

# CURRENT RESEARCH ON ORGANIC ACIDEMIAS



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The Organic Acidemia Association is proud to present the first edition of *Current Research on Organic Acidemias*, an educational publication designed to highlight and promote research studies on the organic acidemias.

We need to attract more graduate students, medical students and young physician scientists to the field of inborn errors of metabolism to hasten the development of better treatments and genetic cures for these disorders. Here, we present a broad cross-section of diseases, professionals and their unique ideas and approaches that tackle some hard questions: how do we best develop viable gene therapies for such disorders, and how do we unite the academic and clinical communities to generate successful clinical trials?

The current reality for patients with organic acidemias is tenuous. Expanded newborn screening methods can detect organic acidemias early in infancy and, in the United States, is being legislated by more and more states. Better dietary and clinical management of patients allows children born with these rare disorders to sometimes live longer than in the past, but it is impossible to measure the amount of ongoing damage a child constantly accumulates as he grows. At some point, the number of organ systems affected is too great for metabolic balance to be maintained, and even the mildest of cases can cascade into a toxic crisis that is often devastating to the patient and his family.

For the past twenty years, the Organic Acidemia Association has provided support to families dealing with the incredible life changes that come when a child with an organic acidemia is diagnosed. Our organization has touched the lives of hundreds of people who might otherwise have been left grasping in the dark for information and emotional support to help deal with such life transitions. Our mission would not be complete, however, without trying to inspire more young researchers to take a look at studying organic acidemias as possible career paths. Additionally, as more of our metabolic physicians are retiring we need more clinical professionals to take their places and to understand and adequately treat the long-term complications of these diseases until cures are found. Genetic cures for the organic acidemias urgently need to be developed, but more people need to study these disorders to make this a reality.

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A handwritten signature in black ink that reads "Janice Stoebner Boecker". The signature is written in a cursive, flowing style.

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