



INSTITUTE OF
HEALTH ECONOMICS

Access With Evidence:

An Evidence-Informed Framework for Rare Disorders

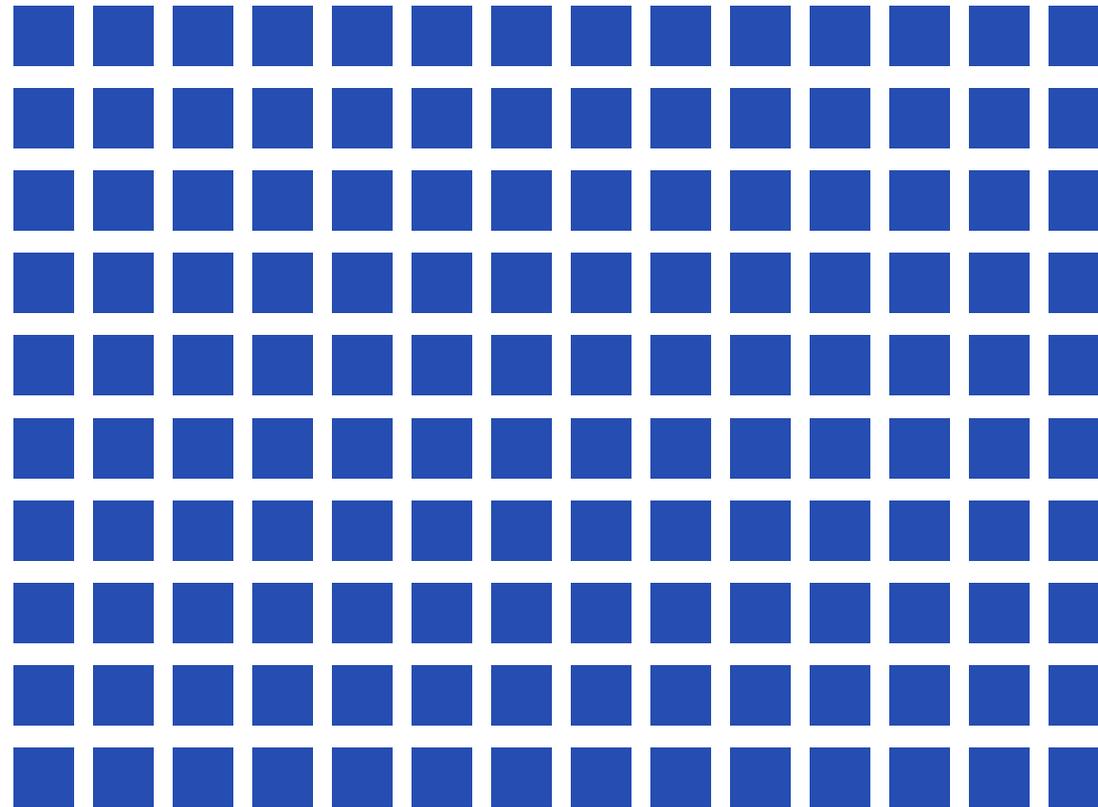
Rare Disease Day Webinar | February 27, 2026

