

SECTION 4

Rare Diseases Drug Coverage Program

RARE DISEASES DRUG COVERAGE PROGRAM

Selected drug products used in the treatment of rare diseases may be considered for coverage for individuals covered under Alberta government-sponsored drug programs. The Minister of Health makes the final decisions regarding coverage under this Program, and may list a drug product under this section when the Minister considers it in the public interest to do so¹.

RARE DISEASES DRUG COVERAGE

In order to be eligible for the Rare Diseases Drug Coverage Program, an individual must:

- have Alberta government-sponsored drug coverage;
- be continuously registered in the Alberta Health Care Insurance Plan for a minimum of five years unless:
 - the individual is less than five years of age at the date of the application, then the individual's parent/guardian/legal representative must be registered continuously in the Alberta Health Care Insurance Plan for a minimum of five years;
- OR
- the individual has moved to Alberta from another province or territory in Canada (the "province of origin"), and immediately prior to moving to Alberta, was covered for a drug product listed in this section in the province of origin by a provincial or territorial government sponsored drug plan, and the individual has been registered in the Alberta Health Care Insurance Plan (the individual must provide supporting documentation from the province of origin to prove prior coverage).
- meet the clinical criteria for a rare disease drug product published on the *List*;
- have a *Rare Diseases Drug Coverage* Application form ("Application") submitted on their behalf to Alberta Blue Cross by the individual's "Rare Disease Specialist";
- have the Application reviewed and approved for coverage by the Alberta Rare Diseases Clinical Review Panel ("Review Panel")
- complete the required forms, and consent to and acknowledge that
 - approval for initial and continued coverage is conditional upon clinical outcomes;
 - regular monitoring of the individual's clinical outcomes will be required, and
 - that coverage will be discontinued if there is inadequate response or the individual's condition deteriorates as outlined in the withdrawal criteria established in relation to a specific rare diseases drug product and/or as assessed by the Review Panel.

Contraindications

In addition to meeting the above criteria, the individual must not have the following contraindications:

- Significant illness, not including one of the rare diseases, likely to substantially alter or reduce life expectancy.

¹ Section 1 of the ADBL does not apply to the Rare Diseases Drug Coverage Program

Rare Diseases Drugs Eligible for Coverage

Drug products approved by Health Canada for the treatment of Rare Diseases may be considered for coverage in accordance with this section.

Rare Diseases are genetic, lysosomal storage disorders occurring at a rate of less than one per 50,000 for the Canadian population for a specific disease (as determined by Alberta Health).

As of April 1, 2009, drug products for the treatment of the following rare diseases are currently under consideration for coverage:

- Gaucher's disease
- Fabry disease
- MPS-I (Hurler/Hurler Scheie)
- Hunter disease
- Pompe disease

Alberta Rare Diseases Clinical Review Panel

The Alberta Rare Diseases Clinical Review Panel ("Review Panel") is a review panel composed of specialists treating rare diseases and other health professionals with clinical expertise, appointed by the Minister of Health.

The Review Panel's functions include:

- Providing advice to Alberta Health regarding the Rare Diseases Drug Coverage Program;
- Reviewing and applying clinical knowledge and skills to individual applications for Rare Diseases Drug Coverage; and
- Providing advice to the Expert Committee on Drug Evaluation and Therapeutics regarding drug products under consideration for coverage under this section, clinical criteria for rare diseases drug products and identifying appropriate "Rare Disease Specialists".

Process for Rare Diseases Drug Coverage

Participating "Rare Disease Specialists" must complete a Rare Diseases Drug Coverage Application form for each individual. The form must be the one specific to the rare diseases drug product being requested. The completed application may be forwarded to Alberta Blue Cross by mail or by facsimile.

To be considered for Rare Diseases Drug Coverage, the "Rare Disease Specialist" must confirm the individual (or individual's parent/guardian/legal representative) has been provided with information regarding the Rare Diseases Drug Coverage Program and have completed the required forms.

**ALBERTA DRUG BENEFIT LIST
RARE DISEASES DRUG COVERAGE PROGRAM**

Alberta Blue Cross, in providing administrative support to the Review Panel, receives and screens each application for completeness, then forwards to Alberta Health to confirm that the individual has met the Alberta Health Care Insurance Plan registration requirement (please see above). Once it has been confirmed that the individual meets the Alberta Health Care Insurance Plan registration requirement, Alberta Blue Cross forwards the application to the Review Panel for assessment. Alberta Blue Cross responds to applicants on the Review Panel's behalf. After an application has been assessed by the Review Panel, Alberta Blue Cross notifies the individual's "Rare Disease Specialist" and the individual or individual's parent/guardian/legal representative by letter of the Review Panel's decision. Eligibility will be effective the date coverage is approved by the Review Panel.

Renewals require a new drug product specific Rare Diseases Drug Coverage Application form that is completed by a "Rare Disease Specialist".

To be eligible for Rare Diseases Drug Coverage, prescriptions must be written by a "Rare Disease Specialist" as identified by the eligibility criteria for the drug product. To avoid wastage, prescription quantities are limited to a one-month supply. Extended quantity and vacation supplies are not permitted. Out-of-country claims will only be reimbursed in accordance with standard rules and regulations; individuals should verify with Alberta Blue Cross these rules and regulations prior to obtaining drug products out of the country.

Government will not be responsible for reimbursement of costs associated with wastage or improper storage of rare diseases drug products.

Prior approval must be granted to ensure coverage. Approval is granted for a specific period, to a maximum of 12 months. If continued treatment is necessary, it is the responsibility of the individual or individual's parent/guardian/legal representative and the "Rare Disease Specialist" to re-apply for drug product coverage prior to the expiry date of the authorization period.