

Pediatric-Onset Opsoclonus Myoclonus Ataxia Syndrome (POOMAS) International Registry

Mark Gorman, MD
on behalf of: *POOMAS-affiliated PIs*



Boston Children's Hospital
Until every child is well™

POOMAS Registry

- ❖ First conceptualized at the 2016 OMS International Workshop (Abingdon, England)
- ❖ Collaborative network of researchers in the United Kingdom (England), European Union (France, Germany), Switzerland, United States
- ❖ Enrolling at BCH since mid-2018
- ❖ Funding: Lauren Mantz Fund for OMS Research, OMS Life Foundation, Boston Children's Hospital OMS Research Fund

POOMAS Registry

❖ **Steering Committee/OMS Study Group Database Task Force**

- ❖ Kumaran Deiva, MD, PhD (Hôpitaux Universitaires Paris Sud, France)
- ❖ Ming Lim, MD, PhD (Evelina London Children's Hospital, United Kingdom)
- ❖ Andrea Klein, MD (University Children's Hospital Basel, Switzerland)
- ❖ Barbara Hero, MD (University Children's Hospital Cologne, Germany)
- ❖ Mark Gorman, MD (Boston Children's Hospital, United States)

❖ **Other Participating Investigators**

- ❖ Sarah Hopkins, MD, MSPH (Children's Hospital of Philadelphia, United States)
- ❖ Yasmin Khakoo, MD (Memorial Sloan Kettering Cancer Center, United States)
- ❖ Elizabeth Sokol, MD (Chicago Lurie Children's Hospital, United States)
- ❖ Timothy Lotze, MD (Texas Children's Hospital, United States)
- ❖ Wendy Mitchell, MD (Children's Hospital Los Angeles, United States)

Specific Aims

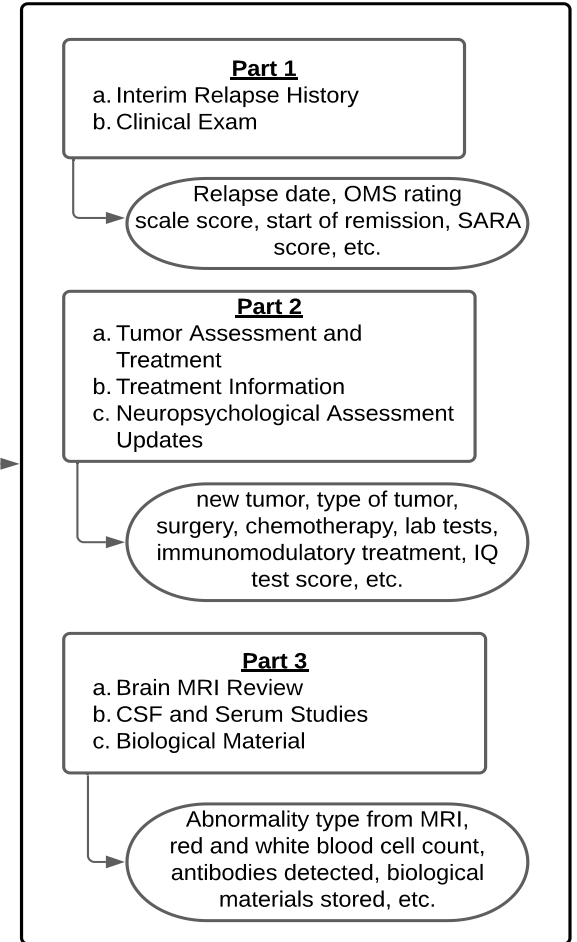
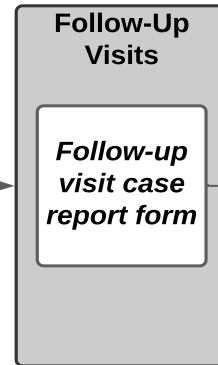
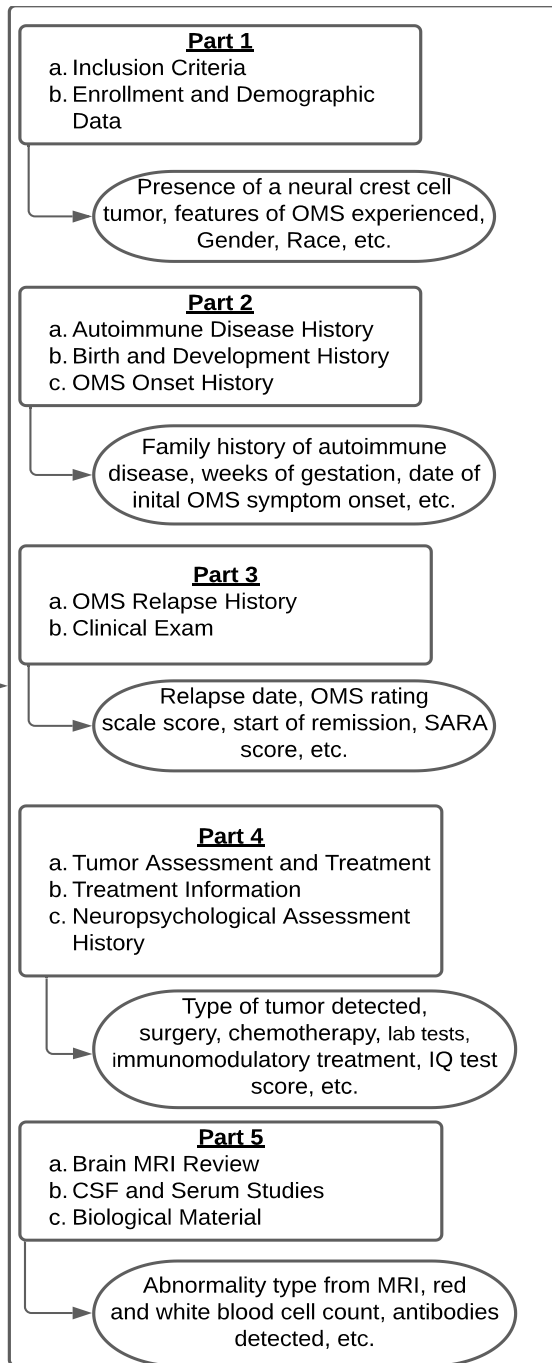
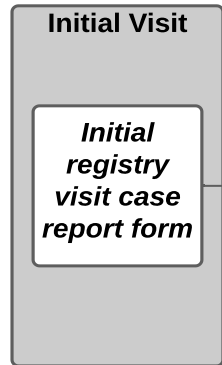
- ❖ To determine the course of illness, prognostic factors, and treatment efficacy in an international database of children with OMS
- ❖ To create a registry of available biological material and MRI linked with clinical information in children with OMS
- ❖ To establish a patient base for future OMS studies, including clinical trials
- ❖ To encourage further academic study, initiative, and publication, accelerating the future of OMS research

