# Development of a real-world evidence (RWE) roadmap to advance OMAS understanding & advocacy

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

Page 2 of 25 Development of a RWE roadmap to advance OMAS understanding & advocacy







What can we accomplish by the next Oxford Conference?

Page 3 of 25 Development of a RWE roadmap to advance OMAS understanding & advocacy





11th International Workshop on vpr 10-12 2025 | Oxford. UK

# WHY REAL-WORLD DATA?

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Page 4 of 25 Development of a RWE roadmap to advance OMAS understanding & advocacy

<sup>1</sup>Alipour-Haris G, Liu X, Acha V, Winterstein AG, Burcu M. Real-world evidence to support regulatory submissions: A landscape review and assessment of use cases. Clin Transl Sci. 2024 Aug;17(8):e13903. doi: 10.1111/cts.13903. PMID: 39092896; PMCID: PMC11295294. <sup>2</sup>CB Insights. The High Cost Of Pharma Real-World Data: Why Are Some Vendors So Expensive? CB Insights website. Published June 1, 2023. Accessed April 7, 2025. <u>https://www.com/research/pharma-real-world-data-vendors-cost/</u> <sup>3</sup>Coherent Market Insights. Real-world Data (RWD) Market Analysis. Coherent Market Insights website. Accessed April 7, 2025. https://www



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data."1	

<sup>1</sup>Alipour-Haris G, Liu X, Acha V, Winterstein AG, Burcu M. Real-world evidence to support regulatory submissions: A landscape review and assessment of use cases. Clin Transl Sci. 2024 Aug;17(8):e13903. doi: 10.1111/cts.13903. PMID: 39092896; PMCID: PMC11295294. <sup>2</sup>CB Insights. The High Cost Of Pharma Real-World Data: Why Are Some Vendors So Expensive? CB Insights website. Published June 1, 2023. Accessed April 7, 2025. https://www.cbinsights.cc pharma-real-world-data-vendors-cost/ <sup>3</sup>Coherent Market Insights. Real-world Data (RWD) Market Analysis. Coherent Market Insights website. Accessed April 7, 2025.



idividual drug manufacturer pend up to \$5MM annually on but "McKinsey estimates that he next three to five years, rage top-20 pharma company unlock more than \$300MM a by adopting advanced RWE ics across its value chain."<sup>3</sup>



# BACKGROUND



# Al advancements

# Patient Advocacy Groups are expanding beyond traditional activities to become hubs for data-driven research.

Page 6 of 25 Development of a RWE roadmap to advance OMAS understanding & advocacy



## No-code data management and analytic tools



# OBJECTIVE

## To develop a roadmap for using real-world evidence to improve outcomes for those affected by OMAS.

# DEPENDENCIES

### Community

### **Experts**

Data

Page 7 of 25 Development of a RWE roadmap to advance OMAS understanding & advocacy

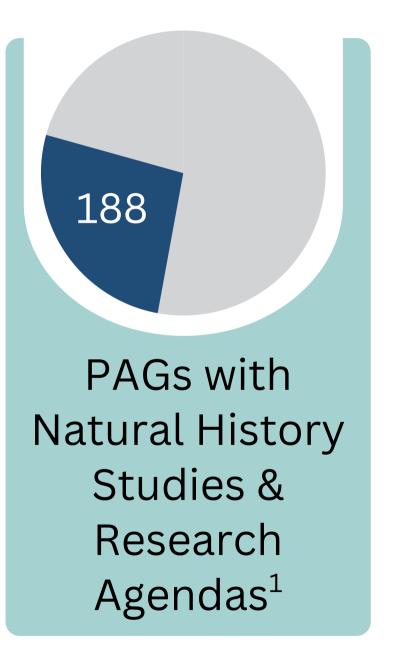


### Funding





# APPROACH Identify the destinations





luch worse Community Input & Individual Goals Assessments<sup>3,4</sup>

+2

Page 8 of 25 Development of a RWE roadmap to advance OMAS understanding & advocacy

