



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

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





How can we leverage our combined strengths
to improve patient lives more quickly?

What can we accomplish
by the next Oxford Conference?



WHY REAL-WORLD DATA?



An individual drug manufacturer may spend up to \$5MM annually on RWD² but over the considerable years, an average company could unconsiderable commercial value year by year by analytics.



WHY REAL-WORLD DATA?



“While nearly half of identified use cases involved an external control arm approach, the review also demonstrated a variety of other approaches including supplementing RCTs and providing primary evidence in lieu of clinical trial data.”¹

An individual drug manufacturer may spend up to \$5MM annually on RWD² but “McKinsey estimates that over the next three to five years, an average top-20 pharma company could unlock more than \$300MM a year by adopting advanced RWE analytics across its value chain.”³



BACKGROUND



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

OBJECTIVE

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

DEPENDENCIES

Community

Experts

Data

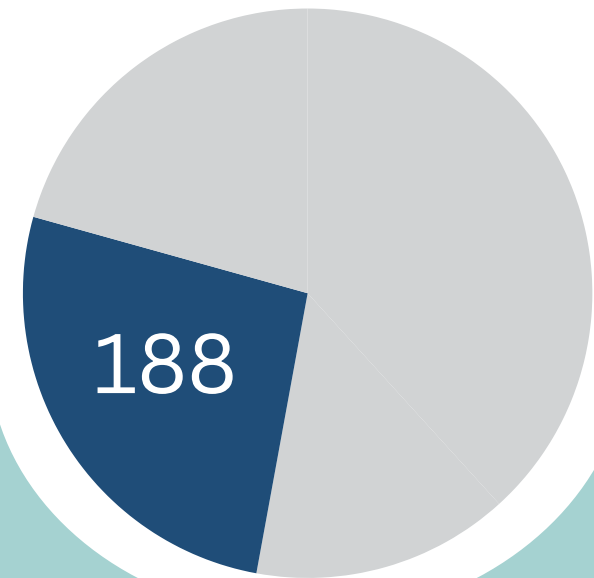
Funding





APPROACH

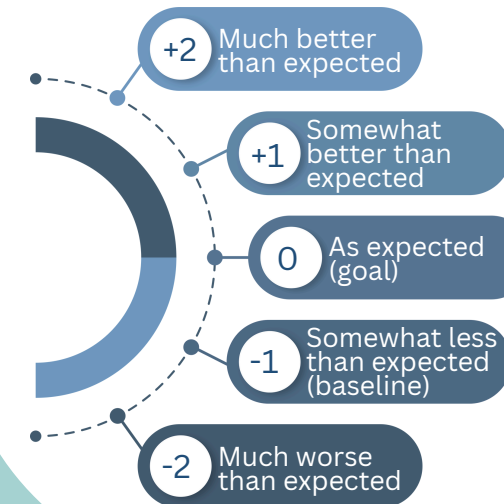
Identify the destinations



PAGs with
Natural History
Studies &
Research
Agendas¹



AI-facilitated
Scoping
Literature
Review²



Community
Input &
Individual Goals
Assessments^{3,4}



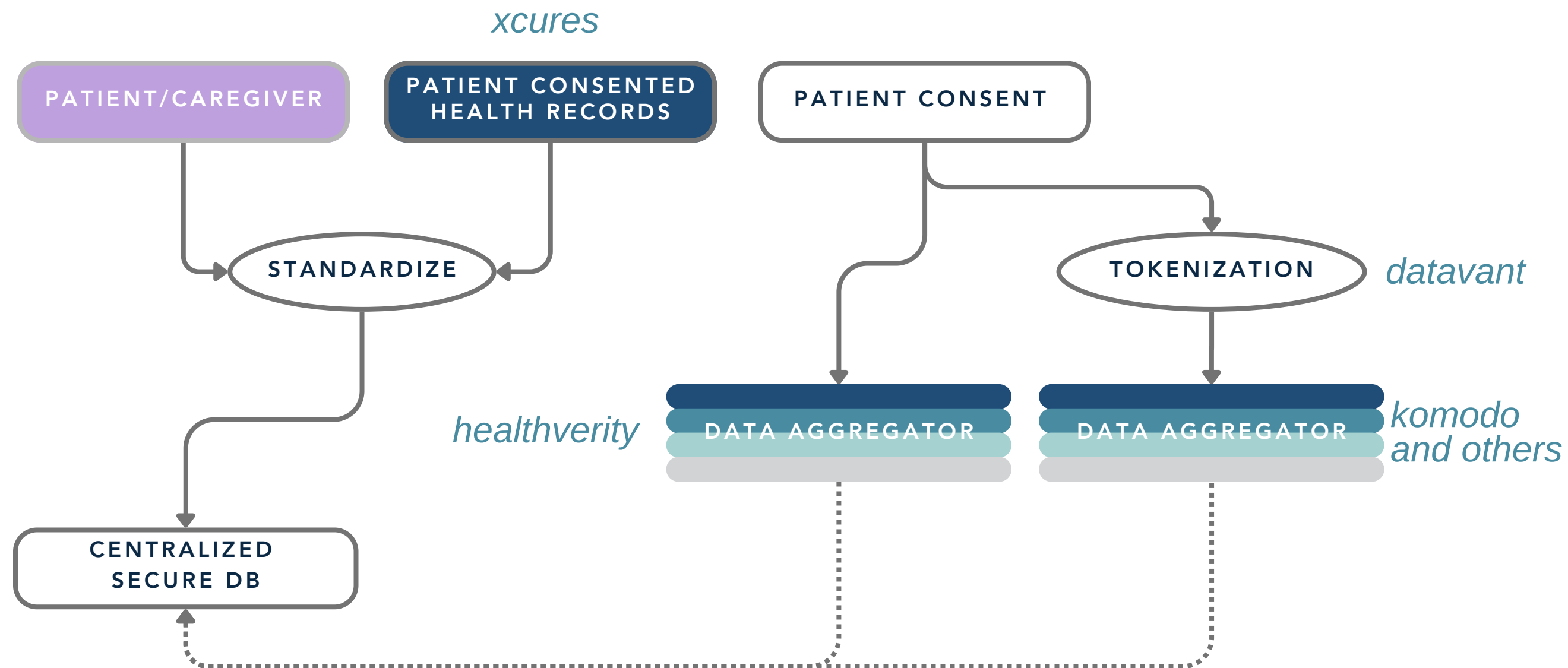
Experts: Oxford
Workshop,
Clinicians,
Payers





APPROACH

Consider the vehicles



- EMR
- LABS/GENOMICS
- IMAGING
- PROCEDURES
- PHARMACY
- CLAIMS-MEDICAL
- CLAIMS-PHARMACY
- CLAIMS - HOSPITAL
- DEMOGRAPHICS
- SDOH
- REGISTRY
- PROs & PREs





APPROACH

Fuel the effort (funding)

