Addressing the authorization of orphan drugs will not fix reimbursement problems: The elephant is still in the room

THE OCTOBER 3, 2012, ORPHAN DRUG INITIATIVE ANNOUNCED by the Federal Ministry of Health aimed at improving availability of information about, and authorization of, orphan drugs may have the potential for accelerating Health Canada's approval of treatments for many rare diseases. However, details about policy formulation or implementation are vague at best, and it is doubtful that it will address the more serious issue of patient affordability. Which begs the question, what is the ultimate result of accelerating authorization if the significant barrier of affordability still exists? Unfortunately, without provincial collaboration on approving reimbursement for orphan drugs, patients will still be left without long-term access.

Given the ambiguity of the federal message, there may be an uncertainty around how to formulate and implement the initiative. Therefore, it is an opportune time for the federal government to reconsider the policy recommendations provided by previous federal task forces, national initiatives and independent scholarly recommendations. These reports provide options for a long-term solution to the current orphan drug reimbursement problem in Canada. Many intelligently include recommendations for a collaborative effort between the federal and provincial governments, a route the current federal government seems reluctant to travel.

Previously, the Romanow report¹ of 2002 suggested that all levels of government contribute to payment that reimburses 50% of costs to the province for drugs costing more than \$1500 per year. As a long-term solution to reduce drug prices, the report included the creation of a National Drug Agency designed to negotiate and monitor drug prices, set up an early warning system to deal with developing expensive therapies and establish a national drug formulary. The report by Kirby and LeBreton² proposed a similar alternative program that had the federal government cover 90% of drug expenses that exceeded \$5000 per year; the provincial governments would cover the remaining 10%.

At the 2004 First Minister's Meeting, goals were set to develop a National Pharmaceutical Strategy (NPS) that entailed catastrophic pharmaceutical coverage, methods for assessing effectiveness and cost options, accelerating access to breakthrough drugs (including orphan drugs) and establishing a national formulary to ensure equal access across the country. In 2006, a ministerial task force was appointed to investigate the best approach to health care renewal, including the development of catastrophic drug coverage, the establishment of a national drug formulary and the facilitation of access to breakthrough drugs.

More recently, in 2008, the task force created decision points that would be a focus for progress, which included a Canadian Access Program for orphan drugs. This would include a transparent decision-making model with public input, similar to the European Union's citizens council. Furthermore, there would be a 50/50 cost-sharing split for drug costs and a national registry for researching and monitoring drug effectiveness. There has been no significant progress with the federal government or industry since the report in 2006.³

Early in 2006, the Canadian Fabry Disease Initiative (CFDI) was formed in response to an increasing demand from physicians and their patients with Fabry disease for access to an orphan drug that was available in almost every other high-income country. The CFDI was a one-of-a-kind nationwide trial that was cofunded by industry, the provinces and the federal government. There were high hopes for the CFDI as a prototype for national access to orphan drugs. However, it was essentially a research study designed to solve a longstanding reimbursement problem; therefore, it is not desirable as an orphan drug strategy.⁴

The purpose of providing this timeline is to demonstrate that significant resources have gone into considering the best options for improving the true problem of orphan drugs:

affordability. It seems reasonable to consider these efforts when formulating a solution. The dominant theme throughout the recommendations in these various reports is that to solve the reimbursement problem with orphan drugs, a federal-provincial (and industry?) collaboration on cost sharing is

needed. As is, the federal statement on improving authorization does not consider the significant barriers to patient access due to exorbitant costs. Without addressing this gigantic issue, the federal government will continue to ignore the elephant in the room. ■

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