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Panel Abstracts

PL1. From Concept to Practice: Advancing Coverage With Evidence Development in Canada

Presenters and coauthors: Scott Gavura, Ontario Health; Angie Wong, Ontario Ministry of Health; Caroline Muñoz, Ontario Health; Winson Cheung, University of Calgary

Coauthors: Jessica Arias, Alayna Brown, Ontario Health

Background: The number of new cancer drugs being approved and funded in Canada is increasing. These drugs often come with large price tags and promising but unclear efficacy based on the initial clinical evidence. Coverage with Evidence Development (CED) agreements support real-world evidence (RWE) generation to address uncertainties that may exist when funding new drugs. A recently completed environmental scan (in press) examined CED structures and operations across 13 national and international jurisdictions, identifying how RWE informs drug funding decisions, with benefits and limitations of this approach identified. While CED supports risk-sharing and evidence generation, challenges identified include unclear decision-making criteria, limited transparency, and feasibility of data collection. These issues, if addressed, can support a robust Canadian model of CED.

Approach: A moderated panel discussion will assess the findings of the environmental scan and the current landscape and enthusiasm for CED in Canada. A summary of the environmental scan will be presented by its author. Panellists invited to respond to the environmental scan will include a clinician-researcher involved in RWE, a provincial cancer agency representative, a provincial ministry of health representative, and an industry representative (to be confirmed). The panel discussion will focus on moving from CED as a concept to routine practice. Panellists and audience members will be invited to identify components of a feasible model for CED in Canada that builds on identified best practices and addresses noted limitations.

PL2. Setting a Target: Antipsychotic Use in Long-Term Care

Presenters and coauthors: Norma Hall, Canadian Institute for Health Information; Elizabeth Carson, Canada's Drug Agency; Tai Huynh, Choosing Wisely Canada; Jonathan Lam, Canada's Drug Agency

Many older adults in long-term care (LTC) are prescribed antipsychotics without a diagnosis of psychosis. In Canada, the rate of potentially inappropriate use has climbed back to prepandemic levels at 24.5%, raising the risk of serious harms such as stroke, falls, hospitalization, and even death in this vulnerable population. Addressing this issue is both a clinical and system priority. This session will explore efforts to address this issue through (1) measuring, exploring current trends, and publicly reporting potentially inappropriate use of antipsychotics; (2) establishing a coalition of pan-Canadian organizations focused on developing partnerships and strategies to address rising rates in the community; (3) reviewing how the coalition is advancing the target by connecting the LTC sector with implementation supports, programs and tools, and

knowledge-sharing webinars; and most importantly (4) setting a national target for appropriate antipsychotic use in Canada using a modified Delphi methodology, whereby an independent panel of clinicians, quality improvement experts, and those with lived and living experience of LTC developed evidence-informed consensus statements on a target for LTC homes in Canada and an associated annual improvement goal. Participants in this session will gain insight into how consensus-driven targets, combined with coordinated mobilization efforts, have the potential to support improvement and promote safer prescribing in LTC.

PL3. Travel to Treatment as an Equity Consideration in Cancer Care: Implications for HTA and Policy in Canada

Presenters and coauthors: Christina Sit, Leukemia and Lymphoma Society; Kathleen Barnard, Save Your Skin Foundation; Louise Binder, Save Your Skin Foundation; Antonella Scali, Psoriasis Canada

Coauthor: Colleen McMillan, Leukemia & Lymphoma Society of Canada

Background: Equitable access to cancer treatment extends beyond drug approval and reimbursement in each province. In Canada, patients with cancers often face extensive travel to access care, creating additional financial, physical, and psychosocial burdens. Travel-related barriers create inequities that are not fully captured by conventional cost-effectiveness analyses.

Objective: This session examines the financial, physical, and psychosocial effects of travel to treatment among cancer patients and caregivers in Canada, with a view to informing how Canada's Drug Agency and decision-makers can better define and integrate equity into health technology assessment (HTA).

Overview: As part of this session, the Leukemia & Lymphoma Society of Canada will present the results of their national blood cancer travel-to-treatment study. The study was conducted between November 2024 and January 2025, and participants reported on financial, physical, and psychosocial costs of travelling to blood cancer treatment. The Save Your Skin Foundation will present an impact analysis from their travel financial assistance program for patients with melanoma and other skin cancers in Canada. Collectively, these findings highlight the need to access and incorporate real-life impacts, such as travel to treatment, systematically in order for "equity" to be a weighted part of the HTA assessment.

PL4. Blind Spots and Bias in HTA: Patient-Centred Drug Assessment to Unlock Better Psychiatric Drug Access

Presenters and coauthors: Aimée Tran Ba Huy, Mood Disorders Society of Canada; Fred Horne, 3Sixty Public Affairs Inc.; Graham Statt, Santis Health; Dr. Pierre Blier, University of Ottawa

Presenter: Adam Haynes, Eisai

Symptom severity, function, quality of life, side effects, survival. These domains shape the framework for guidance on clinically meaningful end points that, when considered, may address blind spots in health technology assessment (HTA)/payer assessments of psychiatric medications beyond quality-adjusted life-years, from health equity, societal, and severity perspectives.

Convened by Mood Disorders Society of Canada, a Canadian mental health patient organization, to help close the gap of inequity in access to psychiatric medications (39% higher rate of negative HTA assessments), a group of diverse health system leaders came together in a working group (WG) that met over 2 years to develop the guidance, keeping front-line voices at the centre of the dialogue. Combining perspectives from people with lived/living experience, patient organizations, public and private payers, pharmacists, nurse practitioners, psychiatrists, the pharmaceutical industry, and HTA (an Institut national d'excellence en santé et en services sociaux view and input from Canada's Drug Agency as a knowledge resources WG member), the WG assessed both a lack of understanding and unconscious bias/stigma to be the underlying factors behind inequities. The codeveloped guidance forms an educational foundation to address these 2 factors.

In this session, WG members (a person with lived/living experience, a clinician, an HTA representative, an industry representative, and a former ministry of health representative as Moderator) will unveil the guidance, including its 7 domains and corollary subindicators. Panellists will showcase real-world applications of the guidance, where audience members interactively take on the role of a payer/HTA reviewer in an anonymous drug review, comparing the current standards using the guidance and demonstrating how differing outcomes are derived.

PL5. When System-Level Challenges Require System-Level Solutions: The Innovation Journey of the Health Technology Expert Review Panel From HTA to HSA

Presenters and coauthors: Leslie Anne Campbell, Dalhousie University; Duncan Steele, Alberta Health Services; Health Technology Expert Review Panel; Gino De Angelis, Canada's Drug Agency; Deirdre DeJean, Canada's Drug Agency; Howard Ovens, Sinai Health

Health technology assessment (HTA) has been invaluable in guiding decisions about the adoption of individual drugs, devices, and interventions. However, today's health care challenges, such as workforce pressures, challenges in access to care, aging populations, and rapid digital innovation, require interventions that extend beyond single technologies. This panel will build upon the 2024 Symposium's panel discussion to further explore the shift from HTA to health system assessment (HSA), which considers the broader impacts of complex technologies and interventions on service delivery, financing, equity, and sustainability. Drawing on real-world examples, panellists will highlight how system-level perspectives can help decision-makers anticipate unintended consequences, align investments with health system priorities, and ensure value for patients and populations. The discussion will focus on practical implications for HTA producers, policy-makers, and health system leaders, including how to adapt deliberative and assessment

frameworks, consider cross-sector collaboration, and use nontraditional forms of evidence to inform complex choices about system design and preparedness. By moving from technology-focused to system-focused assessments, health systems may be better poised to make more strategic, resilient, and equitable decisions in increasingly complex and fiscally restrained environments.

PL6. Smarter Decisions, Stronger Systems: Building Future-Ready Medical Imaging Capacity

Presenters and coauthors: Erin Kirwin, Institute of Health Economics; Nick Neuheimer, Canadian Association of Radiologists; Beth Kidd, Health Coalition of Alberta

Presenters: Rebeccah Marsh, Institute of Health Economics; Laura Weeks, Canada's Drug Agency

Coauthor: Michael Situ, Canada's Drug Agency; Dat Tran, Institute of Health Economics

Medical imaging — like X-rays, CT scans, and MRIs — is essential for timely diagnosis and treatment for a variety of health conditions. Yet many health systems in Canada face serious and increasing challenges with respect to medical imaging: long wait times, staff shortages, outdated machines, and unequal access between cities and rural or remote communities. Additional population factors in Canada such as an aging population and the associated increase in the incidence of cancer, dementia, and other chronic diseases continue to drive up demand. Together, these problems delay diagnosis and care, directly impacting patient health outcomes.

This panel will discuss how health systems can make evidence-informed decisions to address these challenges and prepare for the future. The panel will begin with an overview of current medical imaging challenges in Canada. Panellists will next explore how data can help us understand existing and future demand and make data-driven decisions to improve patient access, outcomes, and the efficiency of the health care systems. By focusing on both the challenges we face today alongside the information and analysis required to inform decision-making around these challenges, this session will show how medical imaging can shift from being a bottleneck to becoming a driver of better, fairer, and more sustainable health care. By starting this conversation, we invite relevant stakeholders across provinces and regions to work together toward more equitable, sustainable, and resilient future-ready health systems.

