



CRITICAL PATH
INSTITUTE

EXPANDING
HORIZONS

ANNUAL REPORT

20
21

for fiscal year ending June 30, 2021

c-path.org

Accelerating the path
to a healthier world

Table of Contents

From Our Chairman	4
2020-2021 Milestones	8
Core Competencies	9
New Leadership	13
Impact: A Call to Action in Parkinson's Research and Care	16
Impact: Generating Cutting-edge Solutions to Accelerate Drug Development	18
Looking Ahead	21
Financial Update	22
C-Path Initiatives	25
C-Path Collaborators	38
People	43

Who We Are

Critical Path Institute (C-Path) is an independent nonprofit, public-private partnership with the U.S. Food and Drug Administration (FDA) created in 2005 under the auspices of the FDA's Critical Path Initiative program.

Our ongoing success is due to a combination of public and private support for our mission.

C-Path provides the legal and scientific infrastructure to create a uniquely neutral environment for industry, academia, regulators and other government agencies, to work together to accelerate and de-risk the medical product development process. Such acceleration is achieved through the collaborative generation of actionable solutions for specific unmet needs in the process. Solutions can be indication specific or indication agnostic.

C-Path orchestrates the development of these actionable solutions through an innovative, collaborative approach to the sharing of data and expertise. It builds consensus among participating scientists from industry and academia with regulatory participation and iterative feedback. Such consensus provides the mechanism to generate the necessary confidence to assure the adoption of the medical product development solutions by sponsors and regulators.

Examples of pathways through which this confidence is achieved include informal and formal regulatory pathways. Through these various mechanisms, sponsors can confidently adopt the solutions generated through C-Path's collaborative approach, thus ensuring the continuous optimization of the medical product development process.



Vision

C-Path is an indispensable partner of excellence in medical product development worldwide, shaping innovative scientific and regulatory pathways to accelerate delivery of therapies for patients in need.



Mission

C-Path is a catalyst for innovation that accelerates the path to a healthier world.



Values

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



Impact

C-Path's successes lead to safer and better treatments, available faster for patients, creating healthier global and local communities.

From Our Chairman

Dear C-Path Friend,

I'm honored to share with you the many accomplishments, connections, partnerships, successes, milestones and impact that C-Path has achieved over the past year.



Together, C-Path has persisted and persevered through the challenges stemming from the pandemic, as well as continued to progress, prosper and foster new and exciting directions for the years to come.



Timothy R. Franson, M.D.

These achievements are a testament to the diligence and commitment of the staff, scientists, academics, organizations, companies and regulatory and research agencies that form C-Path's collaborations.

We have shown our ability to adeptly navigate challenges both anticipated and unforeseen, while remaining steadfast in our commitment to forge ahead into the future and bring about innovative change in medical product development, which will lead to novel and cutting-edge treatments and cures.

Along with the statistics, figures and data that outline C-Path's growth and impact, within this annual report we have included stories describing how our work has affected patients and families on a personal level. These stories of impact remind and reinforce the value of our core mission and inspire us to continue our path of discovery and innovation.

Over the past year, we have continued strengthening our relationships and partnerships, while reaching out and securing new collaborations and allies, building upon our successes and advancing bold new breakthroughs.

What follows is a summary of some of our notable highlights for the past year:



In the summer of 2020, the analytics team from C-Path's Data Collaboration Center program won first place in the validation phase of the Metadata Automation DREAM Challenge, funded by the Cancer MoonshotSM initiative. Designed and run by a community of researchers from a variety of organizations, DREAM (Dialogue for Reverse Engineering Assessments and Methods) Challenges invite participants to propose solutions to fundamental biomedical questions – fostering collaboration and building communities in the process.



September 2020 marked the launch of the Critical Path for Sickle Cell Disease (CP-SCD) Consortium to support collaboration and regulatory endorsement of new medical product development tools for sickle cell disease. These tools will help to optimize and de-risk clinical trials to increase efficiency in developing and delivering safe, effective treatments for people living with sickle cell disease. SCD affects between 90,000 and 100,000 people in the U.S. and millions more around the world. Sickle cell disease research was neglected for many years and while research in this field has now accelerated, currently approved therapies for the disease are not curative. A goal of this consortium is to establish an international forum that engages regulatory agencies around the globe to identify and address issues that impact the development and approval of new therapies for sickle cell disease.



In years past, unmet drug development needs have contributed to the therapeutic orphaning of neonates. C-Path was awarded a multi-year grant by FDA to advance standards and methodologies designed to generate actionable real-world evidence (RWE) from real-world data (RWD) through a neonatal pilot project through the International Neonatal Consortium. This grant will support the integration of patient-level neonatal data from intensive care units from many key stakeholders worldwide, which will be deposited into a Real-World Data and Analytics Platform (RW-DAP). Data will be used to define actionable solutions for neonatal drug development, such as reference ranges of commonly used laboratory values in neonates and a natural history model of bronchopulmonary dysplasia (a chronic lung disease common in preterm neonates).



In December of 2020, C-Path was awarded an FDA contract in support of the development of open-source tools to improve the efficiency of trial design for three neuroscience diseases: Alzheimer's disease, Parkinson's disease and Duchenne muscular dystrophy. C-Path's Quantitative Medicine Program is leading the project through its work in collaboration with its three C-Path Public-Private Partnerships: Critical Path for Alzheimer's Disease, Critical Path for Parkinson's and the Duchenne Regulatory Science Consortium.



Also, in neonatal news, C-Path and Tufts Medical Center announced, in May of 2021, a joint venture to integrate the first patient-level clinical trial data to generate actionable RWE for neonatal drug development, from real-world neonate data. The data sharing collaboration and transfer also marks the fastest on record for C-Path – starting with the Data Contribution Agreement negotiations and receipt of the data in three months' time beginning in March 2021. C-Path's INC will continue to meet and work with collaborators and new data contributors to integrate additional patient-level datasets.

Throughout the fiscal year, C-Path has maintained its position as a globally respected authority, bringing together and directing vital, proactive and essential partnerships that generate efficient cutting-edge answers to the most intractable health care issues affecting the global population.

C-Path continues to expand its horizons, accelerating the pace – and reducing the costs – of medical product development through the creation of new data standards, measurement standards and methods standards that aid in the scientific evaluation of the efficacy and safety of new therapies. Every day, C-Path is opening new doors and forging new paths via the efficacious implementation of data and analytics – extracting the often hidden, yet intrinsically valuable, signal from the noise – while helping to streamline the regulatory processes necessary to foster life-saving drug development for the treatment of rare and debilitating diseases.

We recognize that none of this work would be possible without our collaborators and partners. Our success and growth are inextricably linked to the professionalism and generosity of our cohorts and multi-stakeholder cooperatives. In this past fiscal year alone, C-Path joined with the Pediatric IBD Foundation and ImproveCareNow to establish the Children's Registry for the Advancement of Therapeutics (CREATE™). CREATE™ is a drug-agnostic safety registry designed to support the completion of global post-marketing safety requirements, as well as collect safety information on all therapies used in pediatric IBD patients. Additionally, C-Path joined forces with the Innovative Medicines Initiative 2 Joint Undertaking (IMI2 JU), signing a new contract that will leverage C-Path's global expertise in developing novel product development tools.

In 2020 and 2021, C-Path consortia initiated or partnered on projects with, amongst others, Ataxia Global Initiative, the National Ataxia Foundation, Ataxia UK, Biohaven Pharmaceuticals, Ionis Pharmaceuticals, Roche Pharmaceuticals, Servier Group, Triplet Therapeutics, uniQure and Vico Therapeutics.

We are grateful to have been awarded grants and contracts from FDA and Innovative Medicines Initiative. Our partnerships with these organizations are a reflection of the confidence they have in our mission and in our ability to deliver upon our commitments.

Although we have made great strides and accomplished much, there is still a long road ahead and many obstacles to overcome as we build upon the knowledge and hard-fought wins accumulated during our first 15 years of operation and expand our reach and plot a course for a new horizon.

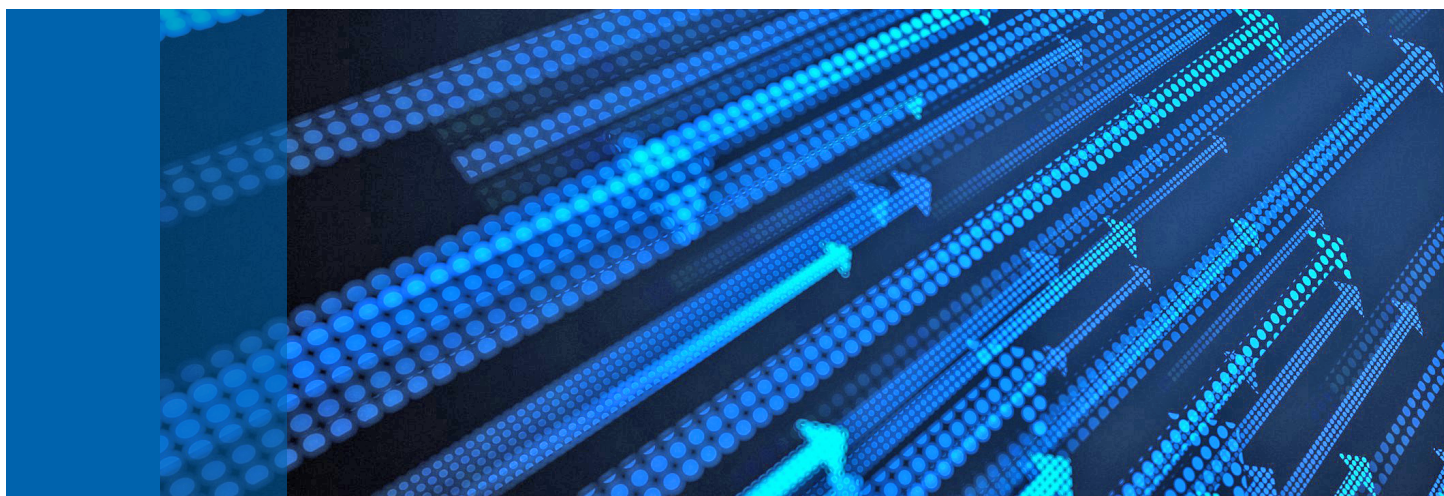
In our 16th year, C-Path was able to safely negotiate the uncertain circumstances brought on by the global COVID-19 pandemic, all while growing, thriving and continuing our pursuit to advance medical innovation and regulatory science based on sound, consensus-based science.

It is with great appreciation and awe that I write these last expressions of gratitude to the members of our staff, our partners, collaborators and supporters around the globe. Each year for C-Path is a new beginning wherein we try again for something that is beyond attainment, but strive on; for any progress attained is a worthwhile endeavor. It is only with your support and commitment that we are able—year after year—to achieve such significant advances toward a substantially healthier world.

It is my hope that these remarks convey to you so much more than thanks.

