

TREATMENT OF RARE DISORDERS: U.S. AND EUROPE ENACT LEGISLATION TO ENCOURAGE INNOVATION AND ACCESS WHILE CANADA LAGS BEHIND.

The European Commission, European Medicines Agency and the U.S. Food and Drug Administration recently adopted a common application form for drug makers seeking orphan designation for their medicines. This will simplify the drug approval process and spur innovation by allowing companies to apply for approval of their new product in both the US and across Europe at the same time. In addition, this new process will help the regulatory agencies to better understand each other's systems.¹

In contrast, patients and advocates in Canada have expressed concern that the Common Drug Review (CDR) – *a government appointed agency that makes recommendations to provinces (with the exception of Quebec) regarding listing decisions for new drugs* – is failing to provide patients with timely access to new medicines and failing to operate in an accountable fashion. The CDR process has recommended against reimbursement for every treatment evaluated for unmet needs.

International Comparison of Public Reimbursement of Products for Unmet Needs²

BRAND ^a	INDICATION	Canada (CDR)	UK	France	Sweden	USA (Medicaid)
Somavert	Acromegaly	No	Yes	Yes	Yes	Yes
Xolair	Severe Persistent Asthma	No	Yes	Yes	Yes	Yes
Aldurazyme	Enzyme Replacement Therapy	No	Yes	Yes	Yes	Yes
Fabrazyme	Fabry Disease	No	Yes	Yes	Yes	Yes
Sensipar	Chronic Kidney Disease	No	Yes	Yes	Yes	Yes
Forteo	Osteoporosis	No	Yes	Yes	Limited	Yes
Replagal	Fabry Disease	No	Yes	Yes	Yes	Yes

^a Products Licensed in Canada, US, and EU

In response to these concerns, the federal House Standing Committee on Health recently conducted a review of the CDR. The government recommendations include³:

- Establishing a specifically designed approach for the review of drugs for rare disorders and for first-in-class drugs;
- Creating a distinct appeal process with a separate group of experts; and
- Increasing the current level of public involvement in the CDR through public attendance at open CEDAC meetings and the creation of a public advisory body.

¹ http://today.reuters.com/news/articlenews.aspx?type=scienceNews&storyID=2007-11-26T183432Z_01_L2677301_RTRUKOC_0_US-DRUGS-REGULATORS.xml

² Biotech Canada – Presentation to the House Standing Committee on Health (April 16, 2007)

³ House Standing Committee on Health Report on the Common Drug Review (Dec. 2007)
<http://cmte.parl.gc.ca/cmte/CommitteePublication.aspx?COM=13189&Lang=1&SourceId=220278>

While these recommendations are encouraging, this review is non-binding and merely suggests that the CDR consider these suggestions. The extent to which CDR implements these, and other Committee recommendations, remains to be seen.

Rare Disorders

Despite the small numbers affected by each disease, there are up to 6,000 rare disorders which affect nearly 10% of the Canadian population, 30 million people in Europe and about 25 million Americans.