

MEMORANDUM

TO: Members of the State Hospital Review and Planning Council

FROM: Thomas Jung
Acting Director, Division of Health Facility Planning

DATE: March 11, 2010

RE: Proton Beam Therapy

I. INTRODUCTION

This memorandum has been prepared by staff of the Department of Health at the request of the State Hospital Review and Planning Council (“SHRPC” or “the Council”). In anticipation of receiving one or more applications for the establishment of a proton beam therapy (PBT) facility in New York State, the SHRPC has been considering the process that should be deployed to evaluate such applications and the policy considerations that should drive the Council’s deliberations.

SHRPC members have raised a variety of issues in their discussion of PBT, including access to this service for New Yorkers, its efficacy and its effectiveness compared to other treatments, advantages and disadvantages of a facility operated by a consortium of hospitals, the legality of establishing a PBT facility without CON approval, and the cost of PBT relative to other treatments. This paper is intended to provide the information needed to inform the SHRPC’s discussion and to recommend a process for considering applications to establish a PBT facility.

II. BACKGROUND

PBT is an emerging form of radiation therapy that can maximize radiation doses to the target tumor, while sparing adjacent healthy tissue. PBT was introduced on an experimental basis in the 1950s, but was not approved as a radiation treatment option by the FDA until 1988, and then only for localized tumors.¹ In 1990, Loma Linda University opened the first hospital-based proton beam clinic in the United States, followed in 2001 by the Northeast Proton Therapy Center at Massachusetts General Hospital. Today, there are eight PBT facilities in operation in the U.S.

PBT has demonstrated efficacy for a number of relatively rare cancers – providing high rates of tumor control and survival, while reducing radiation-related side effects. Due to the physics of proton particles, protons enter the body, deposit most of their energy in the final portion of their trajectory and stop (this is known as the Bragg peak). There is no exit dose. As a result, exposure of normal tissue to radiation is minimized, and higher radiation doses can be administered to the tumor area. It is believed that PBT’s ability to

¹ U.S. Food and Drug Administration, Devices @ FDA, February 22, 1988.

maximize the dose and target it with high precision translates into better tumor control, fewer radiation-induced complications, and better outcomes overall than photon therapy. Definitive outcome studies comparing PBT to conventional electron beam therapy, however, are lacking.

III. INDICATIONS

Based on its precision, PBT is indicated for tumors that are not amenable to surgery or conventional forms of radiation, usually because they are located adjacent to critical tissues or structures and/or because they require comparatively high doses of radiation to provide sufficient cancer control. It is also used as an additional therapy after surgery for certain cancers. It is not recommended when cancer has metastasized.

PBT is indicated in the case of pediatric cancers, where local control is the main objective, but secondary cancers or disruption of pituitary, auditory, visual and intellectual functions as a result of radiation therapy are also of great concern. The increased dosing available through PBT may also reduce the number of treatments required, which is particularly beneficial to children who are often sedated during therapy and face the added risk associated with sedation at each radiotherapy session.

The CMS Medicare contractor serving New Jersey, Highmark Medicare Services, Inc., has issued a local coverage determination (LCD) that PBT is “medically reasonable and necessary” for the following conditions (the analogous LCD covering New York has been retired, and has not been replaced):

- Benign or malignant central nervous system tumors to include, but not limited to, primary and variant forms of astrocytoma, glioblastoma, medulloblastoma, acoustic neuroma, craniopharyngioma, benign and atypical meningioma, pineal gland tumors, and arteriovenous malformations;
- Intraocular melanomas;
- Pituitary neoplasms;
- Benign or malignant conditions of the base of the skull or axial skeleton including, but not limited to, chordomas and chondrosarcomas;
- Malignant lesions of the head and neck;
- Lung cancers, especially NSCLC;
- Unresectable retroperitoneal sarcoma and extremity sarcoma; or
- Solid tumors in children up to age 18.²

In addition, according to the LCD, PBT is indicated when:

- The Dose Volume Histogram (DVH) illustrates at least three (3) critical structures or organs protected by the use of Proton Beam Therapy;
- The dose to control or treat the tumor cannot be delivered without exceeding the tolerance of the normal tissue;

² LCD for Proton Beam Therapy, L30314, available at <http://www.cms.hhs.gov/mcd/overview.asp>.

