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U.S. Food and Drug Administration and European Medicines Agency Reach Landmark Decisions on Critical Path Institute's Clinical Trial Simulation Tool for Alzheimer's Disease

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In a big step forward for Alzheimer's disease (AD) therapy development, both the U.S. Food and Drug Administration (FDA) and the European Medicines Agency (EMA) have independently reached favorable decisions on the value of Critical Path Institute's new disease simulation tool for improving trial design in mild and moderate Alzheimer's disease. The first such instrument to ever receive this regulatory designation, the tool represents an enabling advance to improve the design of future clinical trials in AD. The new tool applies computerized models to simulate "what-if" scenarios for clinical trials. The goal of this virtual tool is to serve as a public resource for sponsors designing trials of new therapies for AD.

On June 12, 2013, the FDA issued a regulatory letter to Critical Path Institute's (C-Path) consortium, the Coalition Against Major Diseases (CAMD). It stated the Agency's decision to deem CAMD's quantitative clinical trial simulation tool a "fit-for-purpose" drug development tool for Alzheimer's disease (AD). "Model-based drug development was one of the goals defined in FDA's 2004 Critical Path Initiative report, and this new tool sets the stage for applying new technologies to accelerating medical product development," said Janet Woodcock, MD, Director of the Center for Drug Evaluation and Research (CDER) at FDA.

On June 27, 2013, EMA's Committee on Human Medicinal Products (CHMP) gave the opinion that "the proposed Disease Progression and Trial Evaluation Model is suitable for use in drug development as a longitudinal model for describing changes in cognition in patients with mild and moderate AD, and for use in trial designs in mild and moderate AD." The CHMP is the EMA committee responsible for preparing opinions on questions concerning medicines for human use.

This new tool will make it possible to simulate clinical trials by integrating all relevant data so future studies will be more efficient and more likely to be successful. A basic explanation of clinical trial simulation tools can be found at <http://vimeo.com/64332443>.

Richard Lalonde, PharmD, Vice President and Global Head of Clinical Pharmacology at Pfizer, stated, "This model was made possible because major pharmaceutical companies participating in CAMD were willing to share de-identified patient-level data for over 6,000 patients who

previously participated in AD trials. The data from these trials were the basis for this model, and the FDA's decision on this tool will allow sponsors to apply modeling and simulation and launch AD trials with a higher degree of confidence. This is a great example of a rising tide lifting all boats."

This effort is testament to the power of stakeholders working together in a non-competitive fashion to pool data and develop methods that will benefit the public health.

"Alzheimer's disease clinical development tools that are derived from real-world findings, and take into account a variety of factors, help to increase our confidence that a clinical study is accurately designed to detect a treatment effect," said Richard Mohs, PhD, Vice President, Early-Phase Neuroscience Clinical Research Eli Lilly and Company and member of the CAMD coordinating committee. "This model provides valuable insight into the relatively slow rate of disease progression in a mild patient population – a critical area of focus for drug development – and guidance for designing an effective study in multiple populations, while emphasizing the need to refine the model as we work to treat patients earlier in the disease process and as new data emerges."

Martha Brumfield, PhD, President and CEO of C-Path stated, "The regulatory decisions on this tool exemplify how C-Path's efforts result in alignment between global regulatory agencies when based on consensus science and supporting data; an alignment that can result in greater efficiency in drug development. This could not have been accomplished by any one entity working in isolation."

The patient community, as active CAMD members, have played a role in the consortium since its inception. Eric Sokol, Director of Alzheimer's Foundation of America stated that, "patients and caregivers should be reassured by the positive decision on this new simulation tool that they can now participate in more efficiently designed clinical trials."

ABOUT CRITICAL PATH INSTITUTE (C-PATH): An independent, non-profit organization established in 2005 with public and private philanthropic support from the Arizona community, Science Foundation Arizona (SFAz), and the U.S. Food and Drug Administration (FDA), C-Path is committed to improving health and saving lives by accelerating the development of safe, effective medicines. An international leader in forming collaborations around this mission, C-Path has established global, public-private partnerships that currently include over 1,000 scientists from government regulatory agencies, academia, patient advocacy organizations, and thirty-five major pharmaceutical companies. C-Path is headquartered in Tucson, Arizona. For more information, visit www.c-path.org.

ABOUT THE COALITION AGAINST MAJOR DISEASES (CAMD): The mission of CAMD (<http://www.c-path.org/camd.cfm>) is to accelerate the development of therapies for neurodegenerative diseases by advancing drug development tools for regulatory approval. The consortium currently consisting of over 150 scientists from pharmaceutical companies, contract research organizations, regulatory agencies, patient advocacy organizations, academic institutions and several government agencies.

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