

MAURY STRAUSS DISTINGUISHED PUBLIC LECTURE

Presented by the Fralin Biomedical Research Institute at VTC



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In Person Lecture: Gene Therapy and Liver Transplants for a Birth Defect

Urea cycle disorders are a group of rare genetic metabolic disorders that are associated with episodes of coma and brain damage. This is due to an accumulation of ammonia that is normally detoxified in the liver through a cycle of enzymes that, when functioning normally, leads to the excretion of toxic waste from protein metabolism as non-toxic urea. While urea cycle disorders occur in only about 1 in 14,000 people, rare genetic disorders as a whole occur in about 1 in 15 people. Using urea cycle disorders as a model of rare genetic disease research, in this lecture the history of the development of therapies for these disorders, the successes and failure, is discussed by Dr. Batshaw, an investigator who has worked in this field for 45 years. The current outcomes are discussed from a neurodevelopmental perspective. A major focus will be on the potential use of gene therapy and gene editing to cure these disorders.

THURSDAY, NOV. 4 at 5:30 p.m.

Room M203, 2 Riverside Circle. Registration required for in person attendance at <http://fralinbiomed.info/batshaw-lecture>. Masks must be worn. Watch live at <https://virginiatech.zoom.us/j/89350646999> or <https://fbri.vtc.vt.edu/events/live-webcast.html>.



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