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December 13, 2023

PMPRB Guidelines Consultation

Thank you for the opportunity to comment on the PMPRB proposed guidelines. I bring to your attention the fact that I also provided comment in December 2019 on the original proposed guidelines.

I am writing as the Chair of the Expensive Drugs for Rare Diseases (EDRD) Advisory Committee of British Columbia. This committee includes specialists physicians, ethicists, pharmacists and pharmacy-economists. Our mandate is to provide expert evidence-informed advice to the Ministry of Health on the exceptional coverage of expensive drugs for rare diseases and related issues.

I have reviewed the "Scoping Paper for the Consultations on the Board's Guidelines - November 2023" and will concentrate my comments on themes 5 and 6.

The chart provided in Box 5 illustrates the current challenge public funders confront and the reality is this trend is very likely to continue over time. The ability of funders to sustain plans where a very small portion of beneficiaries receive vastly disproportionate benefits is an open question. For those in decision-making positions this discussion is complicated by the lack of transparency around price, the often inadequate clinical evidence of effectiveness of EDRD drugs given the inability to mount trials with robust numbers of participants, and the fact that regulatory decisions are made in isolation from decisions around price and coverage.

Theme Five

Canada has made made significant advances over the past twenty years in the evaluation and management of pharmaceuticals through the creation of entities like CADTH and the panCanadian pharmaceutical Alliance. Notwithstanding these advances, the regulatory, approval and listing arrangements in Canada should be improved in at least three ways. First, pharmaceutical prices should be related to proven effectiveness and price should be reset from time to time based on ongoing evaluations of effectiveness. Due to the limited numbers of patients, measures of effectiveness gleaned from initial clinical trials for EDRD tend to be weak; strong evidence of effectiveness can be seen only after real world experience (RWE) with the drug. In countries where the regulatory function is better aligned with pricing and coverage decisions, this is an easier proposition. In Canada, it will be necessary for CADTH and PMPRB to work together to devise requirements and schedules that will allow for temporary pricing with re-evaluations based on real-world experience.

With respect to the subject of potential efficiencies to be achieved by co-ordinating decisions and timelines of the PMPRB with other drug assessment agencies, the major efficiency may be reaching coverage decisions in a more timely manner by sequencing the various reviews more effectively and by sharing common information at the beginning of the review process.

PMPRB and Health Canada should work together to ensure that Health Canada regulations do not allow established products to be registered as drugs and priced as EDRDs. The regulatory

loopholes which allowed Cystadrops to be marketed in Canada at $100,000/patient/year should be reviewed by PMPRB and Health Canada to ensure these loopholes are tightened. Finally, CADTH, PMPRB and CIHI should work together to create national, public patient registries which are linkable to other national datasets and which clinicians and researchers can use for research and patient improvement studies. Given the small number of patients with a particular rare disease, any meaningful analyses will require aggregating information from all patients across Canada.

Theme Six

In providing advice to the Ministry of Health list prices represent the only information the Advisory Committee has on price. Although provinces negotiate price through the panCanadian Pharmaceutical Alliance this information is confidential and not revealed to the committee. The fact that committee members who include transplant specialists, cardiologists and oncologists knowledgeable about the cost of organ transplantation, open heart surgery and radiation therapy do not have information on the actual price of drugs they prescribe is problematic. Not only does it preclude informed assessments of the benefits of drug versus non-pharmaceutical treatments, this lack of transparency stifles informed public discussion about these drugs which is problematic from an open government perspective.

Over the past twenty years many new drugs, including EDRD drugs have significantly improved the quality of many patients' lives and indeed have saved lives. It is understood that additional new drugs can be developed only through research and development expenditures. Using the "lens" of research and development costs is another way that PMPRB can view whether a price is "excessive" and these costs be based on objective information.

Thank you for the opportunity to comment on these proposed guidelines. If you would like further information or to discuss these suggestions I would be happy to set up a mutually convenient time to do so.

Sincerely,

Anne McFarlane