

Medical Devices for Rare Diseases



MEDEC

CANADA'S MEDICAL TECHNOLOGY COMPANIES
LES SOCIÉTÉS CANADIENNES DE TECHNOLOGIES MÉDICALES



INTRODUCTION

On September 2, 2016 the Ontario Government announced the establishment of a Rare Disease Working Group to identify how Ontario's health care system can better manage the treatment of rare diseases, including strengthening diagnosis and treatment for patients living with rare diseases and improving access to care.

MEDEC has produced this position paper on medical devices and rare diseases to provide input to the Ontario Rare Disease Working Group. Our intent is to highlight the need to include medical devices in the Working Group's report to Government. We have provided three recommendations for the Working Group to consider.

MEDEC POSITION

We encourage the Rare Disease Working Group (RDWG) to include medical devices that can better manage the treatment of rare diseases in their deliberations and recommendations. We encourage a focus on a) health technology assessment (HTA); b) reimbursement; and c) health system medical device diffusion and adoption.

OUR RECOMMENDATIONS

1. Health Technology Assessment

We recommend a tailored and transparent assessment system that takes into account the unique characteristics of rare diseases that would be in the best interests of all stakeholders. This Health Technology Assessment (HTA) would facilitate patient access, foster innovation and help publicly funded health plans make more informed reimbursement decisions.

2. Reimbursement

We recommend a reimbursement program designed for rare diseases that would provide coverage with evidence development, and therefore, patient access to medical devices developed for rare diseases.

3. Health System Medical Device Diffusion and Adoption

We recommend the creation of centers of excellence to provide the diffusion and adoption of medical devices for rare diseases. The centres of excellence would be networked to share information and expertise.

BACKGROUND

Medical Devices and Rare Diseases

Medical devices and rare diseases are not usually mentioned together in rare disease initiatives, articles or discussions – the usual focus is therapeutic drugs. For example, Health Canada is developing an orphan drug regulatory framework that seeks to encourage the development of orphan drugs (i.e., drugs for rare diseases) and increase the availability of these products on the Canadian market. There is no mention of a regulatory framework for medical devices to treat rare diseases.

The Ontario Ministry of Health has developed a framework for Drugs for Rare Diseases. This framework recognizes that an innovative approach is required for drugs for rare diseases, but the framework does not include medical devices.

Health Technology Assessment (HTA)

HTA is a multidisciplinary process that summarises information about the medical, social, economic and ethical issues related to the use of a health technology in a systematic, transparent, unbiased, robust manner. Its aim is to inform the formulation of safe, effective, health policies that are patient focused and seeks to achieve best value (source: <http://www.eunethta.eu>). HTAs can result in policy decisions for reimbursement of technologies. While HTA generally relies on a robust assessment of the clinical cost-effectiveness of a new technology, the clinical and economic evidence required for this purpose is often not available for rare diseases, partly because of the challenges related to the recruitment of patients to participate in investigational trials and partly because the economic evaluation results in a high cost per patient for treatment.

With respect to health technology assessment agencies, the Canadian Agency for Drugs and Technologies in Health (CADTH) conducts assessments on drugs, diagnostic tests, as well as medical, dental, and surgical devices and procedures. CADTH does not have an HTA process for assessing medical devices for rare diseases. They use their regular submission and review process. In Ontario, through Health Quality Ontario, the Ontario Health Technology Advisory Committee (OHTAC) assesses a wide range of health interventions, including diagnostic tests, medical devices, interventional and surgical procedures, health care programs and models of care. OHTAC does not have a rare disease HTA pathway; they use their regular submission and review process.

We recommend a tailored and transparent assessment system that takes into account the unique characteristics of rare diseases that would be in the best interest of all stakeholders. This HTA would facilitate patient access, foster innovation and help publicly funded health plans make more informed reimbursement decisions.