<sup>1</sup>Korsunska, A., Bolden, S. E., Repasky, M., Zuccato, M., Fajgenbaum, D. (2023) The ROADMAP Project. https://www.everycure.org/roadmap. <sup>2</sup>Among others - Google Research Accelerating Scientific Breakthroughs with an AI Co-Scientist. Google AI Blog. Published March 21, 2024. Accessed April 8, 2025. https://research.google/blog/accelerating-scientificbreakthroughs-with-an-ai-co-scientist/. <sup>3</sup>Rare Revolution Magazine. Goal Attainment Scaling: Delivering patient-centred clinical trials using personalised outcome measures. Accessed April 8, 2025. https://rarerevolutionmagazine.com/goal-attainment-scaling-delivering-patient-centred-clinical-trials-using-personalised-outcome-measures/. 4Revana A, Sevinc G, George M, Dunn T, Pope K, Stanley J, Rockwood K. Personalized endpoints in Prader-Willi syndrome: a case study with goal attainment scaling. J Clin Sleep Med. 2025 Mar 21. doi: 10.5664/jcsm.11664. Epub ahead of print. PMID: 40114471





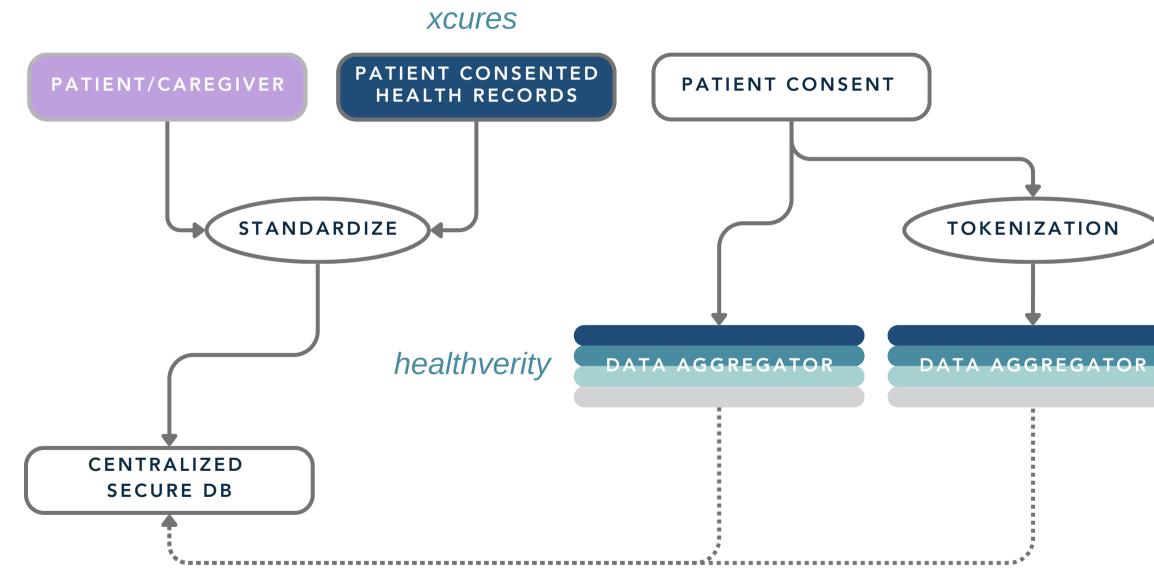


**Experts: Oxford** Workshop, Clinicians, Payers



# APPROACH

## Consider the vehicles



Page 9 of 25 Development of a RWE roadmap to advance OMAS understanding & advocacy





