Canadian Organization for Rare Disorders (CORD)

Submission on the BC Expensive Drugs for Rare Diseases Individual Patient Decision-Making Process

August 2026

The Canadian Organization for Rare Disorders (CORD) appreciates the opportunity to provide input on the principles that should guide British Columbia's individual patient decision-making process for drugs for rare diseases. We understand that the current consultation is specifically focused on individual patient decision-making and that the population-based funding decision process is not under review.

CORD believes this distinction provides an important starting point. **The first principle for an individual patient decision-making process should be that individual review is undertaken only when it serves a necessary and clearly defined purpose that has not already been addressed through the drug-level assessment and reimbursement process.**

We therefore recommend that BC adopt a **fit-for-purpose approach to individual patient review**. Individual adjudication should not be required automatically because a medicine treats a rare disease or has a high per-patient cost. The need for additional review, the questions it is intended to answer, the expertise required and the participation of the patient and family should depend upon the characteristics of the disease, therapy and individual circumstances.

1. Individual patient review should add value, not duplicate previous decisions

Before a rare disease medicine reaches an individual funding application, substantial questions concerning the therapy have already been considered through national and provincial assessment and reimbursement processes, including the evidence supporting its use, the appropriate patient population and conditions for reimbursement, economic and budget considerations and, where applicable, price negotiation.

CORD recommends a clear separation between:

- the **drug or program-level decision** about whether a medicine should be publicly funded and under what conditions; and
- the **patient-level decision** about whether an individual patient meets those established conditions and can appropriately receive treatment.

Issues already determined at the drug or program level should not be reconsidered when an individual patient's application is reviewed.

Accordingly, the Ministry should be able to identify for every individual review:

What new question is being answered that has not already been answered through previous review and funding processes?

Where there is no material patient-specific question remaining, an additional adjudicative committee review creates delay and administrative burden without adding corresponding decision value.

2. Individual review should be fit for purpose rather than automatic

CORD does not suggest that individual patient assessment is never appropriate. Rare diseases and rare disease therapies differ substantially.

For some medicines, eligibility criteria may be objective and readily confirmed by a treating specialist. In these circumstances, individual access should normally proceed through a streamlined authorization process based on confirmation that the patient meets published initiation criteria.

For other therapies, meaningful clinical uncertainty may remain at the patient level. This may occur where:

- disease manifestations and rates of progression are highly variable;
- genotype or phenotype affects treatment eligibility or likely response;
- initiation or renewal criteria require interpretation rather than simple verification;
- validated measures of disease progression or treatment response are limited;
- the therapeutic window is narrow;
- significant irreversible progression may occur while a decision is delayed; or
- CDA-AMC or another appropriate expert body has specifically identified a need for specialist adjudication.

The appropriate level of individual review should therefore be determined **drug by drug and disease by disease according to the clinical need for additional adjudication**, rather than by applying a universal individual review requirement to medicines simply because they fall within the rare disease program.

3. Where expert judgment is required, reviewers must have appropriate disease-specific expertise

This principle is particularly important for rare and ultra-rare diseases.

If the remaining question is sufficiently complex to require expert clinical judgment beyond that of the treating specialist, the adjudication should involve clinicians with relevant expertise in the particular disease and therapy.

A general multidisciplinary committee may provide valuable advice on program policy, consistency, ethics or stewardship. However, membership on a general rare disease committee does not necessarily provide the highly specialized expertise needed to interpret the natural history, phenotype, biomarkers, disease progression and treatment response of a particular rare or ultra-rare condition.

There is an inherent inconsistency in arguing that rare diseases require additional review because their evidence and clinical course are unusually complex, while assigning the individual clinical decision to reviewers who do not have specific expertise in the disease under consideration.

CORD therefore recommends a simple principle:

If an application requires only confirmation that published eligibility criteria are met, additional expert adjudication should not be necessary. If genuine clinical interpretation is required, it should be undertaken by clinicians with relevant disease-specific expertise.

Canadian experience demonstrates that such approaches are feasible. For Fabry disease, disease-specific expertise has been incorporated into assessment and renewal arrangements rather than relying solely on a general drug-review body.

