

## N-of-1 Task Force Education Resources

Name of Resource	Link Resource	Resource Content	Resource Relevance to N-of-1	Resource Ownership	Funding	Audience
ERICA - PROMs Repositories	<a href="https://erica-rd.eu/work-packages/patient-centred-research/proms-repository/">https://erica-rd.eu/work-packages/patient-centred-research/proms-repository/</a>	Patient Reported Outcome Measures (PROMs) Repository	Somewhat relevant: focus is on overall rare diseases. Focus on EU	European Rare Disease Research Coordination and Support Action consortium (ERICA) Coordinating Center/European Reference Network	EU Horizon 2020 research and innovation programme grant	Academics/researchers, patients
Cortellis database	Cortellis database (part of the ClarivateTM offering): <a href="https://access.clarivate.com/login?app=cortellis">https://access.clarivate.com/login?app=cortellis</a> <a href="https://clarivate.com/cortellis/learning/cortellis-training/">https://clarivate.com/cortellis/learning/cortellis-training/</a>	Publication, reports, blogs Training Programs under support and training	Relevant but subscription is required Focus on Biopharma research and development	Clarivate: Cortellis	For profit	Academics/researchers, companies
FDA - Clinical Outcome Assessment Compendium	<a href="https://www.fda.gov/drugs/development-resources/clinical-outcome-assessment-compendium">https://www.fda.gov/drugs/development-resources/clinical-outcome-assessment-compendium</a>	Clinical Outcome Assessment Compendium	Somewhat relevant: including common and rare diseases Focus on USA	FDA	Government funded	Academics/researchers, companies

N=1 Collaborative	<a href="https://www.n1collaborative.org">https://www.n1collaborative.org</a>	Publication, Workshops, Seminars	Relevant: focus on ASO Focus on international knowledge sharing	N=1 Collaborative	Nonprofit	Academics/researchers, patients
THE YU LAB - Antisense Oligonucleotides	<a href="https://www.theyulab.org/antisense-therapy">https://www.theyulab.org/antisense-therapy</a>	Publication, Podcasts	Relevant: focus on ASO and overall precision medicine Focus on research and patient treatment	YU LAB: Boston Children's hospital	Government/Private funding	Academics/researchers, patients
Harvard Medical School - Publication on antisense oligonucleotides	<a href="https://hms.harvard.edu/news/collaboration-propels-research-untreatable-neurodegenerative-disease--see-video-explaining-how-ASOs-work">https://hms.harvard.edu/news/collaboration-propels-research-untreatable-neurodegenerative-disease -- see video explaining how ASOs work</a>	Publications	Relevant: ASO and Gene therapy Focus on research and patient treatment	Harvard Medical School	Private, non-profit	Academics/researchers, patients
Oligonucleotides Therapeutics Society - Rare Disease Task Force	<a href="https://www.oligotherapeutics.org/rare-disease-task-force/">https://www.oligotherapeutics.org/rare-disease-task-force/</a>	Annual Meetings, Webinars, Newsletters	Relevant: focus on ASO Society's mission to foster academia – and industry-based research	Oligonucleotide Therapeutics Society (OTS)	Membership based	Academics/researchers
Global Nursing Network for Rare & Undiagnosed Diseases	<a href="https://www.gnnrd.org/">https://www.gnnrd.org/</a>	Mentoring through roundtables and educational programs	Somewhat relevant: focus on nurses supporting rare disease patients	Global nursing network for rare diseases	To be confirmed	Health professionals, patients

			Focus on international collaboration of nurses			
UNIQUE - Understanding Rare Chromosome and Gene Disorders	<a href="https://rarechromo.org/">https://rarechromo.org/</a>	Information guides, videos, little yellow book	Somewhat relevant: focus on families affected with rare chromosome and gene disorders Focus on international patient support	Unique	Nonprofit registered charity	Academics/researchers, health professionals, patients
UEMS Competency on Rare and Undiagnosed Diseases	<a href="https://www.uems.eu/data/assets/pdf_file/0006/121488/Rare-Diseases-ETR.pdf">https://www.uems.eu/data/assets/pdf_file/0006/121488/Rare-Diseases-ETR.pdf</a>	Publication: Training Requirements for the Competency of Rare and Undiagnosed Diseases	Somewhat relevant: European Standards of Postgraduate Medical Training Focus on EU	European Union of Medical Specialists	Nonprofit	Health professionals
Rare Awareness Rare Education Portal	<a href="https://rareportal.org.au/about/#:~:text=Rare%20Voices%20Australia%20(RVA)%20is,living%20with%20a%20rare%20disease.">https://rareportal.org.au/about/#:~:text=Rare%20Voices%20Australia%20(RVA)%20is,living%20with%20a%20rare%20disease.</a>	Portal is still in development as of November 25, 2023.	Somewhat relevant: focus on information and resources for all rare disease stakeholders Focus on Australia	Rare Voices Australia: Rare Awareness Rare Education - Rare Portal	Nonprofit	Academics/researchers, health professionals, patient advocacy organizations
Ultragenyx - Introducing RARE Entrepreneur Bootcamp	<a href="https://www.ultragenyx.com/rare-entrepreneur-bootcamp/">https://www.ultragenyx.com/rare-entrepreneur-bootcamp/</a>	Event for parents developing rare disease drugs for	Relevant: focus on Nof1 Focus on knowledge	Ultragenyx: RARE Entrepreneur Bootcamp	For profit	Patients

		their children, conducted in collaboration with Global Genes and other companies. Small group of participants	sharing, probably internationally			
American Journal of Medical Genetics - Publication "Gene targeted therapies: equitable diagnosis and early delivery	<a href="https://onlinelibrary.wiley.com/toc/15524876/2023/193/1">https://onlinelibrary.wiley.com/toc/15524876/2023/193/1</a>	Publication: Volume 193, Issue 1 Special Issue: Gene-Targeted Therapies: Early Diagnosis and Equitable Delivery, March 2023	Relevant: focus on personalized medicine International available publication	AJMC: American Journal of Medical Genetics	To be confirmed	Academics/researchers
The Global Guide to compassionate use programs	<a href="https://www.globalcompassionateuse.com/what-is-it/">https://www.globalcompassionateuse.com/what-is-it/</a>	Publication: covers regulatory and legal framework for Compassionate Use and Expanded Access to regulatory mechanisms worldwide.	Somewhat relevant: focus on Use and Access Focus on North and South America, Europe, Middle East and Asia Pacific.	The Global Guide to Compassionate Use Programs	for profit	Patients