komodo and others EMR

LABS/GENOMICS

IMAGING

PROCEDURES

PHARMACY

CLAIMS-MEDICAL

CLAIMS-PHARMACY

CLAIMS - HOSPITAL

DEMOGRAPHICS

SDOH

REGISTRY

PROs & PREs



## APPROACH

# Fuel the effort (funding)

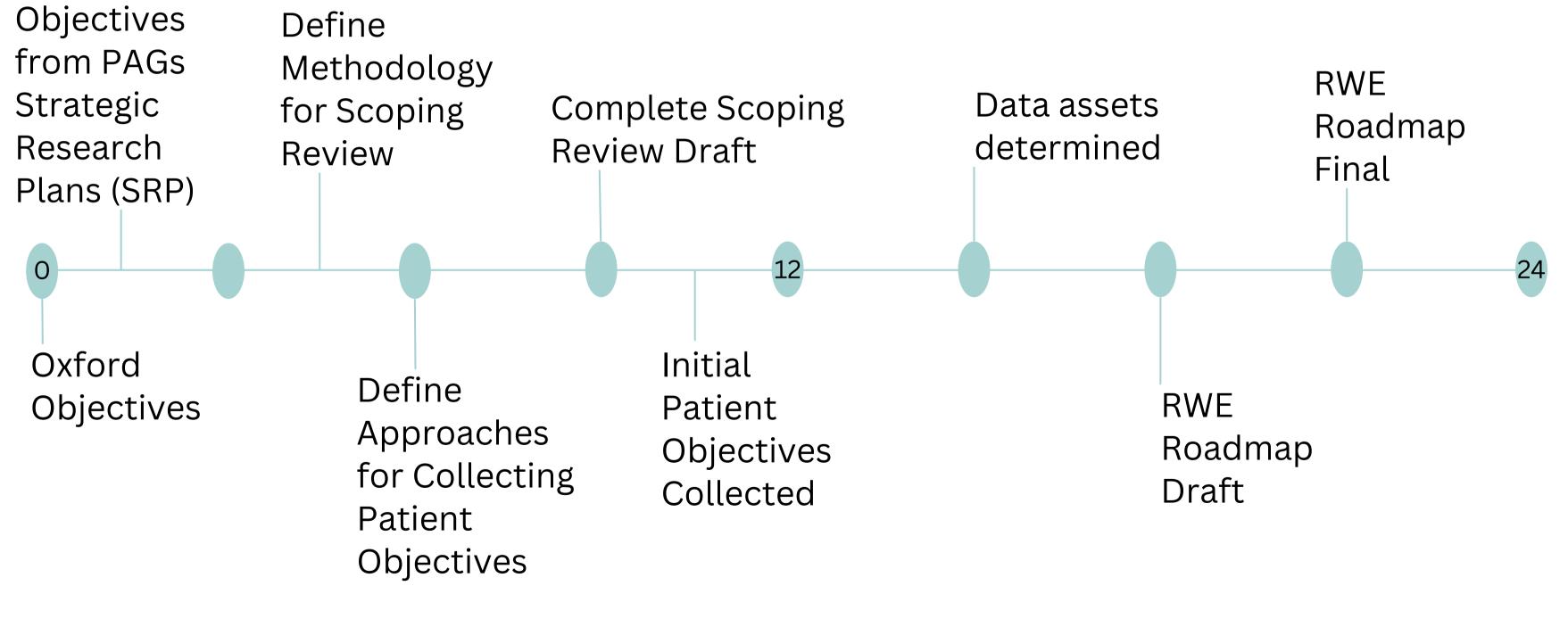
## stay tuned.....

Page 10 of 25 Development of a RWE roadmap to advance OMAS understanding & advocacy





# APPROACH Set a timeline



Page 11 of 25 Development of a RWE roadmap to advance OMAS understanding & advocacy





