WEBINAR 4

From Drug Access to Rare Disease Readiness

What Canada has built — and what Phase 2 must deliver

diagnosis • access • care • data • equity

CORD / CRDN Phase 2 readiness session

Professional Experts

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Session promise

By the end of the webinar, participants should be able to name the weakest links and the Phase 2 actions that would make real pathways work.

1

Readiness lens

shift from drug access alone to the full pathway

2

Changing context

RDDS, Task Force, regulatory alignment, MFN

3

Provincial signals

Quebec implementation + Alberta consultation

4

Two pathway tests

SMA and ATTR-CM as contrasting examples

5

Phase 2 priorities

clear actions for CORD / CRDN follow-up

What are the are practical gaps and what must change next?

Opening: How has the question has changed?

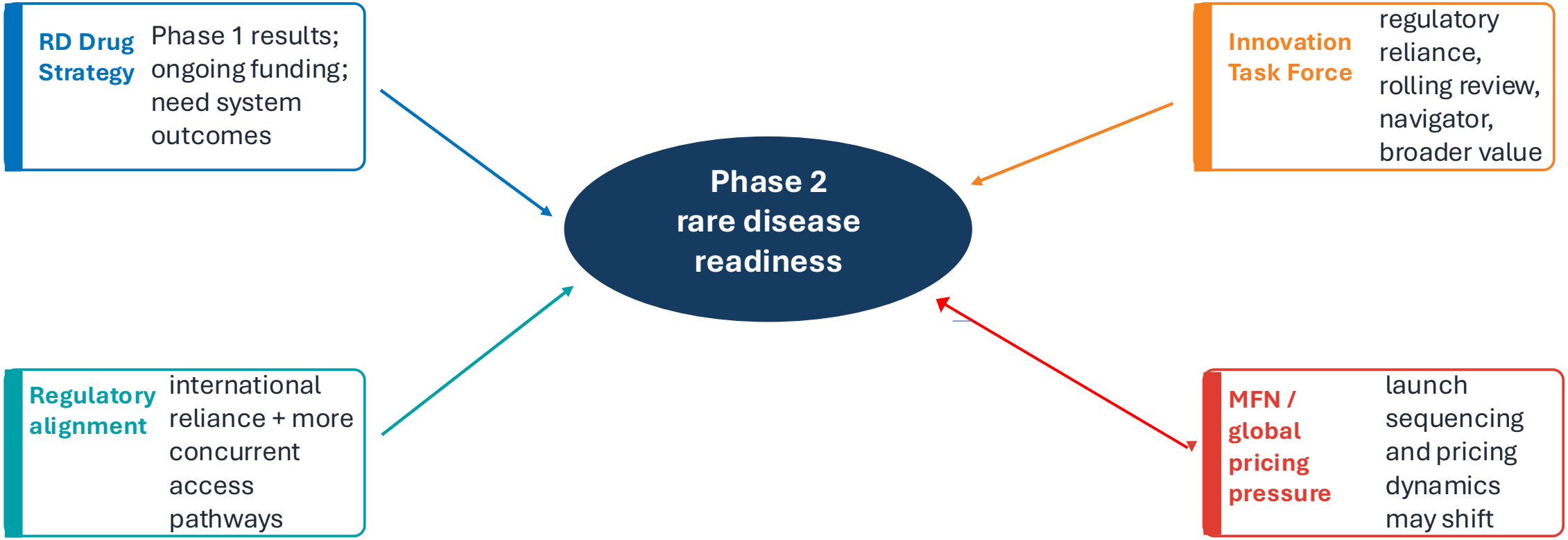
Phase 1 asked whether more drugs could be funded. Phase 2 must ask whether patients can move through the system.

Readiness means the whole pathway works — not only that a drug is listed.

Exercise: identify where the pathway breaks, and what RDDS Phase 2 should fix first.