Study Design

- ❖ Longitudinal, observational natural history of consecutive subject visits at participating study sites
- ❖ Data collected only at clinically indicated visits (i.e. no study specific visits)
 - ❖ Patients on immunotherapy: data entry anticipated every ~3 months
 - ❖ Patients off immunotherapy: data entry anticipated every ~12 months

Study Design

- ❖ ‘Tiered’ enrollment structure
 - ❖ “Prospective”: enrolled with 24 months of OMS onset
 - ❖ “Retrospective”: enrolled >24 months after OMS onset
- ❖ Inclusion criteria:
 - ❖ Formal diagnosis of Opsoclonus Myoclonus Syndrome
 - ❖ Age of onset < 18 years old



Focus areas

- ❖ Describe variations in disease course, prognostic factors, and efficacy of different treatment regimens among children with OMAS
- ❖ Identify and assess the triggers, characteristics, and outcomes associated with relapsing courses of OMAS and the prevention thereof
- ❖ To provide infrastructure and support hypothesis generation for clinical trials and observational studies related to pediatric-onset OMAS

Progress

- ❖ Developed research protocol, CRFs, REDCap database
 - ❖ Manuscript describing registry near finalized
- ❖ Initial IRB approval granted, Boston Children's Hospital (2018)
 - ❖ 72 participants enrolled at BCH
 - ❖ 145 participants enrolled across POOMAS Network (goal 400)
- ❖ Additional Sites Enrolling Participants:
 - ❖ Children's Hospital of Philadelphia
 - ❖ Children's Hospital Los Angeles
 - ❖ Memorial Sloan Kettering Cancer Center
 - ❖ University Children's Hospital Basel
 - ❖ Chicago Lurie Children's Hospital
 - ❖ Texas Children's Hospital
 - ❖ Evelina London Children's Hospital

Progress

- ❖ Active Onboarding in progress at:

- ❖ Hôpitaux Universitaires Paris Sud

- ❖ University Children's Hospital
Cologne

- ❖ Conference calls with Steering Committee (2016-present)

- ❖ Consortium-wide PI & coordinator calls, bi-monthly (2020-present)