- There is documented clinical rationale that doses generally thought to be above the level otherwise attainable with other radiation methods might improve control rates; or
- There is documented clinical rationale that higher levels of precision associated with Proton Beam Therapy compared to other radiation treatments are clinically necessary.³

The use of PBT for prostate cancer is the subject of lively debate among radiation oncologists, payors, consumers, and others in the field. A number of existing and planned PBT facilities in the United States emphasize the treatment of prostate cancer as it allows for high patient volume due to short treatment sessions. While prostate patients praise the outcomes achieved through PBT, the literature does not establish the benefits of PBT over other treatments for prostate cancer.⁴ According to the New Jersey MAC, “[t]here is as yet no good comparative data to determine whether or not Proton Beam Therapy for prostate cancer is superior, inferior, or equivalent to external beam radiation or brachytherapy in terms of safety or efficacy.”⁵

IV. PBT FACILITIES IN THE UNITED STATES

Currently, access by New Yorkers to PBT is quite limited. There are eight operating proton beam centers in the U.S., and two more are expected to open this year.⁶ We understand, anecdotally, that there are waiting lists at the Massachusetts and Pennsylvania facilities.

Currently Operating Proton Beam Facilities in the U.S., 2009

<i>Facility</i>	<i>Date of First Patient</i>	<i>Patients Treated</i>	<i>Dates of Count</i>
Loma Linda UMC	1990	11,414	11/06
Midwest Proton RI, Indiana	1993	379	12/07
University of California - San Francisco	1994	920	03/07
Northeast Proton T.C. - MGH	2001	2,710	10/07
M.D. Anderson, TX	2006	527	12/07
University of Florida, FPTI	2006	360	12/07
Procure Proton, T.C. - Oklahoma	2009	N/A	N/A
University of Pennsylvania	2009	N/A	N/A

Business ventures are beginning to develop proton beam facilities on a “turn-key” basis. One such venture, ProCure, is involved in developing a small facility, with a single gantry, two inclined beam rooms and a fixed beam room, in southern New Jersey, in collaboration with a radiation oncology practice and health system.

³ *Id.*

⁴ Levin, WP, Kooy, H, Loeffler, JS, Delaney, TF, “Proton Beam Therapy,” *British Journal of Cancer*, 2005.

⁵ *Id.*

⁶ Trikalinos, TA, Terasawa, T, Ip, S, Raman, G, Lau, J., “Particle Beam Radiation Therapies for Cancer,” Agency for Healthcare Research and Quality, Technical Brief No. 1, September, 2009.

V. OUTCOMES

Clinical studies of PBT have shown promising results with respect to tumor control and survival rates.⁷ The available evidence suggests that PBT is effective in treating ocular tumors, certain intra-cranial, skull base and cervical spine tumors, and intracranial arteriovenous malformations.

One review of the literature on PBT for ocular tumors described local control rates of over 95 percent, cause-specific survival rates of 85 percent, and eye preservation rates of 90 percent, although it also reported rates of “retained reasonable vision” at less than 50 percent.⁸ At least one study of PBT in treating ocular tumors resulted in eye retention rates for uveal melanoma after PBT approaching 100 percent at 5 years.⁹ Another study of high-risk patients with “extra-large” uveal melanomas who rejected a recommendation of enucleation (removal of the eye), reported at 24 months a 67 percent probability of local control and a 90 percent probability of metastases free survival, and a 54 percent probability of enucleation, with 25 percent of the patients reporting retained vision of 20/200 or better.¹⁰

A growing body of literature also reflects the positive results of PBT in treating the slow-growing bone tumors of the skull base -- chordomas, and chondrosarcomas. These tumors are difficult to treat surgically due to the risk of damage to the surrounding, normal, nerve tissue and the challenge of obtaining clear margins. Outcomes achieved in treating chondrosarcoma surpass those of chordoma. One study of PBT treatment and combined PBT-photon treatment in 30 children with chondrosarcoma or chordoma reported five-year survival rates of 100 percent and 81 percent respectively, with severe late toxicity in only one child.¹¹ A study of PBT for adults with chondrosarcoma or chordoma observed overall survival rates at three years of 94 percent.¹² A systematic review of studies concerning chordoma alone described five-year local control rates ranging from 46 to 73 percent and five-year survival rates from 67 percent to 81 percent.¹³

⁷ E.g., Levin WP, Kooy H, Loeffler JS, Delaney TF, “Proton Beam Therapy”, British Journal of Cancer, Sept. 2005. 93, 849-854; Gotein M, “Trials and tribulations in charged particle radiotherapy,” Radiotherapy and Oncology, June 2009; see also Trikalinos TA, et.al., supra note 6.

⁸ Brada, M, Pijlsp-Johannesma, DeRuyscher, D, “Current Clinical Evidence for Proton Therapy,” The Cancer Journal, July/August 2009.

⁹ Egger E, Zografos L, Schalenbourg A, et.al., “Eye Retention after Proton Beam Radiotherapy for Uveal Melanoma,” International Journal of Radiation Oncology, Biology, Physics, March 15,2003.