PL7. Future-Ready Systems: Accelerating Cell and Gene Therapy Adoption in Canada

Presenters and coauthors: Cate Murray, Stem Cell Network; Stéphanie Michaud, BioCanRx

Presenters: Dr. Bernard Thébaud, University of Ottawa; Josée Champagne, Ottawa Hospital Research Institute; Patrick Bedford, Morphocell Technologies; Jason Guertin, Université Laval; Dr. Denis-Claude Roy, University of Montreal

Coauthors: Omar Tounekti, Health Canada; Harry Atkins, Ottawa Hospital Research Institute; Véronique Moulin, Université Laval

Cell and gene therapies (CGTs) represent a transformative class of therapeutics with the potential to cure previously untreatable diseases, including type 1 diabetes, rare disorders, and specific cancers, and to enable tissue and organ repair. While researchers and companies in Canada are advancing world-class CGT innovations, patients continue to face delays of 3 to 5 years compared to international peers in the European Union, Japan, and the US. These gaps, driven by fragmented regulatory, reimbursement, and implementation systems, contribute to inequities in access, loss of talent and intellectual property, and missed opportunities for Canada's biotech sector.

This panel will explore the design and implementation of a Canadian Accelerated Approval and Adoption Pathway (CAAAP): a nationally coordinated framework to ensure timely and equitable patient access to CGTs. Moderated by Cate Murray (Stem Cell Network), the discussion will bring together diverse expertise spanning patient-focused clinical care (Dr. Harry Atkins, Ottawa Hospital Research Institute), translational science and commercialization (Dr. Stéphanie Michaud, BioCanRx), health policy and system integration (Véronique Moulin, Université Laval), and regulatory oversight (Omar Tounekti, Health Canada). Panellists will address integrated regulatory and health technology assessment reviews, innovative reimbursement and risk-sharing models, system readiness through centres of excellence and digital monitoring, and commercialization supports for bioentrepreneurs in Canada. By highlighting perspectives across science, policy, regulation, and clinical practice, this session will advance a critical dialogue on how Canada can position CGTs as both a pillar of equitable health care and a driver of global competitiveness. CAAAP offers a future-ready systems solution — ensuring patients in Canada benefit first from Canadian discoveries.

Poster Abstracts

PO1. Paramedic Workplace Violence Interventions: A Rapid Review

Presenting author: Gail Thornton, University of Alberta

Coauthors: Devidas Menon, Tania Stafinski, University of Alberta

Workplace violence in emergency medical services occurs prehospital and in emergency departments (EDs) and leads to challenges around workforce retention. A rapid review was conducted to understand what is known about strategies/interventions for preventing or reducing workplace violence in these settings. A structured search for academic evidence published within the last 10 years on strategies/interventions to prevent or reduce worker-to-worker or patient-to-worker violence in prehospital or ED settings was conducted using the following databases: MEDLINE, Embase, CINAHL, and PubMed. Of 8,850 records found, 167 were potentially relevant and retrieved for further consideration. Ten primary studies met the inclusion criteria and ultimately comprised the review. All involved interactions between paramedics and patients who had demonstrated combative or violent behaviour. Eight occurred in prehospital settings (4 Canada; 4 US) and 2 in EDs (2 US). Of those in prehospital settings, 5 assessed adverse events associated with chemical (pharmacological) restraint use, mainly through retrospective chart reviews. Two comprised pre-post studies of violence reporting systems, and 1 reported on the development of a prehospital risk assessment tool for predicting the likelihood of a patient demonstrating violent behaviour in the ED. In the 2 studies of EDs, paramedics represented fewer than 20% of participants. One involved a pre-post study of the implementation of a violence reporting system, while the other surveyed participants about their level of confidence in de-escalation techniques after simulation-based training. Across all 10 studies, positive results were presented, suggesting that while the evidence is limited, the strategies appear promising.

PO2. Towards Patient-Centricity: A Casebook on Using Patient Experience Data in HTA

Presenting author: Patil Mksyartinian, Colorectal Cancer Canada

Patient experience data (PED) is critical for understanding patient priorities in decision-making, particularly in areas of high uncertainty such as cell and gene therapies, targeted therapies, and rare diseases. By embedding patient values into regulatory and health technology assessment (HTA) processes, PED has the potential to reshape health care. Yet, its integration remains debated, with ongoing questions about what qualifies as robust and relevant data for HTA use. This presentation draws on work from the Health Technology Assessment International Rare Disease Interest Group and the From Testing to Targeted Treatment Program (FT3), which developed a PED casebook for patient organizations and industry. The casebook highlights diverse evidence collection methods used in HTA and advocacy, providing practical insights into study design, appropriate methodologies, and novel value drivers. It serves as both a learning resource and a tool to strengthen advocacy and decision-making. The upcoming PED casebook will feature 5 new examples, each offering lessons on data collection and use in HTA. Real-world applications — such as integrating colorectal cancer patient preferences into drug reimbursement decisions in Canada — will be discussed to illustrate methodological approaches (e.g., mixed methods, preference studies, qualitative research, surveys). The presentation will explore how PED can inform decision-making for small or underrepresented populations, provide guidance for study design, and demonstrate how practical tools can support the development of patient-centred evidence bases. Ultimately, the goal is to evaluate how PED can enhance HTA processes and foster more patient-centric value assessments.

PO3. Real-World Data to Support Decision-Making in Multiple Myeloma

Presenting author: Naomi Chouinard, Institut national d'excellence en santé et en services sociaux

Coauthors: Hichem Makhoukh, Oscar Eduardo Molina, Marianne Bouchard, Patrick Dufort, Thomas Mortier, Institut national d'excellence en santé et en services sociaux

Introduction: Multiple myeloma (MM) is a fast-evolving and costly field, with new drugs reshaping patient distribution across treatment lines. Currently, no data source provides a precise count of patients receiving each line of therapy in Quebec. Market size can be estimated using real-world evidence (RWE) — such as public prescription claims and hospitalization records — and Canadian epidemiological data. Leveraging innovative data sources enables faster and more precise cost estimates while reducing uncertainty in health technology assessment to support decision-making.

Objective: Explore RWE sources in Quebec to enhance the estimation of MM market size and evaluate the potential implications for budget impact analyses.

Methods: Data from the Quebec cancer registry on patients diagnosed with MM were analyzed alongside the annual number of autologous stem cell transplants performed in the province for this population. Additionally, prescription claims for MM treatments among publicly insured patients in Quebec were examined. These RWE sources were compared with published Canadian epidemiological data.

Results: Discordance in terms of the proportion of patients starting treatment and receiving an autologous stem cell transplant was observed when compared to the literature. A difference in market size estimation of about 50% in transplant-eligible patients was obtained. This highlights the potential for overestimating market size and budget impact analyses in transplant-eligible patients with MM when using only Canadian epidemiological data.

Conclusion: Using RWE sources enables more accurate evaluations of market size. However, limitations such as the need for data quality assessment and challenges in linking individual-level data may restrict their usability.

PO4. Improving Time to Patient: Insights from the Canadian Cancer Treatment Hackathons

Presenting author: Patil Mksyartinian, Colorectal Cancer Canada

Coauthor: Barry D. Stein, Colorectal Cancer Canada

Canada's drug reimbursement process is complex, involving multiple levels and jurisdictions, resulting in an average wait of 598 days for patients to access new medicines in 2024. To explore ways to improve access, Colorectal Cancer Canada held Canadian Cancer Treatment Hackathons from November 2022 to July 2025, engaging over 200 thought leaders from Canada and internationally.

The first Hackathon in 2022 focused on novel ideas within existing systems and identified actionable, time-saving opportunities at each step of the health technology assessment (HTA) process, such as concurrent negotiations alongside HTA reviews. The second examined critical success factors from 5 leading HTA agencies in Australia, England and Wales, France, Germany, and Italy. The third enabled participants to design a new drug review and reimbursement process, identifying 5 key themes to improve time to patient. The fourth highlighted high-priority ideas to expedite access through international collaboration agreements. The fifth identified actionable changes among key stakeholder groups, including patients, industry, and clinicians. The sixth explored improvements at the negotiation level, including earlier integration of patient and caregiver values. The seventh refined tools for collecting and using patient experience data. The eighth examined outcomes-based agreements to improve access, identifying key success factors for implementation. The latest, ninth Hackathon explored actionable solutions to streamline post–letter of intent processes to ensure faster, more equitable access to new cancer medicines.

Overall, these efforts reflect a strong commitment to improving patient outcomes by refining Canada's drug review and reimbursement processes through strategic collaborations, patient engagement, and global best practices.

PO5. Impact and Lived Experiences With FreeStyle Libre 2 in Quebec Adults Living With Type 2 Diabetes

Presenting author: Yeesha Poon, Abbott Diabetes Care

Coauthors: Jean-François Yale, McGill University; Sylvie Bouchard, Sylvie Bouchard Pharmacienne Consultante Inc.; Abraham Lee, Abbott Diabetes Care; Bertha De Los Santos, Abbott Diabetes Care

Introduction: Sensor-based technology (SBT) allows people with diabetes access to their comprehensive glucose data. Previous SBT studies have demonstrated glycemic improvements in people living with type 2 diabetes (PWT2D) regardless of insulin treatment. However, the behaviour shifts that lead to these improvements are not well described. This study aims to gain short- and long-term insights into the underlying mechanisms driving glycemic improvement in PWT2D.

Methods: Semistructured focus group interviews were conducted. Participants included French-speaking adults residing in Quebec with type 2 diabetes who used SBT (FreeStyle Libre 2 system). Interviews were conducted in 2 groups: (1) PWT2D on basal insulin treatment and (2) PWT2D on noninsulin treatment. Focus group interviews were transcribed, and thematic analysis was performed to identify recurring patterns. Results described here focus on the drivers of long-term behaviour change.

Results: The basal insulin treatment group consisted of 7 participants — 28.5% female and 85.7% used SBT for longer than 12 months. The noninsulin treatment group consisted of 9 participants — 66.7% female and 77.8% used SBT for longer than 12 months. Thematic saturation was reached after analyzing transcripts from 7 participants. Three key themes related to long-term behaviour change were identified: (1)

development of healthy dietary habits, (2) visualizing the glucose impact of exercise led to increased physical activity, and (3) SBT as a communication tool enabled increased health care provider engagement.

