance and accelerate understanding of the utility of novel biomarkers as drug development tools.



The **Data Collaboration Center (DCC)**, which built and manages C-Path's Online Data Repository (CODR), has the goal to enable multiple organizations to work together to share medical research data, in order to optimize its value in creating new insights and tools that accelerate drug development in areas with unmet medical needs. The DCC supports data-sharing projects aligned with specific C-Path consortia, as well as data-sharing initiatives that are independent of C-Path consortia. All of the DCC's work takes place in a neutral, precompetitive environment, utilizing appropriate data standards. The DCC possesses the technical and scientific subject matter and project management expertise necessary to support advanced research efforts.

Launched in February 2019, the **Friedreich's Ataxia Integrated Clinical Database (FA-ICD)** is designed to catalyze and accelerate Friedreich's ataxia (FA) research and drug development by curating and standardizing FA clinical trial and natural history data into CDISC format and making this data publicly available to qualified researchers. These researchers can access and analyze data in aggregate, or filter and view de-identified patient-level data from four clinical trials and a large FA natural history study. The initiative represents a collaborative partnership between the Friedreich's Ataxia Research Alliance (FARA) and C-Path, with a goal of expanding the FA-ICD platform by engaging with other data contributors to secure additional datasets.



The **TB Platform for Aggregation of Clinical TB Studies (TB-PACTS)** is designed to catalyze and accelerate tuberculosis (TB) research by curating and standardizing TB clinical trial data and making this data publicly available to qualified researchers. These researchers can access and analyze data in aggregate, or filter and view individual patient-level data from 17 clinical trials, including REMoxTB, RIFAQUIN and OFLUTBU. This initiative represents a collaborative partnership between the Special Programme for Research and Training in Tropical Diseases (TDR), the TB Alliance, St. George's University of London, Case Western University, the British Medical Research Council and C-Path. The partnership continues to expand the scale of the TB-PACTS platform by engaging with other TB clinical data contributors to secure additional datasets. This and other C-Path TB data platforms will be leveraged in the new IMI funded European Regimen Accelerator for TB consortium.

COMPLETED CONSORTIA



The **Coalition For Accelerating Standards and Therapies (CFAST)**, a joint initiative of C-Path and the Clinical Data Interchange Standards Consortium (CDISC), was founded to advance clinical research and medical product development by creating and maintaining data standards, tools, and methods for conducting research in therapeutic areas that are important to public health. C-Path led the development of the first CDISC Therapeutic Area (TA) Data Standards, in order to advance the data aggregation needs of specific C-Path consortia. This was done in collaboration with CDISC. To date, in partnership with CDISC and the FDA, the National Cancer Institute Enterprise Vocabulary Services (NCI EVS), TransCelerate BioPharma, the European Medicines Agency (EMA), the Innovative Medicines Initiative (IMI), and the Association of Clinical Research Organizations (ACRO), 28 CDISC therapeutic area standards have been published, and C-Path has led or supported the work on 14 of these projects.



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

C-Path's HD-RSC Team



C-Path Collaborators

ACADEMIC INSTITUTIONS

- Baylor Scott & White Research Institute
- Binghamton University
- Brigham and Womens Hospital
- Brighton and Sussex Medical School
- Case Western Reserve University
- City University, London
- Cleveland Clinic
- Colorado State University
- Duke University
- Emory University
- Fraunhofer SCAI
- George Washington University
- Harvard University
- Helmholtz Centre Munich
- Imperial College London
- Indiana University School of Medicine
- Johns Hopkins University
- Karolinska University
- Leiden University Medical Center
- Lund University
- Mayo Clinic
- McGill University
- Mt. Sinai
- Icahn School of Medicine at Mount Sinai
- Newcastle University
- New York University
- North Shore University Health System
- OULU Finland
- Paris Descartes University, Necker Hospital
- RIKEN Institute
- Stanford University
- State University of New York, Buffalo
- Stony Brook Medicine
- Tufts University
- University College London Institute of Neurology
- University of Alabama, Birmingham
- University of Arizona
- University of Bristol
- University of British Columbia
- University of California, Davis
- University of California, Irvine
- University of California, San Francisco
- University of Cambridge
- University of Cape Town
- University of Chicago
- University of Colorado, Denver
- University of Florida
- University of Genoa
- University of Glasgow
- University of Hasslet
- University of Helsinki
- University of Illinois
- University of Iowa
- University of Kansas
- University of Leicester (UK)
- University of Leuven
- University of Liverpool
- University of Ljubljana
- University of Lübeck
- University of Maryland
- University of Massachusetts
- University of Munich
- University of Oxford
- University of Pittsburgh
- University of Pennsylvania
- University of Rochester
- University of Sheffield
- University of St. Andrews
- University of Tampere
- University of Texas
- University of Turku
- University of Utah
- University of Virginia
- University of Washington
- VU University Medical Center
- Washington University
- Western Washington University

