

## National Strategy for Drugs for Rare Diseases should Prioritize Patients not Cost Containment

### Description

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### National Strategy for Drugs for Rare Diseases should Prioritize Patients not Cost Containment

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#### ABSTRACT

The 2019 federal budget raised hope for Canadians living with rare diseases by proposing to invest up to \$1 billion over two years starting in 2022-23, with up to \$500 million per year ongoing, in a national strategy for high-cost drugs for rare diseases. This commitment was reaffirmed in the 2020 fall economic statement. In January 2021, Health Canada published a discussion paper to engage Canadians in building the strategy. The principal emphasis of the discussion paper is cost-containment for high-cost drugs, which Health Canada equates with drugs for rare diseases. This is worrisome. First because the priority should be patients not cost containment. Second, drugs for rare diseases, whether high-cost or not, must be part of a strategy for rare disorders, but they should not be the only component of it. A rare disease strategy must include improving early detection and prevention and providing timely, equitable, evidence-based and coordinated care, as well as providing sustainable access to potentially beneficial treatments. Research into understanding the occurrence, impact and outcome of rare disorders, unmet health needs, and potential and actual therapies for them should be promoted. All these essential elements were proposed by the entire rare diseases community in 2015 coordinated by the Canadian Organization for Rare Disorders. Canadians with rare disorders need their governments to implement a comprehensive national strategy for rare diseases with Ottawa serving as facilitator and funder. What Health Canada proposes is a plan for cost-containment that will disincentivize drug developers from launching innovative therapies in Canada. Breakthroughs, such as messenger RNA technologies for precision medicines for rare disorders move ahead rapidly in other countries but languish in Canada. Spending on drugs for rare diseases is sustainable. By comparison, spending is higher for new cancer drugs, but politicians and government officials are extremely unlikely to suggest that innovative cancer drugs should be withheld from patients. The federal government's cost containment policy deprioritizes the unmet needs of Canadians with rare disorders.

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**DISCLOSURE**

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