

## **Division of Neurology**

change the outcome

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Re: Paul's Life Circle

Every year, more than 20,000 individuals are born with tuberous sclerosis complex (TSC), a genetic condition affecting all major organ systems, including the brain, skin, heart, lungs, and kidneys. Children and adults with TSC have very high risk of developing autism, epilepsy, and cognitive disabilities in addition to tumors that can cause lifethreatening medical complications if not appropriately diagnosed and treated.

Founded in 1992 by David Franz, MD, the TSC Clinic at Cincinnati Children's Hospital has grown to become the largest clinic in the world providing comprehensive, multidisciplinary medical care and social support for TSC children and adults. The clinic started and continues to be dedicated to improving quality of life and achieving best possible treatment outcomes for TSC patients. TSC patients from the Cincinnati region are increasingly joined by patients from around the United States, North and South America, Europe, and Asia to receive personalized, state of the art treatment from the dedicated experts and staff of the Cincinnati TSC Clinic. The clinic has provided care for more than 1400 patients since 2009, including more than 650 last year alone.

The TSC Clinic at Cincinnati Children's continuously strives to improve treatment options and care delivery for patients affected by TSC through optimizing communication with patients and care providers before, during, and after clinic appointments with our team of neurologists (Drs. Darcy Krueger, MD, PhD; David Franz, MD; Jamie Capal, MD), nephrologists (Drs. Jens Goebel, MD; Stuart Goldstein, MD; Janet Boyle, MD), pulmonologist (Francis McCormack, MD), geneticist (Betty Schorry, MD), psychiatrist (Daniel Nelson, MD), and numerous additional specialists at Cincinnati Children's Hospital Medical Center. The clinical program is supplemented by robust research initiatives and collaborations at Cincinnati Children's Hospital and other leading medical centers that are aimed at earliest diagnosis and prevention of TSC-associated disease manifestations and complications, including studies with mTOR inhibitors such as rapamycin and everolimus that led to the first FDA-approved treatment specifically for TSC in 2010 and studies

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funded by the National Institutes of Health aimed at providing the earliest diagnosis and treatment of epilepsy and autism in infants with TSC.

We continue to focus attention on another equally important aspect of TSC that is often overlooked. The behavioral, learning, and social difficulties encountered by patients, family members, and care givers are tremendous demand on financial and emotional resources. In addition, children transitioning to adolescents and adolescents transitioning to adults encounter significant struggles in self-identity, self-esteem, and self-responsibility, all of which create barriers to independence, employment, and happiness. The social support programs of the TSC Clinic at Cincinnati Children's have proven essential to provide financial assistance to qualified families to travel to Cincinnati for evaluations and treatments, participate in our annual summer camp program for TSC children and adolescents, and create numerous additional outreach activities and support programs for our patients.

Thank you for generous support of Paul's Life Circle.

Darcy A. Krueger, MD, PhD

Director, TSC Clinic at Cincinnati Children's Hospital Medical Center Associate Professor of Clinical Pediatrics and Neurology