Discussion: Insights from the perspectives of primarily long-term users of SBT described the sustained impact of glucose data access on health-related behaviour, including diet modifications, exercise encouragement, and increased communication with providers.

PO6. Impact of a Patient-Reported Outcome Measure on Health Care Utilization and Costs of Cancer Patients

Presenting author: Soo Jin Seung, Sunnybrook Research Institute

Coauthors: Anisia Wong, Sunnybrook Research Institute; Zharmaine Ante, Institute for Clinical Evaluative Sciences; Ning Liu, Institute for Clinical Evaluative Sciences; Craig Earle, University of Toronto; Lisa Barbera, University of Calgary; Nicole Mittmann, Canada's Drug Agency

Objective: To evaluate the economic impact of exposure to the Edmonton Symptom Assessment System (ESAS), a patient-reported outcome measure, on the provincial health care system.

Methods: We conducted a population-based retrospective matched cohort study of adults in Ontario with a first-ever invasive cancer diagnosis between April 1, 2010, and March 31, 2022. Patients were classified as exposed if they completed the ESAS after diagnosis and unexposed if they did not. The index date was the first ESAS for exposed patients. Health care resource utilization and mean total health care costs (2023 CA$) per patient-year were assessed.

Results: A total of 139,285 propensity score–matched pairs were analyzed, balanced for age, sex, cancer type, and comorbidities. The mean ESAS score among exposed patients was 16.9, with prostate, lung, and hematologic cancers representing the most assessments. ESAS-exposed patients had fewer hospitalizations (1.8 ± 6.2 vs. 3.8 ± 14.8) and physician visits (71.8 ± 111.8 vs. 89.4 ± 177.6) but more cancer clinic visits (7.6 ± 16.4 vs. 4.4 ± 15.1) and oral medications (62.3 ± 107.8 vs. 61.2 ± 114.7) than the unexposed. Total health care costs per patient-year were significantly lower in the exposed group ($59,230 ± $104,328) than in the unexposed group ($76,447 ± $183,127; $P < 0.0001$). Inpatient care was the main cost driver among unexposed patients, while outpatient services such as cancer clinic visits and medications were used more frequently among the exposed.

Conclusion: ESAS exposure was associated with reduced health care resource utilization and costs, suggesting that patient-reported outcome measures may support earlier outpatient care and reduce costlier inpatient use.

PO7. The Evolving Market Landscape for Rare Disease Drugs in Canada

Presenting author: Jihong Yang, Patented Medicine Prices Review Board

Coauthors: Tuhin Rahman, Shirin Rizzardo, Patented Medicine Prices Review Board

Background: The emergence of a growing number of expensive drugs for rare diseases (EDRDs) presents new treatment possibilities while raising questions around affordability and access. This study analyzes EDRDs launched in Canada over the past 2 decades, using quantitative data and evidence to evaluate EDRD trends in drug approvals, drug prices, and sales revenues in Canada.

Methods: Drawing on IQVIA MIDAS data and treatment cost information from Canada's Drug Agency, we examined 159 EDRDs approved in Canada between 2005 and 2024. The analysis explores launch trends, treatment costs, and market growth from 2010 to 2024, comparing oncology and non-oncology EDRDs. It also compares 2024 Canadian list prices with those in 11 comparator countries.

Results: Our preliminary results show that EDRD approvals in Canada tripled in the past decade compared to the previous one. EDRDs accounted for roughly 28% of the branded pharmaceutical market in 2024, up from less than 1% in 2006. More than 60% of these drugs had annual treatment costs exceeding $200,000, and nearly all drugs priced above $1 million per year were non-oncology EDRDs. In 2024, Canadian ex-factory list prices for EDRDs were, on average, 15% higher than the median of the 11 PMPRB comparator countries, ranking the third highest behind Spain and Italy.

Conclusion: EDRDs in Canada are increasing in number and cost, with prices higher than most peer countries. The rise in approvals and expanding market presence underscore the importance of effective policies that support individuals with rare diseases while maintaining the sustainability of the health care system.

PO8. Universal Newborn Screening for Metachromatic Leukodystrophy: A Budget Impact Analysis

Presenting author: Tania Conte, Canada's Drug Agency

Coauthor: Samantha Verbrugghe, Canada's Drug Agency

Early-onset metachromatic leukodystrophy (MLD) was prioritized by the Newborn Screening Advisory Panel due to its rarity, its severity in infancy and early childhood, and the emergence of gene therapy. To support decision-making on its inclusion in the Recommended Pan-Canadian Newborn Screening List, Canada's Drug Agency conducted an evidence review, including a budget impact analysis from the perspective of the health care system in Canada. We assessed the costs and resource use associated with implementing a 3-tiered newborn screening (NBS) strategy for early-onset MLD, recognizing that patients with late-onset MLD would also be diagnosed. The target population included all infants born over a 10-year time horizon and those projected to develop MLD within this period. We compared universal NBS to the current no-

universal-NBS approach, using literature-based epidemiology, natural history, and test data as well as expert-informed costs and resource estimates. Due to limited data on monitoring and treatment, only costs up to diagnosis were included.

Planning for future-ready health systems for universal NBS for MLD offers a pathway to earlier detection but also raises important considerations around system capacity and implementation. Over 10 years, universal NBS could identify 22 to 42 additional patients with early-onset MLD and 6 to 12 additional patients with late-onset MLD within the treatment window of potentially disease-modifying therapies. Implementation would require incremental investments of $4.5 million to $23.7 million, including an additional 0.6 to 9 mass spectrometers and 1 to 7.2 full-time lab technicians, depending on resource sharing and multiplexing of tier-1 tests. The cost per additional potentially treatable patient spans $85,000 to $832,000, reflecting uncertainties in implementation logistics and MLD incidence.

PO9. Medical Device Economic Evaluation Methodological Quality Assessment Checklist

Presenting author: Ilke Akpinar, University of Alberta

Coauthors: Mike Paulden, University of Alberta; Jeff Round, Eltanin Economics

Background: Economic evaluations are essential for guiding health care resource allocation, yet most existing quality assessment tools were developed for pharmaceuticals and do not address challenges relevant to medical devices.

Methods: A list of 49 candidate items was compiled based on a systematic review and existing methodological guidance and refined with input from an expert committee. A 2-round modified e-Delphi study was conducted with 40 health economists with expertise in the Canadian context (response rates: 100% and 90%). Consensus was defined according to RAND criteria.

Results: The final Device Economic Evaluation Methodological Quality (DEEM-Q) checklist consists of 39 items, refined through 2 e-Delphi rounds in which most items reached consensus. Some items were merged, new items added, and several reworded for clarity. The checklist covers core economic evaluation domains from Canada's Drug Agency guidance (e.g., decision problem, comparators, perspective, time horizon, resource use and costs, modelling, and uncertainty) alongside device-relevant considerations such as limited evidence, learning curve effects, organizational impact, incremental innovation, and diversity. The DEEM-Q thereby reflects both established methodological standards and issues particularly relevant to medical device evaluations.

Conclusions: DEEM-Q is the first consensus-based checklist designed for assessing methodological quality in economic evaluations of medical devices. It provides a transparent framework to guide study design, assist reviewers and HTA bodies in appraisal, and improve the consistency and quality of evidence for health care decision-making.

PO10. Administrative Burden of Laboratory Notification Processes Among Family Physicians

Presenting author: Hayley Lawrence, University of British Columbia

Coauthors: Kate McCammon, Health Quality BC; Leanne Griffiths, Health Quality BC; Craig Mitton, University of British Columbia; Allison Muniak, Health Quality BC; Olivia L. Tseng, Centre for Clinical Epidemiology and Evaluation

Background: Administrative work is essential in medical practice, encompassing paperwork, documentation, and communication to support patient care. However, unnecessary duplicative tasks or overly complex processes can create an accumulated burden, which may lead to burnout among family physicians and constrain their capacity to deliver quality care. This study aimed to understand the impact of the laboratory notification process in British Columbia, Canada, which remains unclear.

Approach: We adopted a qualitative approach and conducted semistructured interviews with 11 family physicians in British Columbia from January to March 2025. Using thematic analysis and NVivo software, we inductively identified themes that describe the impacts and their potential causes.

Findings: We identified 5 themes: physician burnout, notification alert fatigue, delayed care, lack of clarity on action plan, and resource inefficiencies (funding and provider time). Notification alert fatigue is attributed to a high volume of laboratory test result notifications, including repeated and redundant test results. This contributed to cognitive overload, increasing the risk of error, while important findings were sometimes overlooked, resulting in delayed care. The “always-on” expectation to review results, even during vacations, contributes to physician burnout. Ambiguity around responsibility for follow-up created duplication and inefficiency. Unnecessary patient visits, redundant tests, fragmented digital systems, and wasted provider time reflected opportunities to improve the notification process. These inefficiencies disrupt family physicians' work-life balance, diminish job satisfaction, and contribute to burnout.

Conclusion: Findings highlight the impact of administrative burden on efficiency, patient safety, and family physician well-being. Improving the laboratory notification process could reduce burnout and improve care quality.

