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Budget Impact Analysis for Proton Beam Therapy for pediatric population in Poland

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Abstract

Objective: Proton beam therapy (PBT) is increasingly used as an alternative radiotherapy for cancer. It is often used in cases where the position of the tumor in relation to critical structures, sensitive to radiation, significantly limits or prevents the use of classical radiotherapy. Use of the PBT minimizes long-term negative effects of radiotherapy, which is especially important in the treatment of children. The aim of the analysis was to assess the budget impact of using PBT from perspective of public payer in Poland.

Methods: The analysis was carried out in a 3-year time horizon. Cost data reflect the estimated costs incurred by the public payer (NHF) in providing health benefits. The estimation of the financial consequences was based on the Polish current tariffs for the included health benefits. Sources of data, including patient population, were opinions of clinical experts, scientific evidence, NHF.

Results: The analysis was carried out in the base, minimum and maximum scenario. In the base case 102 patients in 9 cancer indications were included. Total incremental costs were: €1.560 million, €1.769 million and €2.045 million in each year of analysis. There were no differences in the model parameters related to the costs of health services. Costs in the minimum scenario were approximately 30% lower and in the maximum scenario 10% higher than in the base case within 3 years.

Conclusion: PBT generated additional cost for treatment of pediatric populations with 9 oncological indications for public payer in Poland. Our findings can be used by decision makers in Poland.

INTRODUCTION

Proton beam therapy (PBT) is a relatively new technique of radiation therapy that uses high-energy proton beams to irradiate neoplastic lesions. It is particularly beneficial in cases where the position of the tumor in relation to critical structures, sensitive to radiation, significantly limits or prevents the use of classical radiotherapy.[1] PBT reduces normal tissue toxicity due to better local control of the radiation dose over the target areas. Despite its high costs many studies show that PBT is cost effective in terms of treatment complications and indirect costs.[2]

Currently, PBT is used in a very small number of cancers. Most countries use PBT in the pediatric population, where the incidence of radiation-related complications is relatively higher due to the longer life expectancy and the higher sensitivity of healthy tissues to radiation. In the adult population, PBT is used for large-scale cancers such as lung or breast cancer. However, the list of indications differs from country to country. In European countries, coverage decisions are most often taken at the national level.[3]

By the end of 2019, approximately 222,425 people worldwide were being treated with PBT. Currently, hospital proton irradiation centers operate in 19 countries, including all G7 countries with publicly funded healthcare systems, except Canada.[4] By the end of 2021, 102 PBT centers were established around the world, including 29 centers in the EU.[5]

According to estimates the importance of PBT will increase due to growing number of cancer cases and the necessity to increase the radiotherapy safety profile. Currently tumors rank second on the list of the most frequent deaths in Poland and Europe. According to forecasts, by 2025 an increase in the incidence of cancer in Poland is expected from 310,000 up to 350,000.[2]

The aim of the study was to evaluate a budget impact of PBT from the public payer perspective in Poland.[5]

METHODS

The budget impact analysis (BIA) concerns the financial consequences of extending the use of PBT from the perspective of the Polish public payer (National Health Fund, NHF) in 9 oncological indications in pediatric population (table 1). The analysis was performed according to the recommendations for conducting BIA.[6,7,8] The financial consequences of introducing the proposed changes were presented as an incremental cost expressed as the difference in costs between the 'new' and 'existing' scenario.

The 'existing' scenario presents the estimated costs of the NHF for services in the field of intensity-modulated radiation therapy (IMRT) and stereotactic teleradiotherapy (RT) in the above-mentioned indications. Under this scenario PBT is not covered by the NHF.

The 'new' scenario presents the estimated costs of treatment with PBT as the basic RT option. Since not all patients eligible for PBT will undergo it, some of them will receive other forms of RT, i.e. IMRT or stereotactic RT. It is caused by: personal preferences of patients or their parents/guardians regarding the optimal form of radiotherapy, or geographic limitations related to access to PBT (only one PBT center in Poland).

