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FDA Endorses Biomarkers for Use in Early Stages of Brain Disease

TUCSON, Ariz., April 27, 2015 — Critical Path Institute (C-Path) announced that the U.S. Food and Drug Administration (FDA) has issued three Letters of Support to C-Path's Coalition Against Major Diseases (CAMD) consortium for the use of certain biomarkers as tools to help select clinical trial participants in the earliest stages of Parkinson's (PD) and Alzheimer's diseases (AD). These are the first Letters of Support the FDA has issued for biomarkers which can be applied in neurological disorders such as AD and PD, and follows the Letters of Support issued by the FDA and European Medicines Agency (EMA) for specific safety biomarkers to C-Path's Predictive Safety Testing Consortium (PSTC).

"These Letters of Support are intended to encourage scientists to collect data from exploratory studies, which may lead to the qualification of these biomarkers," said Janet Woodcock, M.D., director of the FDA's Center for Drug Evaluation and Research. "We are optimistic about how this effort will further advance biomarker development."

The Letter of Support for a Parkinson's disease biomarker – Dopamine Transporter Activity as assessed by single-photon emission computed Tomography (SPECT) imaging – measures the expression of dopamine nerve terminal function. Low levels of dopamine transporter binding have the potential to indicate that people with motor dysfunction are more likely to show clinical decline of PD-specific outcome measures. Using this molecular imaging biomarker is a way to identify people living with Parkinson's disease for enrollment in clinical trials that are likely to be at the earliest stages of PD.

Dr. Ken Marek, principal investigator of the Parkinson's Progression Marker Initiative (PPMI), comments, "We now suspect that previous PD clinical trials, particularly those focused on early stage PD, included many subjects that were unlikely to have PD; The FDA's Letter of Support will serve to encourage sponsors designing new PD trials to use this biomarker as an inclusion criteria and thus increase the likelihood of success of any new candidate therapy."

CAMD also received a Letter of Support for Alzheimer's disease biomarkers – which include amyloid beta protein (A?1-42), tau, and phosphotau (ptau) – proteins that can be measured in cerebrospinal fluid (CSF) as an index of what occurs in the brain. Low levels of amyloid and high levels of tau could indicate that people with mild cognitive impairment are more likely to progress to Alzheimer's dementia. In addition to the CSF biomarkers, CAMD also received a Letter of Support for the use of low baseline hippocampal volume as assessed by MRI for the potential to identify early Alzheimer's disease patients that are more likely to progress to Alzheimer's dementia during the course of an AD clinical trial.

"The development of accurate, reliable biomarkers continues to be one of the most promising methods to enrich clinical trials with populations of subjects who are more likely to have the disease of interest and progress during the course of a clinical trial, which should serve to help expedite the research and regulatory

process," says C-Path President and CEO Martha A. Brumfield, Ph.D.

These Letters of Support are intended to encourage the use of these biomarkers in clinical studies as an enrichment strategy. By using these biomarkers, clinical trial sponsors have more confidence that individuals that lack pathologic hallmarks of the disease will not be enrolled in clinical trials, thereby minimizing exposure for those who do not have the disease and increasing the chance of a successful trial.

The Letters of Support also encourage sponsors to employ Clinical Data Interchange Standards Consortium (CDISC) clinical data standards and sharing of clinical trial data. The Letters of Support for the AD and PD biomarkers are posted on the FDA DDT website.

About the Critical Path Institute

The Critical Path Institute (C-Path) is an independent, non-profit organization established in 2005 with public and private philanthropic support from the Arizona community, Science Foundation Arizona, and the U.S. Food and Drug Administration (FDA). 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 seven global, public-private partnerships that currently include over 1,000 scientists from government and regulatory agencies, academia, patient advocacy organizations, and dozens of major pharmaceutical companies. C-Path is headquartered in Tucson, Arizona. For more information, visit http://www.c-path.org.

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