¹⁰ Conway, RM, Poothillil, AM, Daftari, IK, Weinberg, V, Chung, JE, O’Brian, JM, “Estimates of ocular and visual retention following treatment of extra-large uveal melanomas by proton beam radiotherapy,” Arch Ophthalmology, 2006.

¹¹ Habrand J., Schneider R., Alapetite C. et al., “Proton therapy in pediatric base and cervical canal low-grade bone malignancies,” International Journal Radiation Oncology Biology Physics, 2008.

¹² Weber DC et al, “Results of spot-scanning proton radiation therapy for chordoma and chondrosarcoma of the skull base: the Paul Scherrer Institut experience,” Int J Radiat Oncol Biol Phys, 2005; see also Nguyen, Q, Chang E, “Emerging role of proton beam radiation therapy for chordoma and chondrosarcoma of the skull base,” Curr. Onc. Rep., July 2008.

¹³ Amichetti M, Cianchetti M, Amelio D, et.al., “Proton Therapy in Chordoma of the Base of the Skull: a Systematic Review,” Neurosurgical Review, 2009.

PBT has achieved high rates of tumor control, reduced side effects, and improved survival for difficult or inoperable lesions of the brain and spinal cord, showing marked improvement since the early 1990s.¹⁴ In 1994, Seifert, et.al., cited disappointing results for the stereotactic proton beam treatment of cerebral arteriovenous malformations measuring larger than 3 cm.¹⁵ However, by 2007, studies showed favorable results for proton beam treatment of large AVMs. This includes complete obliteration, at three to five years after treatment, of 30 to 40 percent of the largest lesions (15 ml or greater) and of 67 to 76 percent of lesions measuring less than 15 ml.¹⁶

Proton beam therapy is also being used for cancers of the head and neck,¹⁷ including oral tumors,¹⁸ nasopharyngeal tumors,¹⁹ and lymphomas.²⁰ Although study populations are small, the results have been encouraging. It is also used for hepatic,²¹ pancreatic,²² prostate, and lung cancers²³ with promising trends toward increased survival in the hepatic trials. More recently, it is being utilized for breast cancer where the beam control can limit exposure of the heart and lungs. This is important, as significant mortality is associated with cardiac and vascular damage following radiation therapy.²⁴ Clinical trials involving the use of PBT (alone and in combination with other therapies) for these cancers and others are currently under way, with more than 40 registered at the U.S. National Cancer Institute alone.

¹⁴ Vemmimmen, FJ, Mohamed Z, Slabbert, JP, et.al., “Long-term results of Stereotactic Proton Beam Radiotherapy for Acoustic Neuromas, Radiotherapy and Oncology, Feb. 2009; and also Bush, DA, McAllister, CJ, Loreda, LN, et.al., “Fractionated Proton Beam Radiotherapy for Acoustic Neuromas, Neurosurgery, February 2002. 50 (2) 270-71; and also Gudjonsson O, Blomquist E, Nyberg G, “Stereotactic Irradiation of Skull Base Meningiomas with High Energy Protons”, Acta Neurochirurgica, 1999. 141 (9), 933-40; Boskos C, Feuvret L, Noel G, “Combined proton and photon conformal radiotherapy for intracranial atypical and malignant meningioma. Int. J. Radiation Oncology Biological Physics, 2009.

¹⁵ Seifert V, Stolke D, Mehdorn HM, Hoffman B, Clinical and Radiological Evaluation of Long-term Results of Stereotactic Proton Beam Radiosurgery in Patients with Cerebral Arteriovenous Malformations’, Journal of Neurosurgery, November 1994. 81 (5), 683-9.

¹⁶ Chen C, Chapman P, Petit J, Loeffler J, “Proton Radiosurgery in Neurosurgery”, Neurosurgical Focus, December 2007; see also Silander H, Pellettieri L, Enblad P, et.al., Fractionated, Stereotactic Proton Beam Treatment of Cerebral Arteriovenous Malformations”, Acta Neurologica Scandinavica, 2004.

¹⁷ Chan, AW and Liebsch NJ, “Proton Radiation Therapy for Head and Neck Cancer,” Journal of Surgical Oncology, 2008. 97, 697-700.

¹⁸ Slater JD, Yonemoto LT, Mantik DW, et.al., “Proton Radiation for Treatment of Cancer of the Oropharynx: Early Experience at Loma Linda University Medical Center Using a Concomitant Boost Technique,” International Journal of Radiation Oncology, Biology, Physics, June 1, 2005. 62(2), 494-500

¹⁹ Lin R, Slater JD, Yonemoto LT, et.al., ‘Nasopharyngeal Carcinoma: Repeat treatment with Conformal Proton Therapy – Dose-Volume Histogram Analysis’, Radiology, Nov. 1999.

