

# Proton Beam Therapy for Cancer in Children and Adults: Recommendation

## Final Recommendation

- Ontario Health, based on guidance from the Ontario Health Technology Advisory Committee, recommends publicly funding proton beam therapy for pediatric cancers and a subset of adult cancers requiring curative radiation therapy

## Rationale for the Recommendation

The Ontario Health Technology Advisory Committee has reviewed the findings of the health technology assessment<sup>1</sup> and determined that proton beam therapy may result in fewer treatment-related harms but similar overall survival and progression-free survival compared with conventional photon radiation therapy to treat children and adults with certain types of cancer.

There remains some uncertainty in the evidence. Committee members acknowledged the complexity and challenges of developing high-quality evidence from randomized trials and long-term observational studies that could resolve this uncertainty on long-term harms. The committee also acknowledged that results from ongoing randomized controlled trials for some adult cancers may be available in several years, and longer for observational studies with children. The committee also considered the lived experience of adults and parents of children with cancer requiring curative radiation therapy, including the financial challenges they currently face in accessing proton beam therapy out of the country.

While the committee acknowledged that proton beam therapy would require a significant financial investment, they also recognized that it would improve access for eligible patients in Ontario and that the cost per patient would be less than sending patients out of the country for treatment. There was consensus among the committee that the availability of proton beam therapy in Ontario should be contingent on its substitution for photon radiation therapy within the overall capacity planning for provincial radiation therapy.

In addition, the committee supported having proton beam therapy connected with a tertiary pediatric radiation centre and having Ontario Health (Cancer Care Ontario) prioritize a list of specified cancers eligible for proton beam therapy based on the evolving evidence.

## Decision Determinants for Proton Beam Therapy for Cancer in Children and Adults

Decision Criteria	Subcriteria	Decision Determinants Considerations
<b>Overall clinical benefit</b> How likely is the health technology/intervention to result in high, moderate, or low overall benefit?	<b>Effectiveness</b> How effective is the health technology/intervention likely to be (taking into account any variability)?	Proton beam therapy may result in similar overall survival and progression-free survival compared with photon radiation in children and adults with certain types of cancer (GRADE: Moderate to Very low).
	<b>Safety</b> How safe is the health technology/intervention likely to be?	Proton beam therapy may result in fewer adverse events than photon radiation in some cancers in children and adults (GRADE: Moderate to Very low).
	<b>Burden of illness</b> What is the likely size of the burden of illness pertaining to this health technology/intervention?	Late toxicities and secondary tumours impose a significant burden for cancer survivors, especially children and young adults. According to the Childhood Cancer Survivor Study, <sup>2</sup> the 30-y cumulative incidence for severe, disabling, or life-threatening conditions or death due to a chronic condition following cancer treatment was 42%. Radiation treatment is given daily over 4–6 wk, increasing the burden of illness.
	<b>Need</b> How large is the need for this health technology/intervention?	Approximately 1,627 children and adults with 13 major cancers/tumour conditions could benefit from proton beam therapy in Ontario each year.
<b>Patient preferences and values</b> How likely is adoption of the health technology/intervention to be congruent with patient preferences and values and with ethical or legal standards?	<b>Patient preferences and values</b> Do patients have specific preferences, values, or needs related to the health condition, health technology/intervention, or life impact that are relevant to this assessment? (Note: The preferences and values of family members and informal caregivers are to be considered as appropriate.)	Patients and families report support for proton beam therapy in Ontario, valuing the opportunity to receive effective treatment close to family and other emotional supports.
	<b>Autonomy, privacy, confidentiality, and/or other relevant ethical principles as applicable</b> Are there concerns regarding	To support patient autonomy and independence in decision-making, information about all treatment options should be presented to patients and families. Responsible planning and

