Health Canada Santé Canada

National Strategy for Drugs for Rare Diseases Initiative

Canada - British Columbia Funding Agreement

Between: His Majesty the King in Right of Canada, as represented by the Minister of Health (hereinafter referred to as "Canada")

And: His Majesty the King in Right of the province of British Columbia, as represented by the Minister of Health (hereinafter referred to as "British Columbia")

Canada and British Columbia are also referred to as a “Party” or collectively as the “Parties”

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Preamble

Whereas, the Parties have agreed to targeted federal funding over three (3) years, beginning in 2024-25, for investments to increase access to, and affordability of, promising and effective drugs for rare diseases (DRD) to improve the health of patients;

Whereas, in Budget 2021, the Government of Canada reaffirmed its announced plan to provide ongoing funding of $500 million per year to help Canadians with rare diseases access the drugs they need;

Whereas, in March 2023, the Government of Canada announced the National Strategy for DRD, which will make available up to $1.4 billion over three (3) years to provinces and territories through bilateral agreements;

Whereas, the Government of Canada authorizes the Minister of Health to enter into agreements with the provinces and territories, for the purpose of supporting activities provinces and territories will undertake to help improve access to New DRD, enhance access to existing DRD, invest in governance and infrastructure, and improve Screening and Diagnostics for rare diseases;

Whereas, the Lieutenant Governor in Council authorized the British Columbia Minister of Health under the *Ministry of Intergovernmental Relations Act* to enter into agreements with the Government of Canada under which Canada undertakes to provide funding toward costs incurred by British Columbia, to help improve access to New DRD, enhance access to existing DRD, invest in governance and infrastructure, and improve Screening and Diagnostics for rare diseases;

Whereas, Canada respects jurisdictional roles, and recognizes that British Columbia is responsible for the design and delivery of publicly insured prescription drug coverage to its residents;

Whereas, Canada respects and acknowledges the existing pharmaceutical management systems and the roles of the pan Canadian Pharmaceutical Alliance and British Columbia. The National Strategy for DRD will operate within the existing systems;

Whereas, British Columbia acknowledges to meaningfully engage and work with Indigenous organizations and governments responsible for the delivery of drug benefits to further support access to DRD;

Whereas, British Columbia acknowledges the overall importance of the meaningful support of official languages, including but not limited to the support of official languages minority communities; and

Whereas, British Columbia acknowledges the overall importance of the meaningful support of the use of Sex and Gender-Based Analysis Plus to address the different

needs of diverse populations based on identity factors such as sex, gender, age, disability, Indigeneity, sexual orientation, ethnicity, religion and more.

Therefore, the Parties agree as follows:

1. Definitions

In this agreement,

1.1. "Agreement" means this funding agreement and includes all annexes and any amendments made to this Agreement in accordance with section 9;

1.2. "Common Set" means the list of drugs in Annex A that has been established and is maintained through the collaborative process referenced in section 5.2 of this Agreement;

1.3. "Diagnostics" means the process of determining the nature of a disease or disorder and distinguishing it from other possible conditions in symptomatic patients or those that have screened positive;

1.4. "Election" means the designation of a drug on the Common Set by a provincial or territorial government as a drug to be made publicly available by that province or territory per section 2.2.a. and 5.1, as notified to Canada per section 12 upon the effective date of this Agreement and at any point thereafter up to and including March 31, 2026;

1.5. "Eligible Expenditures" means the costs described in section 5.6 of this Agreement that Canada has agreed to contribute to and that are incurred and paid by British Columbia in carrying out the Initiative;

1.6. "Evidence Collection Mechanisms" mean the processes and activities undertaken to support evidence collection in relation to the drugs in the Common Set as described in Annex B of this Agreement;

1.7. "Fiscal Year" means the twelve-month period beginning April 1st of any year and ending March 31st of the following year, and including parts thereof in the event that this Agreement commences after April 1st or expires or terminates before March 31st;

1.8. "Governance Mechanism" is defined as the Pharmaceutical Executive Group (Assistant Deputy Minister working group comprised of public drug plans which is responsible for making recommendations to Deputy Ministers for decision) and its affiliated sub-groups as may be formed from time to time;

1.9. "Initiative" means funding available to provinces and territories through bilateral agreements as part of the National Strategy for Drugs for Rare Diseases;

1.10. “New DRD” means drugs for rare diseases which have received a Notice of Compliance (NOC) by Health Canada in 2019 or later; and

1.11. “Screening” means to assess the likelihood that an asymptomatic individual in a population has a health problem or condition.