PO11. Estimating Health Utilities in Pediatric Intestinal Failure

Presenting author: Daniela Gattini, Hospital for Sick Children, University of Toronto

Coauthors: Vikram Raghu, University of Pittsburgh School of Medicine Children's Hospital of Pittsburgh; Varsha Lilman, The Hospital for Sick Children; Lisa Lakkis, University of Pittsburgh School of Medicine Children’s Hospital of Pittsburgh; Jessie Hulst, The Hospital for Sick Children; Yaron Avitzur, The Hospital for Sick Children; Wendy J. Ungar, The Hospital for Sick Children; Paul W. Wales, Cincinnati Children’s Hospital Medical Center

Background: Health utilities, a measure of health status required to conduct cost-utility analyses, are lacking in children with intestinal failure (IF). Current economic evaluations extrapolate utilities from adults. This study aims to estimate health utilities in children with IF on parenteral nutrition (PN) compared to children with IF that have achieved enteral autonomy (EA).

Methods: Children with IF were recruited from 2 large intestinal rehabilitation centres in North America. Preference-based instruments were administered by interview to 50 children on PN and 57 children who achieved EA. Proxy assessments were conducted in children aged 5 to 7 years, and self-assessments were conducted in children aged 8 to 17 years. Health utilities, ranging from 0 (death) to 1 (perfect health), were calculated using the Child Health Utility 9 dimensions questionnaire and the Health Utilities Index. Differences in mean utilities between PN and EA groups were assessed with a t test.

Results: For children aged 5 to 7 years, mean utilities on PN were 0.735 (standard deviation [SD] = 0.208), while those who achieved EA had mean utilities of 0.821 (SD = 0.169), P = 0.269. For children aged 8 to 17 years, mean utilities on PN were 0.788 (SD = 0.184), and mean utilities for EA were 0.838 (SD = 0.142), P = 0.326.

Conclusions: This multicentre study involving 107 children with IF provides the first estimation of pediatric health utilities. Estimated utilities for children with IF on PN are notably higher than published adult estimates (0.36 [SD = 0.35]), while utilities of children that achieved EA align with adult estimates (0.82 [0.22]). These findings are crucial for advancing economic evaluations in pediatric IF.

PO12. Drug Listing Outcomes in British Columbia Following CDA-AMC Recommendation and BC Drug Benefit Council

Presenting author: Catherine Hnatiuk, Eversana

Coauthors: Linda Tang, Kamalpreet Mann, Aditya Padmanabhan, Eversana

The British Columbia Drug Benefit Council (DBC) conducts drug reviews following reimbursement recommendations from Canada's Drug Agency (CDA-AMC). Subsequently, the British Columbia Ministry of Health (Ministry) makes listing decisions, in consideration of the DBC recommendation. This analysis aims to understand trends in DBC reviews, Ministry participation in pCPA negotiations, and listing outcomes in British Columbia. Reviews of sponsored non-oncology drugs from January 1, 2020, to May 15, 2025, were included if the recommendation was positive. Out of 167 CDA-AMC recommendations, 54 DBC recommendations were identified. The remaining files were excluded since they were not reviewed by DBC due to similarities to existing treatments (55), are awaiting DBC review (38), not reported (18), or withdrawn from the market (2). The majority of DBC recommendations were "Do not list at the submitted price" (63%; 34). DBC recommended "Do not list" and "Reimburse" in 28% (15) and 9% (5) of files, respectively. The Ministry participated in pCPA negotiations for 85% of "Do not list at submitted price" recommendations, 87% of "Do not list" recommendations, and 80% of "Reimburse" recommendations. Ultimately, 65% of drugs with a "Do not list at the submitted price" recommendation, 47% of drugs with a "Do not list" recommendation, and 60% of "Reimburse" recommendations are listed by BC Pharmacare. In conclusion, the most common DBC

recommendation is “Do not list at the submitted price.” The Ministry participated in pCPA negotiations for most files, regardless of DBC review outcome. The BC Pharmacare listing rate is lowest for drugs that have received a DBC recommendation of “Do not list.”

PO13. Drugs for Advanced Renal Cell Carcinoma: A Treatment Pattern Analysis

Presenting author: Winson Cheung, University of Calgary

Coauthors: Rebecca Mercer, Canadian Centre for Applied Research in Cancer Control; Reka Pataky, BC Cancer; Qi Guan, Ontario Health; Mariet Mathew Stephen, University of Calgary; Nicola Bai, BC Cancer; Katharina Forster, Ontario Health; Christie Farrer Rogers, University of Calgary; Samara Strub, Sanofi; Safiya Karim, Alberta Health Services; Steven Yip, Alberta Health Services; Louis de Léséleuc, Canada’s Drug Agency

Background: Renal cell carcinoma (RCC) is the most common type of kidney cancer and is often diagnosed at advanced stages, making treatment challenging. While recent clinical trials show the benefits of combining immune checkpoint inhibitors with VEGF inhibitors, real-world data on their use and treatment durations remain limited. Understanding these patterns is crucial for optimizing clinical guidelines and planning health care resources.

Methods: This study examined systemic treatment patterns for advanced RCC in 3 Canadian provinces: Ontario, Alberta, and British Columbia. We used population-based data from 2017 to 2023 to analyze treatment sequences, durations, and dosing for patients receiving publicly funded therapies.

Results: Among 2,224 patients, the average age at diagnosis was 67 years and the majority of patients were male. Since their introduction, combination ipilimumab plus nivolumab and combination axitinib plus pembrolizumab have dominated as the first treatment option (first-line treatment) for advanced RCC, almost entirely replacing the older drugs: pazopanib and sunitinib. The average duration of first-line treatment ranged from 10 to 13 months, while second- and third-line therapies were generally shorter, ranging from 6 to 8 months.

Discussion: These findings help illustrate how patients move through treatment lines, offering insights for funders on evolving therapy use. As new drugs enter the market, this data can inform budget planning and policy decisions. These results may also support conversations between funders and clinicians when new therapies become available. Finally, the results highlight the need for further research into the clinical and economic implications of ongoing shifts in the advanced RCC treatment landscape.

PO14. Polypharmacy Trends in GLP-1 and GIP RA Therapy: A Real-World Analysis from Canadian Claims

Presenting author: Tuhin Maity, McKesson Canada

Coauthors: Saina Sehatkar, Mandeep Singh Chopra, McKesson Canada

As evidence grows around the health benefits of glucagon-like peptide-1 (GLP-1) and glucose-dependent insulinotropic polypeptide (GIP) receptor agonist drugs, such as semaglutide (e.g., Ozempic, Wegovy) and tirzepatide (e.g., Mounjaro, Zepbound), their use across multiple indications continues to expand. However, the polypharmacy profiles and medication-use patterns associated with these therapies in the real world remain poorly understood. Exploring the complexity of real-world medication use is critical for stakeholders including patients, providers, and payers. It can inform patient support strategies, anticipate drug-drug interactions, guide value assessments, and improve access and outcomes. This study analyzed approximately 1 million accepted pharmacy claims in 2024–2025 related to GLP-1 and GIP receptor agonists across all Canadian provinces. Descriptive analyses were conducted to profile the patient population. Using unique patient identifiers, concurrent medication use was mapped to uncover polypharmacy patterns. These patterns were then examined in relation to patient demographics and the specific GLP-1 or GIP drug prescribed. To our knowledge, this is the first study of its kind using Canadian real-world claims data to explore polypharmacy in this therapeutic class. The approach presented here offers actionable insights that can support evidence-based decision-making across clinical, regulatory, and market access domains.

PO15. Clinical Meaningfulness in Context: Avoiding Misapplication for Better Decisions

Presenting author: Kayla Scippa, Johnson & Johnson

Coauthors: Sheryl Pease, Kobina Quansah, Peter Coyle, Charles Iaconangelo, Carol Jamieson, Eric Chan, Johnson & Johnson; James McGinley, Carrie Houts, Vector Psychometric Group

The use of meaningful within-person change (MWPC) values has brought the patient voice to the forefront of evidence interpretation, ensuring clinical study results communicate what matters to patients. These MWPC thresholds are crucial for understanding patient experience but are increasingly being misapplied to interpret the difference in average change values between treatment arms (between-group differences [BGD]). Applying an individual threshold to an aggregate value confuses 2 distinct concepts: what is meaningful to an individual patient versus what is the average comparative effectiveness of a treatment. Such misapplication risks distorting treatment effects and diminishing the evidentiary value of patient-reported outcomes (PRO) for reimbursement decision-making.

This poster aims to (a) define terminology related to clinical meaningfulness, (b) explain methodological approaches for deriving meaningful change thresholds, and (c) highlight the consequences of conflating MWPC with BGD. Anchor-based approaches, which link change scores to interpretable patient- or clinician-reported anchors and distribution-based approaches will be included. Recent international initiatives, like those from Setting International Standards in Analyzing PRO and Quality of Life Endpoints (SISAQOL) and evolving regulatory patient-focused drug development perspectives, will be summarized to provide context on best practices.

Results will highlight that MWPC thresholds appropriately characterize meaningful change at the individual patient level, while BGD provide insight into important average population-level effects. Treating these thresholds as interchangeable can lead to misestimation of benefit. Clear distinction and appropriate consideration of clinical meaningfulness is essential. Promoting consistent terminology and methodological rigour will improve interpretation of PROs and support patient-centred evidence-based reimbursement decision-making.

PO16. Examining Real-World Quality of Life at a Pilot Centre in Ontario: Feasibility and Equity Factors

Presenting author: Teresa Tsui, Canada's Drug Agency

Coauthors: Rebecca Mercer, Canadian Centre for Applied Research in Cancer Control; Elena Zhou, Sunnybrook Research Institute; Rahul Desai, Sunnybrook Research Institute; Shreya Chatterjee, Sunnybrook Research Institute; Curtis Yeung, Sunnybrook Research Institute; Eleanor Pullenayegum, The Hospital for Sick Children; Kelvin Chan, Sunnybrook Research Institute

Introduction: Health utilities (HU) that inform health technology assessments (HTA) in oncology are often derived from clinical trials from highly selected, socioeconomically advantaged populations, which may overestimate HUs in the real world. Routine collection of EQ-5D HUs supports real-world evaluations of publicly funded cancer treatments. There is limited real-world data examining which factors impact EQ-5D HUs in oncology.

