BETTER ACCESS TO TREATMENT FOR PEOPLE WITH RARE DISEASES: A QUESTION OF EQUITY

There is an inequity within our health system between those who have common vs. rare diseases

Quebecers who suffer from a rare disease are also burdened with a number of additional disadvantages:

- There are few medications or specific treatments;
- When acceptable treatments exist, many are not available in Canada;
- While some treatments are approved by Health Canada, a large percentage are not reimbursed by our "Régime général d'assurance médicament (RGAM)" in Quebec or are not available through our hospitals due to the inadequate way in which they are evaluated or because of the cost.

Inequity also exists between different rare diseases but also within the same disease.

Some Quebecers who have rare disease can nevertheless succeed in getting access to treatment:

- In Quebec, there is a "patients d'exception" program (Special Access Program) where the attending physician can ask the "Régie de l'assurance-maladie (RAMQ)" for authorization to treat their patient with a medication that is not currently listed on the hospital drug list. However, this is only done on a case-by-case basis. What happens to patients whose physicians do not make the request or when the request is refused?
- Patients in certain hospitals or who live in certain geographical areas, will get their treatment whereas others will not, due to the randomness of the decision makers process (whether the payer is the hospital or the Social Services Agencies ("Agence de santé et de services sociaux")). So access to treatment may depend on your postal code.
- Some rare disease patient support groups get access to medication through different channels even when they have not been evaluated by the "Conseil du médicament/INESSS (Institut national d'excellence en santé et en services sociaux)" or if the medication was rejected for funding: for example, The National Gaucher Foundation of Canada, The Canadian Fabry Disease Initiative (which has a treatment centre at Sacré-Coeur Hospital), the tyrosinemia treatment centre, etc.
- Through private insurance, some patients have access to their medications even if these drugs are not reimbursed by the pubic health system.
- In desperate circumstances, some parents of children with rare diseases go to the media and have succeeded in getting their children treatment.

- Finally as a last resort, those that can afford to do so, travel to other countries to get treatment. In many cases these treatments have been available in these other countries for years if not decades.

We have to find solutions so that our health care system treats Quebecers with rare diseases equitably, with the same standards of care as everyone else, especially when it come to getting access to treatments.

The portion of the Quebec population that is suffering from a rare disease, should not be denied or be restricted access to available treatments. We have to find new approaches in evaluating new medications or other kinds of therapies and to find better ways of distributing the cost of reimbursements, so that equal and appropriate access for people with rare diseases can be realized. The government supports and extols the initiatives that lead to innovative therapeutic approaches, especially for personalized care. Generally, people with rare diseases are rarely given consideration nor do they gain tangible benefits from these efforts.

The Quebec government must adopt an all encompassing and innovative purchasing policy for rare disease medications. Most other developed nations, who are investigating the funding of rare disease medications take a global approach and avoid examining these treatments on a case-by-case basis. Quebec needs to do the same. There are ethical and societal implications with respect to people with rare diseases, who should be able to benefit from equitable treatment[1].

Statistics and facts:
In Quebec, in order to get access to orphan drugs, people with rare diseases face an overwhelming number of obstacles at many levels:

1. Lack of availability : (see also diagram in appendix)
   - There are only about 250 orphan drugs for 400 of the 7000 rare diseases:
   - Only half of the 250 orphan drugs have been approved by Health Canada (~126), even though they are authorized for use in other markets worldwide (these are mostly unavailable because the pharmaceutical company has not submitted the drug for review in Canada. Some of the reasons put forth include: that the market is too small to make a profit, the administrative efforts of dealing with our bureaucracy is too high, etc.;
   - In Quebec, of the 126 orphan drugs that were approved by Health Canada, only slightly more than half (~75) were evaluated for reimbursement by the "régime d'assurance médicament public";
   - Of the 75 drugs, 54 were approved for reimbursement in Quebec, but two thirds of them have restrictive criteria (for example, a drug can only be used for a specific disease or for a sub-portion of the rare disease patient population - (analysis conducted November 2013; see diagram in the appendix)[2]