²⁰<http://www.cancer.gov/clinicaltrials>, “Proton Therapy for Hodgkin Lymphoma”, University of Florida, January 2010

²¹ Nakayama H, Sugahara S, Tokita M, et.al., “Proton Beam Therapy for Hepatocellular Carcinoma: the University of Tsukuba Experience”,

²² Delaney TF, “Clinical Proton Radiation Therapy Research at the Francis H Burr Proton therapy Center”, Technology in Cancer Research and Treatment, Aug. 6, 2007. 6(4 suppl), 61-6.

²³ Nakayama H, Sugahara S, Tokita M, et.al., “Proton Beam Therapy for Patients with Medically Inoperable Stage I Non-Small-Cell Lung Cancer at the University of Tsukuba”, International Journal of Radiation Oncology, Biology, Physics. Jan.5, 2010

²⁴ Weber DC, Ares C, Lomax AJ and Kurtz JM, “Radiation Planning with Photons and Protons for Early and Advanced Breast Cancer: An Overview”, Radiation Oncology, 2008. 1(22).

VI. COMPARATIVE EFFECTIVENESS

Although PBT's ability to precisely target high doses of radiation is believed to result in improved outcomes over more conventional radiation therapy, relatively few clinical trials have compared the two forms of therapy.²⁵ The very limited availability of PBT worldwide and ethical considerations (related to clinical equipoise, or lack thereof, between PBT and other therapies) create real impediments to comparative trials.

AHRQ has released a technical brief surveying the literature on clinical outcomes and adverse events associated with PBT for treatment of cancer. Of 243 papers reviewed, only 17 were comparative, and only 8 were randomized. Of the randomized studies, none compared PBT alone with a conventional therapy. Most compared different types of PBT treatments (e.g., different doses or combinations of photon and proton therapies), rather than comparing charged particle treatments to treatments without charged particles. According to AHRQ, "no study found that charged particle radiotherapy is significantly better than alternative treatments with respect to patient-relevant clinical outcomes."²⁶

While clinical trials testing the efficacy of PBT in comparison with conventional therapies are lacking, systematic reviews of the literature comparing results achieved with conventional therapies to PBT have found evidence of PBT's superiority in treating certain cancers.²⁷ Although some reviewers concluded that, for most of the cancers studied, the benefits of PBT were marginal,²⁸ none found evidence that PBT is inferior. These reviews, of course, typically involve studies with non-comparable patient populations, follow-up periods, and clinical conditions, and therefore lack the scientific rigor of randomized controlled trials.

Some experts in the field maintain that clinical trials are unnecessary or not feasible, given ethical concerns, the size of the population appropriately eligible for PBT, and the limited availability of PBT.²⁹ A key concern is the presence or absence of "equipoise" -- i.e., whether or not PBT and conventional therapies are equally effective. If PBT is clearly preferred, a randomized, clinical trial would be unethical. Because PBT offers greater precision in beam administration and less radiation administration to surrounding structures, some clinicians maintain that PBT is preferred, and a clinical trial would not be ethical. Further, they contend that PBT is just an advanced form of a tested modality (radiation therapy) that, therefore, does not require additional randomized trials.

Others maintain that certain trials, including randomized trials, are both necessary and feasible. AHRQ and others recognize that for a small subset of cancers (e.g., uveal

²⁵ Trinkalinos, T, Terasawa, T, Ip, S, Raman, G, Lau, J, "Particle Beam Radiation Therapies for Cancer," Technical Brief No. 1, AHRQ, Nov. 2009; Gotein, M, "Trials and tribulations in charged particle radiotherapy," *Radiotherapy and Oncology*, 2009.

²⁶ Trikalinos, TA, et al., *supra* note 6.

²⁷ Amichetti, et al., *supra* note 13; Nguyen, et al., *supra* note 12; Trikalinos, TA, et al., *supra* note 6 (uveal melanoma); Brada, M, et al., *supra* note 8, (uveal melanoma).

²⁸ Brada, et al, *supra* note 8.