Reimbursement

Coverage of medical devices for rare diseases is more restricted than for standard conditions due to HTA assessments based on clinical and economic evidence. The main reason that health insurance plans are more reluctant to cover medical devices for rare diseases is because of the higher price-per-patient. While per-patient costs are higher, it should be noted that overall budget impact will generally be lower due to the number of patients with the rare disease being treated. In addition to their limited budget impact on health insurance plans, medical devices for rare diseases offer tangible economic benefits to the healthcare system. Medical devices for rare diseases can reduce overall health care expenditure by replacing or preventing costly health interventions and hospitalization. In addition, patients with rare diseases will realize a higher quality of life. One solution for reimbursement is to provide coverage with evidence development (CED). CED programs can address more than one type of uncertainty including: clinical benefit, value for money, adoption and diffusion, and affordability. In Ontario, OHTAC has significant experience with CED.

We recommend a reimbursement program designed for rare diseases that would provide coverage with evidence development, and therefore, patient access to medical devices developed for rare diseases.

Health System Medical Device Diffusion and Adoption

If the challenges presented by high HTA evidence standards for clinical and economic data and the resulting restricted access to medical devices can be addressed, the adoption of medical devices can still be challenging. One solution is to create centres of excellence for rare diseases where information, proper diagnosis and comprehensive and expert care can be provided to patients. Centres of excellence would be funded to provide rare disease program funding centred on activity based funding or quality-based procedures. It would also be useful for centres of excellence to be networked with similar centers of excellence in Canada and internationally to share information, expertise, education and training.

We recommend the creation of centers of excellence to provide the diffusion and adoption of medical devices for rare diseases. The centres of excellence would be networked to share information and expertise.

Examples of Medical Devices for Rare Diseases

(Source –US FDA Office of Orphan Products - List of CDRH Humanitarian Device Exemptions)

Medical Device Name	Device Description / Indication
Argus® II Retinal Prosthesis System	This epiretinal prosthesis is surgically implanted in and on the eye and includes an antenna, an electronics case, and an electrode array. The Argus® II Retinal Prosthesis System is intended for patients aged 25 years and older with bare or no light perception vision caused by advanced retinitis pigmentosa.
Reclaim™ Deep Brain Stimulation for Obsessive Compulsive Disorder (OCD) Therapy	This device is indicated for bilateral stimulation of the anterior limb of the internal capsule, AIC, as an adjunct to medications and as an alternative to anterior capsulotomy for treatment of chronic, severe, treatment-resistant obsessive compulsive disorder (OCD) in adult patients who have failed at least three selective serotonin reuptake inhibitors (SSRIs).
Impella RP System	This device is indicated for providing circulatory assistance for up to 14 days in pediatric or adult patients with a body surface area ≥ 1.5 m ² who develop acute right heart failure or decompensation following left ventricular assist device implantation, myocardial infarction, heart transplant, or open-heart surgery.
FENIX™ Contenance Restoration System	This device is indicated for the treatment of fecal incontinence in patients who are not candidates for or have previously failed conservative treatment and less invasive therapy options (e.g. bulking agents, radiofrequency ablation, sacral nerve stimulation).
PDGFRB FISH for Gleevec Eligibility in Myelodysplastic Syndrome / Myeloproliferative Disease (MDS / MPD)	This device is indicated for the qualitative detection of PDGFRB gene rearrangement from fresh bone marrow samples of patients with MDS/MPD with a high index of suspicion based on karyotyping showing a 5q31~33 anomaly. The PDGFRB FISH assay is indicated as an aid in the selection of MDS/MPD patients for whom Gleevec (imatinib mesylate) treatment is being considered. This assay is for professional use only and is to be performed at a single laboratory site.

ABOUT MEDEC

MEDEC is the national association representing the medical technology industry in Canada. Our members are committed to providing safe and innovative medical technologies that enhance patient care and advance patient outcomes. The medical technology industry in Canada employs over 35,000 Canadians in close to 1,500 corporate facilities, and has sales of nearly \$7 billion per annum. We are committed to ensuring that Canada has a strong and vibrant medical technology industry.



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405 The West Mall, Suite 900, Toronto, Ontario M9C 5J1
 T: 416-620-1915 F: 416-620-1595
 Toll-free: 1-866-58-MEDEC(63332)
 E: medec@medec.org www.medec.org