

C-Path and Replica Analytics Collaboration Aims to Accelerate Rare Disease Research

NEW YORK, OTTAWA and **TUCSON**, Ariz., June 29, 2022 — <u>Critical Path Institute</u> (C-Path) and <u>Replica Analytics</u>, an <u>Aetion</u> company, announced today a new partnership that involves leveraging synthetic data to further catalyze the generation of actionable solutions to accelerate drug development for rare diseases.

Replica Analytics will help generate synthetic datasets across rare and orphan indications in which patientlevel datasets are often quite small, which heightens considerations regarding data privacy and accessibility.

Together with real data, these synthetic data will help maximize the utility of C-Path's Rare Disease Cures Accelerator-Data and Analytics Platform (RDCA-DAP ®), which provides a centralized and standardized infrastructure to support and accelerate rare disease characterization targeted to accelerate drug development. Additionally, the platform includes a framework that supports the rigorous conduct of natural history studies, with attention to established data quality standards, to be most useful to clinical trial design and regulatory review. It includes a robust, integrated database and analytics hub that allows for the aggregation of rare disease data from various sources and the efficient and effective interrogation of that data. Once added to RDCA-DAP, the synthetic data will also represent valuable real-world, electronic health record (EHR) based data available on the platform, opening doors to modeling and research based on longitudinal focused analyses.

"Our collaboration with Replica Analytics is important because synthetic data can add value to real-world data while observing data privacy considerations, which will help accelerate overall rare disease drug development," said RDCA-DAP Scientific Director Alexandre Betourne, Pharm.D., Ph.D. "The goal of RDCA-DAP is to provide a centralized and standardized infrastructure to support and accelerate rare disease characterization and therapy development, this collaboration is in line with our efforts."

Synthetic data generation (SDG) is a privacy enhancing technology that has been gaining rapid adoption, particularly in the life sciences sector. SDG uses AI to create machine learning models that learn the statistical patterns and properties of real datasets to generate data that retain the same characteristics as the original dataset, but with no one-to-one mapping back to an identifiable person. SDG can help amplify small datasets, simulate virtual patients to augment patients in existing datasets, and optimize the design of small sample clinical trials.

"We are certainly seeing a growing opportunity to partner with organizations like C-Path for generating datasets that are fit-for-purpose," says Khaled El Emam, Ph.D., SVP and GM of Replica Analytics. "Synthetic data, which preserves the integrity and utility of source data, as well as being privacy-protective, can be a very valuable tool to enable this collaboration."

In May 2022, C-Path hosted a webinar in which the Replica Analytics team introduced SDG and discussed the role the technology can play in researching and treating rare diseases. The event was recorded and can be viewed on C-Path's YouTube channel.



About C-Path

Critical Path Institute (C-Path) is an independent, non-profit organization established in 2005 as a public and private partnership. C-Path's mission is to catalyze the development of new approaches that advance medical innovation and regulatory science, accelerating the path to a healthier world. An international leader in forming collaborations, C-Path has established numerous global consortia that currently include more than 1,600 scientists from government and regulatory agencies, academia, patient organizations, disease foundations, and hundreds of pharmaceutical and biotech companies. C-Path U.S. is headquartered in Tucson, Arizona, C-Path in Europe is headquartered in Amsterdam, Netherlands and C-Path Ltd. operates from Dublin, Ireland with additional staff in multiple other locations. For more information, visit c-path.org.

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About Replica Analytics, an Aetion company

Replica Analytics is the premier science-based SDG technology provider to the healthcare industry. The company a pioneer in the development of unique technologies for generating privacy-protective synthetic data that maintain the statistical properties of real-world data (RWD). The company was acquired in late 2021 by Aetion, the leading regulatory-grade real-world evidence (RWE) technology provider. Replica Synthesis software provides a full suite of synthetic data generation and evaluation capabilities that can solve multiple grand challenges facing the life sciences industry, and health research in general. For more information, visit: https://replica-analytics.com



About Action

Action is a healthcare analytics company that delivers real-world evidence for the manufacturers, purchasers, and regulators of medical treatments and technologies. The Action Evidence Platform® analyzes data from the real world to produce transparent, rapid, and scientifically validated answers on safety, effectiveness, and value. Founded by Harvard Medical School faculty members with decades of experience in epidemiology and health outcomes research, Action informs healthcare's most critical decisions—what works best, for whom, and when—to guide product development, commercialization, and payment innovation. Learn more at action.com and follow us at @actioninc.

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