²⁹ See Gotein, M, "Trials and tribulations in charged particle radiotherapy," *Radiotherapy and Oncology*, 2009.

melanoma and pediatric cancers), PBT is preferred, and randomized clinical trials would be unethical.³⁰ Nevertheless, they also conclude that for many cancers and modalities, equipoise exists. In addition, AHRQ and others suggest alternative approaches to studying PBT, such as non-randomized, case-controlled studies performed prospectively or retrospectively.³¹

VII. PUBLIC NEED

The average annual number of new cancer cases in New York between 2003 and 2006 was 99,214.³² Of these new diagnoses, more than 13,000 cancer cases involved tumor sites that could benefit from the precision and higher doses offered by PBT:

*Tumor Sites Considered Suitable for Proton Beam Therapy
(Average Cases in NYS 2002-2006)*

<i>Site</i>	<i>Avg. Annual Cancer Cases</i>
Brain/CNS	3,481 ³³
Oral Cavity and Pharynx	2,005
Liver/bile duct	1,426
Pancreas	2,603
Larynx	826
Nasal cavity & sinuses	137
Trachea	35
Eye & Orbit	193
Pediatric (excluding leukemia)	700 ³⁴
Lung Cancer	2,721

Source: NYS Cancer Registry, Cancer Incidence and Mortality for All Sites of Cancer, NYS, 2002-2006

Since there are only eight PBT facilities in the country at this time, a PBT facility in New York would be expected to attract patients not only from the immediate vicinity, but also from the entire state, surrounding states, and around the world. At a minimum, a proton beam center in New York State would have a secondary service area in New Jersey and Connecticut. This would be expected even if the proposed facility in New Jersey becomes operational.

To the extent that the efficacy and comparative effectiveness of PBT in treating prostate cancer is established, the need for PBT would grow. On average, there are 14,576 new prostate cancer cases in New York each year.³⁵

³⁰ *Id.*; Trikalinos, T, et al, *supra* note 7; Brada, *supra* note 9.

³¹ *Id.*

³² NYS Cancer Registry, available at: <http://www.health.state.ny.us/statistics/cancer/registry/>

³³ This includes benign tumors.

³⁴ This figure includes cases counted in the other categories.

³⁵ NYS Cancer Registry, *supra* note 32.

It is difficult, if not impossible, to predict with precision how many of the non-prostate and prostate cases would, in fact, be appropriate for PBT, and how many of the eligible patients would opt for this therapy. However, assuming that a small fraction (e.g., 10 percent) of the cases in New York, New Jersey and Connecticut would be appropriate for PBT, there are more than enough cases to occupy fully even a large PBT facility with capacity to serve 1,500 patients annually.

VIII. CONSORTIUM MODEL AND GOVERNANCE

The Council has discussed the potential advantages and disadvantages of a consortium approach to the establishment of a PBT facility. Among the advantages are: (1) the ability to pool clinical, financial, and administrative resources in developing an expensive and complex facility; (2) the opportunity to collaborate in developing treatment protocols and clinical studies; and (3) the opportunity to concentrate patients in a single facility, in order to improve clinical experience through high patient volume, rather than dispersing patients to duplicative facilities.

Some Council members have raised concerns about the concept of a consortium. These concerns have focused on governance and administrative issues. Under the Department's regulations, a PBT facility established by a consortium would have to be controlled by a single operator that would be responsible for the management of the facility, the delivery of high quality health care, and compliance with state and federal regulations. The single operator would be owned and controlled by the participants in the consortium. In the event that the Department is presented with a PBT application that involves the establishment of a new operator owned by a consortium, the character and competence of the proposed operator would be assessed by the Department, SHRPC, and the Public Health Council, based on the qualifications of the participants. In addition, the governance structure and any administrative services agreements would be evaluated to prevent any improper delegations of authority.

While the consortium model does present some complexity, it is not inconsistent with state laws or regulations, and also offers the possibility of a more efficient use of resources and improved quality of care.

IX. COST AND REIMBURSEMENT

Based on the technology currently available, the development of a five-gantry PBT facility is estimated to cost about \$200 million. Smaller, lower cost equipment is under development. However, the more compact systems have not been approved by the FDA, and will not serve as many patients.

Medicare reimbursement for PBT varies considerably based on region, service delivery site, and the selected treatment and payment classification code. For PBT services delivered in a hospital outpatient department, the 2010 rate would be either \$942.31 (for APC 664 – Level I Proton Beam Radiation Therapy) or \$1,232.67 (for APC 667 – Level II Proton Beam Radiation Therapy). In free-standing facilities, the rates vary based on

determinations by the CMS contractors.³⁶ A review of PBT rates paid by three contractors for four different CPT codes disclosed rates ranging from \$406.03 to \$1,098.25.³⁷ While private payors do not publicize their provider reimbursement schedules, most pay for radiation therapy based on a percentage of the Medicare rates.

