Canadian Organization for Rare Disorders

From Priority Indicators to Readiness Signals

Webinar 3 – July 28, 2026

Panel

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Most important Indicators (63/136 completed responses)

Bottom line
Treat the results as a tight priority cluster, not a definitive rank order. Scores are extremely close across the top indicators.

Same core, small shifts
11 of the prior 13 indicators remain in the strict updated Top 13. The two new entrants are equity of access and assistive/device supports.

Near-ties still matter
A4 undiagnosed pathways and B2 time to actual access are just outside the strict Top 13 and remain relevant to patient stories and Phase 2 discussion.

Updated strict Top 13: mean importance ranges from 4.71 to 4.54. Items #14–#16 are also tightly clustered: C2 = 4.53, B2 = 4.51, A4 = 4.50.

Entered strict Top 13	E4 Equity of access across populations and regions E3 Assistive devices, medical equipment, digital tools, daily living supports
Moved outside strict Top 13	B2 Time to actual patient access = 4.51 A4 Undiagnosed / complex / multisystem pathways = 4.50
Presentation recommendation	Use the updated Top 13 for the rating table, and allow Factor 14 to be nominated in both rounds. Patient stories can still reference near-tie indicators when relevant.

The 13 top rated indicators + added 14th discussion factor

Mean importance scores are very close. Present the 13 rated indicators as a priority cluster, then add one non-rated discussion factor that can also be nominated as a readiness signal or Phase 2 priority.

#	Code	Indicator	Mean
1	**B1**	Rare disease medicine access pathways	**4.71**
2	**C1**	Clinical genetics + specialist workforce capacity	**4.67**
3	**A6**	RD education + decision support for professionals	**4.66**
4	**A2**	Genetic/genomic testing + counselling	**4.65**
5	**C5**	Pediatric-to-adult transition supports	**4.64**
6	**B5**	Follow-up after treatment initiation	**4.60**
7	**E4**	Equity of access across populations and regions	**4.59**

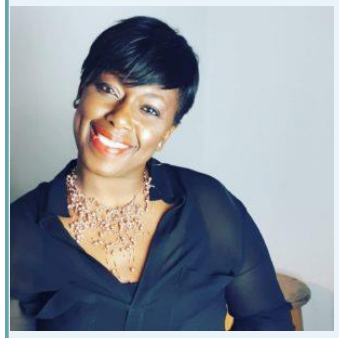
#	Code	Indicator	Mean
8	**A3**	Adult-onset or late-diagnosed pathways	**4.58**
9	**C4**	Shared records, care plans + transfer	**4.58**
10	**E3**	Assistive devices + daily living supports	**4.57**
11	**B4**	Treatment delivery capacity	**4.55**
12	**D2**	RWE infrastructure linked to access/outcomes	**4.55**
13	**A1**	Newborn screening scope + speed	**4.54**

Added discussion factor (14): Rare disease research and technology development capacity — include in both nomination rounds, including Phase 2 priorities. Not rated in the initial scan.

Scoring basis: mean importance from updated advisory scan, 54 total respondents. Top 13 shown as the strict updated ranking; score differences are small and near-ties should be discussed transparently.

Moments That Matter: Live Patient Voices

What was the moment when the system was not ready — and what should have been in place?



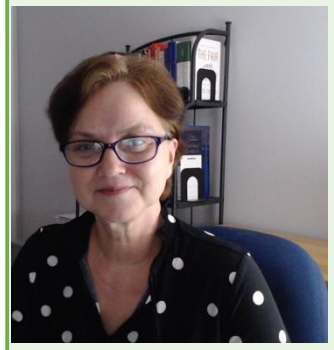
Biba

Sickle Cell Disease

Newborn screening + equity

Key message: Readiness means answers before crisis, not after avoidable pain and trauma.

Readiness signal: Equitable newborn screening and immediate early-care pathways for treatable rare diseases.
Links: A1 / A2 / B5 / Equity



Mirga

BPAN

Genomic diagnosis + caregiver evidence

Key message: Diagnosis came late; family knowledge, research literacy, and caregiver observations became essential to care.

Readiness signal: diagnostic pathways that connect families to research, expertise, and support.
Links: A2 • A6 • C4• C5 • 14

Amber

Hypoparathyroidism

Specialist capacity + innovation ready

Key message: Access required travel, persistence, and a trial pathway; knowledgeable specialists and clear treatment routes were uneven.

Readiness signal: Readiness signal: adult pathways, trial access, specialist knowledge, technology adoption, and follow-up.
Links: A3 • C1 • B1 • B5 • 14

How to discuss the 13 indicators + added factor

Find and diagnose patients earlier
A1 • A2 • A3 • A6
Can patients be found, diagnosed, referred, and supported early enough?