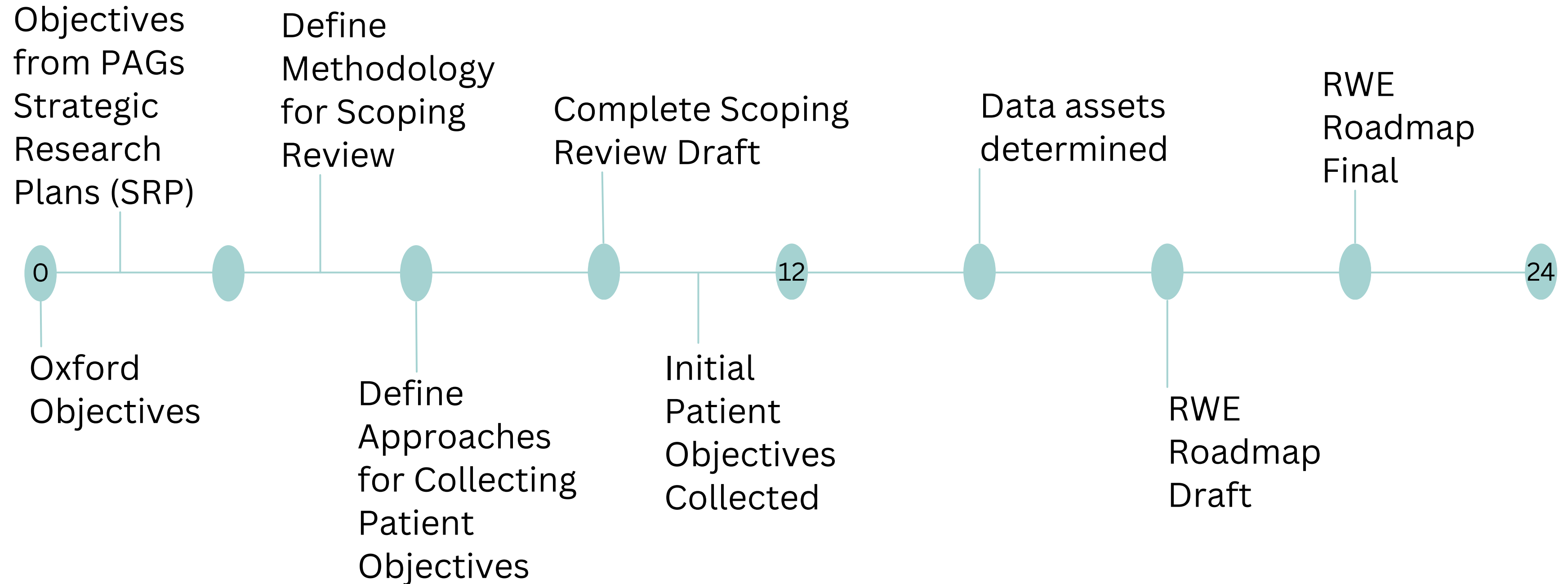
stay tuned.....