It is generally acknowledged that PBT cost and reimbursement exceed the cost and reimbursement for other types of radiation therapy, including intensity modulated radiation therapy with photons (IMRT).³⁸ However, it is difficult to find consistent information quantifying that cost differential. Estimates of the difference in reimbursement rates for PBT versus IMRT, for example, range from 40 percent to 100 percent. One study which used a simulation model to compare the cost-effectiveness of PBT to conventional radiation therapy for children with medulloblastoma concluded that PBT is cost-effective and saves money by reducing adverse effects and morbidity, such as hearing loss, IQ loss and growth hormone deficiency.³⁹ Clinicians who provide PBT indicate that total PBT costs per patient may come down as hypofractionation is refined, and the number of PBT sessions per case is reduced.

X. DEMONSTRATION PROJECTS UNDER 10 NYCRR PART 705

Regulations pursuant to Article 28 allow the Commissioner to authorize the operation of demonstration projects to evaluate the medical efficacy, cost-effectiveness, efficiency of, and public need for, new medical technologies. These rules (10 NYCRR Part 705) provide a basis for the approval of PBT technology in a manner that would help answer the questions of PBT efficacy, cost-effectiveness, access and need that have been raised in the SHRPC discussions of this emerging technology, as well as help assess effective financing mechanisms.

A. Notification of Potential Applicants

Part 705 states that once the Commissioner has determined that a new medical technology would be eligible for evaluation through a demonstration project, he will notify all medical facilities that provide a level and type of care and service for which the technology would be appropriate. The form of such notification is not specified but could take the form of a letter to hospital CEOs announcing the eligibility of PBT service for operation as a demonstration project under Part 705.

The notice would typically include a description of the requirements and deliverables associated with the demonstration project, such as engagement in research, reporting of

³⁶ Caron, J, "Ensuring Appropriate Incentives for Proton Beam Therapy: A Review of the Medicare Landscape," American Health Lawyers Association, Nov. 2009.

³⁷ Id.

³⁸ See Gotein, M, Jermann, M, "The Relative Costs of Proton and X-ray Radiation Therapy," Clinical Oncology, (2003).

³⁹ Lundkvist, J, Ekman, M, Ericsson S, Jonsson, B, Glimelius, B, "Cost-Effectiveness of Proton Radiation in the Treatment of Childhood Medulloblastoma," Cancer, 2005; see also Lundkvist, J, Ekman, M, Ericsson, S, Jonsson, B, Glimelius, B, "Proton therapy of cancer: Potential clinical advantages and cost-effectiveness," Acta Oncologica (2005).

service data to the Department, and providing access to Medicaid beneficiaries and uninsured individuals.

B. Application Process

Applications for demonstration projects under Part 705 are to be subject to full CON construction review, including review by the SHRPC and approval by the Commissioner. In addition, any project that would establish a new operator for the proposed technology would require establishment approval by the Public Health Council (e.g., any multi-hospital consortium or similar organization would be subject to PHC establishment review).

Prior to Council action, applications submitted under Part 705 are to be reviewed by the Commissioner and by a technical advisory group (appointed by the Commissioner) made up of an unspecified number of members with expertise in relevant areas of medicine, medical technology and the health care industry. For purposes of a PBT demonstration project, a technical advisory group might include at least one clinician with expertise in the delivery of PBT therapy, a consumer representative, an expert in health care administration, an expert in radiation safety and an expert in patient safety.

The Commissioner and the technical advisory group are to consider “pertinent factors” in the review of applications for demonstration projects, including but not limited to:

- (a) the extent to which an applicant's proposal meets the goals of the demonstration as set by the Commissioner;
- (b) the adequacy of the methodology proposed for the demonstration;
- (c) the ability of the proposed demonstration to collect data required for an analysis of the project;
- (d) the adequacy and appropriateness of the plan for organizing and carrying out the project;
- (e) the technical qualifications of the principal investigator and the proposed project staff;
- (f) the reasonableness of the proposed budget in relation to the proposed project;
- (g) the adequacy of the facility and resources available to the applicant;
- (h) where an application involves activities which could have an adverse health effect upon individuals participating in the demonstration, the adequacy of the proposed means for protecting against or minimizing such effects;

- (i) the relevance and status of any approvals required by the Federal Food and Drug Administration for the subject of the demonstration project; and
- (j) the number of applications to be approved.

C. Project Operation

Unlike projects involving services where criteria for need, operations and other features are prescribed in regulation or determined by precedent, the Part 705 demonstration status of a PBT project would give the Commissioner and the Councils considerable latitude in setting forth conditions and contingencies for the organization and operation of a PBT service. Approval of the project could therefore require that it be operated in a manner that would:

- Advance research into the efficacy, comparative effectiveness, and outcomes of PBT;
- Assess and address patient safety risks associated with PBT;
- Help ensure equitable access to PBT services, including by Medicaid beneficiaries and the uninsured;
- Assess arrangements for the financing, governance, organization and delivery of PBT services;
- Assist in determining an appropriate level of Medicaid reimbursement for this service.

