Response to "Canadians Need Improved Access to Drugs for Rare Diseases, Not More Denial"

LETTER TO THE EDITOR

Dear Editor

The comments provided by Rawson and Adams (2023) miss the mark of our articles (Sirrs et al. 2023a, 2023b). We agree that the patient perspective is critical and that patients with "rare diseases have a right to health-care and have huge unmet needs …" (p. 7). However, we challenge Rawson and Adams' (2023) thesis that keeping drug prices higher in Canada than in most other countries would solve the problem of access to therapies for rare diseases that have no available treatment.

More recent data (PMPRB 2022) than that cited by Rawson and Adams (2023) show that four out of the six non–European Union and Group of Seven nations spend less than Canada. Countries with similar healthcare systems (such as the UK and Australia) spend 10–12% less than Canada on drugs for rare diseases (DRDs). Rawson and Adams' (2023) contention that "developers … expect to recoup their [development] costs … resulting in high per-patient prices for DRDs" (p. 6) ignores the extensive literature that does not support a positive association between research and development costs and drug prices (e.g., Jayasundara et al. 2019; Wouters et al. 2022).

Rawson and Adams' (2023) statement that "few government drug plans offer any kind of coverage for" (p. 5) Zolgensma is incorrect. All the Canadian provinces, the Yukon and the Non-Insured Health Benefits for First Nations and Inuit provide coverage for Zolgensma (CureSMA Canada 2023). Ontario, Quebec, Alberta, British Columbia, Nova Scotia and Saskatchewan already cover Luxturna, with other jurisdictions working toward agreements (Fighting Blindness Canada 2023). Trikafta is reimbursed in every province (Cystic Fibrosis Canada 2023).

In our opinion, the arguments used by Rawson and Adams (2023) echo those used by manufacturers – and in the complex interplay between industry, healthcare bureaucracies and academia, conflicts of interest may arise. However, we believe that companies are capable of change – indeed, if corporations were never required to deviate from purely profit-based models, we would have never moved away from the use of inappropriate and exploitive labour practices or fossil fuels. However, refusing to acknowledge challenges will not help find solutions for Canadians with rare diseases.

Conflict of Interest

The authors declare no conflicts of interest.

Thank you,
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