Objectives: This Ontario-based study (1) prospectively measured real-world EQ-5D-3L HUs among patients receiving systemic cancer therapy, (2) evaluated the feasibility of routine EQ-5D-3L collection, and (3) examined how socioeconomic factors influence EQ-5D-3L HUs.

Methods: Patients starting publicly reimbursed systemic therapy at an Ontario Cancer Centre were invited to complete the EQ-5D-3L and demographic questionnaires. With a subset of patients, we conducted semistructured interviews and analyzed their content to identify key themes informing the feasibility of future implementation. We conducted multivariable linear regression to assess the association between demographic and socioeconomic variables with EQ-5D-3L HUs.

Results: Of 170 patients enrolled (May 2024 to February 2025), 94% would definitely or very likely complete future EQ-5D-3L questionnaires. Interview participants expressed satisfaction with the EQ-5D-3L and supported its routine collection. Baseline HUs were consistent with previous cancer studies. Factors associated with lower HUs independent of cancer site were low annual family income (< $29,000) and undisclosed income.

Implications: This study supports broader EQ-5D implementation using existing infrastructure. Our findings suggest the need to account for income in EQ-5D analyses, which is pivotal to HTAs. Future research can

investigate widespread EQ-5D collection across Ontario and additional equity-related associations with health-related quality of life in oncology.

PO17. Out-of-Pocket Costs for People With Tuberculosis Disease in Toronto, Canada: A Web-Based Survey

Presenting author: Lauren Ramsay, Canada's Drug Agency

Coauthors: Beate Sander, Health Systems and Policy Research Collaborative Centre, University Health Network; Ezinne Ndukwe, University of Toronto; Elizabeth Rea, Toronto Public Health; Andrea Ackery, University Health Network; Jane McNamee, University Health Network; Thanh Nguyen, University Health Network; Sarah Brode, University Health Network; Nawang Yanga, York University; Amrita Daftary, York University; Kelly O'Brien, University of Toronto; Yeva Sahakyan, University Health Network; Marian Hassan, University Health Network; Christopher Pease, University of Ottawa and The Ottawa Hospital

Background: In Ontario, despite publicly funded health care for people with tuberculosis (TB), the direct and indirect costs to individuals have not been well studied and may be substantial.

Objective: We assessed the economic and social impacts of TB among individuals undergoing treatment at specialized TB care centres in Toronto.

Methods: We evaluated patient-reported costs, work disruptions, and changes in household financial status. We conducted a cross-sectional web-based survey among adults receiving TB treatment at West Park Healthcare Centre and the Toronto Western Hospital TB clinic between April 2023 and April 2025. Respondents completed a modified version of the WHO Tuberculosis Patient Cost Survey, which collected data on out-of-pocket costs, work loss and social outcomes. We used descriptive statistics to summarize key findings.

Results: A total of 42 people with TB disease completed the survey (mean age = 36.7 years; 38% female). The mean cost of a TB-related appointment was $42.82 (SD = $47.89), with variation by treatment centre and transportation method to appointments. More than half (61.9%) of respondents reported missing work due to treatment, with a mean loss of 130 hours. Following diagnosis with TB, 28.6% of respondents experienced job loss, and 45.3% of respondents experienced a worsening of their household financial situation.

Conclusion: Despite publicly funded health care, people with TB face economic challenges. These findings will contribute to a deeper understanding of the financial challenges faced by people with TB and may be useful in informing public health policy.

PO18. Implementing Hospital-Based HTA Recommendations: A Retrospective Review of Barriers and Facilitators

Presenting author: Geneviève Asselin, HTA Unit, CHU de Québec-Université Laval

Coauthors: Sylvain L'Espérance, CHU de Québec-Université Laval; Marc Rhainds, HTA Unit, CHU de Québec-Université Laval

The implementation of evidence-based recommendations from health technology assessment (HTA) in clinical practice is complex and influenced by several factors, such as organizational, clinical, and human factors. A retrospective analysis was conducted to examine the level of implementation of recommendations issued between 2017 and 2023 by the HTA unit of a tertiary care university hospital.

A self-administered questionnaire, developed using Microsoft Forms, was emailed to clinicians and managers of clinical services and departments targeted by the HTA recommendations. The survey focused on the degree of implementation of the recommendations, the perceived benefits, the barriers encountered, and the support needed for their implementation.

Of the 49 HTA recommendations reviewed, 54% were fully implemented, 26% were in progress, and 6% were not implemented. Implementation success was often linked to strong team commitment, alignment with institutional priorities, and ease of integration into clinical workflows. A large majority of respondents (88%) reported barriers to implementing the recommendations, including human resource limitations, organizational constraints, equipment issues, and disruptions related to the COVID-19 pandemic. Additionally, some recommendations have been put on hold or deprioritized due to leadership transitions or financial concerns. Overall, 83% of respondents perceived positive impacts following implementation. However, impact measurements were documented in only 30% of implemented recommendations.

These findings highlight the importance of considering interdisciplinary collaboration, organizational issues, and financial and human support to facilitate the integration of HTA recommendations into clinical practice. Future efforts will focus on prioritizing initiatives with measurable outcomes and high potential for impact on patient health.

PO19. Insight Into Approvals, Marketing, and Pricing of Emerging and Innovative Medicines 2021–2025

Presenting author: Blake Wladyka, Patented Medicine Prices Review Board

Background: High-cost specialty medicines, which include biologics, orphan drugs, and cancer products, are increasingly dominating the global pharmaceutical drug landscape. Many of these medicines have treatment costs of more than $10,000 per year, yet many have limited evidence of therapeutic benefit. The objective of this presentation is to provide an overview of emerging and innovative medicine characteristics, access, and pricing.

Methods: This study explores the market entry dynamics of emerging and innovative medicines approved in Canada and internationally from 2021 to 2025. The analysis details the international availability, pricing, and sales of medicines within a 5-year time frame following first approval and monitors how these metrics compare year over year.

Results: On average, 52 new medicines have been approved each year internationally between 2021 and 2024. Approximately two-thirds of new medicines were submitted to Health Canada for approval, with a 90% success rate. With approximately one-third of new medicines recording sales by Q2 of 2025, Canada ranked 14th out of 31 Organisation for Economic Co-operation and Development countries. Medicines marketed in Canada accounted for most new medicine sales internationally. In recent years, more than half of new medicines approved internationally had annual treatment costs of more than CA$10,000, with a growing share reaching more than CA$1 million. Most of these high-cost drugs received an orphan designation from an international approval body.

Conclusion: The number of new medicines approved internationally is stable, with orphan designations and treatment costs increasing every year. Within the Organisation for Economic Co-operation and Development, Canada ranks in the upper half for access to new medicines.

PO20. The Value Vocabulary: An Analysis of Societal Terms in Canadian HTA Decision-Making

Presenting author: Jessica Moreira, Hoffmann-La Roche Limited

Coauthors: Simon Yunger, Roche Canada; Lori Yin, Hoffmann-La Roche Limited

Objectives: Health technology assessment (HTA) bodies increasingly consider societal impacts beyond direct medical outcomes and costs. Prompted by a recent Canada's Drug Agency (CDA-AMC) initiative to broaden its deliberative framework, this study analyzes the inclusion of societal considerations in HTA recommendations before the framework's expansion across all expert committees.

Methods: A qualitative and quantitative analysis was performed using NotebookLM and author review on the 79 CDA-AMC available recommendations issued in the year before the framework's expansion as of the search cut-off (August 31, 2025). Documents were systematically searched for 17 predefined societal impact terms (e.g., productivity, family spillover) and societal perspective in economic modelling, and the frequency and context of their use were analyzed.

Results: The analysis identified 78 distinct contextual mentions. The most frequent were practical burdens like health system impacts (22), family spillover (15), travel costs (14), productivity (7), and out-of-pocket costs (6), which were considered by all committees (pan-Canadian Oncology Drug Review Expert Review Committee, Canadian Drug Expert Committee, Formulary Management Expert Committee). Formal economic terms like human capital, friction method, and presenteeism were absent as part of their deliberations; however, the "societal perspective" as part of economic modelling was mentioned 20 times as part of 6 distinct recommendations.

Conclusion: Tangible societal burdens like health system strain and family well-being were qualitatively acknowledged by CDA-AMC committees; however, they were not consistently or formally considered in decision-making. The recent initiative to broaden the CDA-AMC deliberative framework is a critical step toward bridging this gap, presenting an opportunity to move from ad hoc discussion to a more systematic integration of societal value in HTA decisions.

PO21. The Patient-Reported Genetic Testing Utility InDEx (P-GUIDE): A Novel Measure of Personal Utility

Presenting author: Stephanie Luca, The Hospital for Sick Children

Coauthors: Robin Hayeems, The Hospital for Sick Children; Elise Poole, The Hospital for Sick Children; Daniel Assamad, The Hospital for Sick Children; Bowen Xiao, The Hospital for Sick Children; Wendy J. Ungar, The Hospital for Sick Children; P-GUIDE Study Team, The Hospital for Sick Children

Introduction: To inform patient-centred care in genomic medicine, it is critical to understand how patients value genetic testing. To this end, we developed the Patient-reported Genetic testing Utility InDEx (P-GUIDE) for pediatric clinical genetics.

Methods: Informed by the literature, experts, and patient partners, a 55-item draft of P-GUIDE was created. Interviews with parents of children who had genetic testing assessed item clarity and relevance. Clinical experts completed content validity surveys on draft 2. Two independent samples of parents completed prospective surveys for item reduction, using exploratory factor analysis (draft 3) and confirmatory factor analysis (draft 4). Performance characteristics of the final tool were assessed.