D. Project Evaluation

The above factors would be addressed and analyzed through examination of data and other information in written progress reports submitted to the Department by the project operator every six months, as required by Part 705.

Following completion of the demonstration project, and if the medical efficacy, cost-effectiveness and safety of, and public need for, the technology are demonstrated to the satisfaction of the Commissioner and the Councils, the operator must apply for permanent certification pursuant to Article 28 CON requirements.

E. Proposed Modifications to Part 705

Part 705 currently restricts demonstration projects to a term of two years. While a two-year period may have been sufficient to assess the then-emerging MRI technology in 1983 when Part 705 was adopted, it is not sufficient to assess emerging technologies and treatments that require long term study due to the nature of the conditions treated, the time required to reach maximum capacity, the number of patients with particular conditions, and/or other factors. The duration of the demonstration project should be dependent upon the nature of the technology and its uses. The Department is proposing to remove the two-year limit and provide the Commissioner with discretion to determine the time limit for each project.

In addition, given that a considerable capital investment may be required for emerging technologies that may preclude their adoption by New York’s largely non-profit hospital sector, the Department proposes modifying Part 705 to allow for innovative financing mechanisms for new technology demonstration projects with a cost in excess of \$100 million.

XI. CERTIFICATE OF NEED REQUIREMENT FOR PBT FACILITIES

Questions have arisen concerning whether medical providers must be licensed pursuant to Article 28 of the Public Health Law to operate a PBT facility, or whether it is permissible for a private physician practice to operate a PBT facility without first obtaining certificate of need (CON) approval. Given the emerging nature of the therapy, the size and cost of a facility, and the necessary organization and management structure to operate and finance such a facility, it is difficult, if not impossible, to conceive of a PBT facility operated by a physician practice that would pass regulatory muster without CON approval. However, the regulations defining a diagnostic and treatment center (D&TC) subject to CON approval require a fact-based inquiry. Thus, any determination concerning the legality of an effort by a physician practice to establish and operate a proton beam facility without first securing CON approval would, of course, depend on the particular facts in question.

A. Identifying a “De Facto” D&TC

Under Article 28 of the Public Health Law, “hospitals” are required to obtain, through the CON process, establishment approval from the Public Health Council, and approval of the construction, acquisition or substantial alteration of their physical plant from the Commissioner (§§2801, 2801-a, 2802). The term “hospital” includes general hospitals, outpatient departments, diagnostic and treatment centers, nursing homes, and other facilities.

The Department’s regulations at 10 NYCRR §600.8 set forth a series of criteria that are intended to inform a determination as to whether an outpatient provider is operating as a physician practice or as a D&TC requiring CON approval and an operating certificate. These criteria include, among other factors:

- Patient contact is made directly with the facility rather than the individual physician; or referral is made to the facility by the physician; or provision is made for services by the physician, not in his [or her] offices but at another location.
- When the physician is not chosen by the patient, the physician is assigned by the facility, or the patient is given a choice among several practitioners associated with or employed by the facility.
- [C]entral services, including but not limited to laboratory, pharmacy, X-ray and narcotics are available with no free choice of the provider of such services by the patient . . .
- [T]he facility insures adherence to standards

- [T]he facility supplies ancillary services . . .
- [T]he responsibility of the facility terminates on discharge of the patient, as distinguished from the continuing responsibility of the physician; follow up care is not provided by or at the facility . . .
- Bills and charges are determined by the facility . . .
- [I]ncome distribution is determined by the facility . . .
- [E]mployees are selected and supervised by the facility . . .
- The structure is so physically extensive that it exceeds the usual space requirements of the private practitioner . . .
- [T]he departmental organization is large enough to require delegation of authority to nonmedical personnel . . .

10 NYCRR §600.8(c)(emphasis added). The regulations provide that the enumerated criteria are not intended to be the sole determining factors in resolving the status of an outpatient provider, but rather should be considered together with other relevant factors. Nor do all of the listed factors need to be present, in order for the Department to find a de facto D&TC. “Professional expertise is to be exercised in the utilization of the criteria.” 10 NYCRR §600.8(d).

Physical Structure and Equipment: Based on the technology currently available, the physical structure and equipment required to provide proton beam therapy clearly exceed the usual requirements of a physician practice. Existing proton beam facilities range in size from about 60,000 square feet to 100,000 square feet. These facilities require the installation of a cyclotron or synchrotron, which accelerates charged particles emitted from a radiation source. The accelerator typically produces protons with energy in the range of 70 to 300 MeV. The cyclotron weighs about 200 tons; the gantries can weigh about 100 tons each, are each about 3 stories high, and typically have wheels that measure 35 feet in diameter. The facility’s walls and ceilings range from 6 feet to 18 feet in thickness. Given the weight of the equipment and the thickness of the walls, specialized construction is required. The building foundation, support beams, and weight bearing structures demand specialized design and engineering to accommodate not only the equipment, but also the massive shielding structure needed to protect patients, staff and the public from the high energy radiation.