2. Ineffective evaluation of the therapeutic value of the drug by INESSS* in order to add it to the list of reimbursable medications.
   The current methodology does not address specific rare disease issues nor does it take into account the problems of conducting clinical trials. [3]
   - Two thirds of the drugs evaluated by INESSS were refused because they were considered of limited therapeutic value;

* *INESSS : Institut national d'excellence en santé et en services sociaux : organization that evaluates drugs for reimbursement in Quebec (in the past, it was called the « Conseil du médicament »)

- In 2007, in the "Politique du médicament du Québec", a recommendation was put forth that an evaluative process be adopted that would "take into account the peculiarities of these diseases and the special needs of these patients with respect to the financing by representatives of the RGAM or by the health and social services institutions."[4]
- Quebecers with rare diseases are still waiting for this evaluation process, and we lag behind other provinces (Ontario, Alberta, British Columbia and New Brunswick).
- Proof of the ineffectiveness of current evaluative process was demonstrated last year, when the scientific committee from INESSS confirmed in their report on a specific rare drug that the disease's inherent characteristics created an obstacle in being able to assess its therapeutic value. Despite this shortcoming in the existing process, they still made a recommendation to the health ministry to reject the medication for reimbursement.[5]

3. High costs:

Despite the financial incentives offered by the government to pharmaceutical companies that develop orphan drugs, the cost of these drugs remains high:

- With the low number of people with rare diseases, we estimate that the cost of the orphan drugs currently available in Canada represents less than 1% of the budget of all reimbursed medications[6]
- Unlike in other provinces, Quebec does not participate in the negotiation with pharmaceutical companies for the cost of medicines, and only Quebec mandates the publication of the negotiated prices. However, Quebec pays more than other provinces for the same medications.

**The RQMO/QCOD works with different organizations to improve access to orphan drugs for Quebecers with rare diseases (patients, patient support groups, physicians and other health care professionals, pharmaceutical companies, etc.)*

***The RQMO/QCOD relies on the support of private, public and corporate donors, including non-restrictive educational subsidies provided by pharmaceutical companies; this allowed for the development of this website.*

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[1] Regroupement québécois des maladies orphelines. Les maladies rares et orphelines, oubliées par le gouvernement - Le RQMO demande au ministre Réjean Hébert la mise en place d'une politique sur les médicaments orphelins. Communiqué de presse, juin 2013. http://www.newswire.ca/en/story/1180901/les-maladies-rares-et-orphelines-oubliees-par-le-gouvernement-le-rqmo-demande-au-ministre-rejean-hebert-la-mise-en-place-d-une-politique-sur-les-medic

[2] Regroupement québécois des maladies orphelines. Pour un meilleur accès aux traitements pour les maladies rares. Une question d'équité. Fiche technique en préparation du dîner-causerie sur l'accès aux traitements pour les maladies rares, 21 février 2014. http://www.rqmo.org/PDF/Document_preparatoire_diner-conference_21_fevrier.pdf

