

Repurposing a Nutraceutical in Myeloproliferative Neoplasms to Improve Disease Markers and Symptoms

UCI Health

趙 Chao Family Comprehensive Cancer Center

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Angela Fleischman MD PhD
MPN Physician-Scientist



Susan O'Brien MD
Key Opinion Leader in Clinical Trials



Brianna Hoover MS
Technical Expertise in Thrombosis

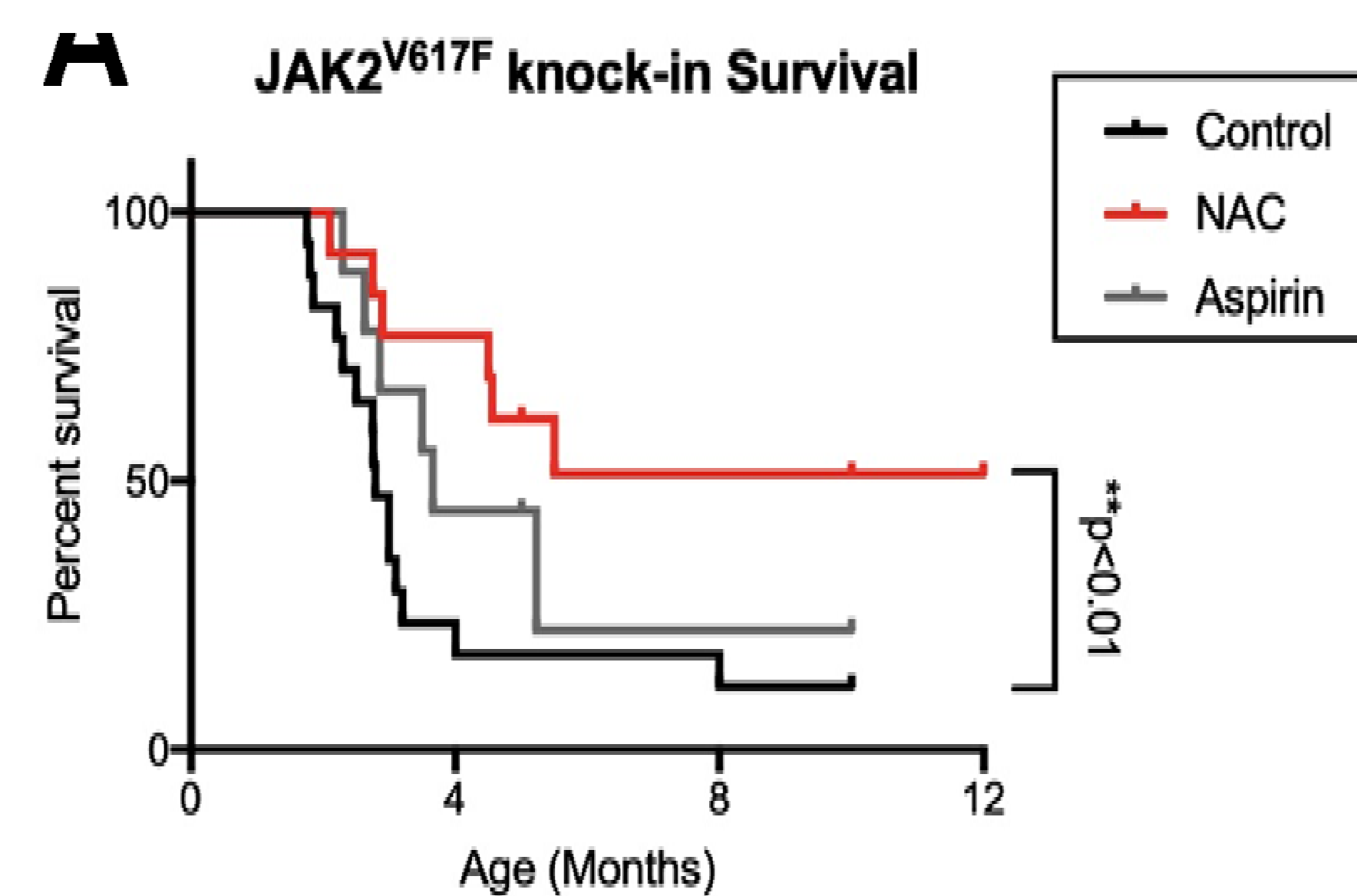
PROPOSED TREATMENT

Repurposing of N-Acetylcysteine (N-AC) in MPNs may provide an easily accessible, low risk, and low-cost therapeutic alternative for patients.

Studies of N-AC administration in high inflammatory environments, such as colitis, have demonstrated improvements in numerous inflammatory symptoms. N-AC has also been proposed to have anti-thrombotic effects in thrombotic thrombocytopenic purpura (TTP), a rare blood disorder where blood clots form in small blood vessels throughout the body. Recent findings have also found that N-AC has a positive effect in animal models of MPNs.

N-AC is currently FDA approved to treat acetaminophen overdoses, and N-AC is available over the counter in tablet and capsule formulations. Thus, N-AC is a novel, easily disseminable, and low-risk therapy to improve symptom burden, prevent thrombotic events, and ultimately reduce the risk of inflammation-driven disease progression of MPNs.