PHARMACEUTICAL COMPANIES

- Abbott Molecular
- AbbVie
- Actelion Pharmaceuticals Ltd
- Alere
- Allergan
- Amgen, Inc
- Astellas
- AstraZeneca Pharmaceuticals LP
- Avrobio
- Baxter Healthcare Corporation
- Bayer Pharma AG
- Becton Dickinson Diagnostic Systems
- Biogen
- BioMérieux
- Biophytis
- Boehringer Ingelheim Pharmaceuticals, Inc
- Bristol-Myers Squibb Company
- Catabasis Pharmaceuticals
- Celgene
- Cepheid
- Chiesi
- Commonwealth Biopharma
- CTI BioPharma
- Cydan Development
- Daiichi Sankyo
- Denali Therapeutics Inc
- Eisai, Inc
- Eli Lilly and Company
- EMD Serono
- Ferring Research Institute
- Galápagos
- Genentech, Inc
- GE Healthcare
- Gilead Sciences
- GlaxoSmithKline Corporation
- Goldfinch Biopharma, Inc
- Hansa Medical
- Healx
- Hoffmann-La Roche, Inc
- Horizon Pharma, Inc.
- IBM
- Inception Sciences Canada
- Ironwood Pharmaceuticals, Inc
- Infant Bacterial Therapeutics
- Innate Immunotherapeutics
- Ixico
- Kadmon Corporation
- Johnson & Johnson Pharmaceutical Services
- Lundbeck
- Mallinckrodt Pharmaceuticals
- Metabolic Solutions Development Co.
- Merck Sharp & Dohme Corporation
- Millenium: The Takeda Oncology Company
- Mitsubishi Tanabe Pharma Corporation
- Neurametrix
- Nuredis
- Novartis Pharmaceuticals Corporation
- Novo Nordisk
- Ocello (NL)
- Otsuka
- Palladio Biosciences
- Pfizer, Inc
- PharmaStat
- Prilenia
- PTC Therapeutics
- Qiagen
- Reata Pharmaceuticals
- Regulus Therapeutics
- Relay Therapeutics
- Sage
- Sanofi
- Santhera Pharmaceuticals, Inc
- Sarepta Therapeutics, Inc
- Sequella, Inc
- Shire Pharmaceuticals, Inc
- Summit (Oxford) Limited
- Sunovion Pharma, Inc
- Takeda Pharmaceutical Company
- TEVA Pharmaceutical Industries, Ltd
- Therabron
- Thermofisher
- Transplant Genomics
- Triplet Tx
- UCB Pharma
- uniQure
- Vaccinex
- Veloxis
- Vertex Pharmaceuticals
- Voyager Therapeutics
- Wave Life Science

PATIENT GROUPS

- Alzheimer's Association
- Alzheimer's Drug Discovery Foundation
- Alzheimer's Research UK
- Bill and Melinda Gates Foundation
- CHDI Foundation
- Cure Parkinson's Trust
- Davis Phinney Foundation
- European Huntington Association
- Graham's Foundation
- Huntington's Disease Society of America
- Huntington Society of Canada
- Huntington Study Group
- Italian MS Society
- JDRF
- KHI Patient Family Partnership Council
- March of Dimes
- Michael J. Fox Foundation
- Movement Disorder Society
- National Multiple Sclerosis Society of Canada
- National Multiple Sclerosis Society
- National MS Society of UK
- Parent Project Muscular Dystrophy
- Parkinson's Disease Foundation
- Parkinson's UK
- PKD Charity
- PKD Foundation
- USAgainstAlzheimer's

