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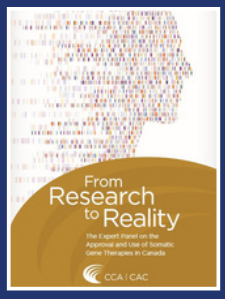
From Research to Reality

The Expert Panel on the Approval and Use of Somatic Gene Therapies in Canada



Gene therapies are being approved for use in Canada, but could strain healthcare budgets and exacerbate existing treatment inequities across the country. However, there are opportunities to control spending, streamline approvals and support fair access through innovation, coordination and collaboration, according to a new expert panel report from the Council of Canadian Academies (CCA).

From Research to Reality describes the stages involved in the approval and use of gene therapies in Canada, and examines the challenges associated with regulatory oversight, manufacturing, access, and affordability. It also identifies emerging solutions that can capitalize on Canadian strengths in research, manufacturing and health-care accessibility.

The diversity of gene therapies requires a flexible and tailored approach to addressing access and affordability challenges

There is substantial diversity among gene therapies — in diseases treated, ways that genetic material is altered, ways therapies are administered, and ways genetic material is delivered to a cell. Each gene therapy will have its own profile across these four dimensions, with implications for safety, effectiveness, cost, and complexity of manufacturing and provision. Availability of treatment alternatives, patient population size, and potential complications add to the complexity of gene therapies.

This diversity necessitates a flexible approach to decision-making as gene therapies move through regulatory approval, funding decisions, manufacturing, and ultimately use in clinics. Clinical trials may be difficult to design, access may be complicated by advanced manufacturing and provision requirements, and value may be enhanced when therapies apply to severe diseases that lack alternative treatments. When regulation, provision, and reimbursement approaches are flexible, decision-makers can accommodate these potential challenges, and ultimately enhance affordability and access. This growing flexibility can be seen in Health Canada's new pathway for reviewing advanced therapeutic products, the emergence of collaborative patient registries, and adjustments to health technology assessment (HTA) processes.

Risk-based purchasing agreements and post-market surveillance could mitigate the significant clinical and economic uncertainties associated with approved gene therapies

The long-term safety and durability of gene therapies are uncertain by virtue of their recent introduction in the market and the short length of clinical trials relative to the anticipated long-term impacts of therapies. This uncertainty is a challenge for regulators, HTA bodies, and public drug plans that must proceed with decision-making despite limited information. Performance-based agreements between payers and manufacturers could be used to share the risks associated with funding gene therapies. Additionally, ongoing monitoring of the performance of approved therapies could help to reduce uncertainty over time. Post-market surveillance could be used to inform regulatory and economic reassessments of approved gene therapies as the evidence base grows.

High prices, complex provision, and the nature of diseases treated by gene therapies exacerbate existing inequities in healthcare access

To date, approved gene therapies have generally been priced in the hundreds of thousands of dollars, and these prices do not include any additional costs of hospital care. This necessitates careful scrutiny of the value and affordability of gene therapies by public drug plans. Jurisdictions may reach different funding decisions based on their own valuations, leading to unequal access across regions. Further compounding access challenges, complex gene therapies will likely only be available in large urban hospitals that have the advanced infrastructure and personnel required to administer them and manage adverse reactions. Patients with rare diseases may confront additional difficulties receiving accurate diagnoses and accessing high-cost treatments in public healthcare systems.

Different conceptions of value may lead to disagreement over the merits of publicly funding individual gene therapies

Inevitably, public payers face trade-offs. On the one hand, they wish to maximize health gains at the population level by funding drugs that offer the greatest improvement in life expectancy and quality of life at the lowest cost. On the other, they also consider funding more expensive drugs based on additional values such as severity, rarity, and novelty of illness, as well as lack of treatment alternatives. These trade-offs have long been debated, with well-established ethical arguments underpinning different conceptions of value. Based on existing research in Canada and Europe, society appears to broadly support spending on those drugs that offer the greatest improvements at the least cost, but favours spending on relatively high-cost drugs in cases where diseases are severe and lack alternative treatments. Enhanced transparency in value assessments could improve consistency and help manage patient and sponsor expectations.

Pan-Canadian coordination could control spending and improve access to gene therapies

Coordinated efforts to manage drug prices are well established. The pan-Canadian Pharmaceutical Alliance (pCPA) negotiates drug prices on behalf of numerous public drug plans to help contain and equalize costs, while the Patented Medicine Prices Review Board (PMPRB) provides federal oversight on the prices of patented medicines, ensuring they are not excessive. Further coordination could be achieved through national pharmacare, which could bring regulatory reviews, HTAs, and price negotiations under a joint approach, potentially reducing overall review time and equalizing access across jurisdictions. Even in the absence of a national program, provinces, territories, and the federal government could work together to establish common principles and approaches to accessing high-cost therapies. Further areas for collaboration include the development and maintenance of registries, training of highly qualified personnel, and manufacturing capacity.

Stewardship of public investments in gene therapy research could alleviate challenges associated with commercialization and high drug prices

Despite early public investments in research, technologies are usually transferred to the private sector as new gene therapies move toward commercialization. In this process, the public sector loses its claims over the intellectual property it helped develop. Patent leasing has been proposed as one mechanism to enhance the value of public research investments. Or, if patents are sold to the private sector, there may be opportunities to build in favourable drug pricing clauses that could enhance public benefit. Public manufacture and commercialization of gene therapies offer additional options for protecting public investments. With these controls, the public could potentially exert greater influence over the price and accessibility of new gene therapies.