# OBJECTIVES

## Initial thoughts

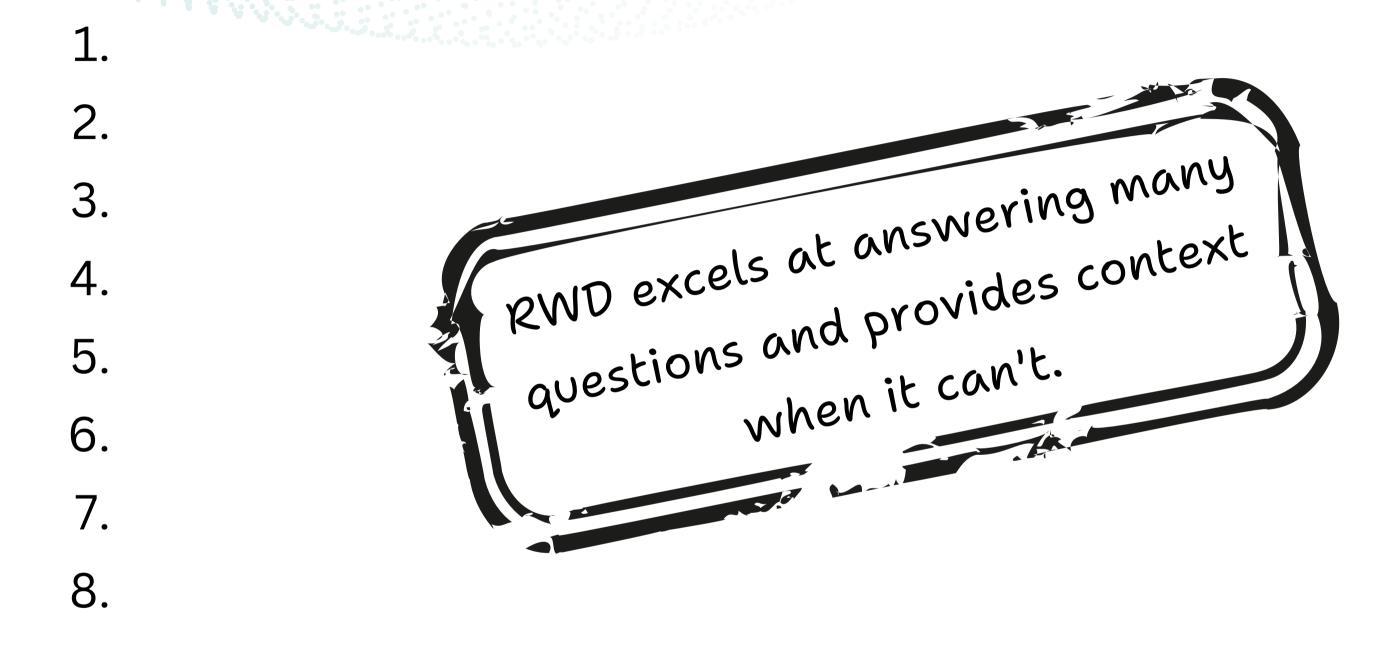
- 1. Comparative effectiveness of upfront vs escalating treatment
- 2. Variation in treatment patterns and concordance with guidelines
- 3. Costs & Health resource utilization home infusion vs. infusion center
- 4.ICD-CM code for OMAS (doesn't belong here, but important)
- 5. Barriers to care
- 6. Development of individual goals /goal attainment scaling

## Now, for Oxford Input





# **OXFORD OBJECTIVES**









# Thank you!

# WHAT CAN YOU DO WITH RWD?\*

Epidemiology & Disease Burden	Treatment Patterns	Safety & Tolerability	Outcomes	Health Economics
Understanding disease prevalence	Actual treatment patterns which	Identifying rare adverse events	Assessing the effectiveness of	Assessing the cost- effectiveness of
and incidence	may differ from clinical trials and	Evaluating the	treatments	treatments
Identifying risk factors:	guidelines	safety of new treatments	Identifying factors that influence	Identifying cost drivers
Understand disease burden		Monitoring the long-term safety	outcomes. Evaluating the quality of care	Economic impact of interventions

\*AND MUCH MORE





# A bit about us....

Page 16 of 25 Development of a RWE roadmap to advance OMAS understanding & advocacy





Principled Research Resources

Real-World Evidence & Insights Generation

Done Differently. Done Better.