Fibrodysplasia ossificans progressiva (FOP) provides another particularly relevant example. In its reimbursement assessment of palovarotene (Sohonos), CDA-AMC identified the challenges involved in assessing patients with a heterogeneous ultra-rare condition and recommended access to expert clinical adjudication. Sohonos is also included on the national Common List. CORD understands that a multi-province expert approach involving, among others, Ontario, Manitoba and Saskatchewan is now being used for FOP.

This is a sensible model for an ultra-rare disease in which expertise is necessarily scarce and distributed across Canada. It avoids requiring every province to reproduce expertise locally while ensuring that decisions requiring specialist interpretation are made by those best qualified to make them.

Importantly, this does **not** imply that a disease-specific panel should be established for every rare disease medicine. The FOP model is appropriate precisely because the characteristics of FOP and palovarotene warrant this degree of specialist adjudication. In other circumstances, treating-specialist confirmation against established criteria may be entirely sufficient.

4. Where an individual review is warranted, patients and families must be participants in the decision-making process

If the Ministry determines that a particular application genuinely warrants individual adjudication, **the individual patient and family must have a meaningful opportunity to participate in that process**.

An individual review should not consist solely of applying population-level evidence and administrative criteria to an individual patient's file. The purpose of retaining an individualized process should be, in part, to understand circumstances that cannot be fully captured through clinical trial averages, standardized eligibility criteria or administrative documentation.

Rare diseases can vary significantly among individuals in presentation, progression, severity and impact. The consequences of disease may extend well beyond conventional clinical measures to mobility, independence, communication, cognition, education, employment, caregiving demands and the ability to participate in family and community life. The significance of a particular treatment benefit or the consequences of delaying treatment may therefore differ substantially among patients who share the same diagnosis.

Where an individual review is conducted, the process should provide a structured opportunity for the patient and, where appropriate, family or caregivers to contribute information about:

- the patient's individual disease course and progression;
- symptoms and impacts that may not be fully reflected in standard clinical measures;
- effects on functional ability, independence and quality of life;
- the burden on family and caregivers;
- previous treatments and the consequences of inadequate disease control;
- the patient's goals and priorities for treatment;
- the consequences of treatment delay or further disease progression; and
- other circumstances relevant to assessing the potential value and appropriateness of treatment for that individual.

This participation could occur through written input, a standardized patient/family submission, direct participation where appropriate, or another accessible mechanism that allows the patient's perspective to be meaningfully considered without creating an additional administrative burden.

The treating specialist should also have the opportunity to provide clinical context, respond to questions from the reviewing experts and explain why treatment is appropriate in circumstances that may not be apparent from standardized criteria alone.

Importantly, patient participation should be **meaningful rather than procedural**. Relevant patient and family information should form part of the evidence considered in reaching the decision and should be reflected, where material, in the reasons provided for that decision.

CORD believes there is a fundamental principle at stake:

If BC retains an individual patient decision-making process because individual circumstances matter, then the individual patient and family must be able to inform that decision.

A process cannot reasonably be characterized as individualized if the person whose circumstances are being adjudicated has no meaningful opportunity to contribute to the assessment.

This principle is also consistent with concerns raised by other stakeholders about the need for patients and treating clinicians to have meaningful opportunities to submit relevant evidence and address uncertainty in the EDRD process.

5. BC should consider a tiered authorization model

CORD recommends that the Ministry consider three pathways.

A. Criteria-based authorization

Where a patient clearly meets established and published initiation criteria, the qualified treating specialist should attest to eligibility and PharmaCare should authorize coverage within a defined service standard. Further committee adjudication should not ordinarily be required.

Patient and family submissions should not become an additional requirement for straightforward criteria-based authorization. The objective is to remove unnecessary steps, not create new ones.

B. Disease-expert individual review

Where eligibility, expected benefit, response or renewal requires substantive expert interpretation, the application should be considered by appropriately qualified disease-specific experts.

For ultra-rare diseases, BC should actively consider participating in multi-provincial or pan-Canadian expert mechanisms rather than establishing separate provincial panels.

Whenever such an individual review occurs, the patient and family should have a defined opportunity to provide relevant information about the patient's circumstances, experience of disease, treatment priorities and anticipated impact of treatment or non-treatment.

C. Exceptional-case review

Where a patient falls outside established criteria but the treating specialist believes there is a compelling clinical rationale for treatment, an exceptional review mechanism should remain available.