APPROACH

Set a timeline



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

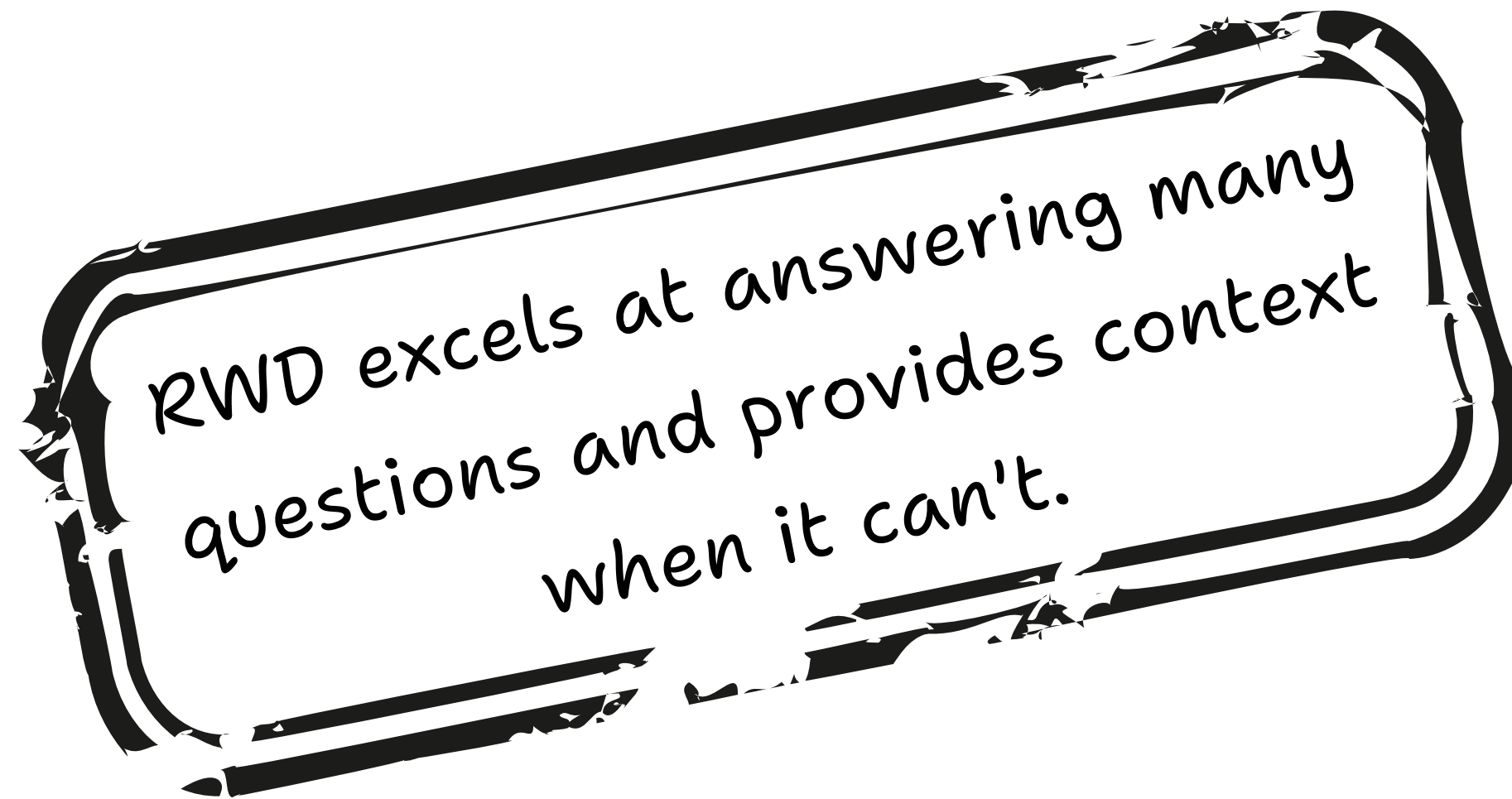
Now, for Oxford Input



OXFORD OBJECTIVES



- 1.
- 2.
- 3.
- 4.
- 5.
- 6.
- 7.
- 8.



Thank you!

WHAT CAN YOU DO WITH RWD?*



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

*AND MUCH MORE





A bit about us.....





Real-World Evidence & Insights Generation

Done Differently.
Done Better.