2. Objectives

2.1. The Parties commit to work together to increase access to, and affordability of, effective DRD to improve the health of patients across Canada.

2.2. The Parties agree that with financial support from Canada, British Columbia will:

a. Improve patient access to drugs on the Common Set through the Election of drugs by British Columbia;
b. Improve coverage for New DRD not in the Common Set and/or other existing DRD; and
c. Work toward improving Screening and Diagnostics for rare diseases.

2.3. Further, the Parties commit to work together to establish and strengthen national governance and data infrastructure for DRD to improve information coordination and evidence for decision-making.

3. Term of agreement

3.1. This Agreement will come into effect when the last Party has signed and will end on March 31, 2027, unless terminated earlier in accordance with section 11 of this Agreement.

3.2. Notwithstanding section 3.1, this Agreement covers Eligible Expenditures incurred to carry out the Initiative during the period between: April 1 of the year in which it is signed and ending March 31, 2027.

4. Financial contribution and obligations

4.1. Canada has designated the following maximum amounts to be transferred in total to all provinces and territories under this Initiative on a “base plus per capita” basis for the duration of this Agreement:

a. $468,774,452 for the Fiscal Year beginning on April 1, 2024
b. $468,774,452 for the Fiscal Year beginning on April 1, 2025
c. $468,774,452 for the Fiscal Year beginning on April 1, 2026

4.2. Fixed annual funding for the duration of this Agreement has been allocated with a base amount of $1,500,000 for each province and territory and the remainder

of the funding allocated on a per capita basis. The final total amount will be paid to British Columbia and has been calculated using the following formula: $1,500,000 + (F – (N x 1,500,000)) x (K/L), where:

F is total one-time funding amount available under this Initiative, which is equivalent to the maximum amount payable listed in section 4.1;

N is number of jurisdictions (all 13) that will be provided the base funding of $1,500,000;

K is total population of the particular province or territory, as determined using the population estimates from Statistics Canada minus the number of eligible clients of the Non-Insured Health Benefits program residing in that particular province or territory; and

L is total population of Canada, as determined using the most recent annual population estimates from Statistics Canada minus the number of eligible clients of the Non-Insured Health Benefits program.

4.3. Allocation to British Columbia for Fiscal Year 2024-25:

Subject to the terms of this Agreement and based on the formula described in section 4.2, Canada will make a contribution to British Columbia of up to sixty-four million five hundred and seventy thousand one hundred and eighty-one dollars ($64,570,181) toward Eligible Expenditures.

4.4. For the purposes of the formula in section 4.2, the population of British Columbia for each Fiscal Year and the total population of all provinces and territories for each Fiscal Year will be determined by population numbers released by Statistics Canada in March of each year. Non-Insured Health Benefit populations will be determined by the most recent figures available from Indigenous Services Canada as of calculation date of March 31. Adjustments to allocations for each Fiscal Year will be made at the beginning of each Fiscal Year in accordance with Statistics Canada updated population figures.

4.5. Payments

Canada's contribution will be paid in semi-annual amounts as follows:

4.5.1 The first installment amount will be paid within fifteen (15) business days from the time this Agreement comes into effect, and on or about April 15 of each subsequent Fiscal Year. The second installment will be paid on or about November 15 of each Fiscal Year.

4.5.2 The amount in the first installment will be equal to 50% of the amount set out in 4.3 for 2024-25 and 50% of the amount determined pursuant to 4.2 for Fiscal Year 2025-26 and Fiscal Year 2026-27.

4.5.3 The amount of the second instalment will be equal to the balance of Canada's contribution to British Columbia for the Fiscal Year as determined under section 4.2.

4.5.4 The sum of both semi-annual installments constitutes a final payment for the Fiscal Year.

4.6. Adjustment

Notwithstanding any other provisions of this Agreement, Canada may withhold or reduce any payments to be made to British Columbia pursuant to this Agreement in the event that:

a. British Columbia has failed to make an Election of any Common Set drugs as outlined in sections 2.2.a. and 5.1 before signing this Agreement in Fiscal Year 2024-25 or has no Common Set drugs Elected at the beginning of Fiscal Years 2025-26 and 2026-27.
b. Any report has not been submitted by British Columbia in accordance with the requirements set out in section 6; or
c. Any such report or audit conducted pertaining to this Agreement indicates that the Province/Territory's actual Eligible Expenditures for the Initiative have been lower than the amount disbursed to British Columbia up to the time of such report or audit.

