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Correspondence

Retrospective analysis of small cohorts does not support therapeutic efficacy of L-arginine



I read the article by Ganetsky et al. [1] with great interest and appreciate their work in the field of mitochondrial medicine. However, I have concerns about authors' conclusion that "...IV arginine may be of benefit in improving the clinical outcome of acute neurologic decompensation in mitochondrial disease patients with stroke-like episodes."

This study is an 8 year retrospective review of medical records of 9 patients < 18 years of age at enrollment who experienced stroke-like episodes, who harbored an array of nuclear DNA and mtDNA pathogenic variants (excluding the m.3243A > G variant), who were receiving non-uniform array of treatments prior to their strokes that included arginine and citrulline, and who received a range IV arginine dosages after their stroke. No control group is available. The authors seemed to rely on statistical analyses without confidence intervals to support statements about therapeutic efficacy. The power of the study is too low and the study design contains too many potential biases for P values to assist the interpretation of the data [2]. In this type of study, the statistics are NOT a hypothesis probability and are subject to a broad array of misinterpretations [3,4].

Given the biases inherent in this study design such as selection bias and confounding, statements about therapeutic efficacy of IV arginine are not possible.

Conflicts of interest

None.

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Author title

Former CEO, Medical Neurogenetics, LLC; Exited 1/2016.

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John Shoffner Neurology, Biochemical Genetics, Molecular Genetics, Georgia, USA E-mail address: jmsiv9903@icloud.com