2026 context: policy is moving on several fronts at once

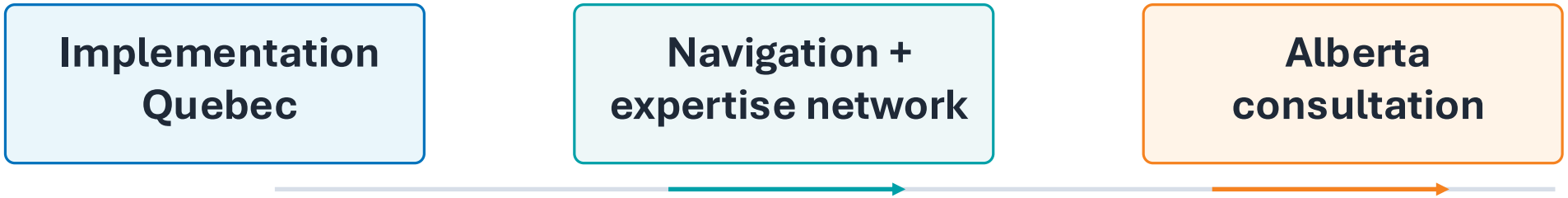
Four current and emerging frameworks create an opening for a broader rare-disease readiness agenda.



Session takeaway: these reforms only matter if they shorten and strengthen real patient pathways.

Provincial systems are beginning to take shape

Quebec and Alberta show two different stages: implementation and design/consultation.



Quebec

Plan d’action 2023–2027 now includes a national clinical navigation network for suspected rare disease and designated expertise centres.

Alberta

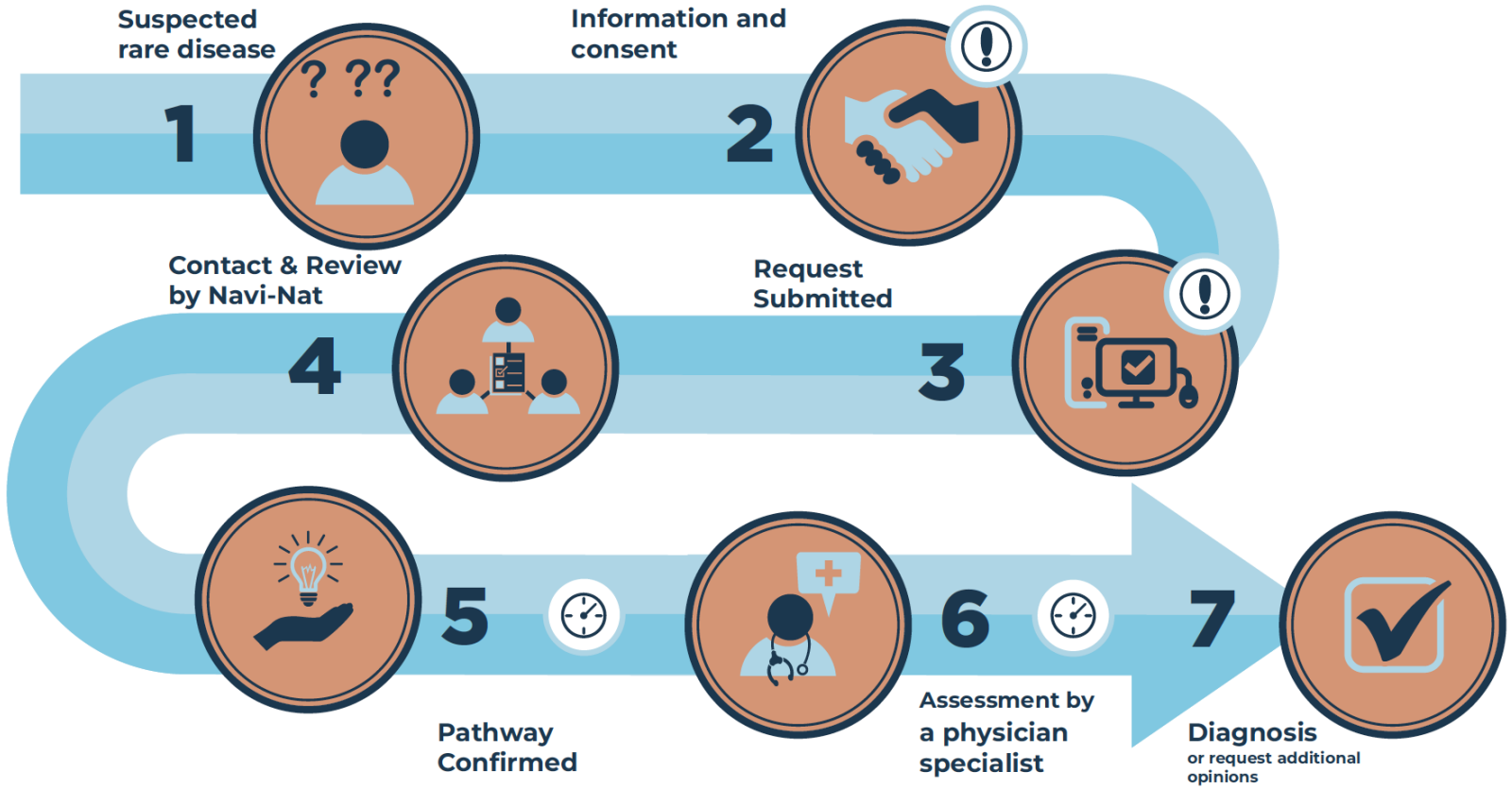
Disease Summit materials focus on gaps in diagnosis, treatment approvals, data/RWE, navigation, coordination and practical next steps.