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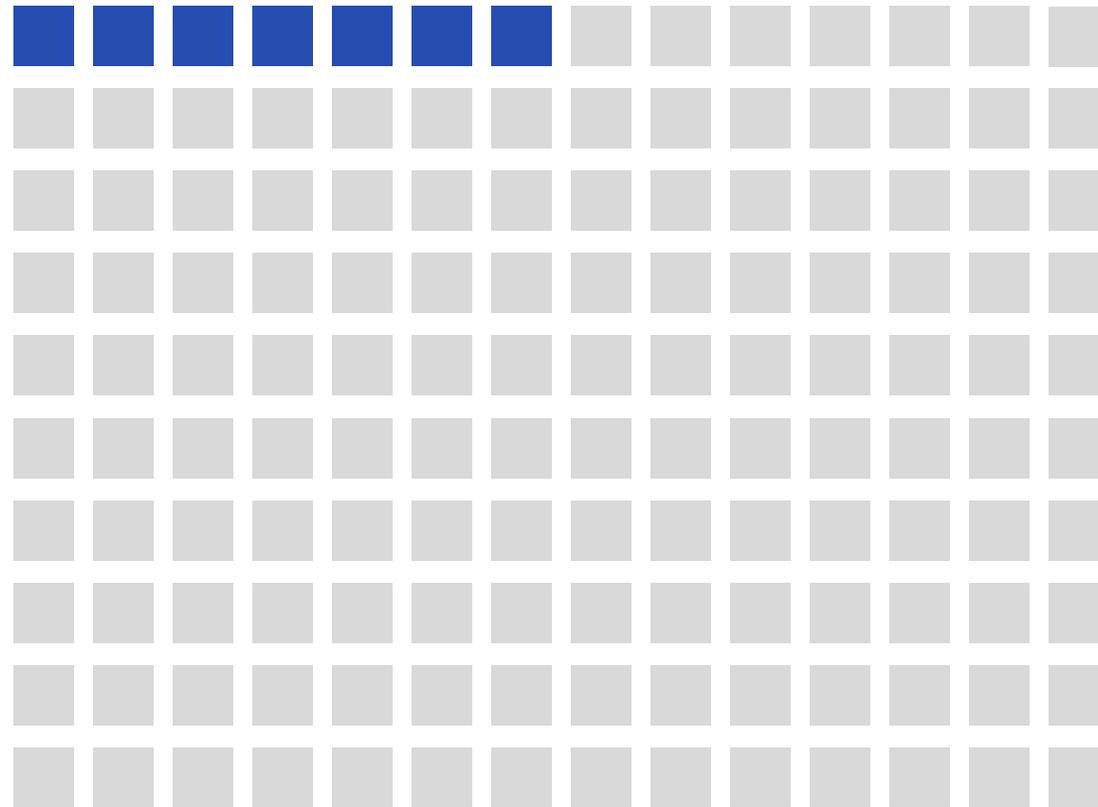
Adjunct Assistant Professor | **University of Alberta**

