Widely-used anti-inflammatory drug shows success in treatment of amyloidosis

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A recent study led by researchers from the Amyloidosis Center at Boston University School of Medicine (BUSM) and Boston Medical Center (BMC) demonstrates that diflunisal, a generic anti-inflammatory drug, successfully reduced neurological decline and preserved the quality of life in patients with familial transthyretin amyloidosis (ATTR). Diflunisal is an inexpensive and safe medication marketed over the past 40 years for arthritis and pain.

This study, published in the Dec. 25 issue of *JAMA*, is one of the first examples of successful repurposing of a generic drug to treat a rare disease. The National Institutes of Health (NIH) has advocated this research strategy as a way to increase the availability of treatments for rare diseases such as amyloidosis.

John Berk, MD, associate professor of medicine at BUSM and clinical director of the Amyloidosis Center, designed the trial, led the international consortium of researchers, and served as the study's corresponding author. Jeffrey Kelly, PhD, a biochemist at The Scripps Research Institute, and Peter Dyck, MD, a neurologist at Mayo Clinic Rochester, were instrumental in development of the study.

Familial amyloidosis is a rare inherited disorder in which mutated transthyretin protein aggregates in the blood and forms insoluble fibrils that cause tissue damage. Patients with hereditary ATTR amyloidosis develop debilitating peripheral and autonomic nerve damage, heart disease and weight loss. Untreated, patients die 10-15 years after disease onset. Investigators at centers in Sweden, Italy, Japan, England and the United States enrolled 130 patients between 2006 and 2010. Patients were randomized to receive diflunisal or placebo treatment for two years.

The investigators found that diflunisal dramatically inhibited the progression of neurologic disease while preserving quality of life when compared to placebo treatment. Known to physicians as Dolobid, generic diflunisal is inexpensive and readily available.

To date, no other drug treatment has achieved this level of benefit for this rare disorder.

"Our results show that diflunisal represents an alternative to liver transplantation, the current standard of care for this devastating disease," said Berk. "We hope that this study prompts the identification of other widely-used generic drugs for treatment of rare diseases."

"We are pleased and optimistic about the results of this study, and are encouraged about the potential for repurposing of generic medication to bring treatments to rare and more common diseases," said Robin Conwit, MD, a Program Director with the National Institute of Neurological Disorders and Stroke (NINDS).

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