

Session 4.2 - Development of a real-world evidence (RWE) roadmap to advance OMAS understanding & advocacy- Scott Milligan

Abstract:

Background & Objective: Readily-available healthcare data and AI advancements have enabled patient advocacy groups (PAGs) to become hubs for data-driven research. Our objective is to establish a strategic plan for using real-world evidence (RWE) to benefit those affected by Opsoclonus Myoclonus Ataxia Syndrome (OMAS).

Methods: The roadmap will be informed by research agendas of PAGs with natural history registries (n=188, The Roadmap Project). Research objectives will be gathered from people affected by OMAS (patient, caregiver, family member), advocates, and researchers directly or via structured online forms. Data acquisition approaches will be primary collection (registry, surveys), consented health records, and/or consented tokenization of patient identifiers. Publication plans will consider all healthcare stakeholder audiences.

Results: The RWE roadmap will have defined objectives, methods, and dissemination plans, among which is the creation of a standardized data repository for use by academic researchers.

Conclusion: This strategic plan will advance OMAS understanding & advocacy by leveraging RWE and facilitating collaborative research initiatives.

Reference: Korsunskaja, A., Bolden, S. E., Repasky, M., Zuccato, M., Fajgenbaum, D. (2023) The ROADMAP Project. <https://www.everycure.org/roadmap>