Due to the limitations of our model and possible variability of some key input parameters the sensitivity analysis was also performed with the minimum and maximum scenarios.

The analysis was carried out in a 3-year time horizon. Cost data reflect the estimated costs incurred by the public payer in providing health benefits. The estimation of the financial consequences was based on the Polish current tariffs for the included health benefits.

All costs are presented in EUR using exchange rates as of June 23, 2022 of the National Bank of Poland (€1.00 = PLN 4.6590, £1.00 = PLN 5.4756, CAD 1.00 = PLN 3.4582, AUD 1.00 = PLN 3.0877). Amounts are shown in full values.

No ethics committee review was required since this research did not include human subject data. Individual patient level information was not used, and the research relies purely on published or simulated data.

Data sources

Sources of data included into analysis were: opinions of clinical experts, scientific evidence, NHF data on the valuation of accounting healthcare products.

Population

The patient population for each indication was estimated based on available epidemiological data and opinions of clinical experts (questionnaire and personal communication). The target population in both scenarios is equal due to: the same eligibility criteria for particular types of radiotherapy, and no patients meeting eligibility criteria only if the indications for PBT were extended.

The analysis of the impact on the payer's budget also assumes 10% annual increase in the target population compared to the previous year (base case). In the minimum and maximum scenario annual increase of population were 5 and 20% respectively. Estimations of the volume of the target population in the each year were presented in table 1.

Table 1. Estimation of the target pediatric population for particular indications included in the analysis

Indication		Number of patients Scenario: 'new' or 'existing'		
		Year 1	Year 2	Year 3
I	Craniopharyngiomas, condition after incomplete surgical treatment or inability of surgical treatment of the primary or recurrent tumor (C75.2)	10	11	13
II	Orbital sarcomas, condition after incomplete surgical treatment or inability of surgical treatment of the primary or recurrent tumor (C69.6)	6	7	8
III	Orbital lymphomas requiring consolidation radiotherapy in the course of oncological treatment (C69.6)	3	4	5
IV	Meningiomas of the brain and spinal cord, WHO stages I and II, condition after incomplete surgical treatment or inability of surgical treatment of the primary or recurrent tumor (C70.0; C70.1; C70.9)	3	4	5
V	Adenomas of the pituitary gland, condition after incomplete surgical treatment or the inability of surgical treatment of the primary or recurrent tumor (C75.1)	3	4	5
VI	Tumors of the external auditory canal and middle ear, condition after incomplete surgical treatment or inability of surgical treatment of the primary or recurrent tumor (C43.2; C30.1)	5	6	7
VII	Pediatric cancers where the optimal photon radiotherapy plan does not safely protect critical organs (various types of cancer)	30	33	37
VIII	Hodgkin and non-Hodgkin lymphoma that requires mediastinal irradiation (C30-C39)	20	22	25
IX	Malignant neoplasms of various histopathology originating from the nasal cavity, paranasal sinuses or pharynx, infiltrating the natural orifices and/or bones of the skull base (diagnosis based on the magnetic resonance imaging of the head and neck) (various types of cancer)	10	11	13
Total		90	102	118

Costs parameters

The BIA includes the costs related to the irradiation treatment itself, its planning and related hospitalization as well as treatment of adverse events. Table 2 presents detailed values concerning evaluation of health services related to radiotherapy in Poland by the NHF.

Table 2. Costs of the health procedures based on the NHF costs

Procedure	Value
Planning PBT	€6,440
PBT	€9,230
IMRT	€3,512
Stereotactic RT	€3,128
Treatment of AEs Grade 3/per day	€35
Treatment of AEs Grade 4/per day	€46
Hospitalization/per day	€117

AE – adverse event; IMRT – intensity-modulated radiation therapy; PBT – proton beam therapy; RT – radiation therapy

In order to correctly estimate the consequences of introducing the proposed changes, the analysis was based on variables determined on the basis of the opinions of clinical experts and scientific evidence. Key assumptions were presented in table 3. One of the important assumption was that in case of % of patients who received PBT

One of the key assumptions was the percentage of patients receiving PBT. In base case we assumed that 100% of children would receive PBT due to the specificity of the target population and the particular significant of clinical efficacy.