Results: Interviewees (n = 22) found most items to be relevant but to require modification. Clinical experts (n = 11) recommended further modifications. Exploratory factor analysis (n = 103) supported a 4-factor solution (Kaiser-Meyer-Olkin = 0.863; Bartlett's P < 0.001) and removed 9 items. Confirmatory factor analysis (n = 103) generated a final model of 19 items across 3 factors: (1) understanding and decision-making, (2) psychosocial benefit, and (3) psychosocial concern. The model demonstrated acceptable fit (posterior predictive P value = 0.05), root mean square error of approximation = 0.08 (90% CI, 0.073 to 0.097), comparative fit index = 0.91, Tucker-Lewis index = 0.90). Standardized factor loadings ranged from 0.63 to 0.88. P-GUIDE scores were positively correlated to published measures of utility, knowledge, empowerment, and positive emotion, demonstrating construct validity. Test-retest reliability was demonstrated (intraclass correlation coefficient = 0.86).

Conclusions: P-GUIDE is a 19-item measure of personal utility for pediatric clinical genetics, available for licensed use. Adaptations for various clinical settings are underway.

PO22. Analysis of the Manitoba Tomorrow Project Cohort With a Focus on Medication Utilization

Presenting author: Noor Breik, College of Community and Global Health, University of Manitoba, Manitoba Tomorrow Project, CancerCare Manitoba

Coauthors: Donna Turner, College of Community and Global Health, University of Manitoba, Manitoba Tomorrow Project, CancerCare Manitoba; Alyson Mahar, Queen's University, Sinclair Cancer Research Institute; Nathan Nickel, College of Community and Global Health, Manitoba Centre for Health Policy, University of Manitoba; Christine Leong, College of Pharmacy, University of Manitoba; Travis Hrubeniuk, Manitoba Tomorrow Project, CancerCare Manitoba, University of Manitoba

Introduction: Reliable, linked real-world data are required to advance medication utilization and pharmacoepidemiology research in cancer and chronic disease. Large cohort studies provide valuable evidence to support system-level decision-making. This study investigates medication utilization, health, and lifestyle data from the Manitoba Tomorrow Project (MTP), a population-based cohort of 10,000 Manitobans aged 30 to 74 years, aimed at building a platform to study the causes of cancer and chronic disease integrated within the national Canadian Partnership for Tomorrow's Health (CanPath) network.

Objectives: (1) Describe the MTP cohort's sociodemographic, health, behaviour, and lifestyle data; (2) link MTP diabetes medications — as a first initiative — with provincial drug and administrative health databases (AHDs); and (3) evaluate concordance and factors influencing agreement.

Methods: (1) Conduct descriptive analyses of baseline characteristics, comparing MTP with the Manitoba population and other CanPath cohorts; (2) analyze all self-reported prescription medications, followed by deterministic linkage of all types of diabetes drugs with AHDs; (3) assess the agreement across data sources, with attention to sociodemographic and health factors.

Results: Among 8,678 participants (mean age 53 years; 67% female; 78% urban), more than one-quarter reported 3 or more chronic conditions. The most common were arthritis (25%), hypertension (23%), cancer history (13%), and diabetes (7.4%), consistent with the Manitoba diabetes prevalence (7.6%). Two-thirds reported prescription medication use, with 28% taking 3 or more concurrent medications.

Next steps: Linking MTP diabetes medication and individual-level data to AHDs will position MTP as a reliable data source, support linked real-world evidence initiatives, and facilitate informed decision-making, thereby enhancing health outcomes.

PO23. Multistate Modelling to Estimate Attributable Health Care Costs Using Administrative Data

Presenting author: Kali Barrett, University of Toronto

Coauthors: John Peel, University of Toronto, Department of Anesthesia; Damon Scales, Sunnybrook Research Institute; Sarah Swayze, ICES; Kuan Liu, University of Toronto; Beate Sander, Health Systems and Policy Research Collaborative Centre, University Health Network

Accurately estimating the costs-of-illness in the presence of right censoring and incomplete follow-up remains a methodological challenge in health economics. A phase-of-care approach, where observed patient costs are allocated to disease-specific phases of care (e.g., active disease, remission, predeath), is commonly used to overcome these challenges. In this method, phases are typically fixed in length and defined by population-level averages of expected disease progression or statistical changepoint methods. These approaches have been successfully applied to studies using health administrative data, where transitions are often unobserved. However, when patient transitions are identifiable in data sources, fixed phase definitions may oversimplify trajectories, obscure heterogeneity in transition timing, and risk misclassifying periods of cost accumulation.

We introduce a novel methodology that uses individual-level data with observed “health state” transitions estimated using multistate transition modelling. Multistate models allow estimation of the probability of moving between clinically relevant states over time, accommodating recurrent and competing transitions and non-Markovian processes. We construct a transition matrix and apply the Landmark Aalen-Johansen estimator to derive phase occupancy probabilities across the observation period. Phase-specific attributable costs are estimated using matched comparator cohorts and modelled with generalized estimating equations. We have successfully used this methodology to estimate costs associated with lung transplantation and COVID-19 using hospital-level data and population-level health administrative records.

This approach directly links individual trajectories to population-level estimates, improving precision in cost attribution and enabling flexible subgroup analyses. It is particularly well-suited to diseases with observable transitions and offers broad applicability for cost-of-illness research.

PO24. Building Evidence for Sustainable CAR T Access: Costing Canadian Academic CAR T for Blood Cancers

Presenting author: Nora Abdelsamie, Ottawa Hospital Research Institute

Background: Chimeric antigen receptor (CAR) T-cell therapy has transformed treatment for hematologic malignancies but remains cost-prohibitive, largely due to centralized and complex manufacturing processes. To address these barriers, the Canadian-led Immunotherapies Collaborative (CLIC) developed an academic, point-of-care compatible manufacturing model.

Methods: We conducted a hybrid-costing study of 78 patients treated in the CLIC-01 trial at 2 Canadian academic hospitals. Costs were estimated from the product developer's perspective and expressed in 2024 Canadian dollars across preinfusion, infusion, and postinfusion phases. A Monte Carlo simulation (10,000 iterations) captured cost variability, and scenario analyses explored potential efficiencies. Standardized regression was conducted to identify cost drivers.

Results: The mean per-patient cost of CLIC CAR T-cell therapy was $155,535 (SD = $10,996). Most costs (65%) occurred in the preinfusion/manufacturing phase, driven by personnel requirements and quality control. Postinfusion monitoring (26%) was dominated by hospitalization ($17,920) and clinical care ($2,235), while infusion-related costs accounted for 9%. Scenario analyses suggested that procurement strategies and process optimization could reduce costs by more than $15,000 per patient. Regression analysis identified facility operations (environmental controls, equipment maintenance, staffing, and quality assurance) and manufacturing processes (T-cell activation, genetic modification, and cell expansion) as the primary drivers.

Conclusions: This is the first detailed costing study of academic CAR T-cell manufacturing in Canada. Our results can inform early-phase trial design, institutional planning, and investment decisions. These findings highlight the potential of point-of-care CAR T-cell therapy to improve sustainability and access to advanced therapies within the health care system in Canada, informing future health technology assessment and policy on cell-based therapeutics.

PO25. Utilizing Real-World Evidence to Advance Health Equity for Patients in Canada With HAE

Presenting author: Michelle Cooper, HAE Canada

Coauthors: Daphne Dumbrille, HAE Canada Inc.; Charbel Daniel, HAE Canada; Stacy Farquhar, HAE Canada; Patricia Quong, HAE Canada

Introduction: Hereditary angioedema (HAE) is a rare genetic disease characterized by painful, debilitating, and disfiguring swelling, which can be life-threatening when it affects the throat. There are 3 subtypes of HAE: HAE-C1 inhibitor types 1 and 2, which are characterized by low or dysfunctional C1, and HAE with normal C1 (HAE-nC1INH). Patients experience inequities due to missed and/or delayed diagnosis, limited specialist care, and variable treatment access across Canada and by HAE subtype, despite comprehensive Canadian and international guidelines for acute management, prophylaxis, and self-administration. Randomized controlled trials are often challenging in rare diseases and may not capture treatment access or quality of life. HAE Canada (HAEC), the national patient organization, highlights this gap to support advocacy for equitable access to current and emerging treatments.

Methods: Since 2017, HAEC has conducted regular comprehensive surveys of our membership. These surveys enabled us to obtain real-world evidence regarding members' HAE attacks, medication access/use, and quality of life. These data are used to prepare patient submissions for health technology assessments and to advocate for access to approved drugs by provincial payers.

Results: Over the past 9 years, HAEC data have contributed to the approval of 5 new therapies. Our data will demonstrate resulting improvements in treatment access and patient quality of life while highlighting the growing disparity for patients with HAE-nC1INH.

Conclusion: Advocacy informed by patient data has improved treatment access and quality of life for patients with HAE; however, greater consideration of real-world evidence is needed to guide decisions about treatment access for rarer HAE subtypes.

PO26. Do Cell Therapy Trial Protocols Provide Regulator-Recommended Preclinical Evidence?

Presenting author: Matthew Jeffers, University of Ottawa, Ottawa Hospital Research Institute

Objective: We evaluated the reporting of preclinical efficacy-related evidence within cell therapy clinical trial protocols based on elements recommended by regulatory agencies. Our aim was to identify potential gaps in reporting that may impact regulatory review and trial justification.