4.7. Overpayments

In the event payments made exceed the amount to which British Columbia is entitled under this Agreement, the amount of the excess is a debt due to Canada and, unless otherwise agreed to in writing by the Parties, British Columbia shall repay the amount within sixty (60) calendar days of written notice from Canada.

4.7.1. For greater specificity and without reducing the general application of the foregoing, a failure to meet the requirements of section 5.3, 5.3.1, or 5.4.2 after funds have been fully paid out will constitute an incidence of payments exceeding the amount to which British Columbia is entitled under this Agreement and the amount of excess payable as a debt due to Canada will be calculated as follows:

4.7.1.1. For section 5.3: 50% x [Total funding amount determined under section 4.2 of this Agreement for Fiscal Year 2026-27] - [Amount of federal funds spent on Common Set per section 5.3] = debt due to Canada under this section (only if positive value).

4.7.1.2 For section 5.3.1: 10% x [Total amount of British Columbia's Eligible Expenditures for the Common Set under section 5.3 in Fiscal Year 2026-27] - [Amount of British Columbia's own funding from sources other than this Agreement spent on Eligible Expenditures for the Common Set under section 5.3 in Fiscal Year 2026-27] = debt due to Canada under this section (only if positive value).

4.7.1.3. For section 5.4.2: 10% x [Total funding amount determined under section 4.2 of this Agreement for fiscal year 2026-27] - [Amount of federal funds spent on Screening and Diagnostics per section 5.4.2] = debt due to Canada under this section (only if positive value).

4.8. Retaining Funds

For Fiscal Years 2024-25 and 2025-26, upon request, British Columbia may retain and carry forward to the next Fiscal Year the amount of up to 10% of the contribution paid to British Columbia for a Fiscal Year that is in excess of the amount of the Eligible Expenditures actually incurred by British Columbia in that Fiscal Year. Any request by British Columbia to retain and carry forward an amount exceeding 10% will be subject to discussion and mutual agreement in writing by their designated officials, at the Assistant Deputy Minister level, and is subject to monitoring and reporting to Canada on the management and spending of the funds carried forward.

4.9. Underspending

British Columbia shall inform Canada in writing of any potential underspending one hundred and twenty (120) calendar days before March 31 for any given Fiscal Year.

4.10. Funding subject to appropriation and initiative funding authorities

a. Notwithstanding any other provision of this Agreement, the amount of funding to be provided to British Columbia pursuant to this Agreement is subject to there being an Appropriation of funds by the Parliament of Canada for the Fiscal Year in which any commitment would come due for payment.

b. In the event that authorities for the Initiative are amended or terminated, or if funding levels are reduced or cancelled by the Parliament of Canada for any Fiscal Year in which a payment is to be made under this Agreement, Canada may reduce or terminate any further payments to be made under this Agreement.

c. Where funding under this Agreement is to be reduced or terminated, Canada shall provide British Columbia with at least sixty (60) calendar days

written notice of the reduction or termination and shall reimburse the British Columbia for any Eligible Expenditures incurred up to the date upon which the reduction/termination is to take effect.

5. Use of funds

5.1. British Columbia will use funds for Eligible Expenditures related to the Common Set of drugs, per all Elections made by British Columbia, for New DRD not in the Common Set and/or other existing DRD, and for the improvement of Screening and Diagnostics for rare diseases.

5.2. British Columbia will participate via the established Governance Mechanism in the collaborative process to:

a. update the Common Set, as required; and
b. contribute to the development and implementation of Evidence Collection Mechanisms for use in decision making on public plan drug listing as outlined in Annex B.

5.3. In Fiscal Year 2026-27, British Columbia will use a minimum of 50% of the federal funding made available under this Agreement for Eligible Expenditures incurred in that Fiscal Year that arise from the making available of Common Set drugs for which British Columbia has made an Election, per sections 2.2.a. and 5.1.

5.3.1 In Fiscal Year 2026-27, British Columbia must pay, using its own funding from sources other than this Agreement, a cost- share equal to a minimum of 10% of Eligible Expenditures incurred in that Fiscal Year that arise from the making available of Common Set drugs for which British Columbia has made an Election, per sections 2.2.a. and 5.1.