SENSITIVITY ANALYSIS

One-way sensitivity analysis was performed for all model inputs. Sensitivity analysis was presented as a minimum and maximum scenario.

RESULTS

Results of the base case

The budget impact results from the perspective of the Polish public payer in three years horizon were presented in table 4. We assumed that the entire pediatric population will be treated with the PBT in the base case. In the base case the total expenditures on treatment of all of the analyzed indications with the PBT were estimated to be €5.374m. over 3 years. What's important most costly component is irradiation therapy (cost of PBT is about 2.6 times higher than IMRT – this include only cost of irradiation without cost of planning or hospitalization).

Table 3. Parameters used in the analysis with their values

Parameter	Scenario		
	Minimum	Base	Maximum
Parameters related to radiation therapy			
Patients receiving PBT (%)	80	100	100
Patients receiving IMRT/stereotactic RT– 'new' scenario (%)	20	0	0
Patients receiving IMRT/stereotactic RT– 'existing' scenario (%)	100	100	100
Patients receiving IMRT in the group of patients receiving IMRT/stereotactic RT – 'new' and 'existing' scenario (%)	85	85	85
Patients receiving stereotactic RT in the group of patients receiving IMRT/ stereotactic RT – 'new' and 'existing' scenario (%)	15	15	15
Patients receiving PBT out of patients subject to planning procedure (%)	100	100	100
Parameters related to hospitalization during radiotherapy			
Patients hospitalized during PBT (%)	60	80	100
Patients hospitalized during IMRT/stereotactic RT (%)	20	40	60
Hospitalization time - therapy (days)	63	84	98
Parameters related to adverse events			
PBT patients with Grade 3 AEs (%)	10	13	15
PBT patients with Grade 4 AEs (%)	0	2	5
IMRT/stereotactic RT patients with Grade 3 AEs (%)	25	33	40
IMRT/stereotactic RT patients with Grade 4 AEs (%)	15	18	20
Hospitalization time PBT/IMRT/stereotactic RT – Grade 3 AEs (days)	15	23	30
Hospitalization time PBT/IMRT/stereotactic RT – Grade 3 AEs (days)	23	30	38
Parameters related to the necessity of repeated radiotherapy			
Patients that undergo repeated PBT (%)	5	10	10
Patients that undergo repeated IMRT/ stereotactic RT (%)	5	10	10

AE – adverse event; IMRT – intensity-modulated radiation therapy; PBT – proton beam therapy; RT – radiation therapy

Table 4. Results of the BIA – base case			
Indication	Year 1	Year 2	Year 3
'Existing' scenario			
I	€86,943	€95,637	€113,026
II	€52,166	€60,860	€69,554
III	€26,083	€34,777	€43,472
IV	€26,083	€34,777	€43,472
V	€26,083	€34,777	€43,472
VI	€43,472	€52,166	€60,860
VII	€260,829	€286,912	€321,689
VIII	€173,886	€191,275	€217,357
IX	€86,943	€95,637	€113,026
Total	€782,486	€886,818	€1,025,927
'New' scenario			
I	€260,293	€286,322	€338,381
II	€156,176	€182,205	€208,235
III	€78,088	€104,117	€130,147
IV	€78,088	€104,117	€130,147
V	€78,088	€104,117	€130,147
VI	€130,147	€156,176	€182,205
VII	€780,879	€858,967	€963,085
VIII	€520,586	€572,645	€650,733
IX	€260,293	€286,322	€338,381
Total	€2,342,638	€2,654,990	€3,071,459
Incremental cost			
I	€173,350	€190,685	€225,355
II	€104,010	€121,345	€138,680
III	€52,005	€69,340	€86,675
IV	€52,005	€69,340	€86,675
V	€52,005	€69,340	€86,675
VI	€86,675	€104,010	€121,345
VII	€520,051	€572,056	€641,396
VIII	€346,700	€381,370	€433,376
IX	€173,350	€190,685	€225,355
Total	€1,560,152	€1,768,172	€2,045,533