Methods: ClinicalTrials.gov was systematically searched for trials of cell therapy products with attached protocol documents. Data were extracted on trial characteristics and elements of preclinical efficacy evidence recommended by regulators. The proportion of protocols containing key regulator-recommended preclinical elements was calculated.

Results: From 532,208 registered trials in ClinicalTrials.gov (as of March 31, 2025), we identified 305 relevant cell therapy trials with available protocol documents. These trials were predominantly focused on cancer indications (64% of trials) and related products (e.g., T cells, tumour-infiltrating lymphocytes, natural killer cells). Other therapeutic areas and products were also represented in the dataset (e.g., mesenchymal stromal cells, 16% of trials; other tissue- or disease-specific cell types, 14% of trials). Preclinical evidence was used to support 64% of trials, with most preclinical evidence being used to support potential mechanisms of action (95% of trials), trial intervention parameters (60% of trials), and disease-modifying effects (57% of trials). Although most regulators recommend providing justifications for how preclinical model systems are relevant to humans, this information was present in only 23% of trial protocols.

Conclusion: Greater focus on clinical relevance of preclinical models, outcome measures, and intervention parameters could better align the presentation of preclinical evidence with regulator recommendations in cell therapy clinical trial protocols.

PO27. Aligning Evidence Synthesis Practices With the Estimands Framework: A Scoping Review

Presenting author: Shomoita Alam, Core Clinical Sciences Inc.

Coauthors: Jay Park, McMaster University; Nathaniel Dyrkton, Core Clinical Sciences; Yichen Yan, Core Clinical Sciences

Meta-analyses of randomized clinical trials are quantitative evidence synthesis methods that make up the backbone of health technology assessment (HTA). In alignment with the estimands framework outlined in the International Council for Harmonisation E9(R1) addendum, which has been adopted by Health Canada and other global regulatory agencies, Canada's Drug Agency recommends in its 2025 methods guidance that all comparative effectiveness evidence be reported using the estimands framework to improve consistency in HTA. Studies indicate that pooling evidence targeting different estimands, even for the same intervention, can yield misleading results. Limited methodological guidance exists for quantitative evidence synthesis with estimands.

Following the Joanna Briggs Institute Scoping Review Methodology guidance, we conducted a scoping review to map methodological considerations when conducting quantitative evidence synthesis within the estimands framework. We considered peer-reviewed publications and methodological guidance from HTA bodies addressing the terms "Estimand" and "Evidence Synthesis" or "Meta-Analysis." Text on methods considerations was extracted and summarized descriptively.

Our search yielded 5 peer-reviewed publications and 3 technical guidelines from Canada's Drug Agency, the International Council for Harmonisation, and the HTA Coordination Group. Articles discussed methodological concerns about potential bias introduced by pooling trials with differing strategies for intercurrent events or mismatched summary measures, and raised challenges related to determining the relevance of intercurrent events. Documents also discussed trade-offs between estimand concordance and network connectivity.

Although the estimands framework brings clarity around study objectives and analytical methods for individual trials, more work is necessary to understand its implications for quantitative evidence synthesis. Best practice recommendations are needed to guide quantitative evidence synthesis within the estimands framework.

PO28. Investigating the Potential for a Canadian Accelerated Pathway for All Drugs With a High Unmet Need

Presenting author: Allison Wills, 20Sense; Arif Mitha, 20Sense

Coauthor: Cathy Lau, independent

Background: In May 2025, Ontario announced a 3-year pilot project to shorten access timelines for select cancer drugs under Project Orbis. While Project Orbis offers 1 category of drugs for accelerated listing, Health Canada's existing expedited pathways Priority Review and Notice of Compliance with conditions (NOC/c) are longstanding mechanisms to accelerate approval for drugs with a high unmet need. This study aims to investigate the potential for a Canadian accelerated pathway for all drugs with a high unmet need, in the categories of Priority Review and NOC/c, including Project Orbis files, to determine the number of files this would apply to in a given year and the potential impact on time to listing, with an objective of understanding the potential to establish a more timely and equitable access framework for patients in Canada.

Methods: Drug submissions completed by Health Canada in 2022 were examined. For each, submission type (New Drug Submission, Supplemental New Drug Submission, Priority Review, NOC/c) and indication were recorded. Submissions were cross-checked with FDA Project Orbis files to identify Canadian participation. Publicly available Canada's Drug Agency records were reviewed to collect dates for submission acceptance, draft recommendation, and final recommendation. Publicly available pan-Canadian Pharmaceutical Alliance records were reviewed for matching indications, negotiation dates, and Canada's Drug Agency project numbers. Ontario formulary listing dates were obtained by contacting the Ontario Ministry of Health and pharmaceutical manufacturers, and from Ontario's Exceptional Access Program Frequently Requested Drugs list. Times from Health Canada approval to Ontario listing were then calculated.

Results and conclusions: Analysis is currently in progress and will be available for the Symposium in April 2026.

PO29. Externally Controlled Clinical Trials for Pediatric Rare Disease: Current Practices and Guidance

Presenting author: Gabrielle Pratt Tremblay, University of Ottawa

Coauthors: Emma Iverson, RareKids-CAN; Alex Bliu, Health Canada; Christopher Gravel, School of Epidemiology and Public Health, University of Ottawa; Beth Potter, School of Epidemiology and Public Health, University of Ottawa

Rationale: In an externally controlled trial (ECT), outcomes in participants receiving the investigational treatment are compared with those in an external untreated group, typically derived from real-world data sources. Although ECTs have important limitations, they are often considered acceptable when assigning participants to an internal control group is not feasible or ethical — for example, due to population size, disease severity, and lack of availability of alternative treatments. This study aims to describe and evaluate current practices in the design and conduct of ECTs in pediatric rare genetic diseases, with the goal of informing recommendations for improved evidence generation.

Methods: We are conducting a scoping review (registered protocol, DOI: 10.17605/OSF.IO/4MBRN), using Joanna Briggs Institute methodology, to identify recent ECTs evaluating treatments for rare pediatric genetic diseases and to describe their methodological approaches. We will compare these approaches with existing recommendations from regulatory and health technology assessment organizations to assess the extent to which current practice aligns with available guidance.

Results: To date, we have identified 12 recent ECTs evaluating treatments for pediatric rare genetic diseases. These trials employed external comparators either as benchmarks or for direct comparison and demonstrated considerable variation in design and analytic strategies used to mitigate potential sources of bias. Preliminary results suggest that many of these trials did not fully incorporate recommended approaches to align trial data with external control data.

Value: Understanding methodological gaps in ECTs that evaluate therapies for pediatric rare genetic diseases is essential to strengthening the evidence base that guides clinical practice and policy decisions.

PO30. Enhancing Transparency of the Drug Reimbursement Process: Publication of CDA-AMC Cost Table Tools

Presenting author: Morgan Karugaba, Canada's Drug Agency

Coauthor: Ian Cromwell, Canada's Drug Agency

Cost comparison tables serve as essential components of drug reimbursement processes by providing estimates of anticipated drug costs over a specific period (i.e., 21-day, 28-day or annually). To enhance understanding and improve alignment with external users of pharmacoeconomic review reports, the Health Economics team aims to publish a collection of cost table tools on the Canada's Drug Agency (CDA-AMC) website to demonstrate the methods used to determine drug acquisition costs during reimbursement evaluations. Currently, the cost tables available in published reports are unmodifiable. Additionally, they are based on public list prices that can change over time and do not reflect the confidentially negotiated prices. Publishing these new tools will provide health care decision-makers with options to input negotiated prices that accurately reflect the costs of the health care system. In alignment with the CDA-AMC guiding principle on integrity, this makes CDA-AMC methodology transparent by showcasing how the CDA-AMC estimates are derived.

Five cost table tools were selected based on their completeness, user-friendliness, and relevance to current reimbursement practices. These tools encompass psoriasis, lymphoma, prostate cancer, and melanoma cost tables. The tools provide standardized monthly or annual cost estimates for selected drugs and treatment regimens within specific disease indications. Depending on the disease indication, the tool accounts for patient weight, differing treatment durations, and different dosing types (i.e., body surface area, infusion, fixed dosing, and body weight dosing). The tools capture drug acquisition costs based on provincial drug formulary prices, exceptional access program list prices, or IQVIA Delta Price Advisor prices where applicable, excluding all other health care-related expenses.

PO31. Health Utility of Neuromuscular Disease: Evidence From the BIND Study

Presenting author: Alyssa Grant, Ottawa Hospital Research Institute

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Objectives: Health state utility values are essential for economic evaluations of interventions for neuromuscular diseases (NMDs), yet robust reference data remain scarce. This study aimed to (1) describe health utility among individuals with inherited and acquired NMDs, and (2) examine sociodemographic, clinical, and economic predictors of health utility.

Methods: We conducted a national cross-sectional survey of 1,454 included individuals with NMD or their caregivers registered with Muscular Dystrophy Canada. Participants completed the Health Utilities Index Mark 3 (HUI3; range: -0.36 to 1). For minors, parent-proxy responses were obtained using the Pediatric Quality of Life Inventory (PedsQL) 3.0 Neuromuscular Module (range: 0 to 100). Multivariable generalized linear models were used to identify predictors of health utility.

Results: The mean HUI3 score was 0.27 (SD = 0.31) among adults living with NMD. For minors, the mean PedsQL was 45.9 (SD = 18.7). Among adults, lower health utility scores were independently associated with longer disease duration, racialized minority status, lower education, being a student, unemployed or unable-to-work status, single or previously married status, worse financial toxicity and specific diagnoses (e.g., ataxias, spinal muscular atrophy, and other NMDs, including amyotrophic lateral sclerosis). For minors with NMD, disease group (e.g., autoimmune myopathies; other NMDs, including amyotrophic lateral sclerosis), financial toxicity, younger age (e.g., 0 to 5 years) and school absenteeism correlated with worse PedsQL scores ($P < 0.05$).