5.4. British Columbia will participate via established Governance Mechanism in a collaborative process to work towards the improvement of Screening and Diagnostics for rare diseases and identifying best practices and lessons related to rare disease initiatives.

5.4.1 In Fiscal Year 2024-25 and 2025-26 British Columbia may allocate a total of up to 10% of the Fiscal Year federal funding under this Agreement, on activities related to the improvement of Screening and Diagnostics for rare diseases.

5.4.1.1 British Columbia, may, in accordance with their need and circumstances, allocate unutilized Screening and Diagnostic funds in Fiscal Year 2024-25 and 2025-26 to other Eligible Expenditures.

5.4.2 In Fiscal Year 2026-27 British Columbia will use 10% of the federal funding made available under this Agreement for Eligible Expenditures incurred in that

Fiscal Year for activities related to the improvement of Screening and Diagnostics for rare diseases.

5.5. British Columbia will allocate the remainder of the federal funding under this Agreement not used in sections 5.3 and 5.4 to incrementally improve coverage for New DRD (not in the Common Set) and/or other existing DRD.

5.6. British Columbia will use funding under this Agreement only for expenditures that support the objectives of the Initiative, and are directly related to the activities set out herein. Eligible Expenditures include:

a. Operating costs including: salaries and benefits, contractual personnel, honoraria, training and professional development, travel and accommodation, materials and supplies, equipment, and rent and utilities;
b. Costs related to changes to public drug plans (e.g., the cost of adding new drugs to formulary, the cost of reducing out-of-pocket costs, the cost of planning, administrative, and communication activities related to DRD coverage changes);
c. Costs associated with improving and broadening rare disease treatments and services (e.g., DRD coverage, screening or diagnostic initiatives, surveillance, evidence collection, and research on enhanced DRD coverage including costs associated with drug pricing or reimbursement pilot projects);
d. Information technology and related investments, including enhancement of data collection, analysis, and sharing capacity; and
e. Activities associated with evaluation and reporting obligations.

6. Performance measurement and reporting

6.1. British Columbia will designate an official or official(s) for the duration of this Agreement to represent its interests related to performance measurement. This includes supporting, through committee structures as they may be established, the establishment and strengthening of national governance and data infrastructure for DRD by:

a. enabling the development of and reporting on performance measurement and other metrics relevant to improving national decision making on DRD, such as data sharing of evidence and outcomes with pan Canadian health organizations; and
b. identifying and sharing best practices and lessons learned related to rare disease initiatives.

6.2. As a condition of receiving annual federal funding under this Agreement, by no later than October 1, 2025, October 1, 2026, and October 1, 2027, British Columbia agrees to:

6.2.1 Provide to Canada an attested annual financial statement of the funding received from Canada under this Agreement during the prior Fiscal Year compared against sections 5.3, 5.4 and 5.5 of this Agreement as outlined in section 6.2.1.1.

6.2.1.1 The financial statement shall show:

i. the total amount of funding received from Canada under this Agreement;
ii. If applicable, the amount of any overpayment that is to be repaid to Canada under section 4.7;
iii. If applicable, the amount carried forward under section 4.8;
iv. the total amount of funding from Canada spent on the Eligible Expenditures for drugs in the Common Set (section 5.3);
v. the total amount of PT funding (from sources other than this Agreement) spent on the Eligible Expenditures for drugs in the Common Set (section 5.3.1);
vi. the total amount of funding from Canada spent on Eligible Expenditures for Screening and Diagnostics (section 5.4);
vii. the total amount of funding from Canada spent on Eligible Expenditures for New DRD not in the Common Set (section 5.5); and,
viii. the total amount of funding from Canada spent on Eligible Expenditures for existing DRD (section 5.5).

6.3. British Columbia also agrees to report, by October 1, 2025, October 1, 2026, and October 1, 2027, and in accordance with the performance measures set out in Annex C, on the outcomes and results achieved using funding received from Canada under this Agreement during the prior Fiscal Year. Which includes:

i. Indicators to measure the performance of the National Strategy
ii. Identification of Newborn Screening tests for rare diseases that are included on PT screening panels
iii. List of drugs in the Common Set with a corresponding evidence generation plan (if applicable) for which PTs have made public funding available for in the last Fiscal Year
iv. Consideration given to measures in support of Official languages, in particular the needs of Official Language Minority Communities under this Initiative
v. Consideration given to measures in support of the use of Sex and Gender-Based Analysis Plus to address the different needs of diverse populations based on identity factors such as sex, gender, age, disability, Indigeneity, sexual orientation, ethnicity, religion and more) under this Initiative

6.4. Audit

British Columbia will ensure that expenditure information presented in the annual financial statement is, in accordance with British Columbia standard accounting practices, complete and accurate.