<https://www.principledresources.com/>





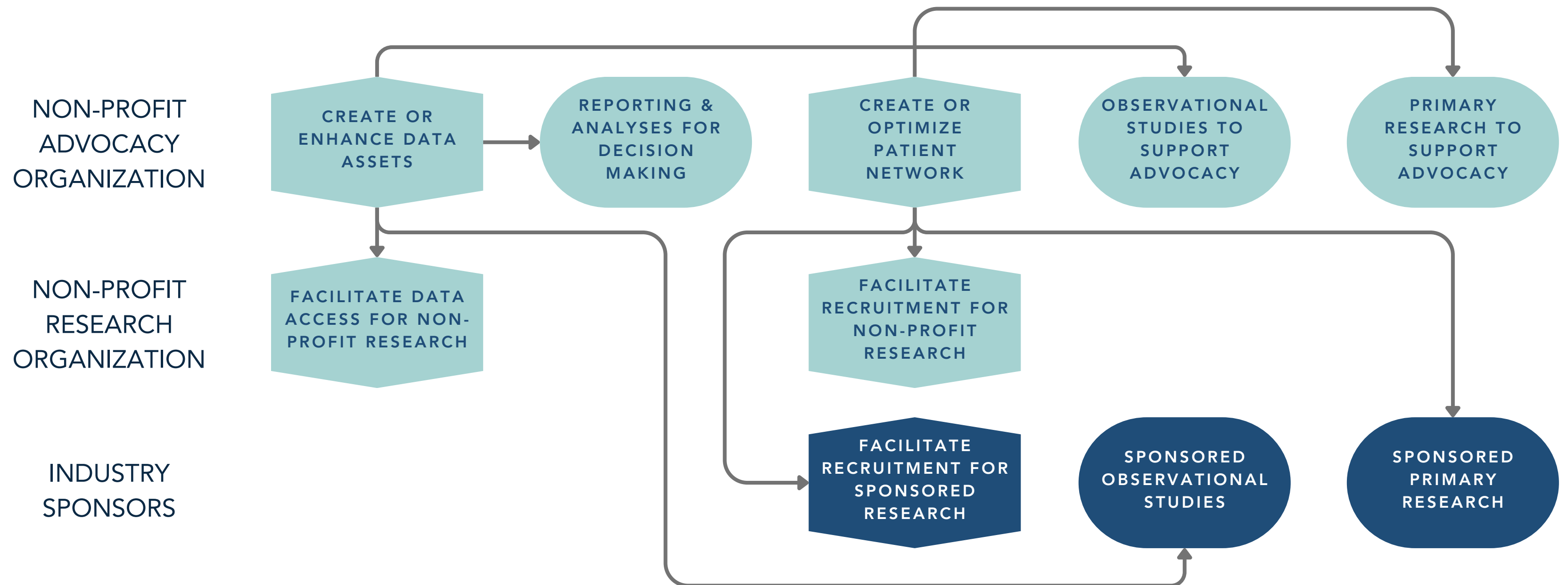
Principled Research Resources is a benefit organization that uses healthcare data and patient/caregiver perspectives to generate impactful real-world evidence and insights.

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.