https://www.principledresources.com/

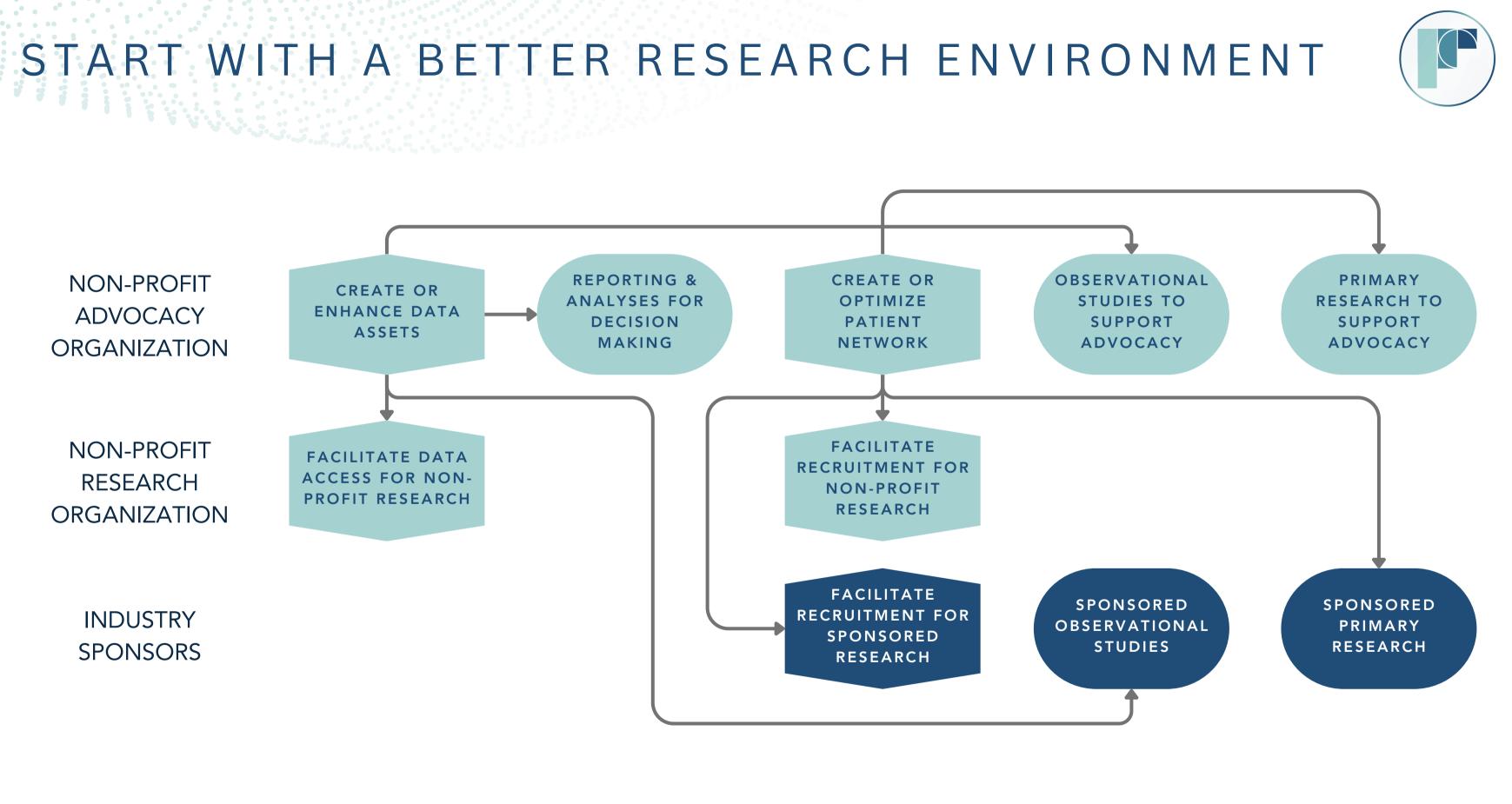
Principled Research Resources is a benefit organization that uses healthcare data and patient/caregiver perspectives to generate impactful real-world evidence and insights.

We partner with non-profit advocacy groups to conduct comprehensive research projects—including prospective, retrospective, qualitative, and quantitative studies—that address critical areas such as clinical outcomes, socioeconomic factors, patient journeys, natural histories, and patient/caregiver priorities.

Through these collaborations, we develop high-quality, cost-effective solutions for sponsors, while delivering sustainable assets to advocacy organizations and the communities they serve.







## END BY SPEAKING THE LOCAL DIALECT



- •Clin Dev • Mkt Research •Commercial • Medical Affairs •HEOR/Access
- Physician Networks
  - Physicians
- Formulary Boards
  - Medical Assoc

- Patients • Caregivers •Other
- Advocates



Page 20 of 25 Development of a RWE roadmap to advance OMAS understanding & advocacy