Navi-Nat

Réseau national de navigation clinique en maladies rares

Québec

Request pathway within the Navi-Nat



Au Québec,

700 000 personnes vivent avec une maladie rare.

Navi-Nat, le premier réseau national de navigation clinique en maladies rares au Canada, aide à orienter les personnes chez qui une maladie rare est soupçonnée.

Wait time varies (specialty waitlists, request complexity, etc.)

Required action by the referring professional

Alberta report-back: what participants heard

Participant report-back prompt

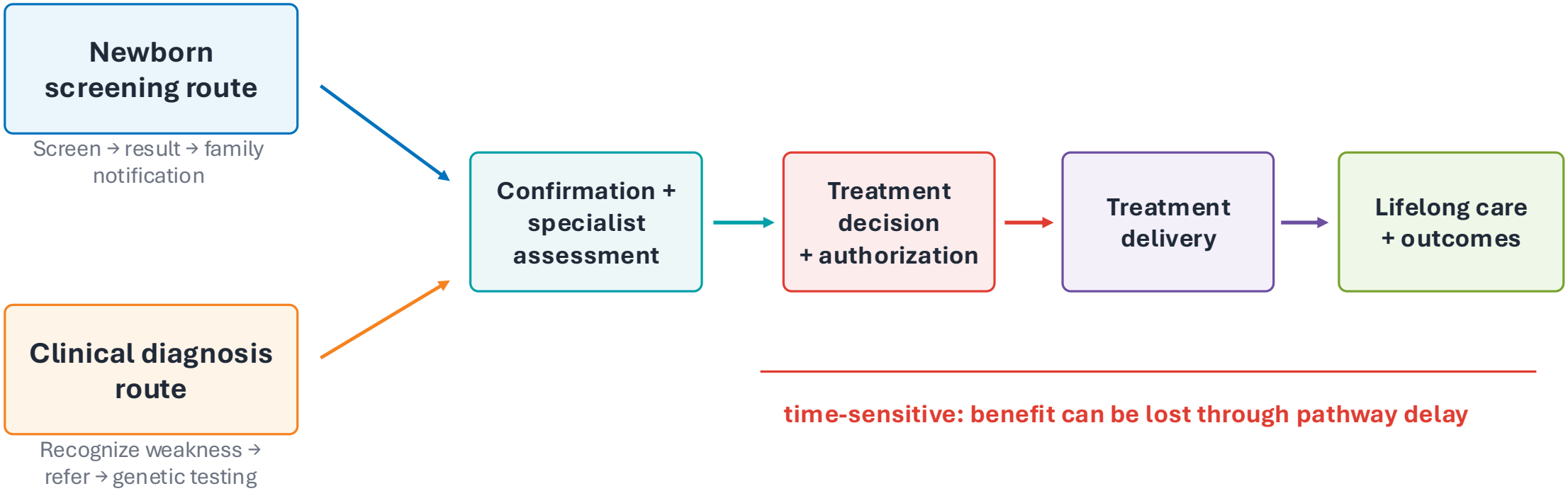
1. What did government ask stakeholders to solve?
2. Which two or three gaps had the strongest consensus?
3. Where did participants see quick wins versus structural change?
4. What should be watched when the outcomes summary is released?