Conclusion: NMDs significantly impact health-related quality of life. These findings provide much-needed reference utility values for individuals with NMDs in Canada and highlight key sociodemographic and clinical predictors of poorer health outcomes.

PO. A Systematic Review of Canadian Cancer Cost-Effectiveness Analyses Studies

Presenting author: Michael Lebenbaum, Canadian Cancer Society

Coauthors: Manuela Vieira, Canadian Cancer Society; Sasha Frost, Canadian Cancer Society; Jeff Hoch, University of California, Davis; Joyce Cheng, Centre for Addiction and Mental Health; Malek Hannouf, Alberta Health Services

Background: The cost of pharmaceuticals has risen sharply, particularly for oncological products. While studies have examined incremental cost-effectiveness ratio (ICER) values and thresholds for cancer cost-effectiveness analyses (CEAs) globally, little research has examined CEAs of cancer in the Canadian context.

Methods: We extracted articles from the Tufts Cost-Effectiveness Registry (1983–2024). We compared the frequency of use of $50,000 per quality-adjusted life-year (QALY) and 100,000 per QALY or higher thresholds by 5 common disease classifications (i.e., International Classification of Diseases chapters), overall and across time (i.e., pre-2015, post-2015). We estimated the median ICER values and intervention costs by cancer diagnosis and by disease classifications, overall and across time.

Results: Among the disease classifications, cancers had the lowest use of the $50,000 per QALY threshold examined and the highest use (approximately 2 to 4 times) of the $100,000 per QALY or higher threshold. Hematological cancers ($85,711), followed by genital cancers ($58,896), had the highest ICERs. Respiratory and blood cancers incurred the highest intervention costs. Cancer intervention costs grew across time, peaking in 2020–2024, while they ranked midrange among disease classifications from 2010–2019. Cancers were the least cost-effective of the disease classifications from 2010–2014 and 2020–2024.

Discussion: The high and growing ICERs and intervention costs, particularly given the frequent use of $100,000 per QALY or higher thresholds, present a challenge for Canada's national drug review bodies as they must reconcile high costs with the demand for equitable and sustainable access to new cancer therapies. These findings also suggest the rising costs of oncological products may put increasing strain on patients with cancer.

Conflict of interest: Manu Veira, Sasha Frost, and Michael Lebenbaum are employed by the Canadian Cancer Society, which employs other individuals actively advocating on drug access and funding.

PO32. Affordability of an HIV Self-Testing Program Among High-Risk Populations in Canada

Presenting author: Lisa Masucci, Ottawa Hospital Research Institute

Coauthors: Sean Rourke, MAP Centre for Urban Health Solutions; Kristin McBain, Unity Health Toronto, MAP Centre for Urban Health Solutions; Kednapa Thavorn, Ottawa Hospital Research Institute

Background: As access to HIV testing in Canada remains limited, HIV self-testing (HIVST) presents a promising solution for early HIV detection. The I'm Ready program is a national, mail-based HIVST initiative targeting key high-risk populations to enhance accessibility. This program has been found to be cost-effective; however, its financial implications are unknown. This study evaluates the budget impact of this HIVST distribution strategy among high-risk populations in Canada.

Methods: We developed a spreadsheet-based budget impact model populated to compare the costs of an expanded care program (I'm Ready program plus usual care) to opportunistic testing at a physician's office using point-of-care testing (usual care). We modelled the impact of HIVST on the yearly testing costs over a 5-year time horizon (2025–2029). Inputs included HIV prevalence and incidence estimates, screening rates, and testing sensitivity and specificity. Program costs were obtained from the I'm Ready program; costs of usual care and follow-up screening visits were obtained from secondary data sources.

Results: The expanded care program resulted in 2,776 additional HIV positive cases diagnosed over 5 years (4,939 HIVST and 2,163 usual care). The program also led to an additional 7.7 million in screening costs over 5 years ($147,011,483 for the expanded care program compared to $139,289,018 for usual care).

Conclusions: HIV self-testing through the I'm Ready program substantially increases case detection among high-risk populations, with modest additional costs. These results can inform national HIV testing strategies and support system-level decisions on the integration of HIV self-testing into routine care.

PO33. Dragon Ambient eXperience (DAX) Copilot: Pediatrics Proof-of-Concept Study

Presenting author: Christina Honeywell, Children's Hospital of Eastern Ontario Research Institute

Coauthors: Jim King, Kathleen Pajer, Jennifer Gillert, Shifra Samber, Lynnette Lywinski, Amanda Helleman, Children's Hospital of Eastern Ontario Research Institute

Introduction: Physicians experience a high level of burnout, often related to clinical documentation issues. AI scribes may reduce these challenges.

Methods: Using a mixed-methods design, this 4-month proof-of-concept study was done with 26 pediatric clinicians to measure effects associated with DAX AI scribe usage. Seven provider work perception constructs were quantified in pre-post surveys — for example, burnout, documentation burden, and workload burden. In-depth work experience data were collected in interviews. Electronic health record signal data metrics tracked constructs such as time in notes and number of in-basket messages, collected 4 months before the study and during it. Family experiences were collected by survey or phone interview. Paired t tests were used for quantitative data, and thematic analysis was used for qualitative data.

Results: DAX use was associated with improvements in 6 out of 7 work perception constructs — for example, burnout, system usability, and task load (all P values < 0.05). DAX was also associated with 5 signal data improvements — for example, shorter progress notes and more provider-generated smart phrases. Based on 444 out of 1,456 patient/caregiver consultations, DAX appointments were positive or neutral. In-depth provider experience data reported reduced cognitive load, shorter documentation time, and improved note quality. Challenges included DAX recording time limit, missing nuanced details in complex cases, and some AI hallucinations, and DAX was not available in French.

Discussion: DAX holds potential for enhancing efficiency, reducing burnout, improving the burden of work and improving note quality, without negative effects on patient appointment experiences. While it is good to satisfactory for most patient appointments, we will discuss recommendations for improvements.

Diverse Perspectives: Patient and provider groups were diverse in race, ethnicity, cultural background, geographic area of residence, and primary language.

PO34. Optimisation du processus de repérage de la littérature à l'INESSS

Presenting author: Alison Wong, Institut national d'excellence en santé et services sociaux

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Introduction : Dans le cadre de ses mandats d'évaluation des médicaments et des technologies en santé, l'Institut national d'excellence en santé et en services sociaux (INESSS) a restructuré son processus de repérage de la littérature, pour mieux répondre aux besoins des professionnels.

Objectif : Mesurer l'impact du nouveau processus sur l'analyse des dossiers.

Méthodologie : Un sondage initial auprès des professionnels cliniques et pharmacoéconomiques a révélé le besoin d'améliorer le processus de repérage de la littérature. Une collaboration étroite entre professionnels et conseillers en informatique a permis de dégager des pistes d'amélioration. Ceci a mené à l'élaboration et l'implantation d'un formulaire de demande adapté, ce qui permet de personnaliser le repérage pour les besoins de chaque dossier. Un sondage post-implantation sera réalisé après 2 mois d'absence de changement du processus. Enfin, une comparaison entre les articles soumis par les fabricants et ceux utilisés dans les avis finaux permettra d'évaluer l'enrichissement des évaluations par le nouveau processus.

Évaluation de la pratique : Un sondage mixte (quantitatif et qualitatif) mesurera l'impact du nouveau processus sur la satisfaction des professionnels (clarté, pertinence, utilité). Les résultats seront comparés à l'ancien processus pour mesurer les gains en efficacité et en valeur ajoutée. Le nombre d'articles retenus dans l'avis final, identifié ou non par la recherche documentaire, sera comparé à ceux soumis par le fabricant.

Conclusion : Les résultats du post-sondage déterminera dans quelle mesure le nouveau processus répond aux besoins des professionnels, améliore la qualité des évaluations et enrichit les données sur lesquelles elles reposent.

PO35. Cost-Utility Analyses in New Therapeutic Areas: A Case Study Examining Geographic Atrophy in Canada

Presenting author: Ewa Wywial, Astellas Pharma Canada, Inc.

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Background: Cost-utility analyses (CUAs) are essential for informing reimbursement decisions in Canada. Conducting CUAs in new therapeutic areas is often challenging due to limited data, uncertainty in disease progression, choice of appropriate comparator(s), and lack of established modelling frameworks.

Objective: To summarize key challenges in conducting CUAs for drugs in new therapeutic areas, supported by geographic atrophy (GA), an ophthalmologic condition with no approved treatments in Canada, as an illustrative example.

Methods: A targeted literature review of CUAs in GA was conducted to synthesize common challenges.

Results: Two US-based CUAs were identified; neither was associated with health technology assessment submissions. Models were heterogeneous with respect to health state definitions, characterizing disease progression, comparator(s) included, and utility data sources. Challenges identified in GA include (1) limited clinical data: small/short-term trials limit precision and generalizability; (2) long-term extrapolation: requires assumptions about treatment durability and disease progression; given patient heterogeneity, these assumptions require more robust real-world data; (3) utility measurement: generic instruments may not capture disease-specific impacts, requiring mapping or alternative measures; (4) modelling approach: selecting appropriate structures and resource use assumptions is difficult without a defined standard of care; (5) comparator(s): choice of standard of care/appropriate comparator(s); and (6) broader value: quality-adjusted life-year–based approaches may miss caregiver burden, patient autonomy, and other societal effects.

Conclusions: CUAs in GA require flexible and innovative modelling, extensive data to develop robust natural history and posttreatment disease progression, extensive sensitivity analyses to address uncertainty, and early stakeholder engagement.

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