

FDA Qualifies C-Path Biomarker GLDH to Detect Liver Injury in Clinical Trials

C-Path's Predictive Safety Testing Consortium led effort to qualify liver-specific biomarker to improve safety monitoring in those with muscle disease.

TUCSON, Ariz., November 19, 2025 — Critical Path Institute® (C-Path) today announced that the U.S. Food and Drug Administration's (FDA) Center for Drug Evaluation and Research, through its Biomarker Qualification Program (BQP), has qualified glutamate dehydrogenase (GLDH) as a safety biomarker to detect drug-induced liver injury (DILI) in clinical trials. The qualification represents a major advancement in drug safety science and marks the culmination of transformative collaborative work led by C-Path's Predictive Safety Testing Consortium (PSTC), in collaboration with global partners across academia, industry and regulatory agencies.

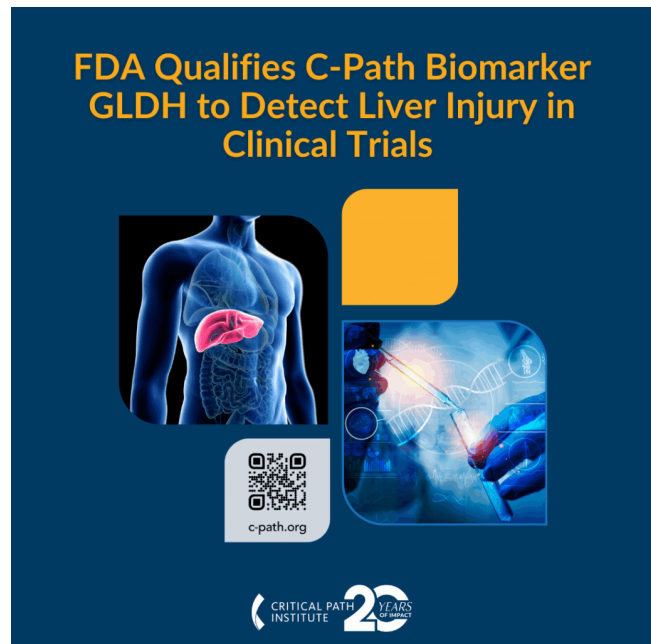
For decades, serum alanine aminotransferase (ALT) and aspartate aminotransferase (AST) have been the standard biomarkers used to detect liver injury. However, because both enzymes are also present in muscle, elevated ALT and AST levels can be difficult to interpret in people with muscle diseases or muscle injury — a challenge that has long hindered safety evaluations in many therapeutic areas, including neuromuscular diseases such as Duchenne muscular dystrophy.

Researchers from C-Path's PSTC and its Duchenne Regulatory Science Consortium (D-RSC) identified GLDH as a promising alternative because it is expressed primarily in the liver, with only trace amounts found in muscle. Unlike ALT and AST, GLDH levels are not affected by muscle damage, making it a more specific indicator of liver cell injury.

"This qualification shows what's possible when the field comes together to solve a stubborn problem," said Nicholas King, PSTC Executive Director.

GLDH can be measured in blood and, when used alongside current liver safety tests such as total bilirubin and alkaline phosphatase, provides a clearer, more reliable understanding of whether a drug is causing harm to the liver — particularly in patients where ALT and AST are elevated for reasons unrelated to liver health.

"This is an exciting step forward," said Mitch McGill, Ph.D., one of the study's lead researchers. "ALT has been the go-to test for liver injury, but it isn't specific enough. GLDH gives us a much more precise way to detect liver damage and monitor recovery."



“Currently, the FDA and pharmaceutical companies rely heavily on ALT to detect liver injury during clinical trials,” McGill said. “ALT has a few weaknesses, including a lack of specificity for the liver. Qualifying GLDH is the first big step to address those problems.”

