



## Health Newsflash

### Fabry Disease



*In the last few months, expensive new drug therapies have been approved by Health Canada's Therapeutic Products Directorate for rare, yet debilitating diseases. Although their prevalence is low in Canada, drugs used to treat these diseases may have a significant impact on private drug plans that include these types of patients. We would like to introduce one of these diseases, Fabry disease, in this issue.*

#### **Fabry Disease**

Fabry disease is a rare genetic disorder caused by a faulty gene in the body, leading to a deficiency of an enzyme (alpha-galactosidase A). This enzyme is involved in the breakdown of lipids (fats) in the body, which accumulates in the veins over time causing damage to the kidneys and heart. This disorder affects more males than females, with an estimated prevalence in Canada of 1 in 250,000. There are currently about 260 patients with Fabry disease in Canada. People with this disease usually survive into adulthood, but are at an increased risk for strokes, heart attacks, and kidney damage.

#### **Current treatments available for Fabry disease**

In the past, treatment has been limited to symptomatic relief. However, the recent availability of enzyme replacement therapy (ERT) has allowed for control, and even reversal, of many complications of the disease. ERT involves replenishing the deficient enzyme by an infusion into the vein. Fabrazyme and Replagal are two ERT drugs recently approved by Health Canada for treating Fabry disease.

#### **Enzyme Replacement Therapy**

Fabrazyme and Replagal are given by infusion into a vein every two weeks. Fabrazyme has a higher incidence of treatment-related reactions compared to Replagal (e.g., fever, shivers or trembling, high blood pressure). Due to these reactions and required monitoring of the patient, Fabrazyme must be given exclusively in hospital while Replagal can be given in or out of hospital. Based on a 70 kg individual, the total annual cost for these drugs is approximately \$263,000 and \$290,000 for Replagal and Fabrazyme, respectively.

#### **Recent developments**

##### **Fabrazyme**

Fabrazyme, manufactured by Genzyme Canada Inc., received its Notice of Compliance (NOC) from Health Canada on January 23, 2004. Prior to this date, Fabrazyme was available to patients through Health Canada's Special Access Programme (SAP). The SAP provides access of non-marketed drugs to physicians who treat patients with serious or life-threatening conditions. Drug manufacturers often supply these drugs free-of-charge. Usually, access of a drug through the SAP terminates once it is marketed in Canada. In the interim, as provincial governments decide on coverage for Fabrazyme, the manufacturer is supplying Fabrazyme at no cost to hospitals.

The provincial governments are awaiting recommendations from the Common Drug Review (CDR), which is expected in July 2004. According to Genzyme Canada, four of five provinces with Fabry disease patients have agreed to cover Fabrazyme if a positive funding recommendation is made; these include Alberta, Nova Scotia, Ontario, and Quebec. British Columbia has not made a decision as of this issue's publication date.

As Fabrazyme is administered in hospital, plan sponsors using the ESI Canada Hospital Drug Program (H) will not experience an impact, as the drug will not be covered on their drug plans. Plan sponsors not using the H program may experience claims for Fabrazyme.

*Optimizing the Value of Drug and Dental Benefits*

Volume 6  
Issue 10

July 21,  
2004



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#### *Optimizing the Value of Drug and Dental Benefits*

#### **Replagal**

Replagal, manufactured by Transkaryotic Therapies Inc., received its Notice of Compliance with conditions (NOC/c) on February 6, 2004. A NOC/c is an approval granted to a product on the basis of promising evidence of clinical effectiveness in a serious, life-threatening, severely debilitating illness. Health Canada granted the NOC/c to Replagal on the condition that the manufacturer continues clinical investigations to find the best dosage. Prior to receiving its NOC/c, Replagal was also available through Health Canada's SAP. Based on the manufacturer's information, patients enrolled in the SAP prior to NOC approval will continue to receive Replagal at no cost. As with Fabrazyme, the provincial governments are awaiting funding recommendations from the CDR, also expected in July 2004.

Replagal has less infusion reactions than Fabrazyme and, therefore, can be given outside a hospital. As such, the manufacturer has made Replagal available to retail pharmacies. According to the manufacturer, about half of the patients in Canada with Fabry disease could be treated with Replagal. Therefore, when the SAP terminates, the private sector will begin to experience claims for Replagal. It may be prudent to place this drug on a prior authorization program as some of these patients will be treated in hospital and therefore, funded through hospital budgets.

#### **Conclusion**

The recent availability of ERT to patients with Fabry disease has given them new hope of substantially improving their quality of life. Although these are life-saving therapies, their prohibitive cost will undoubtedly have a significant impact on provincial and private drug plans.

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Volume 6  
Issue 10

July 21,  
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