Rationale for Modernization

The Canadian Organization for Rare Disorders (CORD) agrees with many aspects of the rationale put forth by the PMPRB as rationale for guidelines modernization. The PMPRB suggests that in today's regulatory environment, the thirty-year old policy of exchanging IP protection for R&D investment and protecting against excessive prices by benchmarking against international references is no longer effective. R&D investment is low compared to certain (European) countries and list prices appear to be higher than in most other OECD countries. With the emergence of effective new pan-Canadian approaches to assessing and negotiating drug prices in Canada, the PMPRB has questioned the continuing relevance of its program.

According to the PMRPB Guidelines Modernization Discussion Paper (June 2016), new guidelines may be necessary to address the shift in drug development from "blockbuster" population-based drugs to "niched" therapies for targeted patients and rare diseases, many of which are marketed at very high prices. From our perspective, these new developments offer life-altering and life-saving solutions to a community where only 5% of patients suffering from nearly 7,000 rare conditions have any effective treatment. In Canada, there are 2.8 million Canadians with rare diseases.

Focus of CORD Response

While CORD is prepared to provide commentary on proposed Guidelines Modernization, we cannot respond to the questions posed. Moreover, we object strenuously to the PMPRB's framing of the current "regulatory environment" for new drugs. We note, in particular, the following concerns:

- Lack of appreciation for the tremendous advancements in health outcomes represented by the so-called specialty therapies, including targeted therapies for "niche" populations and rare diseases as well as curative therapies for Hepatitis C and gene therapies,
- Sweeping generalization and biased characterization of innovative drug developers as "price gougers,"
- Narrow focus on managing the price of individual therapies, both upon entry and continued use without consideration of any other strategies to determine pricing based on value to the patient, the healthcare system, and society as a whole.

The PMPRB suggests that the singular focus on a drug list price has not worked in the past and will definitely not work in the future. Rather than focus on the simplistic task of deciding the right "criteria" for pricing, we would like to challenge the PMPRB to help assure pricing that is reflective of value within a much more complex environment.

Moreover, we feel that Canada cannot and should not shirk its responsibility to promote research and development into new therapies, given both the tremendous need and our research capacities. Again, it is not as simple as providing IP protection or even guaranteeing an entry price for new drugs; Canada needs to foster and incentivize a creative and productive R&D environment. That is the premise underlying Canada's Orphan Drug Regulatory Framework, which would establish Canada as an attractive place to invest in rare disease research, on par with Europe, the USA, and Japan. It would provide incentives for companies to conduct clinical trials and give clinicians an opportunity to develop expertise and patients a chance to access life-saving therapies.

The PMPRB is quite right in saying that benchmarking to reference prices does not guarantee fair or appropriate pricing. Despite all the rhetoric about "value-based" pricing (especially in European countries), one can argue that no country knows how to determine the "right" price, not at launch and not even after widespread use. Nor does any manufacturer. This is true of the increasingly rare "me-too" drugs but also of:

- Curative therapies whose real impact may not be known for decades,
- Incrementally beneficial (cancer) therapies designed to keep patients alive for six months until they develop resistance and a new therapy is introduced,
- Orphan drugs for patients with no natural history for projecting cost-effectiveness because they previously just died,
- Biosimilars that offer attractive prices but potentially serious risks,
- Repurposed drugs whose prices have become so low that R&D investment is not justifiable for new indications.

The establishment of the PMPRB in 1987 predates the mandate for CADTH's predecessor, the Canadian Coordinating Office for Health Technology Assessment, established in 1992. The Common Drug Review became operational in 2003 to assess for all provinces (except Quebec) the cost-effectiveness of a new drug, that is, "whether it should be paid for by the public drug plans at the manufacturer submitted price." Today, as noted, there is also the panCanadian Pharmaceutical Alliance (pCPA), which negotiates a collective "real listing" price on behalf of all public drug plans. Currently, it can be assumed that the negotiated price is lower than the PMPRB allowable price and closer to the "cost-effective" threshold determined by CADTH. The ceiling price determined by PMPRB was (at one time) the list price for Canada's private drug plans, although rebates are often negotiated as well as "compassionate support" to offset patient co-pays. Private insurers have an array of tools available to them.

CORD is particularly concerned that potential PMPRB reforms will create powerful disincentives for rare disease drugs to be available in Canada, or at least, not until they have been successfully stabilized in terms of pricing and use in other jurisdictions. Canada is already one of the last countries in the developed world to receive new orphan drugs, and the directions in new PMPRB Guidelines suggested by the Discussion Paper will certainly erect new barriers. The singling out of specialty drugs and the characterization of a "first-in-therapy" as a potential "monopoly" drug that warrants special focus is not only pejorative but unfounded. It is not CORD's intention to justify current drug prices, including those for rare and ultra-rare indications, but we also have no basis for assuming that these prices are inflated or abusive.

Rather than Canada striking its own path and one that will make us singularly unattractive for innovative therapies, the better option would be to join with other countries to negotiate for a common price.

In Europe, multi-country consortiums are being formed. For example, Belgium, the Netherlands, and Luxemburg are negotiating together with aspirational plans to form the Joint EU Member States Procurement of Rare Disease Medicines Initiative. Similarly, pilot programs under the European Medicines Agency ADAPTSMART initiative are considering not only common criteria for access but also patient registries to monitor outcomes and to assess value using real-world evidence. Thus, the price of the drug, post-market, can be re-negotiated based on measures of real benefits to patients, the health system, and society. Canada, now with CETA in place, should consider joining this consortium.

CORD appreciates the opportunity to provide comments on the Guidelines Modernization Discussion Paper and looks forward to continuing to engage with PMPRB in its consultations.

Submitted by:
Durhane Wong-Rieger
President & CEO
Canadian Organization for Rare Disorders