6.5. Evaluation

Responsibility for evaluating the Initiative rests with British Columbia in accordance with its own evaluation policies and practices.

7. Communications

7.1. The Parties agree on the importance of communicating with the public about the objectives of this Agreement in an open, transparent, effective, and proactive manner through appropriate public information activities. The Parties agree to coordinate their efforts on public communications related to this Agreement to ensure consistency in messaging.

a. The Parties agree that the identity of a drug on the Common Set will not be disclosed prior to the conclusion of the Letter of Intent by the pan Canadian Pharmaceutical Alliance.

7.2. The Parties will each receive the appropriate credit and visibility when investments financed through this Agreement are announced to the public.

7.3. In the spirit of transparency and open government, British Columbia and Canada will make this Agreement, including any amendments, and subject to section 10.4, publicly available on a Government of Canada website, with the exception that Annex A is confidential and restricted from publication.

7.4. British Columbia agrees to make the results under this Agreement, subject to section 10.4, publicly available on its Government of British Columbia website.

7.5. Canada, with prior notice to British Columbia, may incorporate all or any part or parts of the data and information in section 6, or any parts of evaluation and audit reports made public by British Columbia into any report that Canada may prepare for its own purposes, including any reports to the Parliament of Canada or reports that may be made public.

7.6. The Parties reserve the right to conduct public communications, announcements, events, outreach and promotional activities about the National Strategy for DRD and this Agreement, and to give ten (10) business days advance notice and advance copies of public communications related to the National Strategy for DRD, this Agreement, and results of the investments of this Agreement.

8. Dispute resolution

8.1. The Parties are committed to working together and avoiding disputes through government-to-government information exchange, advance notice, early consultation, and discussion, clarification, and resolution of issues, as they arise.

8.2. If at any time either Party is of the opinion that the other Party has failed to comply with any of its obligations or undertakings under this Agreement or is in breach of any term or condition of this Agreement, that Party may notify the other party in writing of the failure or breach. Upon such notice, the Parties will endeavour to resolve the issue and dispute bilaterally through their designated officials, at the Assistant Deputy Minister level.

8.3. If a dispute cannot be resolved by the designated officials, at the Assistant Deputy Minister level, then the dispute will be referred to the Deputy Ministers of the Parties responsible for health, and if it cannot be resolved by them, then the respective Ministers of Canada and British Columbia, most responsible for health, shall endeavour to resolve the dispute.

9. Amendments to the agreement

9.1. This Agreement may be amended at any time by mutual consent of the Parties. To be valid, any amendments shall be in writing and signed, in the case of Canada, by Canada's Minister of Health, and in the case of British Columbia, by British Columbia 's Minister of Health.

10. General

10.1. Governing laws

This Agreement shall be governed by, interpreted and enforced in accordance with the laws in force in British Columbia and the laws of Canada applicable therein.

10.2. Entire agreement

This Agreement, including its preamble sets forth the entire Agreement between the Parties with respect to its subject matter and supersedes and cancels all prior agreements, understandings, negotiations and discussions, both oral and written, between the Parties with respect to the Initiative.

10.3. Members of Parliament

No member of the House of Commons or Senate or of the Legislature of British Columbia shall be admitted to any share or part of this Agreement

or to any benefit arising from it that is not otherwise available to the general public.

10.4. Personal and confidential information

The Parties shall comply with applicable laws, contractual obligations, and policies pertaining to privacy and confidentiality in dealing with information and records related to the Initiative.

10.5. Official languages

British Columbia agrees to consider how they can support Canada's official languages and the needs of official language minority communities in the implementation of this Agreement.

10.6. Diverse populations

British Columbia agrees to consider how they can support diverse populations based on identity factors such as sex, gender, age, disability, Indigeneity, sexual orientation, ethnicity, religion and more) in the implementation of this Agreement.