Summit objectives reflected the readiness lens

- expedite drug availability
- expand navigation supports
- reduce administrative burden
- streamline care pathways
- improve coordination and RWE/data

Pathway test 1: SMA

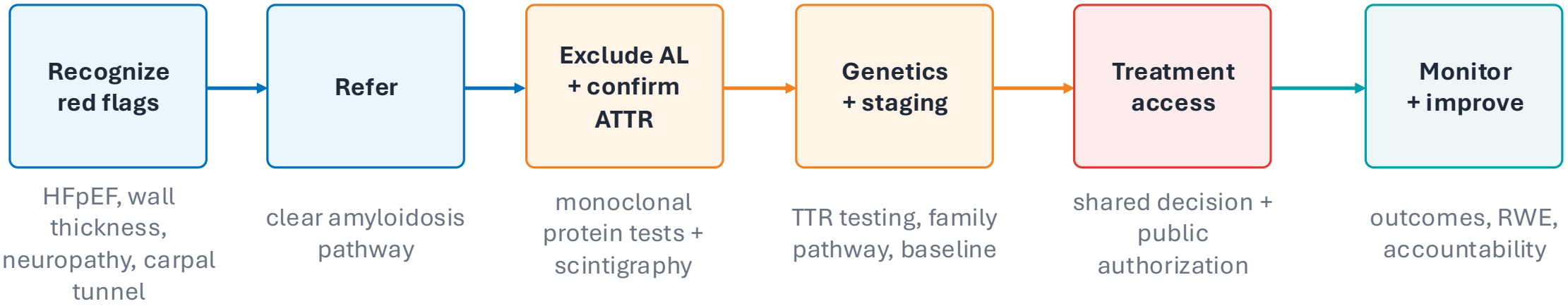
A readiness test for urgent newborn screening, clinical diagnosis across the lifespan, treatment access and lifelong care.



Central question: can early identification become timely treatment and lifelong equitable care at every age?

Pathway test 2: ATTR-CM

A readiness test for recognition, diagnostic coordination, public authorization and long-term monitoring.



Contrast with SMA

SMA tests whether a known patient moves fast enough. ATTR-CM tests whether the system finds the patient early enough.

Central question: can Canada shorten the distance from suspicion to confirmed diagnosis to treatment?

Panel: where do the frameworks meet the patient pathway?

Policy promise	Which reforms could actually reduce patient-level delay?
Pathway reality	Where do patients and clinicians still experience fragmentation?
Evidence + outcomes	What data are needed without overburdening families or clinics?
Phase 2 action	What should be built nationally versus provincially?

Live Readiness Pulse

A. Overall pathway readiness

How ready is your jurisdiction to deliver this pathway reliably?

- Mostly ready
- Uneven / depends where you live
- Major gaps
- Don't know

B. Weakest link

Pick the one pathway component most likely to prevent timely, equitable care.

- SMA: screening-to-action
- SMA: adult/lifelong care
- ATTR-CM: recognition/referral
- ATTR-CM: diagnostic testing
- Both: authorization + outcomes

Final poll: What should RDDS Phase 2 do first to make these pathways work?

Turning webinar input into Phase 2 priorities

Live input

Readiness Pulse + panel priorities

Synthesis

Group gaps into pathway domains

Evidence ask

What provinces / drug plans / programs should provide

Phase 2 platform

Policy asks for September follow-up

Output from Webinar 4

A concise “Rare Disease Readiness: Phase 2 Priorities” document with 5–7 system asks, plus two pathway examples as evidence.

Close: What Phase 2 must deliver

A rare-disease-ready system finds patients earlier, moves them faster, supports them longer and learns from outcomes.

1. Confirm poll results and panel priorities
2. Alberta summit outcomes
3. Refine SMA + ATTR-CM diagrams for final materials
4. Convert findings into RDDS Phase 2 asks

Thank you!