Such cases particularly require:

- relevant disease-specific clinical expertise;
- meaningful input from the treating specialist;
- patient and family participation;
- consideration of individual disease burden and circumstances; and
- transparent reasons for the resulting decision.

This tiered approach would preserve individual review where it contributes meaningful clinical and patient value while substantially simplifying access for routine applications.

6. Affordability and formulary decisions belong at the program level

CORD recognizes the Ministry's responsibility for fiscal stewardship and sustainability. However, these are principally **program-level responsibilities**, rather than patient-level clinical questions.

Once BC has decided to fund a medicine for an identified population and has established eligibility criteria, an otherwise eligible patient's application should not become a second opportunity to reconsider whether the drug is affordable or represents sufficient value.

If availability of an EDRD program budget or other financial considerations can directly determine whether an individual eligible patient receives treatment, this should be stated transparently. More fundamentally, CORD recommends that budget impact, portfolio management and decisions about competing expenditures be addressed explicitly at the program level rather than indirectly through individual patient approvals or denials.

The implementation of the National Strategy for Drugs for Rare Diseases makes this distinction increasingly important. Where BC has made a positive decision to provide access to a Common List medicine, the individual access mechanism should give practical effect to that decision rather than recreate the underlying reimbursement decision.

7. BC Cancer provides a useful provincial precedent

The Ministry may also wish to consider the principles underlying the BC Cancer drug funding model.

BC Cancer makes program-level decisions concerning medicines and approved indications and incorporates funded treatments into its formulary and treatment protocols. Routine access to an approved therapy does not require a general committee to

reconsider the underlying funding decision for every patient. Individual review mechanisms can instead be reserved for restricted access or circumstances falling outside established program criteria.

CORD recognizes that cancer and rare disease programs are not identical. Nevertheless, the model demonstrates an important principle relevant to this consultation:

High cost, specialized medicine and clinical complexity do not inherently require universal patient-by-patient funding adjudication.

A program can make rigorous formulary and indication-level decisions, establish appropriate clinical protocols and criteria, enable routine access for eligible patients and reserve individual adjudication for circumstances in which it genuinely adds value.

CORD believes the same principle should inform modernization of the EDRD individual decision process.

8. Individual decision-making should be transparent and accountable

Whatever model is retained, patients and clinicians should be able to understand:

- why an individual review is required for a particular drug;
- the specific question the review is intended to answer;
- why that question requires adjudication rather than treating-specialist confirmation;
- the expertise of those making the determination;
- how the patient and family can participate;
- what information concerning the patient's individual circumstances and impact will be considered;
- the criteria that will be applied;
- expected decision timelines;
- whether considerations other than individual clinical eligibility can affect the decision;
- the reasons for an approval or denial; and
- the process for reconsideration where relevant new information is available.

These requirements are particularly important where treatment delay can result in irreversible disease progression or loss of therapeutic opportunity.

Conclusion

CORD recommends that British Columbia move from an assumption of **universal exceptional patient review** toward a **fit-for-purpose individual access framework**.

For every therapy, three questions should guide the process:

1. Is there a patient-specific question remaining after the medicine has been assessed and approved for funding that genuinely requires individual adjudication?

2. If individual clinical judgment is required, are the decision-makers appropriately qualified through relevant disease-specific expertise?

3. If the decision is genuinely individualized, does the patient and family have a meaningful opportunity to contribute the circumstances, impacts and priorities that make the case individual?

Where the answer to the first question is **no**, confirmation by an appropriately qualified treating specialist that the patient meets established criteria should ordinarily be sufficient.

Where individual adjudication **is** warranted, the process should be designed around the particular clinical question, involve clinicians with relevant disease-specific expertise and meaningfully incorporate the perspective and circumstances of the patient and family.

Where the issue is instead one of affordability, budget impact or whether the therapy should be publicly funded at all, that decision should be made transparently at the drug or program level—not through reconsideration of an individual patient's request.

This approach would not eliminate individual review. It would ensure that individual review is used **only where it adds value, by those best qualified to provide that value, and with the participation of the patient and family whose circumstances are being considered**.

CORD believes this would produce a more timely, clinically credible, patient-centred, equitable and sustainable pathway for British Columbians living with rare diseases.