With humility and gratitude, on behalf of our entire Board,

Timothy R. Franson, M.D.
Chairman of the Board



2020-2021 Milestones



JUNE 2021

Launch of UNITE4TB Partnership Marks a New Era in Tuberculosis Treatment Development [↗](#)

APRIL 2021

C-Path Opens Access to Duchenne Regulatory Science Consortium Database [↗](#)

MARCH 2021

C-Path Launches Acute Kidney Injury Project with Support from FDA [↗](#)

MARCH 2021

C-Path Receives FDA Qualification for the *Diary for Irritable Bowel Syndrome Symptoms-Constipation (DIBSS-C)* [↗](#)

FEBRUARY 2021

C-Path and Global Partners Launch Ataxia Consortium [↗](#)

JANUARY 2021

Diary for Irritable Bowel Syndrome Symptoms-Constipation is the First Patient-Reported Outcome Consortium Measure Used to Support an FDA-approved Label Claim [↗](#)

DECEMBER 2020

C-Path Quantitative Medicine Program Awarded FDA Contract to Develop Tools for Neuroscience Diseases [↗](#)

NOVEMBER 2020

Pediatric IBD Foundation, ImproveCareNow, C-Path to Establish the Children's Registry for the Advancement of Therapeutics [↗](#)

OCTOBER 2020

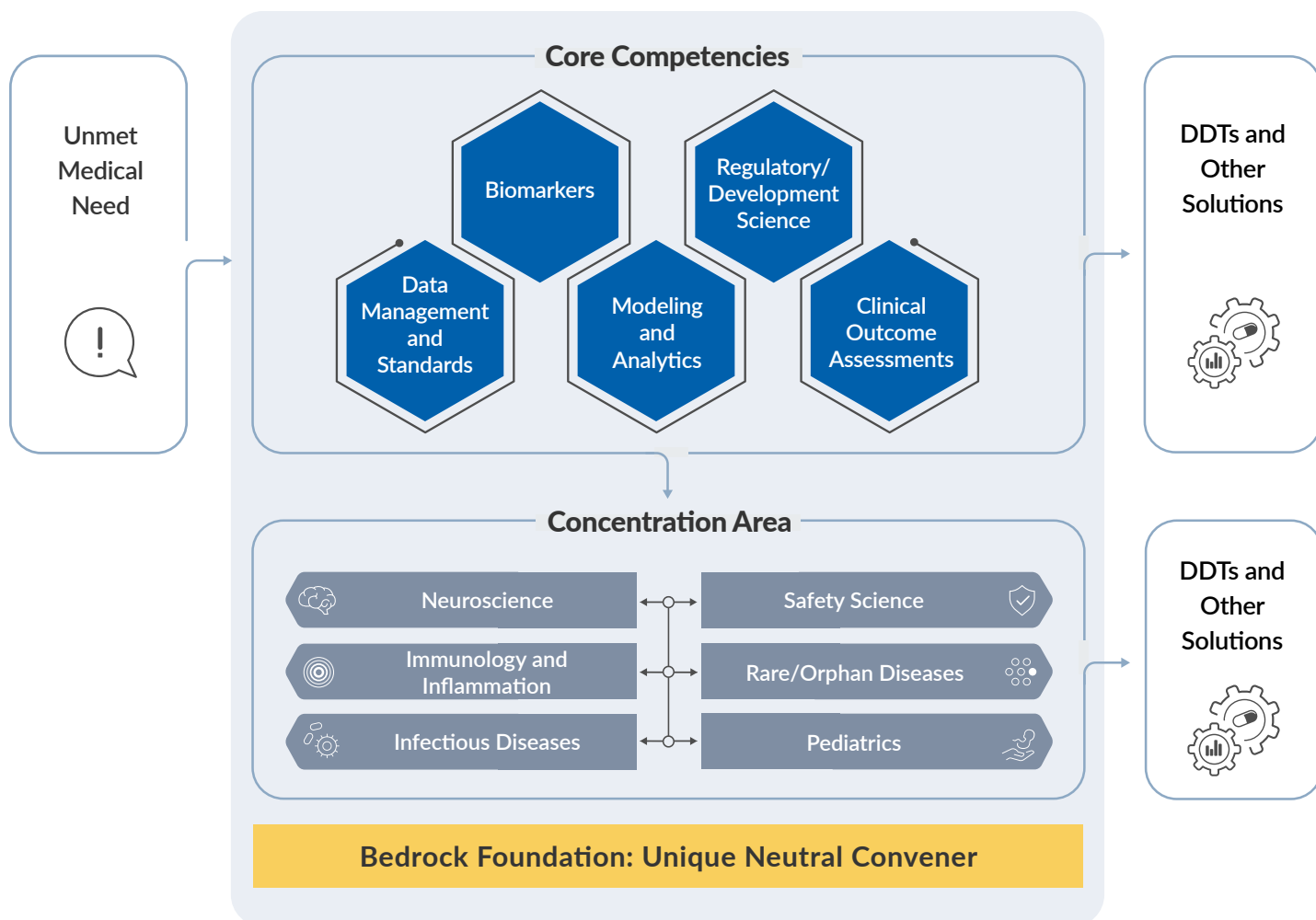
FDA Awards C-Path Grant to Use Real-world Data to Generate Real-world Evidence in Neonates [↗](#)

SEPTEMBER 2020

C-Path Launches Consortium to Accelerate Medical Product Development in Sickle Cell Disease [↗](#)

Core Competencies

C-Path is an independent entity that provides the legal, scientific, and regulatory infrastructure to generate a unique neutral environment for relevant stakeholders in the medical product development ecosystem to collaborate. This collaboration allows the sharing of information and data, which serves as the foundation for C-Path to spearhead the transformation of such information and data into actionable solutions that address specific unmet needs in the medical product development process. Such solutions can include data resources, biomarkers, clinical outcome assessment tools, clinical trial simulators and other quantitative tools. These tools and solutions help de-risk decision making in the development and regulatory review process of novel medical products. 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

DATA MANAGEMENT AND STANDARDS

C-Path's core competency in data management and standards development enables the effective integration of multiple diverse data sources into actionable databases that allow the generation of solutions to expedite medical product development and facilitate 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 whose 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 more, 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 [link](#). More recently, C-Path has begun working with Observational Health Data Science and Informatics (OHDSI) to contribute to the clinical trials and registries working groups of the Observational Medical Outcomes Partnership Common Data Model (OMOP CDM). We are also contributing to and reusing ontologies from the Open Biological and Biomedical (OBO) Foundry.

C-Path's Data Collaboration Center (DCC) was instituted to provide large-scale data resources as the foundation for the generation of actionable solutions to optimize the medical product development process. The DCC team has more than a decade of experience in data standards development, ontology development, platform development, platform hosting, data curation, stewardship of patient-level data privacy, data security and access control methodologies. 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 all

indication-focused consortia at C-Path, of which those for Alzheimer's disease, Parkinson's disease, tuberculosis, multiple sclerosis, Duchenne muscular dystrophy, Friedreich's ataxia and polycystic kidney disease are also accessible to qualified researchers here [link](#). DCC's unique abilities have been instrumental in the generation of a modern, cloud-based data and analytics platform which is being utilized in the Rare Disease Cures Accelerator and Alzheimer's disease efforts.

Additional information about three types of databases C-Path has generated can be found here [link](#), they include:

- Integrated patient-level databases for multiple diseases
- Genetic sequencing of pathogens
- Collection of nonclinical and clinical biomarker data

2

BIOMARKERS

Transforming translational sciences is another key component of the modernization of the medical product development process. C-Path leads the way in regulatory qualification of biomarkers and 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:

- Predictive Safety Testing Consortium (PSTC): FDA, EMA and PMDA qualified six nonclinical urinary biomarkers for the detection of acute drug-induced nephrotoxicity in rodents.
- Critical Path for Alzheimer's Disease: 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. In 2015, the FDA provided a Letter of Support (LOS) for cerebrospinal fluid analytes and hippocampal volume as exploratory prognostic biomarkers for enrichment in early-stage AD trials, and more recently, EMA gave the first-ever LOS for the pre-dementia disease progression model and clinical trial simulator that incorporates hippocampal volume.

- Polycystic Kidney Disease (PKD) Outcomes Consortium: FDA and EMA qualified Total Kidney Volume as a prognostic biomarker for enrichment of clinical trials in autosomal dominant 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.

3 MODELING AND ANALYTICS

Model-Informed Drug Development (MIDD) has become a main area of investment to modernize the medical drug development process. MIDD is based on the discipline of modeling and simulation, which has been a key area of expertise for C-Path since its inception. The vision of C-Path's Quantitative Medicine (QuantMed) Program is to transform drug development through methodological innovation and MIDD. QuantMed 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, the American Society for Clinical Pharmacology and Therapeutics, and the American College of Clinical Pharmacology.

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 the first endorsed quantitative drug development platform:

- 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 (also known as the "Conrado model"), which incorporates HV as an enrichment biomarker (first-ever quantitative drug development tool to receive a Letter of Support from EMA).
- Development of several quantitative drug development tools for tuberculosis, including the EMA-qualified hollow-fiber system for this disease (HFS-TB).
- Development of the model-based qualification of total kidney volume as an enrichment biomarker of trials in polycystic kidney disease, which also provided the supporting evidence for its declaration as a reasonably likely surrogate.
- The quantitative clinical trial enrichment tool for early-motor Parkinson's disease utilizing dopamine transporter imaging as an enrichment biomarker, qualified by EMA.

Currently, QuantMed is developing clinical trial simulation tools for Parkinson's disease, Duchenne muscular dystrophy, Alzheimer's disease, type 1 diabetes 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/
DEVELOPMENT SCIENCE**

The core competencies described above are facilitated by C-Path's strengths in integrating all efforts for the benefit of advancing regulatory science through multiple avenues. This includes not only the support for formal submissions for regulatory review and potential endorsement of solutions to accelerate medical product development, but also the development of consensus among experts and stakeholders within and across C-Path efforts. Such consensus is exemplified through white papers, peer-reviewed publications and other documents that help disseminate the institute's efforts, as well as inform the generation of optimized scientific paradigms for an efficient medical product development process. 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 the regulatory endorsement of specific drug development tools through the appropriate regulatory mechanism at specific regulatory agencies. This is one of the most challenging issues in the drug development tool qualification process.

In April 2016, key stakeholders including FDA's Center for Drug Evaluation and Research, C-Path and the Foundation for the National Institutes of Health Biomarkers Consortium 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 May of 2021, a collaborative group of authors from the pharmaceutical industry, the CRO sector, NIH,

FDA, academia and C-Path published a peer-reviewed article pertaining to perspectives on statistical strategies related to the process of regulatory qualification of biomarkers.