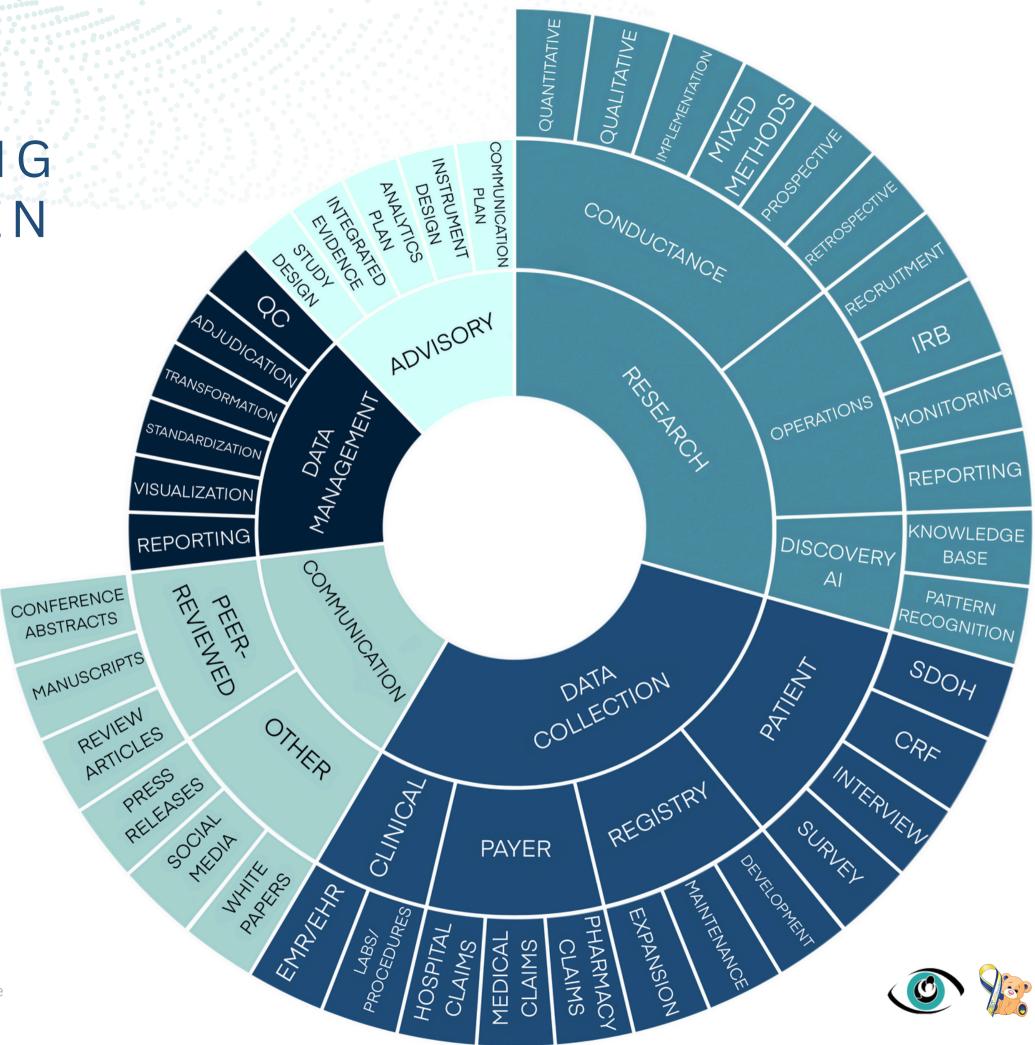


•Commercial •CMS, CHIP, etc •State Medicaid

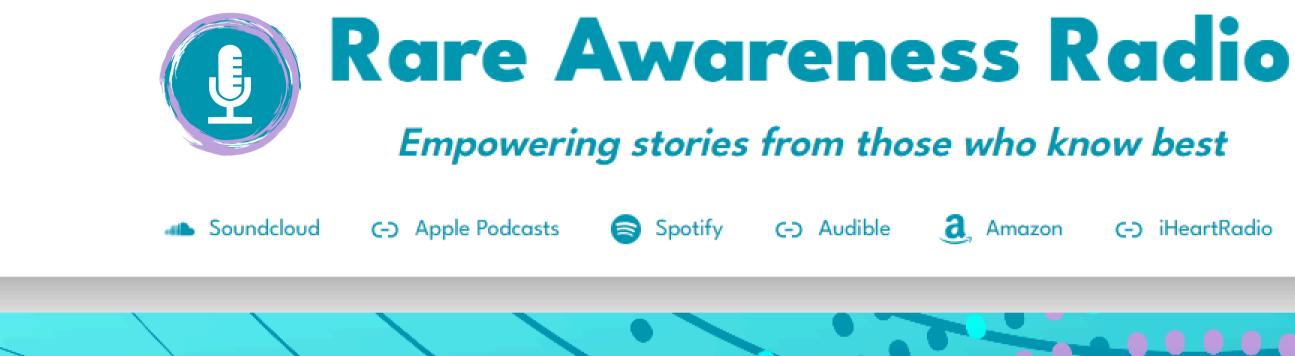
• FDA •Local and National Legislatures



## PROVIDE EVERYTHING IN-BETWEEN



Page 21 of 25 Development of a RWE roadmap to advance OMAS understanding & advocacy 



## Amplifying the voices of those united against rare diseases

"Rare Awareness Radio," an ongoing podcast, amplifies voices from the rare disease community. Each episode features unique perspectives from patients, caregivers, healthcare providers, researchers, or advocates. Through candid conversations and personal stories, we hear lessons learned, successes, and challenges to address.