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	<p>accepted ethical or legal standards related to patient autonomy, privacy, confidentiality, or other ethical principles that are relevant to this assessment? (Note: The preferences and values of the public are to be considered as appropriate.)</p>	<p>management of health resources is aligned with the ethical principle of resource stewardship.</p>
<p><b>Equity and patient care</b> How could the health technology/ intervention affect equity of access and coordination of patient care?</p>	<p><b>Equity of access or outcomes</b> Are there disadvantaged populations or populations in need whose access to care or health outcomes might be improved or worsened that are relevant to this assessment?</p> <p><b>Patient care</b> Are there challenges in the coordination of care for patients or other system-level aspects of patient care (e.g., timeliness of care, care setting) that might be improved or worsened that are relevant to this assessment?</p>	<p>Current access to proton beam therapy outside the province, through Ontario’s Out-of-Country Prior Approval Program, can present patients and families with financial challenges related to travel, meals, and accommodation. It can mean being away from work for up to 6 wk, adding to the economic burden of treatment. For eligible patients who cannot afford the costs of out-of-country care, access to proton beam therapy would improve if this treatment were available in Ontario. Health outcomes for these patients may also improve.</p> <p>Under the Out-of-Country Prior Approval Program, patients must currently travel to the United States and stay there for several weeks to receive care. Some patients are too sick to travel or require immediate treatment, which precludes travelling or waiting for out-of-country treatment. For these patients, timeliness to treatment would improve if proton beam therapy were available in Ontario.</p>
<p><b>Cost-effectiveness</b> How efficient is the health technology/ intervention likely to be?</p>	<p><b>Economic evaluation</b> How efficient is the health technology/intervention likely to be?</p>	<p>We did not identify any published cost-effectiveness analyses directly applicable to our research question. Further, we did not conduct a primary economic evaluation because of the uncertainty in the clinical evidence. Existing clinical evidence may not reflect treatment with the latest advancements in proton beam therapy, which could provide some improvement in clinical outcomes. Therefore, the cost-effectiveness of proton beam therapy in Ontario is unknown.</p>

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<p><b>Feasibility of adoption into health system</b> How feasible is it to adopt the health technology/intervention into the Ontario health care system?</p>	<p><b>Economic feasibility</b> How economically feasible is the health technology/intervention?</p>	<p>The average cost to provide proton beam therapy through the Out-of-Country Prior Approval Program is approximately \$326,800 CAD per patient. To treat patients in Ontario, we estimate the average cost per patient would be reduced to between \$40,028 and \$48,217. Over the next 5 y, we estimate the cost of building and operating a 4-room proton beam therapy centre in an existing hospital in Ontario would be \$127.8 million for capital (device and construction) costs and between \$2.4 million and \$15.6 million per year for operating costs. The total 5-y budget impact would be \$124.8 million. The cost of building and operating a 1-room proton beam therapy centre in an existing hospital in Ontario would be approximately \$32.4 million in capital (device and construction) costs and between \$2.4 million and \$4.8 million per year for operating costs. The total budget impact would be an additional \$20.5 million over 5 y. If we assume building proton beam therapy centres will replace building new photon radiation therapy centres, then the 5-y budget impact could be reduced to approximately \$13 million (1 room) or \$94.8 million (4 rooms).</p>
<p><b>Organizational feasibility</b> How organizationally feasible is it to implement the health technology/intervention?</p>	<p><b>Organizational feasibility</b> How organizationally feasible is it to implement the health technology/intervention?</p>	<p>At the time of this analysis, proton beam therapy is not available within Ontario. Current access is through the Ministry of Health Out-of-Country Prior Approval Program on a case-by-case basis. Implementing proton beam therapy in Ontario will involve building dedicated physical space for the centre, installing new large-scale equipment, and training medical personnel (e.g., radiation therapists, nurses, radiation oncologists, medical physicists) to safely operate treatment rooms.</p>

Abbreviations: CAD Canadian dollar; GRADE Grading of Recommendations Assessment, Development, and Evaluation; wk, week; y, year.

## References

- (1) Ontario Health. Proton beam therapy for cancer in children and adults: a health technology assessment. Ont Health Technol Assess Ser [Internet]. 2021 May;21(1):1–142. Available from: <https://www.hqontario.ca/evidence-to-improve-care/health-technology-assessment/reviews-and-recommendations/proton-beam-therapy-for-cancer-in-children-and-adults>
- (2) Oeffinger KC, Mertens AC, Sklar CA, Kawashima T, Hudson MM, Meadows AT, et al. Chronic health conditions in adult survivors of childhood cancer. N Engl J Med. 2006;355(15):1572-82.

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