Organization and Management: A PBT facility is likely to satisfy the D&TC organization and management criteria set forth in section 600.8. The delivery of a highly complex form of radiation therapy will require a large multi-disciplinary medical staff, including anesthesiologists, radiologists, oncologists, dosimetrists, medical physicists, radiologic technologists, nurses, and pharmacists, as well as an extensive administrative staff.

Patient Relationships: With only eight facilities in the U.S. at present, patients will travel long distances for PBT services. Patients who have no connection to, or knowledge of, the facility’s physicians will be referred to the facility for its PBT services and will receive follow-up care closer to home. Further, central and ancillary services will be delivered by the facility without offering the patient any choice of provider.

While a physician affiliated with the facility will direct the treatment planning and prescribe the doses of radiation to be administered, he or she may not even be present when the radiation therapy is administered.

Other Considerations: The regulations at section 600.8 require the consideration of “other factors that may be pertinent in particular instances” in determining whether medical services are being provided by a facility that must be licensed as a D&TC. In the case of proton beam therapy, the Department would consider the emerging nature of the technology and therapy. Much remains to be learned about the efficacy of the treatment in treating a broad range of cancers, its effectiveness in comparison with more conventional therapies, optimal doses and combinations of treatments, risks posed to patients and health care workers and necessary measures to mitigate them, and the appropriate use of highly sensitive and complex equipment.

B. Remedies Available to Respond to a Scofflaw PBT Facility

The Public Health Law, Education Law, and associated regulations set forth a variety of penalties that may be imposed in the event that a physician practice operates a de facto D&TC without first securing CON approval. First, the physician(s), or the professional corporation, could be charged with professional misconduct pursuant to section 6530(16) of the Education Law. In addition, the Department could obtain an injunction requiring the facility to cease operations.⁴⁰ Under section 2801-c of the Public Health Law, any finding by the Commissioner is to be considered prima facie evidence of the facts in any action for injunction. Further, the Department could impose fines on the operator(s) of the scofflaw D&TC. Finally, any willful violation of the Public Health Law is punishable by a term of imprisonment of up to one year.⁴¹

CONCLUSION

PBT provides a promising approach for patients with cancers that lack satisfactory curative treatments. However, it is still in its infancy and continues to evolve. Improved patient positioning, the use of a stereotactic approach, intensity modulation, and pencil beam may further refine proton therapy. The use of PET scanning for treatment verification and adjustment of treatment plans may also enhance the utilization of proton beam therapy. The use of carbon ions is also under investigation.⁴²

Proponents and skeptics alike call for additional study of PBT, both to compare PBT to other treatments and to identify optimal doses, fractionation, or combinations of therapies, whether through randomized trials or alternatives such as case-controlled, non-randomized studies. Clearly, a critical factor in conducting studies is the development of additional PBT capacity, but cost considerations have hindered the establishment of new facilities.

⁴⁰ N.Y. Public Health Law §12.

⁴¹ N.Y. Public Health L. §12-b.

⁴² See Moya del Pino, B, “Proton Therapy for Cancer: A New Technology Brief,” National Cancer Institute Bulletin, Sept. 8, 2009.

As more compact, lower cost equipment is developed and approved by the FDA, capacity will ultimately grow. However, there is a risk that smaller entrepreneurial ventures will predominate and that those facilities will have neither the inclination nor capacity to conduct much-needed research, nor will they achieve the volume necessary to assure quality. There is also a risk that bottom-line pressures on these facilities will drive inappropriate use of PBT and/or inappropriate hypofractionation to increase patient through-put.⁴³ In addition, the use of lower cost equipment, involving lower energy or fixed beams (rather than gantries), may be inappropriate in a significant number of cases and result in less than optimal outcomes.⁴⁴

A demonstration project would provide an opportunity to control the entry of this emerging technology into the New York market. It would allow the Department to select an appropriate, qualified provider of PBT therapy, with the capacity to conduct rigorous research. The project would provide the information necessary to assess the safety and efficacy of this therapy and determine public need for these facilities in New York State. Through the demonstration project, New Yorkers with difficult-to-treat cancers would gain access to a treatment that has the potential to offer long term survival and a better quality of life.

⁴³ Gotein, supra note 7.

⁴⁴ Id.