11. Terminating the agreement

11.1. Either Party may terminate this Agreement at any time if the terms of this Agreement are breached by the other Party by giving at least six (6) months written notice of its intention to terminate.

11.2. As of the effective date of termination of this Agreement, Canada shall have no obligation to make any further payments.

11.3. All obligations under this Agreement shall expressly, or by their nature, survive termination or expiration of this Agreement until, and unless, they are fulfilled, or by their nature expire.

12. Notice

Communications, including reporting and any notice, information, document, request or other communication, shall be in writing and sent to the coordinates below. Communications that are delivered in person shall be deemed to have been received upon delivery; communications transmitted by facsimile or by e-mail shall be deemed to have been received one (1) business day after having been sent; and communications that are sent by mail shall be deemed to have been received eight (8) business days after being mailed.

Any notice to Canada shall be addressed to:

Associate Assistant Deputy Minister, Health Policy Branch
Health Canada
70 Colombine Driveway, Tunney's Pasture
Building Brooke Claxton Building
Ottawa, Ontario
K1A 0K9
Email: michelle.boudreau@hc-sc.gc.ca

Any notice to the British Columbia shall be addressed to:

Assistant Deputy Minister, Pharmaceutical, Laboratory and Blood Services
Ministry of Health
PO Box 9637 Stn Prov Govt
Victoria BC
V8W 9P1
Email: mitch.moneo@gov.bc.ca

13. Signing in counterpart

This Agreement may be signed in counterparts and each counterpart shall constitute an original document; these counterparts taken together shall constitute one and the same Agreement.

In witness whereof, this Agreement is duly executed by the authorized representatives of the Parties.

Signed on behalf of Canada by the Minister of Health this 23rd day of July, 2024.

The Honourable Mark Holland, Minister of Health

Signed on behalf of British Columbia by the Minister of Health this 23rd day of July, 2024.

The Honourable Adrian Dix, Minister of Health

Annex A - Confidential

Annex B

Evidence collection

Purpose:
This document serves as an Annex to the *National Strategy for Drugs for Rare Diseases Initiative Canada-British Columbia Funding Agreement* (the "Agreement") as referenced in section 5.2(b) of that Agreement.

1. Definitions:

"Evidence" is Real World Evidence and Real World Data

"Evidence Collection" – means the processes by which Evidence is generated and collected for the further evaluation of drugs in the Common Set and may include, but is not exclusive to, work done by individual PTs, the PT DRD Working Group and other Strategy partners

"Evidence Collection Work Plan" – means the Evidence Collection Work Plan approved each quarter throughout the term of the agreement by the Pharmaceutical Executive Group

"Real World Data" are data relating to patient status and/or the delivery of health care routinely collected from a variety of sources

"Real World Evidence" is evidence on the use, safety, effectiveness, and cost of health technologies that is derived from real-world data.

2. All other terms are defined as outlined in the Agreement

3. British Columbia will support the inclusion of Evidence Collection as part of the pan-Canadian Pharmaceutical Alliance negotiation process, as and where appropriate.

4. British Columbia will participate in the development of the Evidence Collection Work Plan, including the development and identification of mechanisms/options to support Evidence Collection in respect of drugs in the Common Set.

5. British Columbia will fulfill the duties of a PT in regard to Evidence Collection, as outlined in the Evidence Collection Work Plan, including but not limited to:

a. British Columbia will support Evidence Collection Mechanisms in relation to drugs in the Common Set according to the Evidence Collection Work Plan;

b. British Columbia will support projects / pilots related to drugs on the Common Set;

c. British Columbia will participate in partnerships with identified organizations to support the collection/generation of evidence on Common Set drugs as and where appropriate / as determined by the Evidence Collection Work Plan;

d. British Columbia will support data collection and sharing, as outlined in the Evidence Collection Work Plan.

6. British Columbia may also participate, at its option, in Evidence Collection, as developed under the Evidence Collection Work Plan, for New DRD not in the Common Set and/or other existing DRD.

Annex C

Reporting requirements for Provinces and Territories
under the National Strategy for Drugs for Rare Diseases

To measure the performance of the provincial/territorial component of the National Strategy for Drugs for Rare Diseases (National Strategy) over time, Provinces and Territories (PTs) will be required to report specific data on a yearly basis covering the previous Fiscal Year. The data will be used to report internally on the progress of the Strategy and may be used to report publicly or communicate on National Strategy implementation pending notification to PTs (pursuant to sections 7.4 and 7.5 of the Agreement, which allow for this).