There are > 7,000 known rare disorders



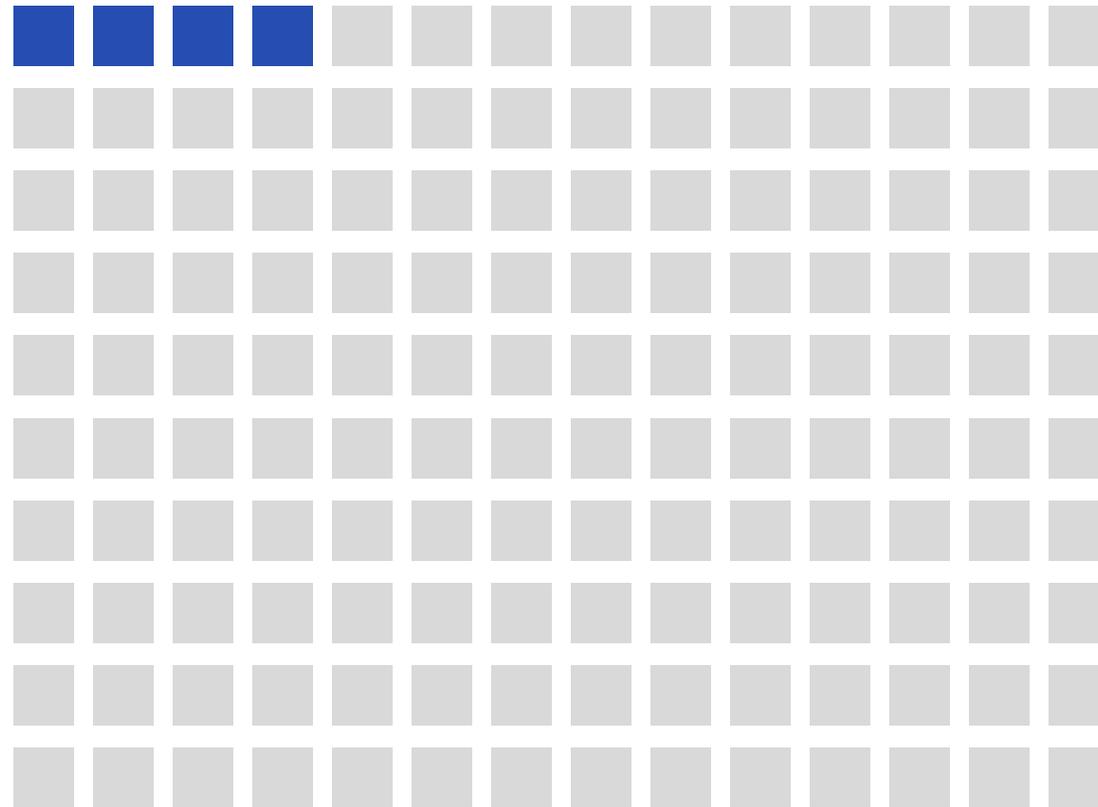
□ = 50 rare conditions

Only 5% have an effective drug therapy



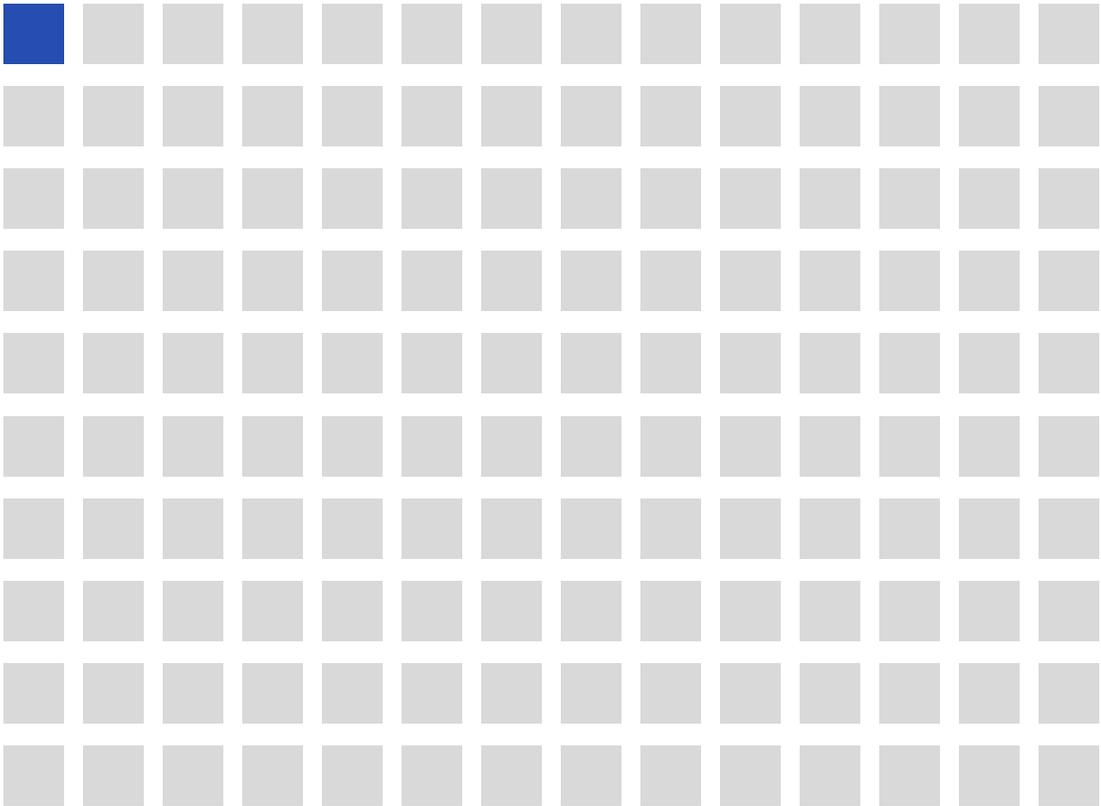
 = 50 rare conditions

Of these, only 60% are approved by Health Canada



□ = 50 rare conditions

Of these, only 25% are listed by public drug plans



□ = 50 rare conditions

The Traditional Evidence-to-Access Pathway



Robust clinical trial data
establishes safety and effectiveness

Value assessed
relative to alternatives and budget impact

Coverage granted
based on demonstrated value

Why Rare Disease Therapies Strain the Traditional Model



- Small sample sizes
- Lack of comparators
- Lack of long-term data



- Delayed
- Denied
- Inconsistent

Shifting To An Adaptive Approach

Standard Listing :



Access With Evidence:



Examples of Access With Evidence Models

Coverage With Evidence Development

Access with research

Performance-Based Agreements

Pay for what works

Milestone-Based Agreements

Pay as value is delivered

Appropriate Use Agreements

Target the right patients

When Access With Evidence Is Most Useful



One-Shot
Curative Therapies



Breakthrough
Life-Extending
Therapies



Disease-Modifying
Chronic Therapies



Heterogenous
Or Variable
Responders



Repurposed
Therapies In Underservice
Populations

What We Need to Move Forward

1. Shared commitment to learning

Access decisions become adaptive, not final.