**5/31/2018
START DATE**

POOMAS Registry Enrollment Overview

Subject Enrollments, Site-Specific

ENROLLMENT TIMELINE

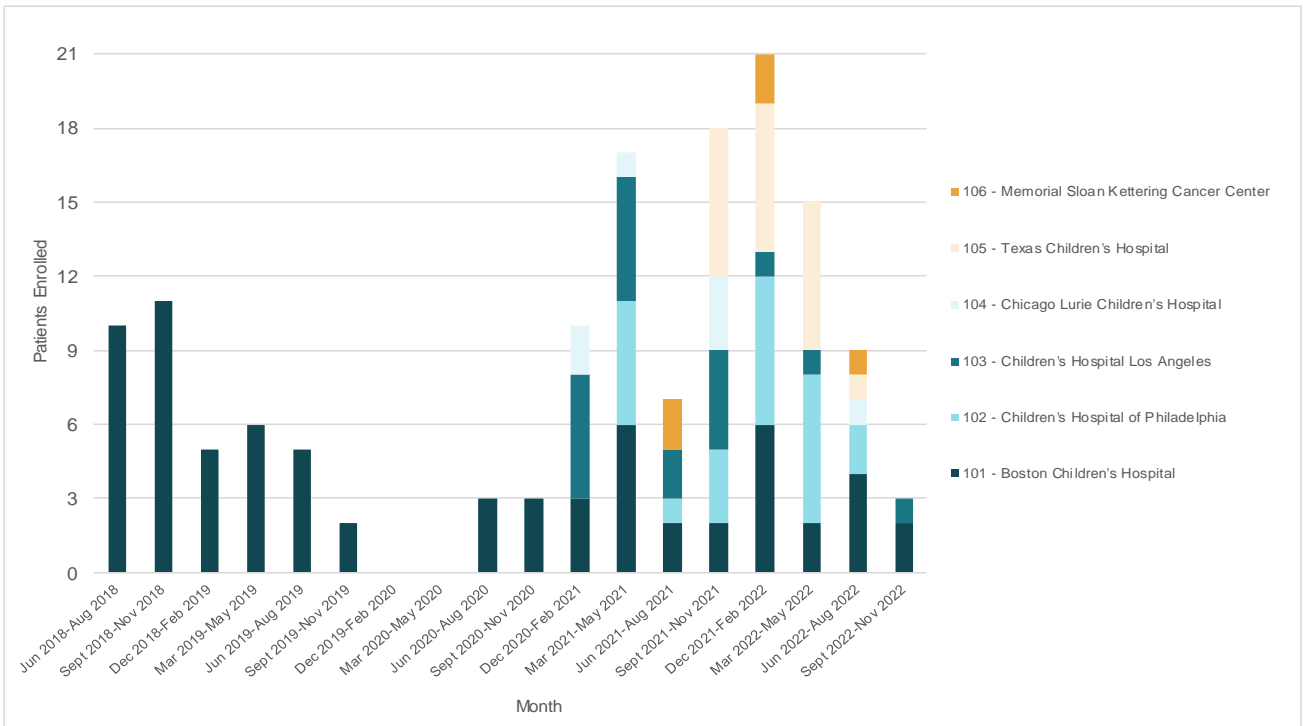
72
BOSTON CHILDREN'S ENROLLMENTS

145
TOTAL ENROLLMENTS

350
TARGET ENROLLMENTS

8 of 10

SITES ACTIVATED



Site	Date Activated	Subjects Enrolled	Months Active	Patients/Month
101 - Boston Children's Hospital (New)	5/31/2018	72	52	1.4
102 - Children's Hospital of Philadelphia	6/8/2021	23	16	1.4
103 - Children's Hospital Los Angeles	1/15/2021	19	21	0.9
104 - Chicago Lurie Children's Hospital	10/15/2020	7	24	0.3
105 - Texas Children's Hospital	1/15/2021	19	21	0.9
106 - Memorial Sloan Kettering Cancer Center	4/21/2021	5	17	0.3
201 - Evelina London Children's Hospital	9/26/2022	0	0	-
301 - University Children's Hospital of Basel	9/26/2022	0	0	-
401 - Hôpitaux Universitaires Paris Sud	-	-	-	-
501 - University of Cologne	-	-	-	-

Enrolling about 5 patients per month

Preliminary data (N=86)

Female		53 (61.6%)
Age of OMAS onset (months)		26.74 ± 22.57
Disease duration at enrollment (years)		4.86 ± 4.38
Prospective enrollment (<2y from onset)		35 (40.7%)
Tumor detected		51 (59.3%)
Course		
	Multiphasic	43 (50.0%)
	Monophasic	39 (45.3%)
	Unknown	4 (4.7%)
Parental History of Autoimmunity		26 (30.2%)

Next steps

- Submit registry description manuscript
- Validate all data on 145 enrolled patients
- Project focused on relapses by Dr. Laura Saucier (neuro-immunology fellow at BCH)
 - Number, rate, timing, and severity
 - Predictors of relapse rate
- Continue to expand the cohort at existing sites, potentially additional sites and/or via virtual recruitment (possibly through OMS Life)