The data will cover New drugs for rare diseases (DRD) (including those in the Common Set), other drugs for rare diseases, and Screening and Diagnostics.

Reporting deadline: October 1 for the period covering April 1 to March 31 of the previous Fiscal Year

- First reports to be submitted by October 1, 2025 covering April 1, 2024 to March 31, 2025
- Count indicators measure a point in time – i.e., the current status as of March 31 (e.g., on March 31, how many drugs in the Common Set have there be an Election for by the PT PT)

Data required from PTs on a yearly basis:

Item	Outcome	Indicator
1	Improved collaboration across public drug plans related to DRD decision-making Canadians have improved access to DRDs	Number of drugs in the Common Set for which the PT has made an Election
2		Number of existing DRD not in the Common Set for which the PT makes public funding available Note: DRD Secretariat compiling a list of DRDs (New and existing) against which PTs would report.
3		Number of New DRD not in the Common Set for which the PT makes public funding available
4	Canadians with rare diseases have better access to Screening and Diagnostic services they need	List of newborn Screening tests for rare diseases that are included on PT Screening panels
5		Number of publicly-funded clinical settings routinely offering Genome-wide sequencing
6	Canadians have quicker access to DRDs	List of drugs in the Common Set for which the PT has made an Election with a corresponding evidence generation plan in the last Fiscal Year

7	Canadians have quicker access to DRDs	Date the PT makes public funding available in the last Fiscal Year for each drug for which the PT has made an Election in the Common Set
8		Date the PT makes public funding available in the last Fiscal Year for each New DRD not in the Common Set
9	Canadians have an understanding of the impact of the National Strategy	A description of improvements made to the coverage of drugs in the Common Set
10		A description of improvements made to the coverage of New DRD not in the Common Set.
11		A description of improvements made to the coverage of existing DRD.
12		A description of improvements in screening and diagnostics.
13		A description of how the different needs of diverse populations based on identity factors such as sex, gender, age, disability, Indigeneity, sexual orientation, ethnicity, religion, official language minority communities, and more were addressed.

Definitions:

DRD – a drug for a condition with an orphan designation by the European Medicines Agency or US Food and Drug Administration.

Existing DRD – means a DRD that obtained Health Canada approval prior to 2019.

Genome-wide sequencing (GWS) – refers to whole exome sequencing (WES) and whole genome sequencing (genome sequencing refers to sequencing the entire genetic code of a person and exome sequencing refers to sequencing only the parts of the genome that contain protein-coding genes). Genome-wide sequencing technologies: A primer for paediatricians - PMC (nih.gov)

Routinely offering – means offered regularly as a normal part of diagnostic processes (even if testing is completed outside of the PT).

Reporting template for Provinces and Territories
under the National Strategy for Drugs for Rare Diseases

Province or territory:

Reporting period: [April 1, YYYY to March 31, YYYY]

Table 1: PT input on indicators

Item	Indicator	PT input	Source of PT data (e.g., name of database(s))
1	Number of drugs in the Common Set for which the PT has made an Election		
2	Number of Existing DRD not in the Common Set for which the PT makes public funding available		
3	Number of New DRD not in the Common Set for which the PT makes public funding available		
4	List of newborn Screening tests for rare diseases that are included on PT Screening panels	Please complete Table 2 below.	
5	Number of publicly-funded clinical settings routinely offering Genome-wide sequencing		
6	List of drugs in the Common Set for which the PT has made an Election with a corresponding evidence generation plan in the last Fiscal Year	Please complete Table 3	
7	Date the PT makes public funding available in the last Fiscal Year for each drug for which the PT has made an Election in the Common Set	Please complete Table 4 below.	
8	Date the PT makes public funding available in the last Fiscal Year for each New DRD not in the Common Set	Please complete Table 5 below.	
9	A description of improvements made to the coverage of drugs in the Common Set	Please complete Table 6 below.	
10	A description of improvements made to the coverage of New DRD not in the Common Set.	Please complete Table 6 below.	
11	A description of improvements made to the coverage of existing DRD.	Please complete	

Item	Indicator	PT input	Source of PT data (e.g., name of database(s))
		Table 6 below.	
12	A description of improvements in screening and diagnostics.	Please complete Table 6 below.	
13	A description of how the different needs of diverse populations based on identity factors such as sex, gender, age, disability, Indigeneity, sexual orientation, ethnicity, religion, official language minority communities, and more were addressed.	Please complete Table 6 below.	