Home Contact in

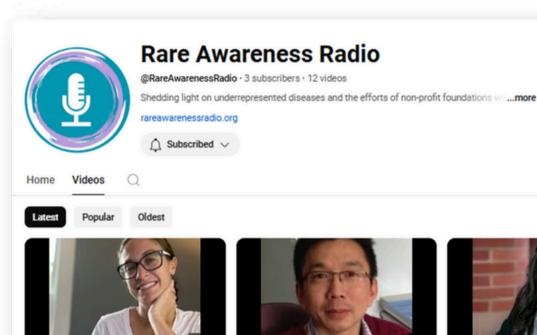
- (-) iHeartRadio YouTube

https://rareawarenessradio.org/

# RADIO FOR GOOD

RARE AWARENESS AWARENESS RADIO RADIO	RARE AWARE RADIO Empowering stories from those with
RADIO Empowering stories from those who know best. Empowering stories from those who know best. Rare Awareness Radio is a recurring podcast that amplifies voices from the rare disease that amplifies voices from the rare disease community. Each episode features unique community. Each ep	The Founder Series explores the journeys that he create rare disease advolution about their motivation successes, inspiring insight to start their own foundation
stories, we hear of and remaining challenges to be a und remaining challenges to b und rem	The Patient Series delves into what matters to patients today - a tit time of their life and in the ever changing car environment. Critical information for all
Hosted by Richard Juknavorian Creator and host of "Meeting You Where You're At", a platform dedicated Where You're At", a platform dedicated Where You're At", a platform dedicated Where You're At", a platform dedicated	The Caregiver Sr gives the perspectives of the family member with a dise they cope with complex me emotional stress, sleep de the constant fear of the un
Contact Us	The Researcher Series provides insights into the the key challenges ar promising areas of study in rare disease research The Provider Se
Rare Awareness Radio is made percentation construction of the provide statement of the provide s	Explores the challenges a experienced by healthcar deal with diagnostic obst no adblocks, among other
www.rareawarenessradio.org	www.rareawareness

#### https://rareawarenessradio.org/



Episode 13 Maura McNamara: OMS Life Foundation 9 views • 12 days ago

Episode 12 Ming Lim: OMS Life Foundation 8 views • 2 weeks ago



Episode 9 Sunitha Malepati: CACNA1A Foundation 16 views • 1 month ago



13 views · 4 months ago

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Episode 5 Kate Vinokurov: Cure OTCD 20 views · 3 months ago

Episode 3 Carolina Sommer: Born A Hero

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Page 23 of 25 Development of a RWE roadmap to advance OMAS understanding & advocacy









Episode 11 Bhavna Dias: OMS Life Foundation 46 views • 3 weeks ago



Episode 10 Rachel Heilmann: The Rory Belle Foundation

15 views • 1 month ago



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Episode 7 Zeke and Chanin Zaragoza: **OMSLife Foundation** 14 views · 2 months ago



Episode 6 Adam Clatworthy: CRELD1 Warriors

85 views • 2 months ago

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Episode 2 Jeff Kramer: Chondrosarcoma Foundation 8 views • 5 months ago