OTHER

- ADPKD Paediatric Registry
- Alberta MS Research Foundation
- .assisTek
- American Society of Transplantation (AST)
- American Society of Transplant Surgeons (ASTS)
- American Society for Clinical Pharmacology and Therapeutics (ASCPT)
- Bambino Gesù Children's Hospital
- Benaroya Research Institute
- BLISS
- Children's Hospital of Philadelphia
- Cincinnati Children's Hospital Medical Center
- Clinical Ink
- COINN
- Consortium of MS Centers
- Drink Clinical Trial
- EFCNI
- ERT
- FIND
- ICON
- International Society of Pharmacometrics
- IQVIA
- Istituto Mario Negri
- Japan National Institute of Health Sciences
- Kessler Foundation
- MedAvante
- Medidata Solutions
- NANN
- NEC Society
- PATH
- PhRMA
- Premie Parent Alliance
- Scientific Institute H.S. Raffaele, Italy
- Signant Health
- TB Alliance
- TB Clinical Diagnostics Research Consortium
- Terasaki Research Institute
- The Leona M. and Harry B. Helmsley Charitable Trust
- The Transplantation Society
- Treatment Action Group
- Working Group on New TB Drugs (Stop TB Partnership)
- Y-Prime

Leadership Team



Lewis Barbieri, JD

Director - Contracts, Legal Services and Compliance



Steve Broadbent, MBA

Special Operations Advisor,
(Chief Operating Officer through June 2019)



Martha A. Brumfield, PhD

Special Advisor, (President and CEO through March 2019)



Stephen Joel Coons, PhD

Program Officer, Clinical Outcome Assessment Program
Executive Director, Patient-Reported Outcome Consortium



Sonya Eremenco, MA

Acting Director, Electronic Patient-Reported Outcome Consortium



Lynn D. Hudson, PhD

Chief Science Officer
Executive Director, Multiple Sclerosis Outcome Assessments Consortium
Executive Director, International Neonatal Consortium



Jane Larkindale, D Phil

Executive Director, Duchenne Regulatory Sciences Consortium



Rick Liwski

Chief Technology Officer and Director, Data Collaboration Center

Ariana P. Mullin, PhD

Executive Director, Huntington's Disease Regulatory Science Consortium



Inish O'Doherty, PhD

Executive Director, Transplant Therapeutics Consortium
Executive Director, Type 1 Diabetes Consortium



Klaus Romero, MD, MS

Executive Director of Clinical Pharmacology and Quantitative Medicine



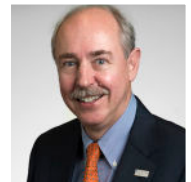
John-Michael Sauer, PhD

Program Officer, Biomarkers Program
Executive Director, Predictive Safety Testing Consortium
Executive Director, Polycystic Kidney Disease Outcomes Consortium



Joseph Scheeren, PharmD

President and CEO (since April 2019)



Sudhir Sivakumaran, PhD

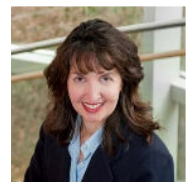
(since July 2019)

Executive Director, Critical Path for Alzheimer's Disease Consortium



Diane Stephenson, PhD

Executive Director, Critical Path for Parkinson's Consortium



Kristen Swingle, MS

Chief Operating Officer (since July 2019)



Advisors



Raymond Woosley, MD, PhD

Founder and Former CEO
Current Senior Advisor

Board Advisors



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Center for Drug Evaluation and Research (CDER)
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Peter B. Corr, MD, PhD

Senior Advisor
Co-Founder and General Partner, Auvén Therapeutics
Management LLLP



The Honorable James C. Greenwood

President & CEO, Biotechnology Industry
Organization (BIO)



Janet Woodcock, MD

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Mr. Peter Barton Hutt, LLB, LLM

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Principal, Tucson Pharma Ventures LLC



Mr. Shaun A. Kirkpatrick, MA

President & CEO, Research Corporation
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Mr. Alan G. Levin, MS, CPA

Executive Vice President & CFO (Retired),
Endo Health Solutions Inc.





Mr. Richard T. Myers, Jr.

CEO, Tempronics
Member, Arizona Board of Regents



Paula J. Olsiewski, PhD

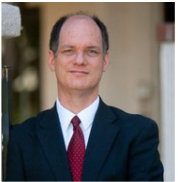
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Enda Conolly, BA

Strategic Advisor, Economic, FDI and Research Development



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