Table 2: Newborn Screening tests for rare diseases that are included on PT Screening panels

In order to simplify reporting we have provided a list of conditions that are included on newborn Screening panels in Canada. The Intergovernmental Newborn Screening Working Group Report Back to Ministers of Health – Provincial-Territorial Health Ministers' Meeting (January 2016) included a list of 22 diseases for the recommended national core panel. These diseases are highlighted in Table 2. All of these were also included in a recent document prepared by the Canadian Agency for Drugs and Technologies in Health (CADTH) for discussion purposes at its Newborn Screening Panel meetings, with the exception of Methylmalonic acidemia (Cbl A, B). The additional conditions were included in CADTH's document.

This list is not exhaustive. Please add rows as needed.

Item	Condition	Included on PT newborn Screening panels (YES or NO)
1	Argininosuccinic acidemia	
2	Biotinidase deficiency	
3	Carnitine palmitoyltransferase 1 deficiency	
4	Carnitine palmitoyltransferase 2 deficiency	
5	Carnitine-acylcarnitine translocase deficiency	
6	Carnitine uptake deficiency	
7	Citrullinemia	
8	Cobalamin A disease	
9	Cobalamin B disease	

Item	Condition	Included on PT newborn Screening panels (YES or NO)
10	Congenital adrenal hyperplasia	
11	Congenital hypothyroidism	
12	Cystic fibrosis	
13	Galactosemia	
14	Glutaric acidemia type 1	
15	Guanidinoacetate methyltransferase deficiency	
16	Homocystinuria	
17	Isovaleric acidemia	
18	Long chain L-3-OH acyl-CoA dehydrogenase deficiency	
19	Maple syrup urine disease	
20	Medium chain acyl-CoA dehydrogenase deficiency	
21	Methylmalonic acidemia: mutase deficiency	
22	Methylmalonic acidemia (Cbl A, B)	
23	Mupolysaccharidosis type 1	
24	Permanent hearing loss risk due to congenital cytomegalovirus infection	
25	Permanent hearing loss risk due to genetic factors	
26	Phenylketonuria	
27	Propionic acidemia	
28	Severe combined immunodeficiency	
29	Sickle cell disease	
30	Spinal muscular atrophy	
31	Trifunctional protein deficiency	
32	Tyrosinemia type 1	
33	Very long chain acyl-CoA dehydrogenase deficiency	
34	X-linked adrenoleukodystrophy	
35	3-hydroxy-3-methylglutaryl-CoA (HMG) CoA lyase deficiency	

Table 3: List of drugs in the Common Set for which the PT has made an Election with a corresponding evidence generation plan (if applicable) **from** [April 1, YYYY to March 31, YYYY]

Please add rows as needed

Item	Brand name of drug	Description (e.g., outcome-based agreement, registry, other)
1		
2		

Item	Brand name of drug	Description (e.g., outcome-based agreement, registry, other)
3		

Table 4: Date the PT makes public funding available from [April 1, YYYY to March 31, YYYY] for each drug for which the PT has made an Election in the Common Set

Please add rows as needed

Item	Brand name of drug	Date the PT makes public funding available
1		
2		
3		

Table 5: Date the PT makes public funding available from [April 1, YYYY to March 31, YYYY] for each New DRD not in the Common Set

Please add rows as needed

Item	Brand name of drug	Date the PT makes public funding available
1		
2		
3		

Table 6: Descriptions of impact of funding under the National Strategy

Item	Component	Description Please provide qualitative details describing the impact funding has had on each identified component. For example, if funding was used to modernize an existing drug plan, please describe the advancements made. Additionally, if funding was used to hire a consultant or additional staff, describe the improvements these staff were able to make to the identified components.

		(suggested word count per item is less than 300 words)
1	A description of improvements made to the coverage of drugs in the Common Set	
2	A description of improvements made to the coverage of New DRD not in the Common Set	
3	A description of improvements made to the coverage of existing DRD	
4	A description of improvements in screening and diagnostics	
5	A description of how the different needs of diverse populations based on identity factors such as sex, gender, age, disability, Indigeneity, sexual orientation, ethnicity, religion, official language minority communities, and more were addressed	