START WITH A BETTER RESEARCH ENVIRONMENT



END BY SPEAKING THE LOCAL DIALECT



Manufacturers



- Clin Dev
- Mkt Research
- Commercial
- Medical Affairs
- HEOR/Access

Providers



- Physician Networks
 - Physicians
- Formulary Boards
- Medical Assoc

Patients & Advocates



- Patients
- Caregivers
- Other Advocates

Payers



- Commercial
- CMS, CHIP, etc
- State Medicaid

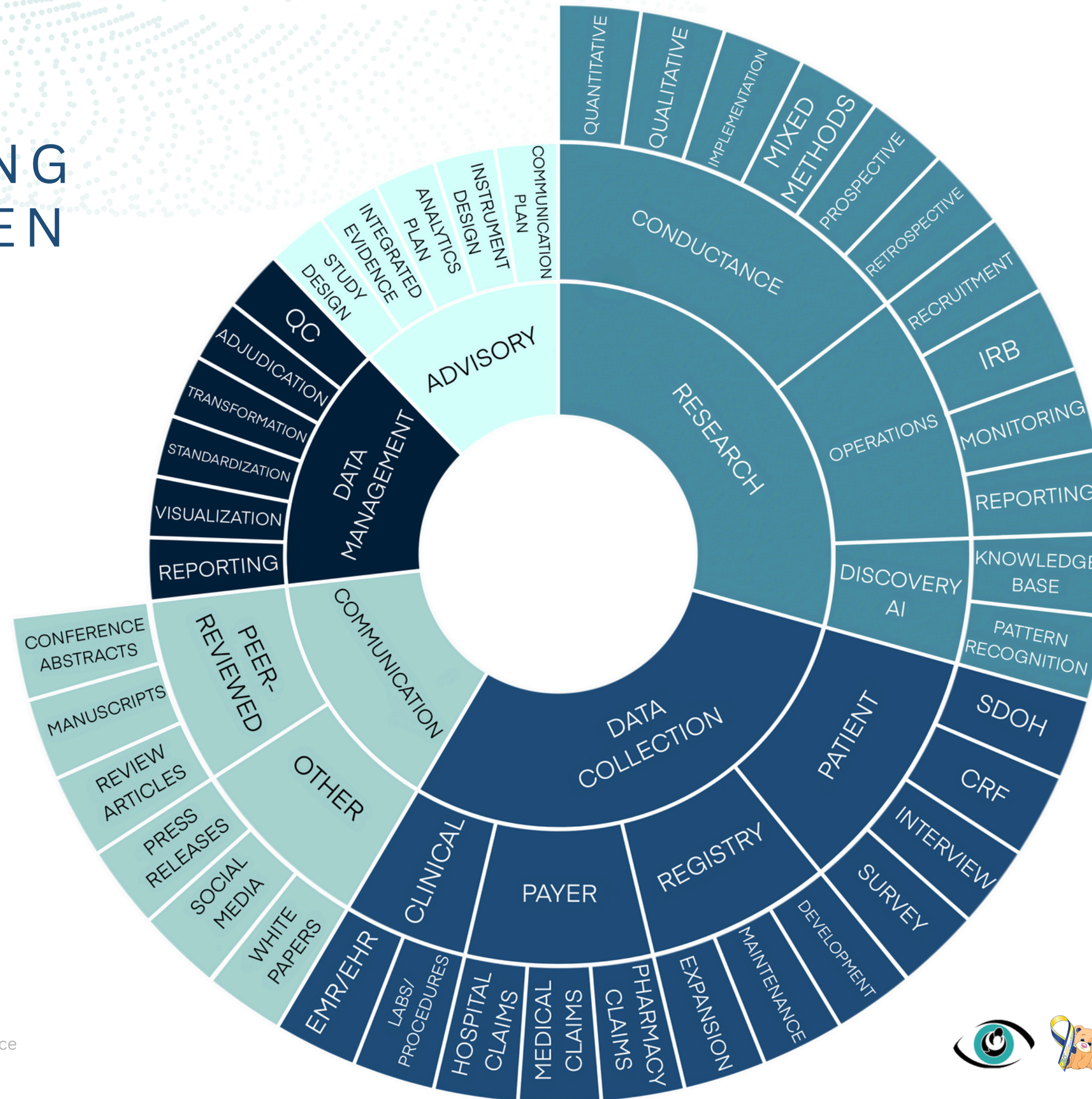
Gov't Agencies



- FDA
- Local and National Legislatures



PROVIDE EVERYTHING IN-BETWEEN





Rare Awareness Radio

Empowering stories from those who know best



