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## Canada needs orphan drug policy

*Medicines for rare diseases are not always available, are expensive, and may not be covered by health insurance*

By: David Gratzer – January 19<sup>th</sup>, 2013

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Over the holidays, an actor who played one of TV's most famous doctors died. For seven seasons, starting in the late 1970s, Jack Klugman played Dr. Quincy, a medical examiner. Klugman was an American, and played an American doctor on TV, but his legacy tells us something important about Canadian health care. Is anyone listening?

Many Americans remember Klugman as more than just a fictional doctor - the New York Times obituary, for instance, describes how he used his fame to promote better health care in the real world. Klugman lobbied for storylines on his show - and lobbied Congress in person - to promote an orphan drug policy in the United States.

The model for drug development is simple: Private firms invest hundreds of millions of dollars in research and drug trials. In exchange, they expect that patents and big consumer markets will deliver a return on their investment. The problem: People with rare diseases lose out. Since a "rare disease" is defined by the U.S. Food and Drug Administration as a disorder that affects fewer than 200,000 Americans, and since Europe defines "rare" to mean any disease that affects fewer than five in 10,000 people, there aren't enough of these patients for the cost-recovery model to work. So most modern countries have policies to promote research, approval, and access to so-called orphan drugs, ensuring that these patients aren't forgotten.

Klugman's on-and off-screen campaign made these policies possible. America's 1983 Orphan Drug Act removed many barriers to orphan drug research, and offered incentives to balance the fact that such pharmaceuticals would only be needed for a small pool of patients. Groups that advocate for patients with rare diseases openly credit Klugman for that outcome, and one tribute described him as "one of the best friends the rare disease community has ever had." Yes, it's true that the U.S. does not provide universal health insurance. But for the vast majority of Americans who do have health insurance, drugs for rare diseases are commonly covered; for the uninsured, at least the meds are approved and available within the U.S. market.

Not so in Canada. Orphan drugs have been an orphaned political issue. Canadian health care may be universal but it is heavily tilted toward a 1950s, hospital-first approach to care. Emergency care can be very good, but primary care is harder to find. Life-saving operations are easy to access, but waiting lists are longer for elective or preventive surgeries. And so it is with orphan drugs. While common medications are cheap and easy

to access, drugs for rare diseases aren't as likely to be available in Canada, let alone covered by public health insurance.

In 2007, Winnipeg North MP Joy Smith tabled a report of the House of Commons Standing Committee on Health that reviewed this issue. In a widely-praised report, the committee called for a Canadian orphan drug policy.

More than five years later, patients are still waiting. In October, Federal Health Minister Leona Aglukkaq announced that the government was "taking action." Months have now passed.

At home and abroad, references to Canadian health care in popular culture are almost always about how great our system is. Few actors or activists are willing to speak out to help to fix its flaws, and their relentless praise makes it easier for politicians to paper over the weaknesses. In Canada, we could use a Jack Klugman to help people see how our system sometimes fails patients. So far, we're getting something quite different.

Take Michael Moore. In late December, the American filmmaker and activist went on a tear on Twitter, arguing that the United States should be more like Canada. One of his arguments: he asked rhetorically "how come not a single person in Canada last (year) went bankrupt over medical bills?"

Moore's premise simply isn't true. Far fewer Canadians face medical bill challenges than Americans, but many people north of the 49th parallel do go bankrupt each year because of treatment costs. One of the most common causes is lack of public insurance coverage for drugs, especially the lack of access to and coverage for orphan drugs.

As the Cato Institute noted in November, since 2005, "of the 63 orphan drugs approved in the United States of America, only 42 were approved in Canada. Of the 49 orphan drugs approved in the European Union, only 29 have been approved in Canada."

Desperate families are forced to import treatments from abroad, pushing them to the financial brink or beyond.

Whatever your preferred solution to the orphan drug problem, something should have been done years ago; Canada is one of the last countries in the industrial world without a policy. Maybe Canada needs a Klugman to call our own - a celebrity voice with enough courage to speak out.

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