5

**CLINICAL OUTCOME
ASSESSMENTS**

Patient-centric drug development is a key aspect of optimal medical product development. By working with numerous stakeholders around the globe, C-Path has emerged a leader in the development and regulatory endorsement of patient-reported outcome measures and other clinical outcome assessments (COAs). C-Path's Patient-Reported Outcome (PRO) Consortium provides a collaborative framework for the development and qualification of COAs that can be used to optimize the evaluation of efficacy of novel medical products. In addition to efforts aimed at advancing electronic collection of COA data on existing and emerging data capture technologies more broadly, C-Path's Electronic Patient-Reported Outcome (ePRO) Consortium works closely with the PRO Consortium to make the COAs advanced by its therapeutic area working groups available in various modes of data collection. While multiple COAs are in development or other stages of qualification, the following PRO measures have obtained formal FDA qualification and are being actively deployed in clinical trials today:

- *Asthma Daytime Symptom Diary (ADSD) and Asthma Nighttime Symptom Diary (ANSDD)*
- *Diary for Irritable Bowel Syndrome Symptoms-Constipation (DIBSS-C)*
- *Non-Small Cell Lung Cancer Symptom Assessment Questionnaire (NSCLC-SAQ)*
- *Symptoms of Major Depressive Disorder Scale (SMDDS)*

New Leadership



COO Kristen Swingle Named Interim President

C-Path Chief Operating Officer Kristen Swingle expanded her role and was appointed Interim President in March of 2021, succeeding Joseph Scheeren, Pharm.D., C-Path's President and Chief Executive Officer since April 2019. Swingle has served as COO since July 2019 and is a leading voice in the Arizona bioscience community. She previously served as Vice President of Stem Cell Operations for Cord Blood Registry, a part of California Cryobank Life Sciences, specializing in newborn stem cell collection, processing and cryopreservation, bringing nearly two decades of experience in the medical and molecular sciences industry to her role leading the daily operations of the organization and the development and implementation of C-Path's global strategy and goals.



KRISTEN SWINGLE, MS



INISH O'DOHERTY, PhD

New Sr. Vice Presidents and Vice Presidents Are Selected

In March 2021, Clinical development scientist Inish O'Doherty, Ph.D. was promoted to Vice President of C-Path's newly formed Immunology and Hematology Program and Sudhir Sivakumaran, Ph.D. was promoted to Vice President for C-Path's Neuroscience Program.



SUDHIR SIVAKUMARAN, PhD

Additionally, Program Officer Stephen Joel Coons, Ph.D., was named as Senior Vice President of the Clinical Outcome Assessment Program and John-Michael Sauer, Ph.D., as Senior Vice President of Translational and Safety Sciences Program.

Jeff Barrett, Ph.D., F.C.P., was named Senior Vice President and lead for C-Path's Rare Disease Cures Accelerator-Data and Analytics Platform initiative.



STEPHEN JOEL COONS, PhD

Co-Directors Appointed to CURE Drug Repurposing Collaboratory Advisory Committee

David Fajgenbaum, M.D., M.S., MBA, and Marco Schito, Ph.D., were appointed co-directors of the advisory committee for C-Path's CURE Drug Repurposing Collaboratory (CDRC) program.

Dr. Fajgenbaum is the co-founder and executive director of the Castleman Disease Collaborative Network and one of the youngest individuals to be appointed to the faculty at the University of Pennsylvania where he directs the Center for Cytokine Storm Treatment & Laboratory.

Dr. Schito is executive director of CDRC at C-Path and an adjunct professor at the University of Arizona James E. Rogers College of Law.

New Leaders of Data Science and Quantitative Medicine Selected

In April of 2021, C-Path named Amanda J. Borens, M.S., as Executive Director of Data Science within C-Path's Data Collaboration Center and Jackson Burton, Ph.D., as Executive Director of C-Path's Quantitative Medicine Program.

Borens has more than 20 years of development, analytics and scientific experience in academia, clinical settings, health care informatics and biotech companies and has been with C-Path for nearly five years.

Prior to joining C-Path, Dr. Burton worked in industry settings conducting modeling and statistical analyses for a variety of mission-driven quantitative solutions in oncology and business decision science. He has been with C-Path for four years.

Clinical Technology Expert and Experienced Neuroscientist Join C-Path in Executive Director Roles

Scottie Kern was named as both Executive Director of the Electronic Patient-Reported Outcome Consortium and Associate Director of the Patient-Reported Outcome Consortium and Terina N. Martínez, Ph.D., as Executive Director of both the Duchenne Regulatory Science Consortium and Critical Path to Therapeutics for the Ataxias.

Kern is an expert in patient-based clinical technologies, with more than 25 years of pharmaceutical sector experience. Dr. Martínez is a neuroscientist and an expert in science communication, research program management and leadership, as well as biomarker and drug development in neurodegenerative diseases.

Prior to joining C-Path, Dr. Martínez was a Senior Associate Director, Research Programs at The Michael J. Fox Foundation for Parkinson's Research, in New York, where she led the Foundation's programs for preclinical tools and animal models, emerging targets and inflammation.



JOHN-MICHAEL SAUER, PhD



JEFF BARRETT, PhD, FCP



DAVID FAJGENBAUM, MD, MS, MBA



MARCO SCHITO, PhD



AMANDA BORENS, MSc

Thereafter, Martínez was a field application and collaboration scientist with Taconic Biosciences based out of Cambridge, MA, where she provided expert technical and scientific consultation across all research sectors for preclinical model selection, application, translational and IND-enabling study design, encompassing diverse disease and therapeutic areas.

Former FDA Commissioner and Alphabet Clinical Policy and Strategy Head appointed to Board of Directors

Robert M. Califf, M.D., MACC, head of Clinical Policy and Strategy for Google parent company Alphabet's Verily Life Sciences and Google Health divisions, was named to C-Path's Board of Directors. Dr. Califf served as the FDA Commissioner under President Barack Obama's administration from 2016-2017.

A nationally and internationally recognized expert in cardiovascular medicine, health outcomes research, health care quality and clinical research, he has led many landmark clinical trials and is one of the most frequently cited authors in biomedical science, with more than 1,200 publications in the peer-reviewed literature.

Dr. Califf was appointed FDA Commissioner in February 2016 where he was committed to strengthening programs and policies that enabled the agency to carry out its mission to protect and promote the public health.

Health Care Industry Leader Joins Board of Directors

C-Path was pleased to welcome health care industry leader and active civic involvement pioneer Mara G. Aspinall, MBA, to its Board of Directors.

Aspinall is Managing Director and Co-Founder of BlueStone Venture Partners, LLC, a venture fund investing in life sciences technology companies in the U.S. Southwest. She also serves as CEO of Health Catalysts Group.

Aspinall has extensive experience in the risk-based capital industry. In addition to BlueStone, she has served as advisor on life sciences transactions for private equity firms. As President and CEO of Tucson-based Ventana Medical Systems, now Roche Tissue Diagnostics, Aspinall led her world-class team to new financial success, more than two dozen major instrument and assay launches as well as global leadership in companion diagnostics. Previously, she served as President of Genzyme Genetics and Genzyme Pharmaceuticals. Genzyme Genetics grew into one of the country's leading diagnostic testing companies and was sold to LabCorp for \$1 billion.

She is also a Board of Directors member on Abcam, Allscripts, Castle Biosciences, OraSure, AZBio and Blue Cross Blue Shield Arizona.

We are extremely fortunate to be able to have these extraordinary and talented experts on our team.



JACKSON BURTON, PhD



SCOTTIE KERN



TERINA N. MARTÍNEZ, PhD



ROBERT CALIFF, MD, MACC



MARA G. ASPINALL, MBA

Impact

A Call to Action in Parkinson's Research and Care



Researcher Sue Dubman knew cancer ran in her family and had braced for the possibility of an eventual diagnosis. In 2009, however, she was blindsided by a diagnosis of Parkinson's disease. Undeterred, Sue knew she could make use of her experience as a researcher to provide a fresh perspective on the disease.

Sue became a patient advocate, focusing on helping accelerate the pace of delivering new treatments to patients. As a former researcher at the National Cancer Institute, she wanted to apply those same skills and experiences to a chronic and progressive neurological disease. Her goal: for Parkinson's patients to be able to have a conversation with their health care team around treating the disease, not just the symptoms.

In her opinion, Parkinson's research is stagnated by limited data-sharing. Due to a series of data-sharing issues, including studies not reaching publication and publishing delays. She says, *"The cumulative effect is that patients, neurologists, other healthcare professionals and the research community are placed in the position of making clinical care and research decisions with access to only a fraction of the relevant clinical evidence that might otherwise be available."* Sue notes that as a patient herself, she can understand both privacy concerns and the patient experience. She believes that shared data, easier access and clear documentation would go a long way towards increasing data circulation and collaboration within the scientific community.



"After a few years of denial, I knew I needed to do something and believed that my experience, as both a patient and as someone in biomedical research and care, could be put to good use so that, in the future, others and their families wouldn't have to suffer like mine."

-Sue Dubman

Data sharing is at the heart of C-Path's Critical Path for Parkinson's (CPP) [🔗](#) consortium. CPP's focus on sharing pre-competitive, patient-level data from observational cohorts and legacy clinical trials, and implementing consensus data standards, aligns with the needs of the Parkinson's community. Additionally, Parkinson's UK, a strategic partner with CPP, has been a leader in bringing together many participants in the Parkinson's research excellence network to find ways to share data and work collaboratively.

Sue served to catalyze the genesis of CPP by submitting a whitepaper that outlined CPP's mission and supporting CPP's roadmap to achieve this mission. Now, six years since CPP's launch, more than 12,000 individual records from people with Parkinson's from around the world have been integrated and shared with researchers on the cutting edge of the Parkinson's space. This database is now being used to design promising new trials and new tools.

One of CPP's latest workstreams, 3DT, works to advance digital health tools for Parkinson's clinical trials by bringing together industry, academia and patient organizations to work collaboratively. The stakeholders in this collaborative workstream see the importance of this work and share risks, learnings and costs to move drug development for Parkinson's disease forward. CPP works closely with regulatory agencies to make sure the tools generated meet regulatory requirements and standards and can be adopted as quickly as possible. The guidance from regulatory agencies has positively impacted this initiative already and further solidifies the notion that working collaboratively is the only way to move digital health tools and better clinical trials forward. The 3DT workstream is currently working with University of Rochester's Watch-PD study, funded by Biogen and Takeda, to develop and understand digital tools in early Parkinson's. All of the data from the Watch-PD study in early Parkinson's will be used to develop quantitative tools that can positively impact therapeutic development in Parkinson's.

By sharing and integrating patient-level data, the Parkinson's community can respond to Sue's call to action. Parkinson's patients and advocates are counting on worldwide collaborations like CPP to foster data-sharing and collaboration. After all, Sue says, *"For those who question, 'Why now?', I say 'Why not sooner?' I, and patients like me, can't wait 10 to 20 years for new drugs or devices to be approved."*



Impact

Generating Cutting-edge Solutions to Accelerate Drug Development



As a global leader in regulatory science, the generation of actionable quantitative solutions to accelerate drug development lies at the heart of the Critical Path Institute's (C-Path) core competencies, led by the Institute's Quantitative Medicine (QuantMed) [Program](#).