FDA’s qualification means GLDH may now be used in clinical trials to help diagnose liver injury in participants with muscle disease or suspected muscle degeneration — an important advancement for drug developers, and for patient safety. Over time, researchers hope GLDH will be incorporated more broadly across the drug development process as a standard tool to evaluate liver health.

“This achievement reflects years of dedication by scientists across the public and private sectors,” said Jiri Aubrecht, Pharm.D., Ph.D., a lead researcher on the project and first author of a 2025 manuscript published in [Toxicological Sciences](#) summarizing this work. “It has taken more than 10 years from our first study to this qualification, and we’re thrilled to see GLDH recognized as a trusted biomarker for liver safety.”

The FDA announcement can be viewed on the agency’s [website](#), and the [submission](#) determination documentation are also publicly available.

Groups interested in joining this ongoing effort or accessing relevant data and materials may contact Nicholas King at nking@c-path.org or visit c-path.org/pstc.

Aubrecht is an adjunct professor in the Department of Oncology and the Department of Biochemistry and Molecular & Cellular Biology at Georgetown University Medical Center in Washington, D.C.

McGill is an associate professor of Environmental Health Sciences, Pharmacology and Toxicology, and Pathology at the University of Arkansas for Medical Sciences.

About Critical Path Institute

Critical Path Institute (C-Path) is an independent, nonprofit established in 2005 as a public-private partnership, in response to the FDA’s Critical Path Initiative. C-Path’s mission is to lead collaborations that advance better treatments for people worldwide. Globally recognized as a pioneer in accelerating drug development, C-Path has established numerous international consortia, programs and initiatives that currently include more than 1,600 scientists and representatives from government and regulatory agencies, academia, patient organizations, disease foundations and pharmaceutical and biotech companies. With dedicated team members located throughout the world, C-Path’s global headquarters is located in Tucson, Arizona and C-Path’s Europe subsidiary is headquartered in Amsterdam, Netherlands. For more information, visit c-path.org.

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About PSTC

C-Path’s Predictive Safety Testing Consortium (PSTC) was founded in 2006 to serve as a pre-competitive collaboration for the independent assessment, advancement, and validation of novel drug safety tests. PSTC was formed and officially announced by Health and Human Services Secretary Michael Leavitt, U.S. Food and Drug Administration (FDA) Commissioner Dr. Andrew von Eschenbach, and FDA Deputy Commissioner Dr. Janet Woodcock. Upon its inception, Woodcock described the consortium as “unprecedented” and a “shining example” of the type of work the FDA would like to see conducted. PSTC’s goal is to obtain regulatory acceptance of novel drug safety tests. PSTC brings together pharmaceutical companies to share and validate innovative safety testing methods under advisement of the FDA, its European counterpart, the European Medicines Agency, and the Japanese Pharmaceutical and Medical Devices Agency. Currently, PSTC is focused on developing and obtaining regulatory qualification of improved clinical safety biomarkers for use in drug development.

For more information: [Predictive Safety Testing Consortium](#)

About D-RSC

The Duchenne Regulatory Science Consortium (D-RSC) is a global, pre-competitive partnership founded by the Critical Path Institute (C-Path) in collaboration with Parent Project Muscular Dystrophy (PPMD) that unites industry sponsors, academic experts, patient-advocacy organizations, and regulatory agencies to accelerate therapy development for Duchenne and Becker muscular dystrophies. D-RSC develops and integrates regulatory-grade Drug Development Tools (DDTs) such as disease-progression models, clinical trial simulation platforms, imaging- and fluid-based biomarkers and standardized data frameworks, to strengthen evidence generation and improve the design, efficiency, and interpretability of clinical trials. By harmonizing diverse datasets, advancing quantitative methodologies, and fostering multi-stakeholder consensus, the Consortium supports more predictable, informative, and patient-centered development programs. Working closely with FDA, EMA, and international regulators, D-RSC drives the adoption of innovative tools and approaches that reduce uncertainty, enable smarter decision-making, and ultimately accelerate the availability of safe and effective therapies for individuals living with DMD and BMD.

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