

C-Path's PSTC Receives Positive FDA Response for Drug-Induced Pancreatic Injury Biomarkers

Safety biomarkers aim to provide an additional tool for detecting acute drug-induced pancreatic injury (DIPI) in phase 1 clinical trials

TUCSON, Ariz., June 13, 2023 — [Critical Path Institute \(C-Path\)](#) today announced that the Biomarker Qualification Program (BQP) at the Center for Drug Evaluation and Research (CDER) in the [U.S. Food and Drug Administration \(FDA\)](#) issued a [Biomarker Letter of Support](#) (LOS) for four pancreatic injury safety biomarkers identified and evaluated by C-Path's Predictive Safety Testing Consortium ([PSTC](#))'s Pancreatic Injury Working Group (PIWG).

This set of biomarkers will help increase the ability to detect and monitor drug-induced pancreatic injury (DIPI) to better inform dosing-related decisions in clinical trials for new drugs.

The biomarkers in question are microRNAs (miRNAs), namely – miR-216a, miR-216b, miR-217, and miR-375. In conjunction with the current standard biomarkers, amylase and lipase, the four novel biomarkers represent sensitive tools to detect DIPI in phase 1 clinical trials, when preclinical information suggests the potential to cause DIPI. Sensitive DIPI biomarkers permit earlier detection of potential injury and determine if a novel therapy can be monitored for potential injury in humans better than the current options. This will improve the accuracy of clinical monitoring and increase the safety of healthy volunteers and patients, also optimizing the overall cost of clinical development of new drugs.

In its LOS, FDA stated, “We support PSTC’s initiative to encourage the voluntary and complementary use of these miRNAs in conjunction with amylase and lipase as exploratory nonclinical and clinical biomarkers of DIPI. We also support PSTC’s generation of additional nonclinical toxicology data and plan for exploratory early clinical studies to enable future formal qualification of these safety biomarkers.”

PSTC Executive Director Nicholas King, M.S. stated, “Collaboration and data sharing between PSTC’s members advanced us to this important step in the path to implementing safety biomarkers with the potential to improve detection of DIPI in drug development.”

Senior Director, Systems Toxicology, Nonclinical Drug Safety at Merck & Co., Inc. and PSTC PIWG Co-Chair Warren Glaab, Ph.D., indicated, “The FDA Letter of Support is a significant milestone demonstrating the added value of emerging safety biomarkers for drug-induced pancreatic injury. This also represents the first endorsement of miRNAs as safety biomarkers to monitor the onset of drug-induced injury. The Letter of Support also provides the foundation for further translation to clinical settings and will further enable clinical qualification of these safety biomarkers.”

Michael Ringenberg, Ph.D., Senior Scientific Director, Pathology, at GlaxoSmithKline and PIWG Co-Chair, said, “Through the identification of promising monitorable parameters, this LOS reinforces our consortia’s commitment to strengthened vigilance over drug safety in patients.”

The LOS for these DIPI biomarkers is posted on the [FDA DDT](#) website and is also accessible via C-Path’s [PSTC webpage](#).

As part of the 21st Century Cures Act, passed into law in December 2016, public-private partnerships consisting of government entities, including FDA, the biopharmaceutical industry, healthcare providers, academic researchers and patient advocacy organizations have been encouraged to work together to foster innovation in development of new therapies by qualifying new drug development tools that can accelerate the process of making new therapies available to patients. Since its formation in 2006, C-Path's Predictive Safety Testing Consortium efforts to improve drug safety monitoring have resulted in two Qualifications of 10 safety biomarkers by the FDA and European Medicines Agency (EMA) and a combined seven sets of Letters of Support from the FDA and EMA.

Any groups that would like to join this effort or obtain information or data that may be useful may contact Nicholas King (nking@c-path.org) or visit <https://c-path.org/pstc>.



About Critical Path Institute

Critical Path Institute (C-Path) is an independent, nonprofit 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.

Critical Path Institute is supported by the Food and Drug Administration (FDA) of the Department of Health and Human Services (HHS) and is 55% funded by the FDA/HHS, totaling \$17,612,250, and 45% funded by non-government source(s), totaling \$14,203,111. The contents are those of the author(s) and do not necessarily represent the official views of, nor an endorsement by, FDA/HHS or the U.S. Government.

Contact:

Kissy Black

C-Path

615.310.1894

kblack@c-path.org