In the context of medical product development, quantitative models are mathematical tools that describe the complex relationships between relevant aspects of product development (exposure/response, disease progression, trial design, etc.).

These tools provide the basis for Model-Informed Drug Development (MIDD), or the application of a wide range of quantitative models to aid in decision-making and reduce uncertainty in medical product development. MIDD facilitates an optimized, more efficient approach to bringing novel products to patients, by reducing costs, time and risks. The U.S. Food and Drug Administration (FDA) formally recognized MIDD as an important component of the Prescription Drug User Fee Act (PDUFA) VI reauthorization, committing to advance many key FDA activities through increased MIDD efforts. However, to provide well-built quantitative models capable of achieving the many goals of PDUFA VI and of product developers, collaborative knowledge sharing efforts must take place.

Due to their complex nature, robust regulatory-ready quantitative models require significant methodological expertise, intimate knowledge of the product development ecosystem, and expertise in the intended area of application for given models.

With the rising regulatory focus on MIDD globally, C-Path formed QuantMed to lead the field in the generation of such MIDD solutions. QuantMed's goal is to leverage resources from a network of experts in industry, academia, nonprofit and regulatory sciences to develop solutions that incorporate quantitative methodologies in pharmacometrics, statistics, systems pharmacology, artificial intelligence and digital data analytics. Since its inception, QuantMed has led the generation of 10 novel tools, in close collaboration with C-Path's consortia and Data Collaboration Center. These tools are available to sponsors and regulators through various mechanisms, and six have been successfully reviewed and endorsed for use by FDA or the European Medicines Agency (EMA).

Through the generation of these solutions, C-Path's QuantMed Program has made a significant impact across a variety of chronic illnesses:



Tuberculosis [↗](#)

C-Path contributed to the approval of the first new drugs and drug-regimens against tuberculosis in over 50 years; the quantitative translational and clinical solutions now support a well-established and solid pipeline of new drugs and drug-regimens under development.



Polycystic Kidney Disease [↗](#)

C-Path paved the way for the approval of the first drug indicated to slow progression of polycystic kidney disease through the use of an imaging-based reasonably likely surrogate endpoint. The availability of a reasonably likely surrogate endpoint in this orphan indication has resulted in a healthy pipeline of new products under development.



Alzheimer's Disease [↗](#)

C-Path helped transform paradigms in clinical trials involving Alzheimer's through quantitative clinical solutions. These solutions are being implemented by developers to support the design of many clinical trials and are expected to contribute to successful new drug approvals.



Parkinson's Disease [↗](#)

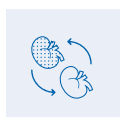
C-Path helped improve trial efficiency by generating the evidence to support a model-based imaging biomarker tool for patient selection deemed acceptable by regulators for use in Parkinson's disease clinical trials.



Type 1 Diabetes [↗](#)

C-Path further optimized patient selection for T1D prevention clinical trials by developing quantitative models that predict disease progression according to patient features and autoantibody status.

Existing efforts by QuantMed in other disease areas are also ongoing:



Kidney Transplantation [↗](#)

C-Path is generating a series of quantitative solutions to optimize the implementation of novel metrics to evaluate the efficacy and safety of novel medical products to extend the useful lifespan of kidney transplant grafts for patients in need.



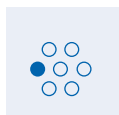
Duchenne Muscular Dystrophy (DMD) [↗](#)

C-Path recently completed a clinical trial simulation platform, comprised of quantitative solutions for multiple clinically relevant measures of DMD progression, transforming drug development paradigms for this rare condition.



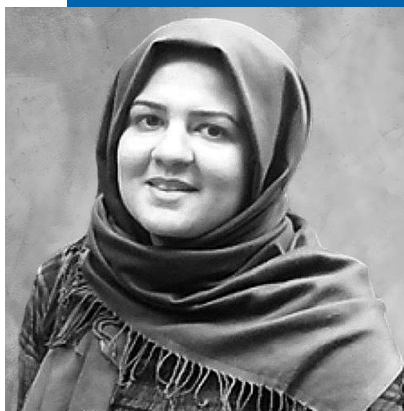
Neonatal Drug Development [↗](#)

C-Path is transforming real-world data into actionable real-world evidence in neonates, to help optimize drug development for bronchopulmonary dysplasia, as well as to improve the understanding of actionable laboratory values for future clinical trials in neonates.



Rare Diseases [↗](#)

C-Path's work is helping accelerate drug development in rare diseases, by generating actionable quantitative insights across multiple conditions, which can inform the design of clinical trials in diseases with very limited sample sizes.



Maedeh Beheshti, PhD,
Post-doctoral Fellow

"The MIDD post-doctoral fellowship at C-Path has transformed my knowledge and experience in MIDD. I have been able to work closely with experts in the field of quantitative modeling and continue to receive guidance, direction and training to better hone my modeling skills and become involved in other important projects that I wouldn't have otherwise had the chance to be a part of. Having been included in a project involving cutting-edge computer medical image analysis with increasing size and demand is an opportunity I might not have received anywhere, but C-Path. This post-doctoral fellowship has advanced my knowledge and skillset in a multitude of areas and has boosted my self-esteem and confidence with projects, especially in therapeutic areas I had previously had little exposure to. The Quantitative Medicine Program at C-Path will have a lasting impact on my career well beyond the conclusion of my fellowship."

Looking Ahead

As the world continues to grapple with the ongoing COVID-19 pandemic, it seems that there is light at the end of the tunnel thanks to the power of science. Even in the face of the pandemic, C-Path didn't slow down; our leaders, members and staff have been hard at work defining its four emerging priorities and associated initiatives that will serve to define its strategic direction for the future.

It is evident C-Path has continued to make an impact through its core competencies in data management and standards, advanced quantitative analytics, biomarkers, clinical outcome assessment tools and regulatory science. Now, in the era of knowledge management, decision science and personalized medicine, C-Path is presented with the best opportunity to grow its impact in supporting the transformation of smart data into actionable cutting-edge knowledge to de-risk and expedite medical product development.

After working with a leading global management firm, pulling in experts from across the drug development industry and health regulatory sector, and identifying internal workgroups to access, articulate and refine the process, C-Path remains laser-focused on fulfilling its vision.

C-PATH'S FOUR MAJOR INITIATIVES

- 1 Accelerating on C-Path's core purpose of advancing the science of drug development by reinforcing its core competencies
- 2 Ensuring business continuity with a sustainable business model
- 3 Growing its globalization plan and solidifying strategic partnerships
- 4 Support our stakeholders and refining processes

The focus today is on scaling-up and modernizing operations to deliver on new project commitments comprising of more than \$24 million in awards, while taking a measured approach to expand C-Path's European footprint.

The Institute is poised to build an organization optimized for scale by engaging in proactive expansion opportunities, governed by cross-discipline decision making that will enable C-Path to manage its larger portfolio with operational excellence to broaden its impact as we move toward completing our second decade together.

Our thanks go out to our community of stakeholders. We are proud of all we have accomplished together, but we know there is still work to be done. With multiple projects in the beginning stages, we look forward to the opportunities ahead to collaborate and deepen our connection with you.

Financial Update

C-Path's regulatory successes, expanding partnerships and scientific expertise have brought the Institute to an inflection point with increased revenue of 33% in fiscal year 2021 over fiscal year 2020.

Financial highlights this year include:



↗ **Design of Clinical Trials in New-Onset Type 1 Diabetes**

Together with the FDA, European Medicines Agency and in collaboration with multiple stakeholders, C-Path will provide a forum for the community and regulators to publicly engage to bridge the gap between scientific understanding of outcome measures, such as C-peptide, and their implementation in registration studies for new onset type 1 diabetes.

↗ **The Development of Tools for Neuroscience Diseases**

C-Path's Quantitative Medicine Program will develop open-source tools to improve the efficiency of trial design for three neuroscience diseases: Alzheimer's disease, Parkinson's disease and Duchenne muscular dystrophy. The team will carry out the project through its work in collaboration with C-Path's three public-private partnerships: Critical Path for Alzheimer's Disease, the Critical Path for Parkinson's and the Duchenne Regulatory Science Consortium.

↗ **Real-world Data to Generate Real-world Evidence in Neonates**

C-Path will advance standards and methodologies designed to generate real-world evidence from real-world data through a neonatal pilot project through the International Neonatal Consortium. The pilot project will include the development of a Real-world Data and Analytics Platform [↗](#).

↗ **Interoperability of Alzheimer's Disease and Related Dementia Data to Accelerate Therapeutic Innovation in Alzheimer's Disease**

C-Path partnered with the Alzheimer's Disease Data Initiative (ADDI) [↗](#), to support data accessibility to data and data-driven solutions aimed at accelerating Alzheimer's disease drug development. The Critical Path for Alzheimer's Disease [↗](#) database available to qualified researchers across the globe, is comprised of integrated, standardized and fully curated high-quality patient-level data from industry clinical trials and observational studies. This will be a relevant component of ADDI's Alzheimer's Disease Workbench [↗](#) providing enhanced access for researchers studying AD and related dementias to relevant data resources.

↗ **Launch of Two New Consortia to Address Unmet Need in Sickle Cell Disease and Ataxias**

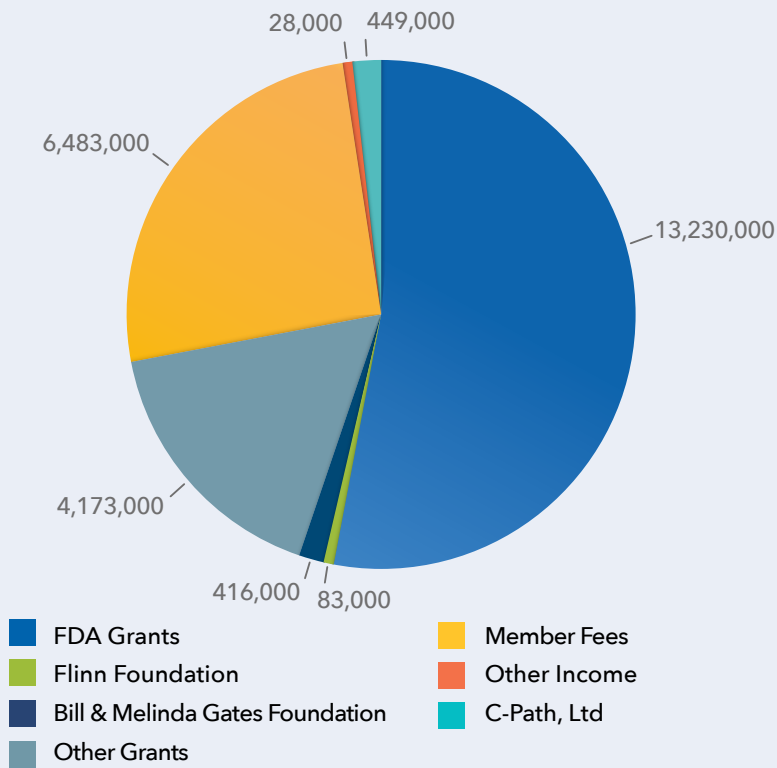
Engaging with global regulatory agencies, C-Path will work to identify and address regulatory issues that impact the development and approval of new therapies for sickle cell disease. The initiative aims to identify drug development tools, develop new trial designs using model-informed drug development and standardize data collection for adoption of master protocols.

