Record of Recommendation

Re: Funding lenalidomide (Revlimid®) in the treatment of Myelodysplastic Syndrome

September 9, 2009

Discussion facilitated using the Decision Making Framework.

- All present will vote electronically when the vote is called by the chair. The voting process will be completed by 5 pm on September 21, 2009. The decision will be made by a majority with dissenting voters given the opportunity to record their opinion. Dissenting opinions must be recorded within seven days of the result of the vote being announced.

- Core values and principles were reviewed and discussed along with competing obligations, constraints and relevant information.

- Options for a recommendation to the Deputy Minister were reviewed and each option was discussed. Two options were identified at this time:
  1) Approval of funding with restrictions
  2) Denial of funding

- An analysis of the projected benefits and burdens of each option was discussed.

Background:

- ✓ Patients with myelodysplastic syndrome (MDS) have an abnormal development of blood cells.
- ✓ Median age of diagnosis of MDS is 65 to 70 years. Most patients present with anemias at diagnosis with the majority eventually requiring blood transfusions.
- ✓ Current treatments for MDS include supportive care (blood transfusions, antibiotics, and growth factors), immunosuppressive therapy, stem cell transplantation, chemotherapy and other novel agents.
- ✓ For older patients, therapy is usually practically limited to supportive care (including blood transfusions) with or without erythropoietin stimulating agents (ESAs).
- ✓ Disadvantages of chronic transfusions include inconvenience and impact on

quality of life, cost, iron overload, allergic reactions, alloimmunization, and infection.

- ✓ Lenalidomide (Revlimid®) has been studied as a novel treatment for MDS to reduce the need for blood transfusions.
- ✓ A phase II study evaluated lenalidomide in the treatment of transfusion-dependent low or int-1 risk MDS with deletion 5q abnormality with or without additional cytogenetic abnormalities. The primary endpoint was transfusion independence and secondary endpoints were cytogenetic response and safety. This was a one-armed study with no control group.

Projected Benefits:

- ✓ Sixty-seven percent (67%) of patients who were treated with lenalidomide achieved transfusion independence.
- ✓ There is no data to support any effect on survival.

Projected Burdens:

- ✓ Lenalidomide is associated with serious treatment-related side effects such as neutropenia, thrombocytopenia and deep vein thrombosis. Three deaths in the study were possibly treatment-related (neutropenic infections)
- ✓ The cost of lenalidomide is $10,830 per month per patient. Treatment of two patients would cost approximately $259,920 per year.
- ✓ The economic analysis provided by the manufacturer suggests a cost per QALY of $61,774; however an independent re-analysis suggests a cost per QALY of over $200,000.

Result of Vote:

The vote was conducted electronically. The question the Committee was asked to vote on is:

"Should the Committee support a recommendation to the Deputy Minister of Health to fund lenalidomide (Revlimid®) as a single agent in adult patients with transfusion dependent anemia due to low or intermediate-1 risk MDS associated with a deletion of 5q cytogenetic abnormality with or without additional cytogenetic abnormalities"

- o **The result of the vote was a majority in favor of <u>not</u> recommending funding.**

This recommendation has been accepted by the Deputy Minister of Health.