ACMT Position Statement: Use of Leucovorin for Autism Spectrum Disorder

PHOENIX, AZ, UNITED STATES, July 8, 2026 /EINPresswire.com/ -- The position of the American College of Medical Toxicology (ACMT) is as follows:

American College of Medical Toxicology

The U.S. Food and Drug Administration (FDA) bypassed its typical rigorous evidentiary standard for approval of leucovorin for treatment of a rare, specific form of cerebral folate deficiency (CFD) that has significant overlap with autism spectrum disorder (ASD), a much more common diagnosis. FDA typically requires clinical trials for approval, but in this case the approval for the new indication was based on case reports and a mechanistic rationale. We are concerned that off-label use of this drug for ASD will increase health care costs and impact leucovorin availability for its established role in cancer treatment, methotrexate toxicity, and certain vitamin deficiencies. Patients and families affected by ASD have already been the target of pseudoscientific treatment and information. In some cases, these therapies have been harmful. ACMT supports use of leucovorin for ASD only in the context of clinical research until more supporting data becomes available. Robust FDA process for labeling changes should not be bypassed.

Recommendations
The American College of Medical Toxicology supports continued research on the role of folate metabolism in ASD and development of novel treatments for that condition. ACMT also recognizes that the robust framework in place for drug approval and labeling changes should not be bypassed. ACMT, along with the American Academy of Pediatrics [20] stands firmly against the premature use of leucovorin in treatment of ASD.

To read the full statement click on the link below;

https://www.acmt.net/wp-content/uploads/2026/07/Leucovorin-Position-Statement.pdf

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