To advance research for the ataxias, C-Path will create a neutral, precompetitive space where stakeholders from across different fields and backgrounds can come together and share expertise, insights and data, which will be leveraged for the generation of actionable solutions for drug development. The integration of data on C-Path's Rare Disease Cures Accelerator-Data Analytics Platform [↗](#) will multiply the impact of the combined ataxia data and facilitate advanced data analysis approaches that would be impossible from individual studies in isolation.

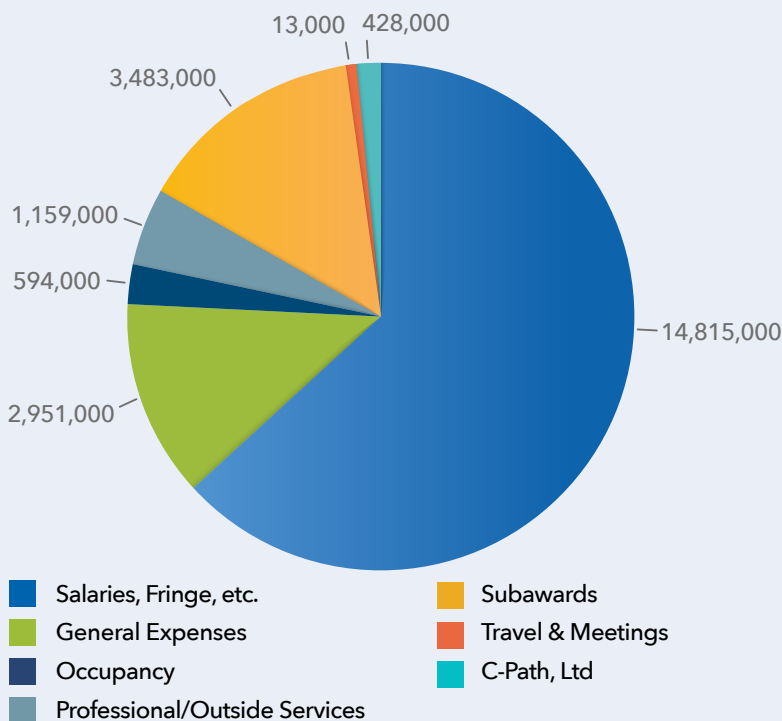


Fiscal Year 2021

C-PATH 2021 FISCAL YEAR REVENUE: \$ 24,864,000



C-PATH 2021 FISCAL YEAR EXPENSES: \$ 23,443,000



ASSETS

Cash and Cash Equivalents	\$ 16,242,943
Certificates of Deposit	\$ 249,017
Accounts Receivable	\$ 1,985,717
Property and Equipment, Net	\$ 90,400
Other	\$ 54,256

TOTAL ASSETS \$ 18,622,333

LIABILITIES AND NET ASSETS

Liabilities

Accounts Payable	\$ 654,018
Accrued Expenses	\$ 955,578
Deferred Revenue*	\$ 6,480,103
Deferred Rent	\$ 83,930

Total Liabilities \$ 8,173,629

Net Assets

Undesignated	\$ 3,816,867
Board Designated**	\$ 2,875,543
Coordinating committee designated	\$ 3,396,642
Property and Equipment	\$ 90,400
Donor Restricted	\$ 269,252

Total Net Assets \$ 10,448,704

TOTAL LIABILITIES AND NET ASSETS \$ 18,622,333

*Pre-awarded funds received for grants and consortia

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

C-Path Initiatives



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. The data is used to support research that leads to the submission of documents to worldwide regulatory agencies to qualify novel safety biomarkers for new Context 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 **Critical Path for Alzheimer's Disease (CPAD) Consortium** 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 public health 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 high-quality industry clinical trial and observational study datasets with the ultimate goal of developing a series of comprehensive disease progression models across the entire continuum of AD – from the earliest stages to severe AD – providing an invaluable tool that will aid in optimizing trial design and execution, reduction of cost and time, and reduced patient burden, with significant impact on regulatory science.

“CPAD’s data collection efforts have produced the best data source in the world for exploring predictive modeling and understanding how patient selection and trial design impact Alzheimer’s drug development and help us all do better and more efficient clinical trials.”

- Lars Lau Raket, Principal, International Project Statistician, Novo Nordisk & Special Advisor to CPAD

2020-2021 Impact

- ✓ CPAD aims to obtain regulatory endorsement for quantitative model-informed drug development (MIDD) tools that will aid in optimizing clinical trial design and execution for AD and related diseases, for specific context of use corresponding to the stages of AD as described in the 2018 FDA draft guidance "Early Alzheimer's Disease: Developing Drugs for Treatment: Guidance for Industry."
- ✓ Initiated development of statistical analysis plans, leveraging the quantitative expertise of CPAD members and the C-Path Quantitative Medicine Program, for generation of quantitative solutions in the form of disease progression models and/or time-to-event models for AD stages across the disease continuum. Preliminary modeling efforts started in August 2021.
- ✓ Spearheaded by the C-Path industry data sharing initiative, contemporary patient-level Alzheimer's clinical trial data are acquired for incorporation into a series of comprehensive disease progression models used to generate MIDD tools that will inform trial design. The AD clinical trial data repository continues to grow significantly and now contains 55 studies with 36,344 individual anonymized patient records. It has been utilized by 481 approved applicants from around the globe, covering over 200 distinct organizations from pharmaceutical industry, government agencies, nonprofit organizations, academia, and independent researchers, from more than 50 different countries.



The **Critical Path for Parkinson's (CPP) Consortium** was created in partnership with Parkinson's UK, one of the largest charity funders of Parkinson's research in EU. 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 sharing data pre-competitively aimed to optimize and improve the design of clinical trials for Parkinson's disease. CPP is focused on accelerating drug development for patients with early motor manifestations of Parkinson's disease (PD) by quantifying disease progression along the continuum and advancing drug development tools for regulatory endorsement. Early intervention holds promise in the target population, given that delaying disease progression is a priority need as expressed by people with PD. CPP also leads CPP's Digital Drug Development tool (3DT) initiative to address gaps in the advancement of digital health technologies for use in PD clinical trials by engaging FDA and EMA early and often to optimize the use of digital health technologies in PD clinical trials.

In 2021 CPP is launching two new workplans with CPP members:

1

CPP Stage 3 Core Workplan will be fully funded by industry member fees for the next three years (November 2021 to November 2024). The goal of this work plan is to address gaps in PD Drug Development by enhancing the sharing of rich data available to CPP members, including multiple stages of the disease continuum, embarking on a path to generate inventories of biomarkers, and patient-centric measures to define regulatory readiness and integrate into the PD model of disease progression.

2

CPP Digital Drug Develop Phase 3 work plan is a pay for project fully funded by industry member and nonprofit member fees over the next year (October 2021 to October 2022). The goal of this initiative is to advance the regulatory maturity of DHTs for use in PD clinical trials by enhancing the sharing of rich data available to 3DT members, embarking on a path to generate qualitative data to address clinical meaningfulness.



The **Critical Path for Sickle Cell Disease (CP-SCD)** was launched in May of 2020. It is a collaboration set up to develop community consensus on how to optimize and accelerate medical product development for this rare disease. Development of therapeutics for SCD was neglected for many years, but has recently accelerated. Maintaining momentum and rapidly developing new, more effective therapies for patients requires improved drug and therapeutic development tools to help clinical development occur faster and effectively. However, the path to regulatory approval of these new therapeutics is challenged by limited understanding of the natural history of the disease and how best to measure therapeutic effects. The mission of CP-SCD is to support collaborative development and regulatory endorsement of new medical product development tools. These tools will help to optimize and de-risk clinical trials to increase efficiency in developing and delivering safe, effective treatments for people living with sickle cell disease.



The **Critical Path to TB Drug Regimens (CPTR)** initiative was created through a partnership between the Bill & Melinda Gates Foundation (BMGF), TB Alliance and C-Path to promote innovation in regulatory science needs to accelerate development of novel drug regimens for tuberculosis (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 has been enabled by a global data-sharing initiative originally funded by the Bill & Melinda Gates Foundation and led by C-Path. This effort will be sustained for the foreseeable future through a worldwide collaboration of TB research organizations, funded by the European Innovative Medicines Initiative (IMI) AMR Accelerator program and associated partners,

including BMGF. C-Path has partnered with more than 50 organizations to participate in the European Regimen Accelerator for Tuberculosis (ERA4TB) project and the Academic and Industry United Innovation and Treatment for Tuberculosis (UNITE4TB), which are public-private initiatives working to optimize and speed up the development of new TB drug regimens. Funding for these initiatives will provide long-term, sustainable funding for the TB Data Collaboration Platform, which will include the TBPACTS database as well as data generated by IMI-funded TB projects as well as associated TB research initiatives, such as the Project to Accelerate New Treatments for Tuberculosis (PAN-TB) and the TB Drug Accelerator (TBDA).

2020-2021 Impact

- ✓ Led deployment of a TB Drug Development Information Management system to collect, organize and disseminate curated, standardized preclinical and clinical data and support data collaboration amongst consortium members
- ✓ Migrated data from the US-based TB-PACTS Data Collaboration Platform to the new EU-based TB Data Archive for use by TB research partners
- ✓ Created a new database of curated and standardized preclinical data, called the TB Platform for the Aggregation of Preclinical Experiments Data (TB-APEX) for use by TB research partners.



The **Critical Path to Therapeutics for the Ataxias (CPTA)** officially launched in February of 2021 and is a public-private partnership with the mission to optimize clinical trials for inherited ataxias. CPTA is a collaborative effort between C-Path, the Ataxia Global Initiative, the National Ataxia Foundation, Ataxia UK and key partners in the industry and academic and clinical research communities to address unmet drug development needs in the inherited ataxia space. Inherited ataxias are a diverse collection of genetic disorders that are characterized by ataxia (impaired coordination of voluntary movements, such as gait and speech). The clinical presentation is typically associated with atrophy of the cerebellum brain region. Each individual ataxia has a unique genetic cause and spectrum of clinical presentation, affecting many different body systems. For example, spinocerebellar ataxias (SCAs) often include dysphagia, stiffness, hyperreflexia, spasticity, and some cognitive impairment, while autosomal recessive ataxias often include peripheral nerve neuropathy and seizures, but also many additional symptoms specific to each disease. CPTA aims to aggregate and share existing clinical data across the inherited ataxias in order to better

inform the field-wide understanding of the natural history of these diseases. We further aim to analyze and optimize existing and new measurements of disease progression and clinical outcomes in the inherited ataxias and drive towards regulatory acceptance of new endpoints and biomarkers, which would catalyze therapeutic development in this space.