Soundcloud



Apple Podcasts



Spotify



Audible



Amazon



iHeartRadio



YouTube

Amplifying the voices of those united against rare diseases

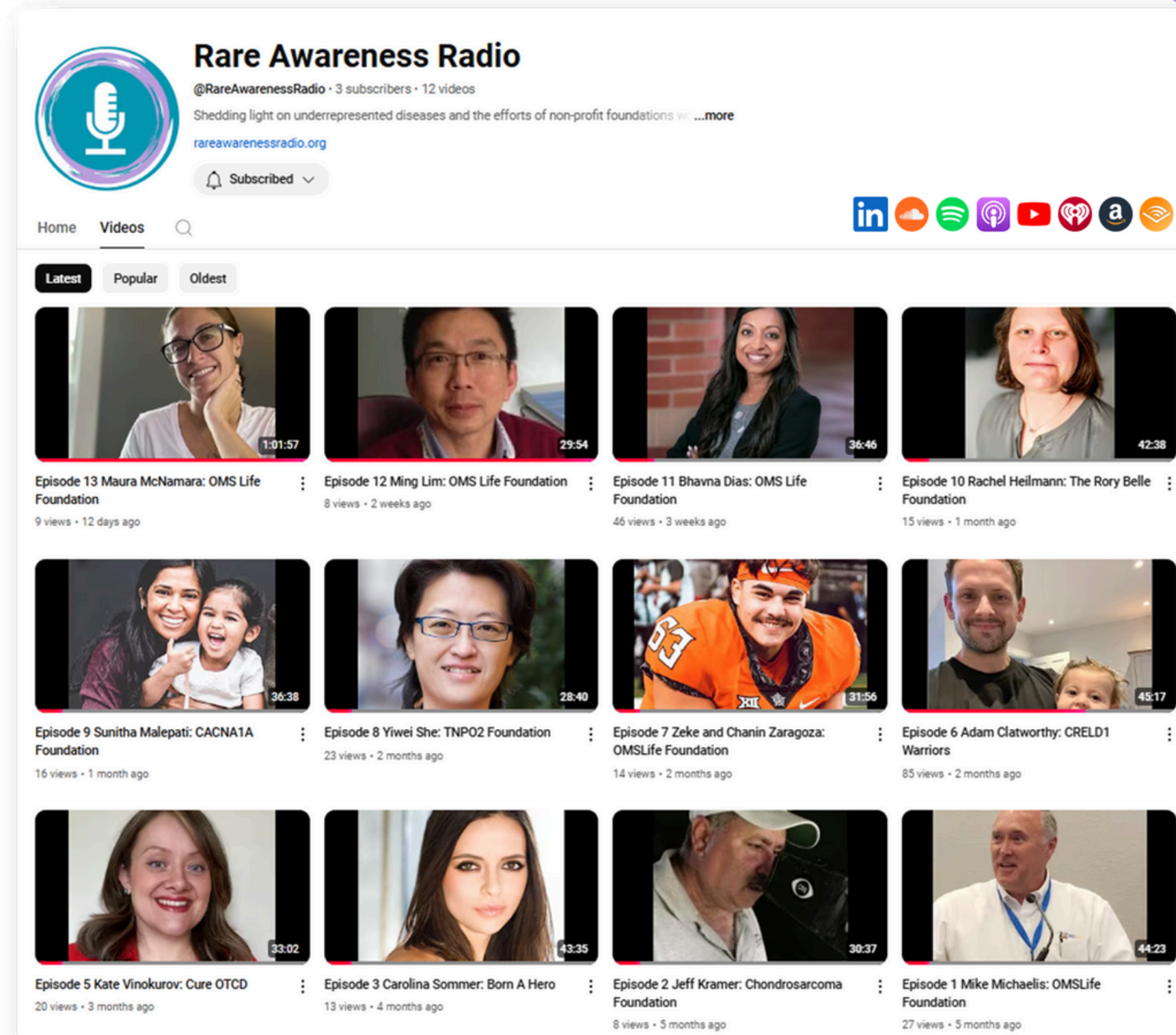
“Rare Awareness Radio,” an ongoing podcast, amplifies voices from the rare disease community. Each episode features unique perspectives from patients, caregivers, healthcare providers, researchers, or advocates. Through candid conversations and personal stories, we hear lessons learned, successes, and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

