

Expensive gene therapies raise challenges and opportunities: Expert panel report

News Release

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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).

"Rapid scientific advances mean potentially life-changing treatments are approaching the clinic at an accelerated pace," said Janet Rossant, PhD, C.C., FRSC, and Chair of the Expert Panel. "These new therapies, however, pose a number of challenges in terms of their introduction into the Canadian healthcare system and ensuring access to those who would most benefit."

<u>The National Research Council of Canada</u> asked the CCA to examine the key legal, regulatory, ethical, social and policy challenges specific to the approval and use of somatic gene and engineered cell therapies in Canada.

Somatic gene and engineered cell therapies treat disease by removing or modifying existing genetic material in a patient, or introducing new genetic material, without passing these changes to future generations. Most treat rare genetic disorders and cancers, however they can cost more than \$1 million and have limited evidence of long-term safety and durability.

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 approval and use of gene therapies challenges payers, regulators, industry, patients and families," said Eric M. Meslin, PhD, FCAHS, President and CEO of the CCA. "This report covers many of these challenges as well as possible paths forward, and will help to support evidence-informed decision making in Canada."

Visit www.cca-reports.ca to download the report.

From Research to Reality will be featured at **Breaking Through**, the 2020 Gairdner International Ontario Symposium on Thursday, December 3, 2020. Information and registration is available at www.breaking-through.ca.

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About the Council of Canadian Academies

The CCA is a not-for-profit organization that undertakes independent, evidence-based expert panel assessments to inform public policy development in Canada. The CCA was founded by three independent organizations that represent some of the finest minds in Canada — the Royal Society of Canada, the Canadian Academy of Engineering, and the Canadian Academy of Health Sciences. Their Fellows and senior decision-makers sit on CCA's Board of Directors and Scientific Advisory Committee, and they are a key source of membership for expert panels. The CCA's founding Academies also provide key guidance and input throughout the assessment process, including expert panel nominations and dissemination processes. For more information about the CCA or its assessments, please visit www.cca-reports.ca.

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