Results of the sensitivity analysis

Due to the limitations of our model and assumptions, a sensitivity analysis was performed in two scenarios: minimum and maximum.. There was no difference in the model parameters related to the costs of the health benefits. Result of the sensitivity analysis were presented in the table 5 as an incremental cost between scenarios. Results of the analysis suggest that the total cost in the minimum scenario is about 30% lower than total cost in base case. Comparing base case with maximum scenario we observed approximately 10% higher total cost over 3 years.

Table 5. Results of the sensitivity analysis – minimum and maximum scenario			
Indication	Year 1	Year 2	Year 3
Incremental cost – minimum scenario			
I	€125,327	€137,860	€150,392
II	€75,196	€87,729	€100,261
III	€37,598	€50,131	€62,663
IV	€37,598	€50,131	€62,663
V	€37,598	€50,131	€62,663
VI	€62,663	€75,196	€87,729
VII	€375,981	€401,046	€426,111
VIII	€250,654	€263,187	€288,252
IX	€125,327	€137,860	€150,392
Total	€1,127,942	€1,253,269	€1,391,128
Incremental cost – maximum scenario			
I	€178,988	€214,785	€268,482
II	€107,393	€143,190	€178,988
III	€53,696	€71,595	€89,494
IV	€53,696	€71,595	€89,494
V	€53,696	€71,595	€89,494
VI	€89,494	€107,393	€143,190
VII	€536,964	€644,356	€787,547
VIII	€357,976	€429,571	€519,065
IX	€178,988	€214,785	€268,482
Total	€1,610,891	€1,968,867	€2,434,235

Cost per patients

In order to better presentations and interpreting the results, we calculated the costs per patient. Based on the BIA we estimated the average cost per patient both for 'existing' and 'new' case – respectively €8,694 and €26,029. The average cost per patient is about 3 times higher for 'new' case in comparison to 'existing' case. The incremental average cost per patients in base case was €17,335. The average costs per patients were presented in in table 6.

Table 6. Estimations of average annual cos per patient	
Scenario	Average annual cos per patient
Minimum scenario	
'existing' scenario	€5,485
'new' scenario	€18,017
Incremental cost per patient	€12,533
Base case	
'existing' case	€8,694
'new' scenario	€26,029
Incremental cost per patient	€17,335
maximum scenario	
'existing' scenario	€12,218
'new' scenario	€30,117
Incremental cost per patient	€17,899

PBT – proton beam therapy

DISCUSSION

This study is the first economic analysis in Poland to show the real impact of the PBT for the treatment of pediatric patients with specific cancers. The base-case model of the current analysis revealed that the PBT would generate the additional cost each year for public payer in Polish settings. Furthermore annual additional costs for NHF (€1.5 million up to €2.0 million) were related to only 90-118 pediatric patients with nine cancer indications eligible to the treatment with the PBT. The main cost component was associated with the expenditure for indication VII and VIII – respectively: pediatric cancers where the optimal photon radiotherapy plan does not safely protect critical organs (various types of cancer) and Hodgkin and non-Hodgkin lymphoma that requires mediastinal irradiation.

According to the available data the average costs for treatment per patient (together for the adult and pediatric populations) for PBT were estimated around: €148,452 (Canada) €117,527 (United Kingdom) and €132,548-€185,567 (Australia).[10] The average costs are much higher than estimated cost in Poland. The above differences could be related to the treatment of other population (indications, numbers of patients) but also with other values of direct costs affecting the value of the total cost.

