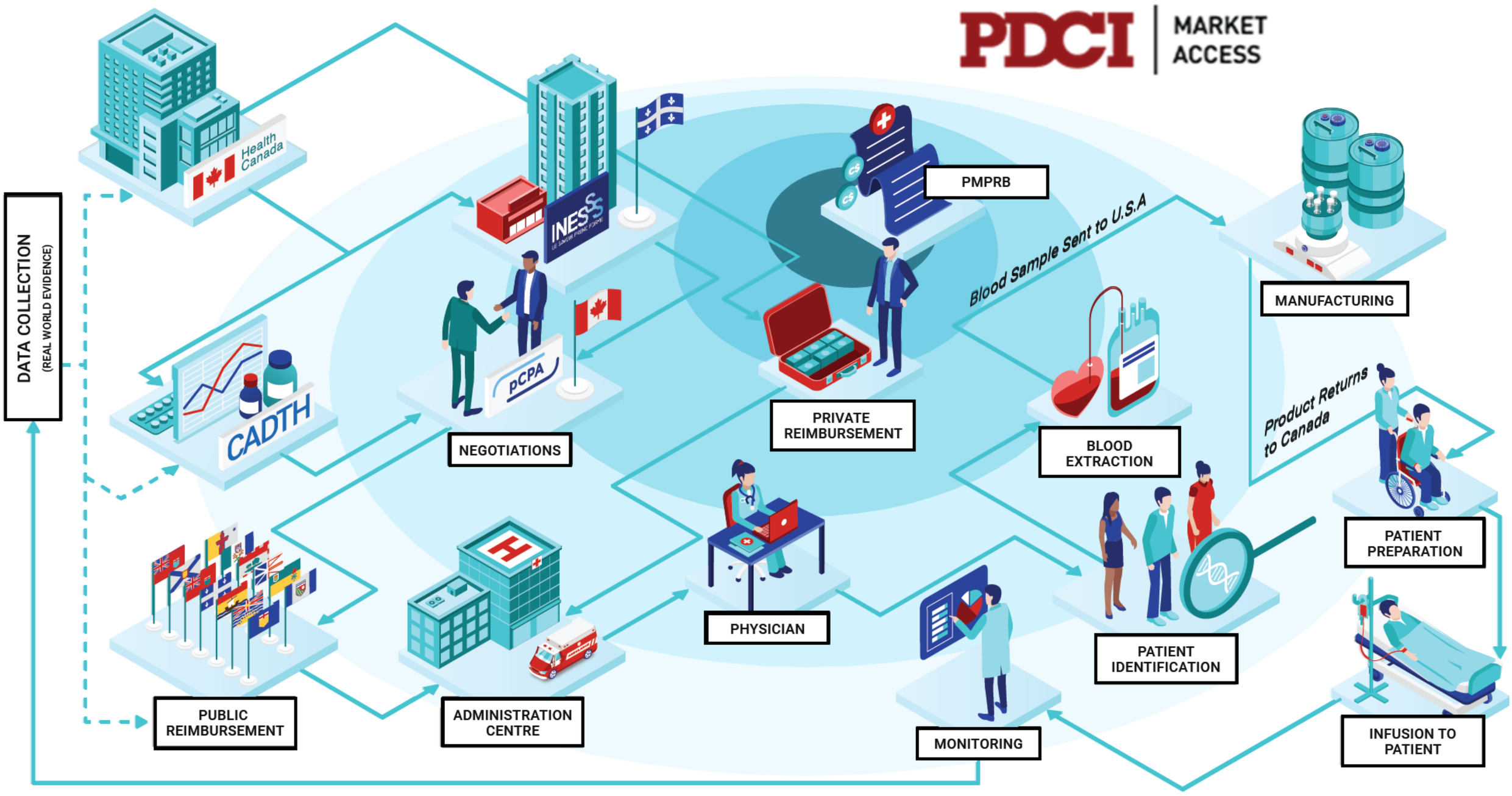
CHALLENGES AND GAPS IN GENE & CELL THERAPY ASSESSMENT AND REIMBURSEMENT PATHWAY

The reimbursement pathway for gene therapies in Canada is complex and evolving. As the framework for moving gene therapies through Health Canada reviews to public and private reimbursement continues to develop, many stakeholders can expect to face obstacles and uncertainty securing patient access to these novel therapies. Gaps and potential challenges include ethics considerations, funding and reimbursement models, administration and distribution infrastructures, equity in small patient populations and the need for long-term data. It is important that these issues are resolved in a way that balances the sustainability of the Canadian healthcare system with the need for timely access for patients. With an increasing number of gene and cell therapies in the pipeline, a structured pathway from regulatory approval to reimbursement on public and private plans must be developed to accommodate these novel therapies.



