



## **Award from Patient-Centered Outcomes Research Institute to Study Pediatric Transverse Myelitis**

The first multi-center, innovative, pediatric transverse myelitis study led by University of Texas Southwestern in Dallas, along with the Transverse Myelitis Association, Johns Hopkins Transverse Myelitis Center, Children's Hospital of Philadelphia, Kennedy Krieger Institute, and Hospital for Sick Kids in Toronto, has recently received a research award from the Patient-Centered Outcomes Research Institute (PCORI). The study is part of a portfolio of projects that will advance the field of patient-centered comparative effectiveness research and provide patients with information that will help them make better-informed decisions about their care.

Dr. Benjamin Greenberg, MD, MHS, Director of the TM and NMO Center will lead the research project at UTSW in Dallas. The proposed study, entitled the 'Collaborative Assessment of Pediatric Transverse myelitis: **Understand, Reveal, Educate**' or **CAPTURE**, will be the first to combine assessments from health care providers and patients relative to pediatric TM outcomes. The *collaboration* will involve multiple health care centers across North America, the Transverse Myelitis Association and most importantly, patients. It will *assess* the current state of *Pediatric TM* in terms of diagnosis, treatment and outcomes. Ultimately, it will lead to an improved *understanding* of the current status of care for individuals afflicted with TM and *reveal* what are the current best practices. Patients will *educate* clinicians and the study will *educate* the broader health care system about what outcomes are important and achievable. It will develop a multi-metric outcome measure based on combined patient generated and provider generated data that can be used in future controlled trials

"Pediatric transverse myelitis is a rare potentially devastating condition that affects children of all ages. To date, there has never been a coordinated effort to understand the patient experience or clinical outcomes," said Dr. Greenberg. "This PCORI grant is the first opportunity for an international effort to collect data about pediatric TM with an intense emphasis on patient and family reported outcomes. Working with outstanding institutions and the TMA, we plan to enroll families from around North America and gain a better understanding of this condition. Partnering with our patients and families is an exciting and welcome opportunity to make meaningful advances!"

Dr. Sanford Siegel, President of the TMA added, "The parents in our community are incredibly well educated about transverse myelitis and they are informed and effective advocates for their children's medical care. They are well positioned and highly motivated to make a significant contribution to the pediatric study of this rare and challenging neuro-immunologic disorder. To date there has not been a single study or clinical trial to guide any treatment of transverse myelitis or to guide the treatment of any of the symptoms from this disorder. The CAPTURE project represents the first study to systematically analyze the most effective acute treatments and symptom management practices in transverse myelitis. The results of this work will have critical and far-reaching benefits for everyone who receives the transverse myelitis diagnosis."

"This project was selected for PCORI funding not only for its scientific merit but also for its potential to fill an important gap in our health knowledge and ultimately help patients and those who care for them

make more fully informed decisions about their care,” said PCORI Executive Director Joe Selby, MD, MPH. “The project reflects PCORI’s commitment to support patient-centered comparative effectiveness research, a new approach to health research that emphasizes the inclusion of patients and caregivers at all stages of the study process. We look forward to following the study’s progress and working with UTSW and the collaborating organizations to share the results.”

The study is one of 71 projects totaling more than \$114 million approved for funding by PCORI’s Board of Governors on Tuesday, Sept. 10. The awards were a mix of projects that included studies specifically targeting improvement of research methods. All were selected through a highly competitive review process in which scientists, patients, caregivers, and other stakeholders helped to evaluate more than 570 proposals that responded to five PCORI funding announcements.

Proposals were evaluated on the basis of scientific merit, how well they engage patients and other stakeholders, their methodological rigor, and how well they fit within PCORI’s national research priorities. The awards are part of PCORI’s latest round of primary research funding. Through previous funding cycles, including a round of pilot projects, and other initiatives, PCORI has committed a total of \$304 million since 2012 to support patient-centered comparative effectiveness research.

For more information about PCORI funding, visit <http://pcori.org/funding-opportunities>.

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#### **About TMA**

The Transverse Myelitis Association (TMA) is a not-for-profit international foundation dedicated to the support of children, adolescents, and adults with a spectrum of rare neuro-immune disorders – Transverse Myelitis, Neuromyelitis Optica and Acute Disseminated Encephalomyelitis. Membership of the TMA includes individuals with these rare disorders, their family members and caregivers, and the medical professionals who treat individuals with these disorders. The TMA currently has over 10,500 members from more than 80 different countries and has a large number of support groups across the United States and around the world. More information is available at <http://myelitis.org>.

#### **About PCORI**

The Patient-Centered Outcomes Research Institute (PCORI) is an independent, non-profit organization authorized by Congress in 2010. Its mission is to fund research that will provide patients, their caregivers and clinicians with the evidence-based information needed to make better-informed health care decisions. PCORI is committed to continuously seeking input from a broad range of stakeholders to guide its work. More information is available at [www.pcori.org](http://www.pcori.org).