Additionally PBT is still growing technology around the world but with increasing role in the treatment of some cancer indications especially in pediatric populations. PBT provides the opportunity to deliver a therapeutic dose of radiation directly to the tumor with simultaneous protection of normal tissue, including critical structures such as: heart, brain. Thus it is recommended in certain cancer indications.[11, 12] However, due to both, high cost and lack of high quality evidence, further research is necessary

PBT in Poland is still under development. Currently only one center named: the Cyclotron Center at Bronowice (Kraków) – CCB – as a branch of the Institute of Nuclear Physics of the Polish Academy of Sciences in Kraków, is available for the patients. The treatment is conducted in cooperation with the University Hospital in Kraków or the National Institute of Oncology in Kraków.[13] PBT was performed in 559 patients (average per year 112 patients) in the years 2016-2020 based on the CCB data. Moreover more in years 2019 and 2020 the number of patients undergoing PBT is on the stabile level – around 146 patients per year. List of the cancers that can be treated with the PBT is finally established by the Ministry of Health. In 2016, the list was relatively narrow and included only seven indications. In 2019, the list of indications qualifying for PBT was increased by nine groups of neoplasms located outside the eye.[13]

Our BIA has both limitations and strengths. One of the limitations was the lack of data. Our model was strongly associated with clinical experts assumptions and lack of high quality clinical data. The BIA did not take into account possible differences in therapy (including: irradiation time, number of cycles, radiation dose received) between the individual indications included in the analysis. Another limitation of our analysis was associated with a small sample size in some indications. Above limitations can affect the uncertainty of cost estimates. Due to adopted short horizon of the analysis (3 years), we did not take into account adverse events that appeared at least 3 years after radiotherapy. The above approach was also adopted due to the limited clinical data from relatively low-quality studies with usually short follow-up periods. The cost of treating adverse events lower than grade 3 was also not taken into account. It should also be noted that the analysis does not take into account the maximum capacity of the Polish PBT center.

In the longer term, it is possible to estimate not only the costs of influencing the payer's budget resulting from the therapy itself, but also the costs related to depreciation. Additionally we can estimate costs from the patient's perspective e.g. travel costs, child's and guardian's accommodation; and from a social perspective e.g. indirect costs defined as costs of lost productivity of patients and their informal carers. The above described approach would make it possible to comprehensively estimate the total costs associated with introduction of PBT in pediatric population in Poland.

High cost of BIA for PBT in Poland could suggest that this technology is not cost saving for public payer but other analyses have revealed that the 5-year budget impact for four-room PBT center in Canada was about €92 million (cost per patient €35,790). Increasing the number the PBT rooms by one generated a lower budget impact of €11.3 million within the 5-year time horizon. If we assume building PBT centers would substitute for new photon therapy centers, then the 5-year budget impact could be further reduced to approximately €9.7 million (one room) or €70 million (four rooms).[4] Nevertheless above could suggested that PBT is potentially cost saving not only for a patient based on the clinical data but also for a payers based on the reduction of total cost in case of building PBT centers with a wide range of health services. Additionally, the PBT cost per patient is closely related to: number of patients treated, included cost (direct, indirect), tariffs.

CONCLUSIONS

PBT generated additional costs for treatment of pediatric populations with 9 oncological indications for public payer in Poland in 3-year time horizon. Our findings can be used by decision makers in Poland. High incremental costs of PBT are associated mostly with the small potential number of patient eligible for therapy with PBT.

HIGHLIGHTS

1. First comprehensive BIA of the PBT in the Polish setting.
2. Total expenditures on treatment with the PBT were estimated to be €5.374 million within 3 years.
3. Irradiation therapy occurred the most important cost driver.

AUTHORS' CONTRIBUTIONS:

Paweł Moćko, PhD and Radosław Rudź, PhD – study design and provide all calculations; all authors – assisted in writing and editing of manuscript, data check-up, critical revision and final approval.

CONFLICT OF INTERESTS:

The authors declare no conflicts of interest

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