ROADmap to Rare Disease Outcomes

Process to developing the resources

Project launch: Core team, patient and family (PF) partners, and international advisory board (IAB) established

Scoping Review

Explore Clinical Outcome Assessments (COAs) in recent pediatric rare disease clinical trials, and the nature of co-design by patients / families

Output: Candidate items & evidence gaps identified

Search for existing tools & resources for outcome design and selection

Output: Existing tool gap documented

International Needs Survey

Trialists' perspectives on gaps & needs in outcome design and selection in pediatric rare disease trials

Output: Priority needs trial community etablished

Patient and Family-Rare Disease Clinical Trial Outcome Assessment and Design Template (ROAD-PF)

Workshop 1 (February 5, 2026)

- Onboarding with F-words tool; 12 patient and family partners
- 22 candidate outcome design considerations (ODCs) across 6 themes, items suggested and prioritized

Workshop 2 (February 19, 2026)

- Wording refined and content validated (11 sessions in French)
- Consensus: 12 items included after revisions

Rare Disease Clinical Trial Outcome Assessment and Design - Template for Trialists (ROAD-TT)

IAB Meeting 1 (February 9 and 12, 2026)

- Draft ROAD-Trialists template with 6 sections and candidate items reviewed by 17 international advisors

IAB Meeting 2 (April 1 and 2, 2026)

- Advisors' feedback consolidated
- Revisions agreed and template updated

Pilot testing of pre-final templates
Finalising both Templates

RareKids CAN

SickKids

v1.0 - 21 Oct 2026