Move from decision to access
B1 • B4 • B5
Can patients move from eligibility or prescribing decision to treatment and follow-up without delay?

Equip and connect care teams
C1 • C4 • C5
Can local, specialist, emergency, pediatric, and adult systems work together?

Equity and daily living readiness
E3 • E4
Do supports, devices, geography, language, income, disability, and population equity barriers get measured and addressed?

Learn while delivering care
D2
Can Canada collect evidence on safety, outcomes, continuation, equity, and value?

Cross-cutting factor (14)
Rare disease research and technology development capacity. This can sit across all clusters and be nominated in both rounds.

What do these clusters suggest in terms of what types of indicators are important? Note how other items such as undiagnosed pathways or time-to-access emerge naturally through the patient stories.

Panelist preparation: two nomination rounds

Panelists receive13 rated indicators plus the added 14th discussion factor in advance and asked to prepare concise, evidence-informed choices.

Round 1: nominate five readiness signals

- Which five of the 14 indicators would tell us the most about state of Canada rare disease readiness?
- Why are these cross-cutting, patient-relevant, accountable, and useful over the next 12–18 months?
- Which indicators reveal system gaps that would otherwise stay invisible?

Round 2: nominate three Phase 2 priorities

- Which three indicators should Phase 2 of the Rare Disease Strategy focus as priority?
- What would improve patient experience, system readiness, and access if acted on now?
- What would make Canada more credible for trials, launch, RWE, and investment?

Guide: panelists can choose the same item for both rounds, but must explain whether it is a measurement priority, an action priority, or both. They may include the added 14th factor in either round.

Panel Top Indicators

Jlda	Leanne	Fred
A2 Genetic/genomic testing and counselling	C1. Clinical genetics and (disease) specialist workforce capacity	C1 - Clinical genetics + specialist workforce capacity
C1 Clinical genetics and specialist workforce capacity	D2. RWE infrastructure linked to access/outcomes	B1 - Rare disease medicine access pathways
A6 Rare disease education and decision support for professionals	C5. Pediatric to adult transition supports	B4 - Treatment delivery capacity
C4 Shared records, care plans and transfer of information	B4. Treatment delivery capacity	D2 - RWE infrastructure linked to access/outcomes
D2 Real-world evidence infrastructure linked to access and outcomes	E3. Assistive devices and daily living supports	A6 - RD education + decision support for professionals

Audience Engagement: Informed Choice of What to Measure and Why

Timing	Question	Output
After panel round 1	Choose up to five indicators Canada should use as a readiness check, from the 13 rated indicators plus the added 14th factor.	Audience readiness-signal ranking across 14 items
After result comparison	Where did audience and panel align or differ?	Moderator synthesis of consensus and tension
After panel round 2	Choose up to three indicators Phase 2 should act on first, including the added 14th factor if warranted.	Audience Phase 2 priority ranking across 14 items
Optional final pulse	What type of action is most needed next? Standards, data, navigation, access pathways, workforce, trials, RWE.	Bridge questions for W4

Audience not asked to select “realistic measures” but to choose the readiness signals and priorities that matter after hearing panel rationale. The research/technology item is “added discussion factor, not rated in initial scan.”

Outputs to carry directly into Webinar 4

- Panel top five readiness signals and audience top five readiness signals across 14 discussion items.
- Panel top three Phase 2 priorities and audience top three Phase 2 priorities, including the 14th factor if selected.
- A short narrative explaining where the priorities align with patient moments.
- A bridge question for W4: what would make Canada credible for trials, launch, equitable access, RWE, and investment?

Webinar 3	Webinar 4
Which indicators tell us if Canada is ready?	How do we improve readiness on the chosen indicators, including research/technology capacity?
Which indicators — and whether the added 14th factor — should Phase 2 prioritize?	How does readiness make Canada more attractive for trials, launch, access, and investment?
What do patient moments show is missing?	How do systems collect evidence on current status and progress?

Closing line: "Today we chose the signals. Webinar 4 asks what Canada must do with them."

Webinar 4 **August 11, 2026 12:00 EDT**

Preparing Canada to be Ready for Rare Disease Innovation

Applies readiness to MFN-style global pressures, clinical-trial competition, launch sequencing, innovative therapies, managed access, RWE, and equitable benefit.

RD Strategy + readiness:
Prepares Canada to identify patients, activate sites, deliver access, generate evidence, and return value to patients, developers, and the health system.

Webinar 4 Registration:

https://3sixtypublicaffairs.zoom.us/webinar/register/WN_JD-ISJRrTfuwzVoRV5QwDA

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