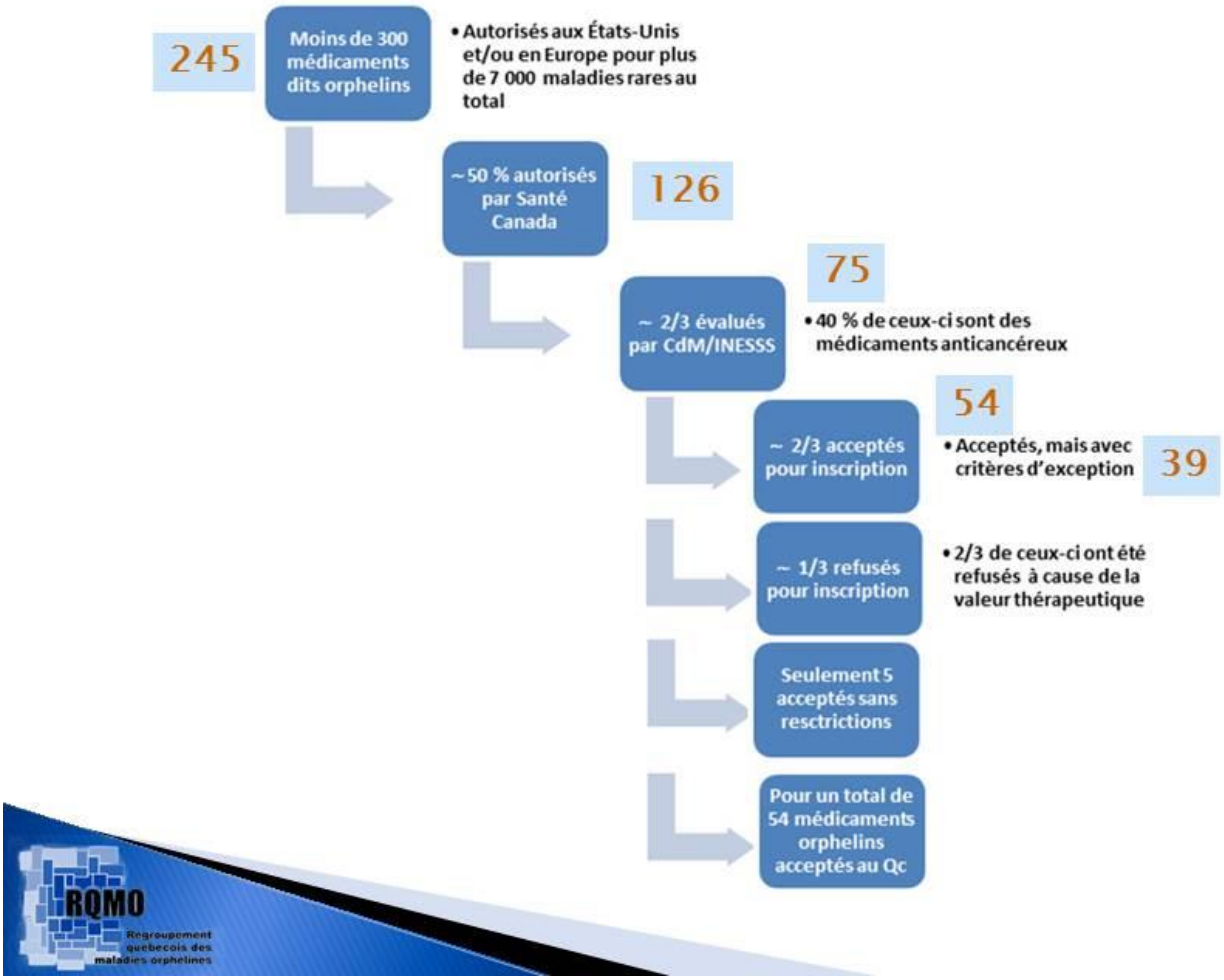
[3] INESSS. Evaluation Process and Criteria. http://www.inesss.qc.ca/en/activites/drug-products/evaluation-process-and-criteria.html

[4] Gouvernement du Québec. La Politique du médicament. 2007. https://www.inesss.qc.ca/fileadmin/doc/INESSS/DocuAdmin/Lois_Politiques/Politique_medicament.pdf

[5] Institut national d'excellence en santé et en services sociaux. Extrait de l'avis au ministre sur VPRIV- La maladie de Gaucher type I, juin 2013, file:///C:/Users/Gail.MININT 624C2FQ/Downloads/vpriv_2013_06_CAV%20(1).pdf

[6] Priest, Lisa. "National drug policy for rare diseases has fallen between the cracks." The Globe and Mail. April 4, 2011. www.theglobeandmail.com/news/national/time-to-lead/national-drug-policy-for-rare-diseases-has-fallen-between-the-cracks/article575248/

Disponibilité des médicaments orphelins



(November 2013)