N-AC extends survival of MPN mice



SUMMARY STATEMENT

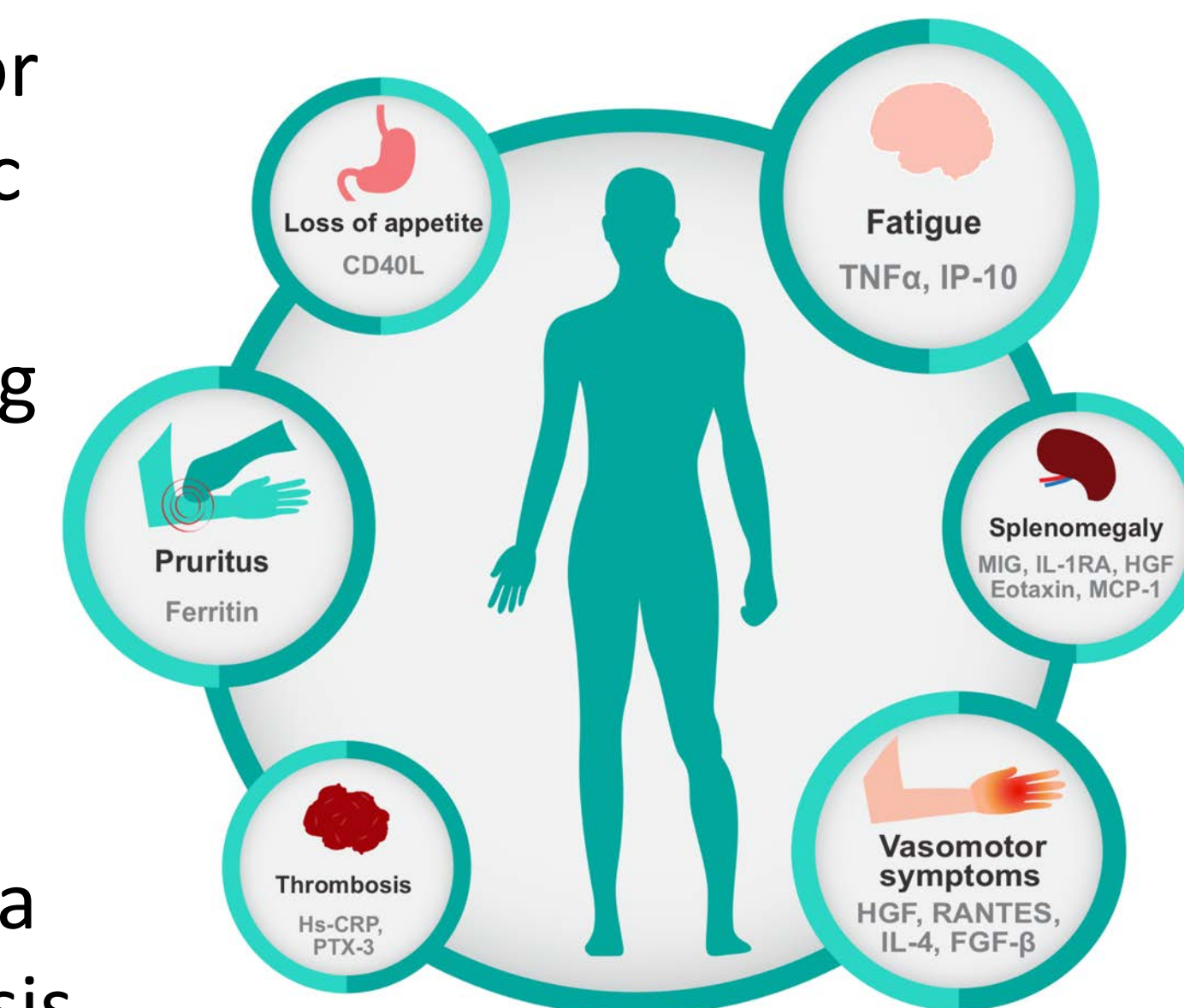
A pilot clinical trial in 50 patients with myeloproliferative neoplasms (MPNs) to measure the safety and efficacy of N-Acetylcysteine (N-AC), an inexpensive and widely available nutraceutical used to treat acetaminophen overdose

DISEASE/CONDITION

Myeloproliferative neoplasms (MPNs) are a group of chronic hematologic malignancies that include polycythemia vera (PV), essential thrombocythemia (ET), and myelofibrosis (MF). MPNs are characterized by uncontrolled production of blood cells.

MPN patients are at greater risk for blood clots and experience chronic inflammation which is responsible for debilitating symptoms including fatigue, headache, and severe itching.

MPN patients are also at risk of developing acute myeloid leukemia (AML), which has a dismal prognosis.



CURRENT TREATMENT

Current treatments for MPNs address either blood clots or symptoms, many times at the expense of each other.

There are no treatments that prevent the progression to AML.

There is a need for low-cost, low-risk treatments for early stage MPNs that address the major clinical issues.

PROJECT

We propose a clinical trial to examine the impact of N-AC in reducing thrombosis, inflammation, and symptoms in MPNs.

This is a crossover clinical trial design with an accrual goal of 50 MPN patients. Each patient will receive N-AC for 8 weeks and placebo for 8 weeks. This study is the first interventional study of N-AC in patients with MPN and is expected to achieve the following outcomes:

- Primary Outcome: reduction in symptom burden
- Secondary Outcome: reduction in laboratory surrogates for thrombosis

We anticipate this work will provide the basis for much larger studies to detect an impact on thrombosis in MPNs. Moreover, we hypothesize that N-AC has long-term beneficial effects in MPNs to prevent tumor heterogeneity that drives progression to AML.



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