<https://rareawarenessradio.org/>

RADIO FOR GOOD



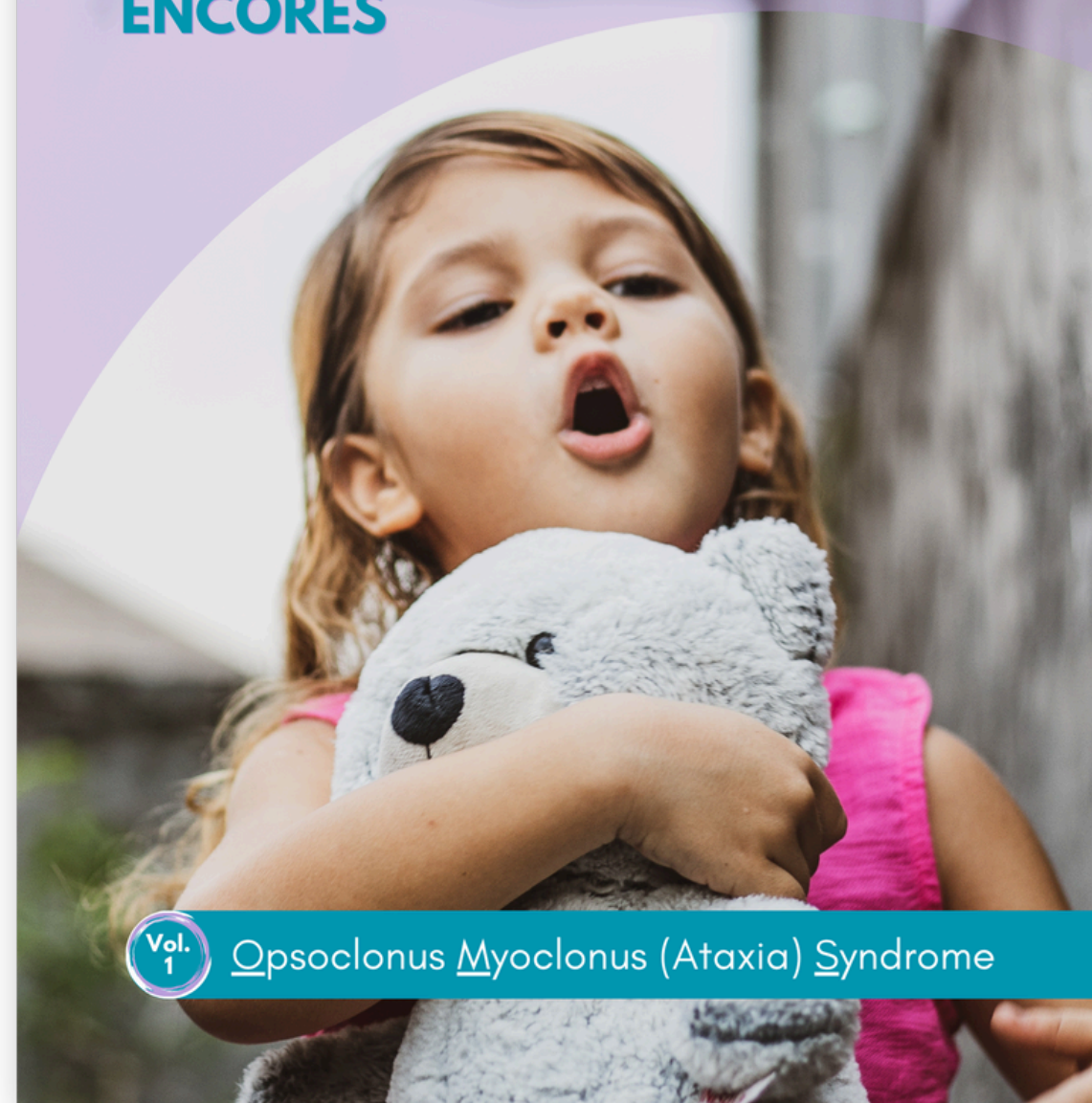
<https://rareawarenessradio.org/>



ENCORES



RARE AWARENESS RADIO ENCORES



Vol. 1 Opsoclonus Myoclonus (Ataxia) Syndrome



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



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.

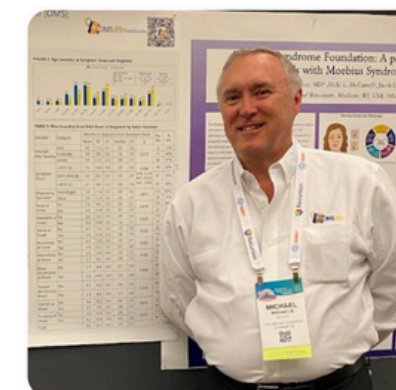
It wasn't until a serendipitous connection led the family to Texas Children's Hospital that Alexa was finally diagnosed with the rare neurological disorder, opsoclonus-myoclonus syndrome (OMS).

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.



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 co-sponsorship of the Abingdon Conference with Dancing Eyes Syndrome Support Trust (DESSST) 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 LLC.

Rare Awareness Radio

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