2. Trust and transparency across sectors

Data governance and clarity on expectations.

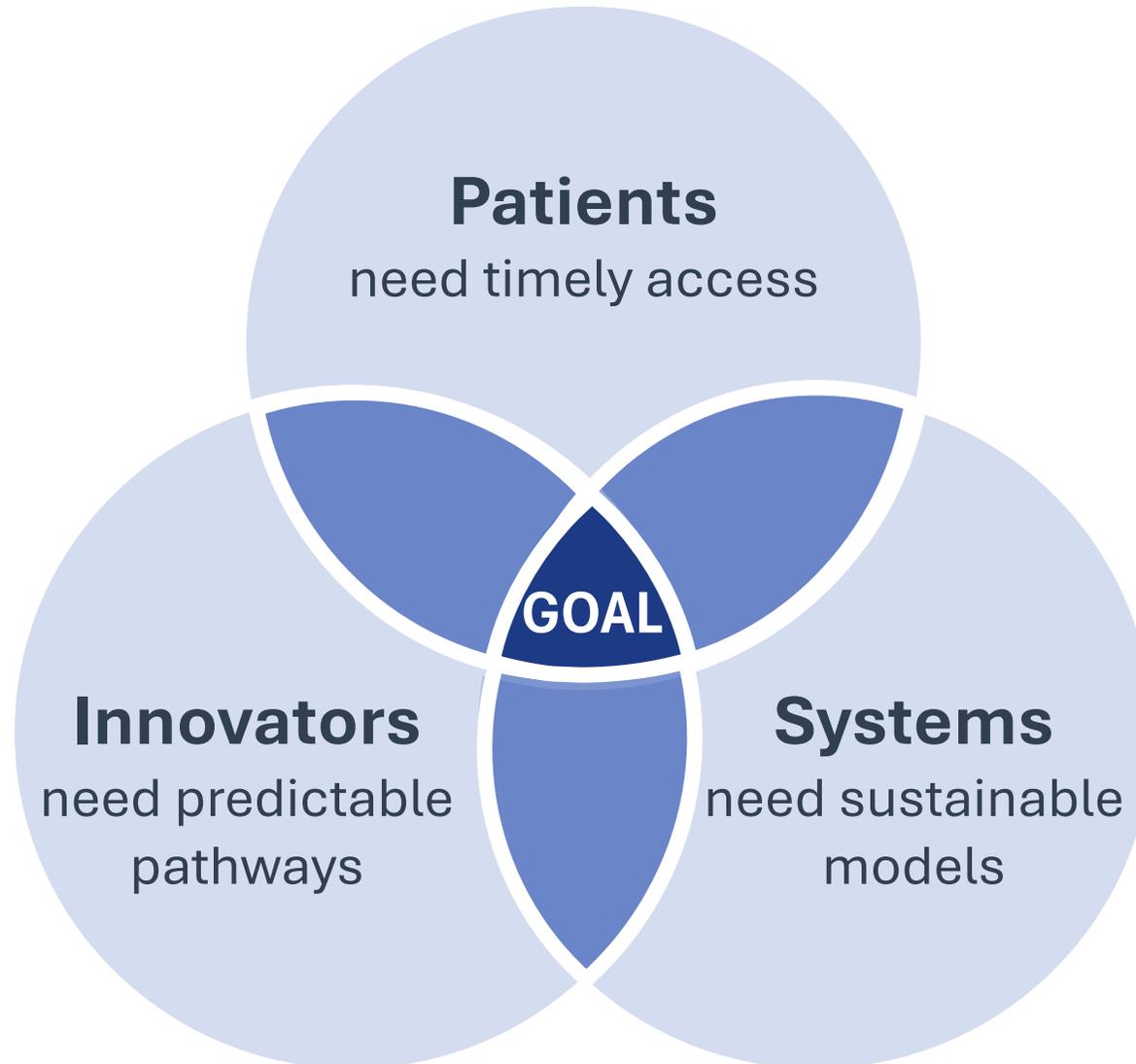
3. Practical guidance

Playbooks on when and how to use these agreements.



*Explore the Outcomes-Based
Agreements Playbook!*

Access With Evidence Balances Three Imperatives



**“The importance of diagnosing individuals
and supporting proper care, research,
and therapeutics development
cannot be overstated.”**

Quote From Caregiver Respondent

Health Technology Innovation Platform (HTIP)

Thank You



HEALTH COALITION
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Santé
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