



**Summaries of UW ICTR Funded Patient-Centered Outcomes Research Awards, 2015**

**1. PI: Matthew Halanski, MD, UW School of Medicine & Public Health**

**Title:** *Improving the Care of Children with Spinal Muscular Atrophy*

Academic Collaborators: Mary Schroth, MD, UW SMPH; Karen Patterson, MS, UW SMPH

Community Collaborators: Families of Spinal Muscular Atrophy, UW Hospitals and Clinics

**Summary**

Spinal Muscular Atrophy (SMA) is a deadly childhood disorder without a cure, and children with this disorder require 24/7 care. Due to the incidence of the disease (1/6,000-10,000), no single center cares for enough patients to develop evidence-based guidelines, and no system exists to compare the effectiveness of the various treatments being used to treat the SMA population. Our goal is to develop an electronic multicenter, multidisciplinary database containing validated outcome measures, and clinical data to allow comparisons between different treatments and outcomes to determine the optimal care for the complex children. In addition to medical data, we will gather patient/family-entered data and quality of life measures to evaluate how treatments affect patient and family life. With the data and lessons learned from this pilot project, we will submit a PCORI grant to expand the database allowing clinicians to compare the effectiveness of various interventions in the SMA population.



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