STAKEHOLDER	RESPONSIBILITIES	GAPS & POTENTIAL CHALLENGES FOR THE FUTURE
REGULATORY (HEALTH CANADA)	Evaluates and monitors the safety, efficacy and quality of pharmaceuticals. Currently assesses gene therapies as biologic drugs.	• Is the appropriate infrastructure set up to regulate the many steps involved in the cell and gene therapy reimbursement pathway? • Will there be a new regulatory framework for these therapies?
HEALTH TECHNOLOGY ASSESSMENT BODY (CADTH)	The Canadian Agency for Drugs and Technologies in Health (CADTH) conducts clinical and pharmacoeconomic reviews of pharmaceuticals and medical devices for all provinces except Quebec.	• The assessment that is currently performed through Medical Devices "Optimal Use" review pathway lacks transparency, solid deadlines, strong recommendations and is essentially the same as a Common Drug Review (CDR) or pan-Canadian Oncology Drug Review (pCODR). • Is there consideration of a provisional algorithm? In theory, gene therapies could displace many treatments.
HEALTH TECHNOLOGY ASSESSMENT BODY (INESSS)	The Institut national d'excellence en santé et en services sociaux (INESSS) conducts clinical and pharmacoeconomic reviews of pharmaceuticals and medical devices for the province of Quebec in a process similar to CADTH.	• How will INESSS align with CADTH and Health Canada reviews? • Will the collection of real-world evidence (RWE) be mandated?
NEGOTIATION BODIES (CANCER AGENCIES, PCPA, PROVINCES)	The pan-Canadian Pharmaceutical Alliance (pCPA) negotiates net prices for branded drugs on behalf of the provinces. Cancer Agencies can also play a role in negotiation.	• Who will be the negotiating body (e.g., pCPA, proposed Cancer Drug Agency [CDA], Canadian Blood Services [CBS])? • What is the most appropriate payment model? • Will the costs of these therapies be sustainable over time for the Canadian governments (Federal, Provincial, Territorial)?
PRICING BODY (PMPRB)	The Patented Medicine Prices Review Board (PMPRB) is mandated to ensure pricing for patented medicines is "non-excessive".	• How will pricing be determined and value re-evaluated? • How will the Human Drug Advisory Panel (HDAP) reviews be assessed? Will there be revised guidelines?
ADMINISTRATION CENTRE	Accreditation sites (FACT; Foundation for the Accreditation of Cellular Therapy) are used in the extraction and infusion of therapies. They are also involved in the provision of care and monitoring.	• How will centers be certified to ensure safety and standardization of the administration and monitoring process. • Will there be involvement of administration centres RWE collection and generation?
PATIENT JOURNEY	Patients must navigate the drug reimbursement pathway while undergoing diagnosis and testing. Once a therapy is approved, patients may need to spend time in the hospital for blood extraction, chemotherapy and blood infusions.	• Who will have the responsibility for the payment of adjunct therapies, transportation, and other costs associated with cell and gene therapies? • Will distribution channels designed to handle specialty drugs (Specialty Pharmacy) play a role? • How will equity in access be supported across Canada? • How will side effects of non-specific gene insertions be managed? • What happens upon relapse of a condition?
PUBLIC REIMBURSEMENT	Public drug plans (provincial and federal) currently reimburse a limited formulary of drugs.	• Which governments (Provincial, Federal, Territorial) will be involved in providing funding? • How often will payments occur? Will outcomes based agreements (OBA) be explored? • Is paying for these therapies viable under a single-payer Pharmacare regime?
PRIVATE REIMBURSEMENT	Generally has a more comprehensive formulary than public plans.	• Will the high costs of gene therapy be sustainable for private systems, especially small employers? • Do private infusion clinics have the necessary infrastructure for the provision and monitoring of gene and cell therapies (e.g., cell transport, cell extraction, cell preservation, hospital care management teams)?
DATA COLLECTION	Some manufacturers have a patient registry in place to monitor patients but this is not required by Health Canada, health technology assessment and negotiation bodies.	• Who will be responsible for setting up the infrastructure and data analytic capabilities necessary to collect and analyse RWE? • Who will determine what outcomes are relevant and provide real world value to stakeholders? • How will data collection be standardized?