



DATE: February 7, 2023 **COMMITTEE:** Senate Finance
BILL NO: Senate Bill 188
BILL TITLE: Public Health - Rare Disease Advisory Council
POSITION: Support

Kennedy Krieger Institute supports House Bill 188 - Public Health - Rare Disease Advisory Council

Bill Summary:

Senate Bill 188 establishes the Rare Disease Advisory Council to study and make recommendations on matters relating to individuals with rare diseases in the State.

Background:

Kennedy Krieger Institute is dedicated to improving the lives of children and young adults with developmental, behavioral, cognitive and physical challenges. Kennedy Krieger's services include inpatient, outpatient, school-based and community-based programs.

Kennedy Krieger treats more than 50 rare diseases in pediatric patients, including leukodystrophy, muscular dystrophy, Kabuki syndrome, Rett syndrome as well as other disorders that impact the nervous system. The Institute is home to several rare disease programs that also have been designated as Centers of Excellence by various organizations, such as the Center for Genetic Muscle Disorders, the Moser Center for Leukodystrophies, the Rett and Related Disorders Clinic, and the SYNGAP clinic.

Rationale:

For decades, medicine has offered little hope to patients with rare genetic diseases such as DYT1 torsion dystonia and X-linked adrenoleukodystrophy. There were too few patients with any one rare disease to be able to study it thoroughly, and it wasn't always possible to tell what disease a patient had. But this is just the sort of challenge that Dr. S. Ali Fatemi and members of Kennedy Krieger's neurogenetics research team take on.

Increased awareness about rare diseases through the proposed legislation is an effective approach to educating the broader community about the real-world experiences of patients living with rare diseases and their families. This approach will also help to educate individuals throughout Maryland about rare diseases and increase the services and resources available to them.

Kennedy Krieger Institute requests a favorable report on Senate Bill 188.