2020-2021 Impact

- ✓ CPTA officially launched, bringing together experts from different stakeholder groups across the ataxia field, in February 2021.
- ✓ CPTA members elected Irina Antonijevic as their Industry Co-Director and Michelle Campbell was appointed CPTA's FDA/CDER Liaison in May 2021.
- ✓ CPTA identified key clinical datasets that could support its activities and began negotiating data contribution agreements for two of these datasets in July 2021.
- ✓ CPTA completed due diligence and developed its official research plan, which will dictate consortium strategy and key priorities, over Q2-Q3 2021; the research plan will be submitted for formal approval in Q4 2021.



The **CURE Drug Repurposing Collaboratory (CDRC)** is a public-private partnership initiated in June 2020 by C-Path and the FDA in partnership with the National Center for Advancing Translational Sciences (NCATS), part of the National Institutes of Health (NIH).

CDRC, in partnership with the FDA-NCATS CURE ID [🔗](#) platform, is a dedicated initiative designed to capture real-world clinical outcome data to advance drug repurposing and inform future clinical trials for diseases of high unmet medical need. The initiative includes emerging/reemerging diseases, anti-microbial drug-resistant infections and neglected infectious diseases as well as rare oncology diseases where there are limited treatment options. The Collaboratory is strongly interested in capturing data from diverse populations including pediatric and pregnant women. C-Path leads CDRC, with participation from a diverse set of global stakeholders including, but not limited to, clinicians, scientists, U.S. Health and Human Services (HHS) agencies, non-government organizations, foundations and societies.

This year, CURE ID has secured a \$9.2 million grant through the HHS Assistant Secretary for Planning and Evaluation (ASPE) Patient-Centered Outcomes Research Trust Fund (PCORTF) to automate the extraction of a limited subset of deidentified data from sites around the world for the purpose of:

- Expanding the open-access CURE ID platform to include EHR/registry data
- Development and provision of a tool that will automate the extraction of data elements from EHRs and registries into CURE ID
- Procurement and synthesis of data for all COVID-19 positive patients (with future expansion capabilities to other diseases) from at least 300 sites in the US and around the world.

This infrastructure will be built for COVID-19 but will be designed in a sustainable manner so that it can be promptly deployed for future outbreaks of existing and emerging infectious diseases as well as leverage the infrastructure for diseases in the interpandemic period. This will provide real-time access to the global clinical experience of repurposing drugs when there is an immediate need to identify potential existing treatments in the absence of novel drug development. Efforts are under way to use this leave-in infrastructure to embed adaptive platform trials in clinical practice to engage community health care while minimizing the clinical burden especially during a pandemic situation.



The **Data Collaboration Center (DCC)** has the mission 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 management and analysis, creates advanced data processing workflows, builds customized data sharing platforms, and develops data standards, ontologies, and tools for interoperability.

2020-2021 Impact

- ✓ Deployed a new TB data collaboration platform in Europe in support of the ERA4TB project. This repository will serve as the main TB clinical and preclinical data archive for IMI-funded TB programs and will help to accelerate the development of new regimens for the treatment of tuberculosis in future IMI projects.
- ✓ Worked in partnership with Translational Genomics Research Institute (TGen) to develop a proof-of-concept approach to address shortcomings in the ability to track hospital-acquired antimicrobial resistant microbes impacting Arizona's health care systems.

- ✓ Developed the Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP) to accelerate the understanding of disease progression for rare diseases by providing a centralized and standardized data infrastructure and a collaborative analysis platform.
- ✓ Continued development of the Alzheimer's Disease Workbench (ADWB), in collaboration with the Alzheimer's Disease Data Initiative (ADDI), to further the mission of data sharing and collaboration for advancing AD research and establish an additional layer of analytics capabilities and data hosting in an Alzheimer's Disease Data and Analytics Platform (AD-DAP). This platform will connect directly to ADWB to bolster the analytics and modeling capabilities for use by ADWB users globally.



The **Duchenne Regulatory Science Consortium (D-RSC)** was formed in partnership with Parent Project Muscular Dystrophy (PPMD) to promote the acceleration of drug development tools and regulatory process engagement for Duchenne muscular dystrophy (DMD). DMD is a rare genetic disease that causes progressive loss of muscle, cardiac issues, the inability to walk, loss of upper body function, inability to breathe, and ultimately results in premature death. D-RSC continues to pursue its mission with its collaborative membership spanning academic and clinical researchers, nonprofit research and patient advocacy groups, and the drug development industry. After achieving the original goals of building an integrated database including data from clinical trials, natural history studies, and clinical data collections and developing a Therapeutic Area User Guide for DMD meeting Clinical Data Interchange Standards Consortium (CDISC) requirements, D-RSC's current data focus is on the integration of patient-level data from observational studies and clinical trials to generate a comprehensive clinical trial simulation (CTS) tool. D-RSC has developed mixed effects models delineating disease progression in DMD patients from the age of four and up as described by a series of endpoints.

By improving stratification approaches, trial duration, sample size, and selection of inclusion/exclusion criteria, the CTS platform will optimize clinical trial enrichment and design of studies to investigate efficacy of potential therapies for DMD.

In addition to the original goals of the consortium, D-RSC has undertaken many additional projects including support of protocol development for the Duchenne Adaptive Platform Trial (in collaboration with PPMD and the Institute for Advanced Clinical Trials for Children [I-ACT]), development of quantitative models of muscle fat fraction imaging biomarkers (with the University of Florida and ImagingDMD) and development of glutamate dehydrogenase (GLDH) as a liver safety biomarker in DMD patients (in collaboration with the Predictive Safety Testing Consortium [PSTC] at C-Path).

2020-2021 Impact

- ✓ D-RSC developed the first disease progression models incorporating five endpoints used in DMD trials. Each model explains at least 30% of the variance in progression in the population (endpoint dependent), allowing users to design shorter or smaller trials that will demonstrate if a therapy is effective.
- ✓ Submitted the final qualification package for EMA review of the clinical trial simulation platform for early-motor DMD. Feedback from EMA regarding this submission was received in April 2021, and a final qualification opinion meeting being planned.
- ✓ Type B meeting with the FDA to discuss development of a Master Protocol for the Duchenne Adaptive Platform Trial (March 2021)
- ✓ Opened the D-RSC database to approved external researchers (April 2021)
- ✓ Extended collaboration with the University of Florida and ImagingDMD to work on development of disease progression models of striatal muscle fat fraction longitudinal change, as measured by MRI (April 2021)
- ✓ Renewed support with the Institute for Advanced Clinical Trials for Children (I-ACT) to work on the Master Protocol for the Adaptive Platform Trial for Duchenne Muscular Dystrophy in collaboration with Parent Project Muscular Dystrophy (PPMD) (June 2021)
- ✓ Submitted a Fit-for-Purpose Initiative Letter of Intent for a Drug Development Tool to the FDA in partnership with ImagingDMD and the University of Florida: A modeling-based clinical trial simulation tool focused on non-invasive magnetic resonance spectroscopy-based muscle fat fraction and functional outcome measures to optimize trial design in Duchenne muscular dystrophy (July 2021).



The **Electronic Patient-Reported Outcome (ePRO) Consortium** was established to advance the science surrounding electronic collection of clinical outcome assessment data in clinical trials. 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.

FA-ICD

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 Huntington's Disease Regulatory Science Consortium (HD-RSC) was created in partnership with CHDI Foundation, a nonprofit biomedical research organization dedicated to collaboratively developing therapeutics that substantially improve the lives of those affected by HD. HD is a late-onset autosomal-dominant inherited neurodegenerative disorder characterized by motor impairment, cognitive decline, and neuropsychiatric disturbances. With the goal of accelerating the development, review and approval of medicines for Huntington's disease, this global initiative brings together biotech and pharmaceutical industry partners, academic opinion leaders and regulatory agencies to work towards regulatory acceptance of drug development tools, biomarkers and better clinical outcome assessments. HD-RSC is also building an integrated and well-curated database of HD patient-level data derived from natural history and clinical trial data from around the world. This database will enable a better understanding of disease progression dynamics across the full temporal range of HD and provides a foundation for model-informed drug development serving to de-risk HD programs and accelerate the 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. Due to reasons related to a complex and rapidly changing physiology, lack of reference standards (e.g., unknown laboratory value reference ranges, an unsystematic adverse event reporting system, etc.), and differences in perception of the importance of neonatal research between key stakeholders, drug development in neonates remains a perpetual challenge. The last drug that significantly impacted survival in preterm neonates was approved

over 30 years ago. Clinical trial activity in the neonatal population remains minimal (<1% of currently registered trials are in neonates), and the vast majority of drugs (>90%) in this population are used off-label, which greatly impacts an objective evaluation of safety and efficacy of the drugs. INC aims to accelerate the development of safe and effective therapies for neonates. INC 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 develops tools that can be incorporated into clinical trials for neonates.



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 a 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 **Patient-Reported Outcome (PRO) Consortium** brings together drug developers, measurement scientists, patients, caregivers, and other advocates, 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 (PRO) 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. This year, the PRO Consortium achieved FDA qualification of its fifth PRO measure, the *Diary for Irritable Bowel Syndrome Symptoms–Constipation (DIBSS-C)*, on December 18, 2020. In addition, the DIBSS-C abdominal symptoms subscale was included in the LINZESS® label expansion in September 2020. In addition to the five PRO measures previously qualified by FDA, PRO Consortium had 15 COAs in FDA’s COA Qualification Program at the end of this fiscal year.



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 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.



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 translational safety biomarkers (nonclinical and clinical safety biomarkers) across several organ systems for application in the development of drugs.

PredicTox KE

The aim of the **PredicTox Knowledge Environment (KE)** project is to advance the science of adverse event prediction by creating an integrated web-based knowledge environment consisting of multiple interconnected databases that capture quantitative multiscale biology of drug action. We refer to this as PredictTox-KE. The databases will contain information on clinical indications, preclinical physiological

data, and cellular regulatory networks. Although many datasets relevant to drug-induced toxicities currently exist, the advantage of a centralized repository is that data can be normalized to ontologies to ensure consistent definitions. As such, all data in PredictTox-KE will be organized within a computable framework that enables integration and analysis.

Creation of the PredicTox-KE will enable an integrative approach to prediction of major adverse events associated with therapeutic agents. This will be done as a collaborative project between researchers at Icahn School of Medicine at Mount Sinai (ISMMS) and Critical Path Institute (C-Path). Data from academia, industry and publicly available databases will be integrated, and querying tools and analytical models will be developed to enable predictive toxicology. In this initial two-year demonstration project, we propose the creation of an initial proof of concept database containing clinical, cellular, and animal data related to adverse events (i.e., cardiomyopathy) caused by small-molecule (the "NIB" class; e.g., imatinib, sorafenib, sunitinib) or antibody (the "MAB" class; e.g., trastuzumab) protein kinase inhibitors used to treat cancer. Although these targeted therapeutics are being successfully used to treat several cancers, mechanisms underlying cardiotoxicity are poorly understood, which makes improved prediction of these serious adverse events an urgent issue.