Episode 1 Mike Michaelis: OMSLife Foundation 27 views • 5 months ago

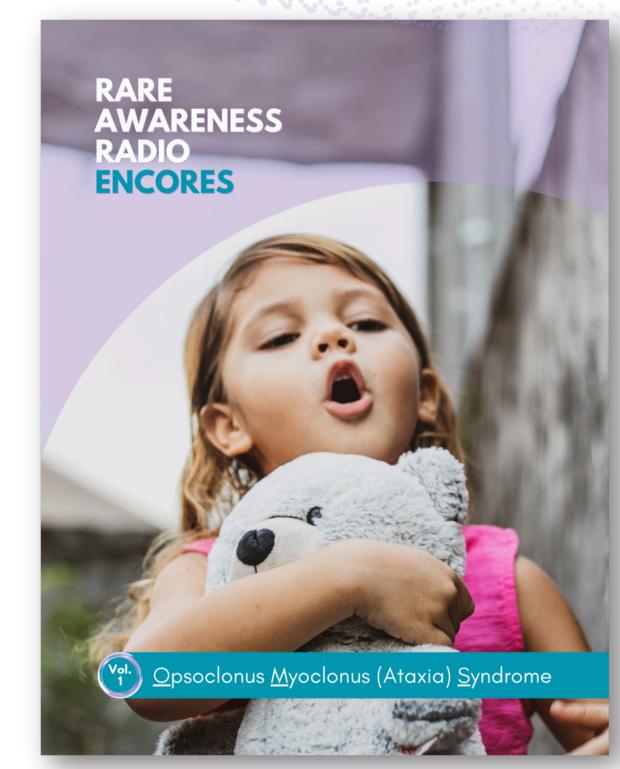
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# ENCORES



RARE AWARENESS RADIO ENCORES

#### Welcome

to Rare Awareness Radio Encores, a magazine dedicated to amplifying the voices of those affected by rare diseases. Here, you'll find insightful summaries of interviews with extraordinary individuals tirelessly working to improve the lives of patients and their families.

From passionate founders of advocacy groups to renowned physicians, dedicated caregivers, resilient patients, and groundbreaking researchers, these stories offer hope, inspiration, and a deeper understanding of the rare disease community's challenges and triumphs.

#### **Our Host**



Richard Juknavorian, is an advocate for rare disease awareness and women's empowerment.

In addition to hosting Rare Awareness Radio, he also hosts the podcast "Meeting You Where You're At", highlighting the stories of inspiring women entrepreneurs.

### Our Sponsors



Page 24 of 25 Development of a RWE roadmap to advance OMAS understanding & advocacy



#### OPSOCLONUS MYOCLONUS (ATAXIA) SYNDROME

# Mike Michaelis & The OMSLife Foundation

When Mike Michaelis' 14-month-old granddaughter, Alexa, suddenly lost the ability to walk and sit up, her parents rushed her to the emergency room multiple times, only to be met with uncertainty from medical professionals.

It wasn't until a serendipitous connection led the family to Texas Children's Hospital that Alexa was finally diagnosed with the rare neurological disorder, opsoclonusmyoclonus syndrome (OMS).

#### This pivotal moment sparked the creation of the OMSLife foundation.

Driven by Alexa's parents' desire to ensure no other family would have to endure the same harrowing experience, the Michaelis family set out to raise awareness, provide resources, and advocate for the OMS community.

Initially, OMSLife started as a simple Facebook page, but as more families reached out seeking support, the organization quickly evolved into a formal 501(c)(3) non-profit. A key focus of OMSLife has been educating frontline medical staff and the public about OMS, a disease that typically affects young children between 6 months and 3 years old, with a slightly higher incidence in girls.

Continuing in the quest to educate others about OMS and recognizing the importance of data collection for research, OMSLife collaborated with the National Organization for Rare Disorders (NORD) to establish a patient registry. This database aims to shed light on the multifaceted challenges of OMS, from the initial symptoms to the obstacles encountered as patients age and their care needs change.

Rare Awareness Radio



Participation in the registry has largely been driven by the community created by OMSLife. Since 2014, OMSLife has organized annual caregiver conferences, providing a vital platform for families affected by OMS to connect, share experiences, and learn from leading specialists. These conferences have lessened the isolation felt by caregivers due to the rarity of the condition.

OMSLife has also been central to connecting researchers and physicians, through cosponsorship of the Abingdon Conference with Dancing Eyes Syndrome Support Trust (DESST) in the United Kingdom. Beyond this conference, OMSLife has provided research grants to fund in whole or part 12 different projects conducted at leading academic institutions.

As OMSLife looks to the future, the foundation is exploring technologic avenues including artificial intelligence to aid in the diagnosis and treatment of OMS, with the goal of providing more consistent and accessible care for all those affected.

Rare Awareness Radio is made possible by support from Meeting You Podcast, OMSLife Foundation, and Principled Research Resources L3C.

- October 2024







PRINCIPLED RESEARCH RESOURCES L3C

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