The **Quantitative Medicine (QuantMed) Program** focuses on the development and innovation of quantitative solutions for medical product development and clinical needs, with the aim of improving population and individual health by transforming drug development through methodological innovation. The program goal is to leverage knowledge from a network of experts in industry, academia, nonprofit and regulatory sciences combined with data acquired from multiple sources to develop quantitative methodologies in pharmacometrics, statistics, systems pharmacology, artificial intelligence, and digital data analytics. The solutions developed by QuantMed are often formally reviewed by regulatory agencies as fit-for-purpose in a drug development context. Because QuantMed's philosophy is one of open science, solutions developed are publicly available as open-source platforms. QuantMed is highly collaborative, partnering with both internal consortia and external members, and seeks to advance the development of novel treatments for patients with unmet medical needs.

2020-2021 Impact

- ✓ Awarded BAA contract from FDA to execute a new project focusing on Model-Informed Drug Development in the Evaluation and Development of Neurological Drug Products
- ✓ QuantMed and the T1D consortium finalized the qualification opinion document for the model-based EMA qualification of Islet Autoantibodies as enrichment biomarkers for T1D prevention

studies, thereby enabling sponsors to efficiently select patient populations for interventional trials intended to halt or stop onset of T1D.

- ✓ Completed the first-in-class comprehensive clinical trial simulation (CTS) platform for Duchenne muscular dystrophy (DMD) currently in use by sponsors to optimize trial design in this disease. The CTS platform represents the first-ever quantitative solution for five clinically meaningful outcome measures across the continuum of DMD.
- ✓ Completed the first-in-class CTS platform for Parkinson's disease (PD) currently in use by sponsors to optimize trial design in this disease. The CTS platform represents the first-ever comprehensive quantitative solution for the two most frequently used components of primary PD trial endpoints.
- ✓ The QuantMed team finalized a briefing document for FDA's Fit-for-Purpose pathway regarding a clinical trial simulation tool for Huntington's disease (HD) of which in the interim has enabled multiple industry partner-based discussions trial design considerations for active HD programs.
- ✓ The QuantMed team developed in-house algorithm-based processes to automate continued redistribution of digital data from 3DT's cosponsored WATCH-PD study, enabling key analyses to take place for advancing digital technology in Parkinson's drug development.



RARE DISEASE CURES ACCELERATOR-DATA AND ANALYTICS PLATFORM

The **Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP)** is an FDA-funded initiative that provides a centralized and standardized infrastructure to support and accelerate rare disease characterization, with the goal of accelerating therapy development across rare diseases. This platform is made possible through a collaborative grant from the FDA and in partnership with the National Organization for Rare Disorders (NORD).

RDCA-DAP promotes the sharing of existing patient-level data and encourages the standardization of new data collection. By integrating such data in a regulatory-grade format suitable for analytics, RDCA-DAP accelerates the understanding of disease progression (including sources of variability to optimize the characterization of subpopulations), clinical outcome measures and biomarkers, and facilitates the development of mathematical models of disease and innovative clinical trial designs. RDCA-DAP is positioned to generate solutions to drug development bottlenecks. As such, the utility of the patient-level data is maximized, and data may be used to develop tools that will be accessible to the community in order to optimize and accelerate drug development across rare diseases.

2020-2021 Impact

- ✓ The RDCA-DAP team increased global recognition of the RDCA-DAP initiative and drove engagement and data sharing by introducing the platform with the rare disease community (patients, patient groups, industry and academics) through meetings. Hundreds of meetings with potential data contributors and initiative collaborators were held and nearly 1,000 individuals attended RDCA-DAP's webinar series and the 2020 annual meeting.
- ✓ RDCA-DAP signed 13 Data Contribution Agreements and is currently negotiating agreements with 21 additional groups. As of June 30, 2021, the platform includes over 60 rare disease datasets in-house from 18 disease areas.
- ✓ RDCA-DAP signed memoranda of understanding with five rare diseases data companies (U.S. and international) to frame collaborations that will help increase the pool of data available and the platform functionality, visibility and value to drug development.
- ✓ RDCA-DAP completed two prototype projects to demonstrate the value of the platform by demonstrating the ability to integrate data across related diseases (polycystic kidney disease and kidney transplant) and development of a disease progression model for polycystic kidney disease. RDCA-DAP also advanced pilot analytical work for disease modeling and data-driven solutions for Freidreich's ataxia in collaboration with FARA.
- ✓ In collaboration with the DCC team, RDCA-DAP helped advance the platform development including beta-testing of the platform's first iteration (with testers from academic, data science/quantitative modeling and platform/patient group segments of our end user base) and ongoing building of an Application Programming Interface with NORD's IAMRARE® registry platform.



The **Type 1 Diabetes (T1D) Consortium** is a public-private partnership initiated in March 2017. Current membership is composed of the following industry and 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 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 **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 27 clinical trials. 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 are being leveraged by the new IMI-funded European Regimen Accelerator for TB (ERA4TB) consortium and the Academic and Industry United Innovation and Treatment for Tuberculosis (UNITE4B) consortium.



The **Trial Outcome Markers Initiative in T1D (TOMI-T1D)**, formed in May 2020, is a JDRF and Diabetes UK-funded international partnership between researchers from academic institutions, pharmaceutical industry and independent nonprofit organizations. TOMI's mission is to accelerate drug development and optimize immune intervention trials in T1D through the development of a composite outcome measure which 1) improves clinical interpretability and patient acceptability, 2) shortens the time to primary outcome and 3) minimizes the number of participants required in trials.



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.

C-Path Collaborators

ACADEMIC INSTITUTIONS

- University of Florida
- Children's Hospital of Philadelphia
- Cleveland Clinic
- University of Colorado Denver
- University of Iowa
- University of Leuven
- Children's National Health System
- Indiana University School of Medicine
- Stanford University
- University of Arizona
- University of California, Davis
- University of Leicester, UK
- Vanderbilt University Medical Center
- Case Western Reserve University
- Duke University
- Imperial College London
- Johns Hopkins University
- Mayo Clinic
- Tufts University
- Univ College London Institute of Neurology
- University of California San Francisco
- University College London Hospital
- University of Alabama - Birmingham
- University of Helsinki
- University of Pennsylvania
- University of Rochester
- University of Washington
- Washington University
- Binghamton University
- Children's National Heart Institute
- Cincinnati Children's Hospital Medical Center
- Hadassah Medical Center
- Leiden University Medical Center
- UMass Memorial
- Besta Institute
- Charité University of Medicine, Berlin
- Federal University of Rio Grande do Sul
- Houston Methodist Hospital
- Massachusetts General Hospital
- Medical University of Innsbruck
- Paris Brain Institute
- Sorbonne University
- University of Bonn
- University of Chicago
- University of Tübingen
- Akron Children's Hospital
- Brigham and Womens Hospital
- Brighton and Sussex Medical School
- City University, London
- Colorado State University
- Diderot University Paris
- Emory University
- Gazi University
- George Washington University
- Harvard University
- Helmholtz Centre Munich
- Karolinska University
- Keio University
- King's College, London
- Kyorin University
- Lund University
- McGill University
- Monash University
- Mt Sinai
- Nagoya University Hospital
- New York University
- Newcastle University
- North Shore University Health System
- OULU Finland
- Paris Descartes University-Necker Hospital

- Plymouth Univeristy
- Queen Mary University of London
- Radboud Univeristy
- Showa University
- Southern Illinois University
- St. Marianna University
- State Univ of New York - Buffalo
- Stony Brook Medicine
- Thomas Jefferson University
- Univeristy of Kansas
- University College Cork, Ireland
- University of Birmingham, England
- University of Bristol
- University of British Columbia
- University of Calgary
- University of California - Irvine
- University of California - San Diego
- University of Cambridge
- University of Capetown
- University of Chicago
- University of Genoa
- University of Glasgow
- University of Gothenburg
- University of Hasslet
- University of Illinois
- University of Liverpool
- University of Louisville
- University of Lübeck
- University of Manitoba
- University of Maryland
- University of Melbourne
- University of Miami
- University of Michigan
- University of Missouri Kansas City
- University of Montreal
- University of Munich
- University of North Carolina
- University of Oxford
- University of Pittsburgh
- University of Sheffield
- University of Siena, Italy
- University of St. Andrews
- University of Tampere
- University of Texas
- University of Toronto
- University of Turku
- University of Ulm
- University of Utah
- University of Virginia
- University of Wurzburg
- University of Zurich
- Western Washington University

PHARMACEUTICAL COMPANIES

- Novartis Pharmaceuticals Corporation
- Sanofi-Aventis US, Inc
- Takeda Pharmaceuticals North America, Inc
- Johnson & Johnson Pharmaceutical Services
- AbbVie
- Pfizer, Inc
- Biogen
- GlaxoSmithKline Corporation
- Merck Sharp & Dohme Corporation
- AstraZeneca Pharmaceuticals LP
- Eli Lilly and Company
- Genentech, Inc
- Hoffman-La Roche, Inc
- Otsuka
- Sarepta Therapeutics, Inc.
- Daiichi Sankyo
- IXICO
- UCB Pharma
- REGENXBIO
- Ultragenyx Pharmaceutical

- Vertex Pharmaceutical
- Ionis Pharmaceuticals, Inc.
- PTC Therapeutics
- Triplet Tx
- uniQure
- Amgen, Inc
- Astellas
- Bayer Pharma AG
- Bristol-Myers Squibb Company
- EMD Serono
- TEVA Pharmaceutical Industries, Ltd.
- Integral Medicines
- Global BloodTherapeutics
- IMARA Therapeutics
- Catabasis Pharmaceuticals
- Edgewise Therapeutics
- Epirium Bio
- NS Pharma
- Biohaven Pharmaceuticals
- Roche
- Servier Pharmaceuticals
- VICO Therapeutics
- Abbott Molecular
- AceLink Therapeutics Inc
- Aeglea BioTherapeutics, Inc.
- Agios Pharmaceuticals Inc.
- AKCEA Therapeutics
- Alere
- Alnylam
- Annexon
- Apellis Pharmaceuticals, Inc.
- Applied Therapeutics
- argenx
- Audentes Therapeutics
- Avrobio
- Becton Dickinson Diagnostic Systems
- BioMarin
- BioMerieux
- bluebird bio, Inc.
- Boehringer Ingelheim Pharmaceuticals, Inc
- Bridgebio
- Cabaletta Bio, Inc.
- Calico Life Sciences
- CareDx
- Celgene
- Cepheid
- Cervel Therapeutics
- Chiesi
- CSL Behring
- Cydan Corporation
- Denali Therapeutics Inc.
- Eisai, Inc
- Galapagos NV
- GE Healthcare
- Gilead Sciences
- Goldfinch Biopharma, Inc.
- Handl Therapeutics
- Hansa Medical
- Harmony Biosciences, LLC
- Healx
- Horizon Pharma, Inc.
- Immucor, Inc.
- imvaria
- Infant Bacterial Therapeutics
- Innate Immunotherapeutics
- Inoviv
- Ironwood Pharmaceuticals, Inc
- Jazz Pharmaceuticals, Inc.
- Kadmon Corporation
- LoQus23
- Lundbeck
- MeiraGTx
- Metabolic Solutions Development
- Millenium: The Takeda Oncology Company
- MindMed, Inc.
- Mironid
- Mitsubishi Tanabe Pharma Corporation
- Momenta Pharmaceuticals
- Natara
- Navitorpharma
- Nephrogen
- Neubase Therapeutics
- NeuExcell Therapeutics
- Neurametrix
- Neurocrine Biosciences, Inc.
- Novo Nordisk

- Ocello (NL)
- One Lambda, Inc.
- OrbiMed
- Palladio Biosciences
- PellaPharm, Inc.
- PharmaStat
- Prilenia
- ProventionBio, Inc
- Qiagen
- Reata
- Regeneron
- Regulus Therapeutics
- Relay Therapeutics
- Sage
- Sangamo Therapeutics
- Sequella, Inc.
- Sunovion Pharma, Inc
- Thermofisher
- Transplant Genomics
- Trove Therapeutics
- Vaccinex
- Veloxis
- Voyager Therapeutics
- Wave Life Science
- Actelion Pharmaceuticals Ltd
- Allergan
- Baxter Healthcare Corporation
- Biophytis
- CTI BioPharma
- Cycleron
- Imbria Pharmaceuticals
- Lysogene
- Mallinckrodt Pharmaceuticals
- Ovid Therapeutics, Inc.
- Oxford Brain Diagnostics
- Santhera Pharmaceuticals, Inc.
- Shire Pharmaceuticals, Inc
- Summit (Oxford) Limited
- vTv Therapeutics

PATIENT GROUPS

- CHDI Foundation
- Movement Disorders Society
- CureDuchenne
- Parent Project Muscular Dystrophy
- Ataxia Charlevoix-Saguenay Foundation
- Ataxia UK
- National Ataxia Foundation
- Alzheimer's Association
- Alzheimer's Research UK
- Astarte Ventures
- BLISS
- Cure Parkinson's Trust
- Davis Phinney Foundation
- European Huntington Association
- European Huntington's Disease Network
- Families Blossoming
- Huntington Society of Canada
- Huntington Study Group
- Huntington's Disease Society of America
- Huntington's Disease Youth Organization
- Italian MS Society
- JDRF
- KHI Patient Family Partnership Council
- March of Dimes
- Michael J Fox Foundation
- National MS Society of UK
- National Multiple Sclerosis Society
- National Multiple Sclerosis Society of Canada
- NEC Society
- NICU Parent Network
- Once Upon a Preemie
- Parkinson's Canada
- Parkinson's Disease Foundation
- Parkinson's UK

- PKD Charity
- PKD Foundation
- PMD Alliance
- Preemie World
- Speaking for Moms and Babies, Inc.
- Alzheimer's Drug Discovery Foundation
- USAgainstAlzheimer's

OTHER

- BioClinica
- Ataxia Global Initiative (AGI)
- .assisTek
- ADPKD Paediatric Registry
- Alberta MS Research Foundation
- American Society of Transplant Surgeons (ASTS)
- American Society of Transplantation (AST)
- ARegPKD Paediatric Registry
- Bambino Gesù Children's Hospital
- Benaroya Research Inst.
- Bill and Melinda Gates Foundation
- Children's Hospital of Pittsburgh
- Clinical Ink
- COINN
- Consortium of MS Centers
- EFCNI
- ERT
- FIND
- Hackensack Meridian Health
- Helmsley Trust
- Hospital do Rim - UNIFESP
- I-ACT
- IBM
- Imeka
- IQVIA
- Istituto Mario Negri
- Kayentis
- Kessler Foundation
- Koneksa Health
- Mapi Research Trust
- MedAvante
- Medidata Solutions
- Medrio
- Nagano Children's Hospital
- NANN
- National Center for Child Health and Development
- National Organization of Rare Disorders (NORD)
- Osaka City General Hospital
- Ottawa Hospital Research Institute
- PATH
- PhRMA
- Rady Children's Hospital
- Saint-Pierre University Hospital
- Saitama Medical Center
- Santa Barbara Nutrients
- Science 37
- Scientific Institute H.S. Raffaele, Italy
- SickKids
- Signant Health
- TB Alliance
- TB Clinical Diagnostics Research Consortium
- The Transplantation Society
- Treatment Action Group
- Unlearn.AI Inc.
- Versant Ventures
- Working Group on New TB Drugs (Stop TB Partnership)
- Y-Prime
- Biomedical Systems Corporation
- Terasaki Research Institute
- Health Economics

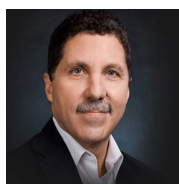
People

Leadership Team



LEWIS BARBIERI, JD

Director, Contracts, Legal Services, and Compliance



JEFF BARRETT, PhD, FCP

Senior Vice President; RDCA-DAP Lead



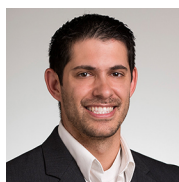
AMANDA BORENS, MSc

Executive Director of Data Science



MARTHA A. BRUMFIELD, PhD

Special Advisor, Former President and CEO



JACKSON BURTON, PhD

Executive Director, Quantitative Medicine Program



STEPHEN JOEL COONS, PhD

Senior Vice President, Clinical Outcome Assessment Program;
Executive Director Patient-Reported Outcome Consortium



GRAHAM HIGSON, MSc

Executive Director of European Office



SCOTTIE KERN

Executive Director, Electronic Patient-Reported Outcome Consortium



RICK LIWSKI

Chief Technology Officer and Director, Data Collaboration Center



TERINA N. MARTINEZ, PhD

Executive Director, Duchenne Regulatory Science Consortium;
Executive Director, Critical Path to Therapeutics for the Ataxias



INISH O'DOHERTY, PhD

Vice President, Immunology and Hematology Program; Executive Director,
Transplant Therapeutics Consortium; Executive Director, Type 1 Diabetes
Consortium; Executive Director, Critical Path for Sickle Cell Disease Consortium



KLAUS ROMERO, MD, MS, FCP

Chief Science Officer; Executive Director of Clinical Pharmacology



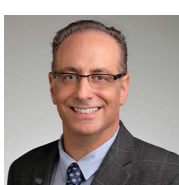
JOHN-MICHAEL SAUER, PhD

Senior Vice President, Translational and Safety Sciences Program; Executive Director,
Predictive Safety Testing Consortium, Inflammatory Bowel Disease Group and
Polycystic Kidney Disease Outcomes Consortium



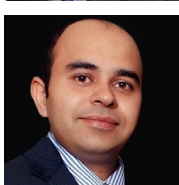
JOSEPH SCHEEREN, PharmD

Special Advisor, Former President and CEO



MARCO SCHITO, PhD

Executive Director, CURE Drug Repurposing Collaboratory



KANWALJIT SINGH, MD, MPH

Executive Director, International Neonatal Consortium



SUDHIR SIVAKUMARAN, PhD

Vice President, Neuroscience Program; Executive Director, Critical Path for
Alzheimer's Disease Consortium; Interim Executive Director, Huntington's Disease
Regulatory Science Consortium



DIANE STEPHENSON, PhD

Executive Director, Critical Path for Parkinson's Consortium



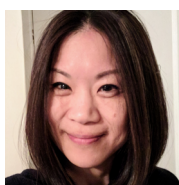
KRISTEN SWINGLE, MS

Interim President,
Chief Operating Officer



RAYMOND WOOSLEY, MD, PhD

Founder and Former CEO
Current Senior Advisor



REBECCA YUAN

Chief of Staff

Board of Directors



MARA G. ASPINALL, MBA

Managing Director and Co-Founder, BlueStone Venture Partners



D. CRAIG BRATER, MD

Vice President of Programs, Walther Cancer Foundation, Inc.



ROBERT M. CALIFF, MD, MACC

Head of Clinical Policy and Strategy, Verily Life Sciences and Google Health

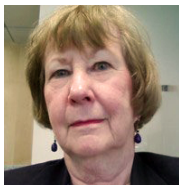


M. WAINWRIGHT FISHBURN, JR.

Founding Partner, Cooley LLP



TIMOTHY R. FRANSON, MD
Principal, Faegre Drinker Consulting



KAY HOLCOMBE, MS
Sr. Vice President for Science Policy (Retired), Biotechnology Industry Organization (BIO)



PETER BARTON HUTT, LLB, LLM
Senior Counsel, Covington & Burling LLP



JEFFREY JACOB, SM
CEO, Cancer Prevention Pharmaceuticals, Inc.
Principal, Tucson Pharma Ventures LLC



SHAUN A. KIRKPATRICK, MA
President & CEO, Research Corporation Technologies, Inc.



ALAN G. LEVIN, MS, CPA
Executive Vice President & CFO (Retired), Endo Health Solutions Inc.



JAMES W. NEWMAN, CPA
Founder, Victory Pharma, Inc.



PAULA J. OLSIEWSKI, PhD
Program Director, Alfred P. Sloan Foundation



TOMAS SALMONSON, PhD, MSc
(Former) Chair of the Committee for Medicinal Products for Human Use (CHMP),
European Medicines Agency (EMA)



ALASTAIR J.J. WOOD, MB, ChB

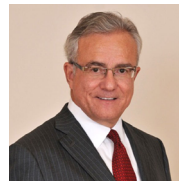
Professor of Medicine, Professor of Pharmacology, Weill Cornell Medical College, NY
Partner, Symphony Capital LLC

Advisors to the Board



SHAAVHRÉE BUCKMAN-GARNER, MD, PhD

FDA Advisor
Director, Office of Translational Sciences, Center for Drug Evaluation and Research (CDER)
U.S. Food and Drug Administration



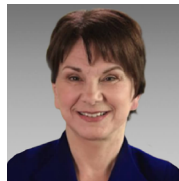
PETER B. CORR, MD, PhD

Senior Advisor
Non-Executive Director, Board of Directors, ADC Therapeutics
Co-Founder and Managing General Partner, Auen Therapeutics Management LLLP



The Honorable JAMES C. GREENWOOD

Senior Advisor
President & CEO, Biotechnology Innovation Organization (BIO)



JANET WOODCOCK, MD

FDA Advisor
Acting Commissioner of Food and Drugs, U.S. Food and Drug Administration





 1730 East River Road, Suite 200
Tucson, Arizona 85718

 520.547.3440

 c-path.org