

Embargoed until 1:45 p.m. ET, Sunday, September 22, 2013

Contact: Michelle Kirkwood
703-286-1600
michellek@astro.org

Nancy Mayes
Mayes Communications
703-772-2510
nancy@mayescommunications.com

Press Room in Atlanta
September 22-25
404-222-5303
404-222-5304

Proton therapy is a cost-effective treatment for pediatric brain tumor patients

Due to decreased side effects, it may also be cost-saving

Atlanta, September 22, 2013—Proton therapy, an external beam radiotherapy in which protons deliver precise radiation doses to a tumor and spare healthy organs and tissues, is cost-effective in treating medulloblastomas, fast-growing brain tumors that mainly affect children, when compared to standard photon radiation therapy, according to research presented today at the American Society for Radiation Oncology's (ASTRO's) 55th Annual Meeting.

The study used a first-order Monte Carlo simulation model to examine a population of 18-year old survivors of medulloblastoma brain tumors who were assumed to have been diagnosed at age 5 and at risk of developing 10 adverse health events, including various hormone deficiencies, coronary artery disease, congestive heart failure, ototoxicity, secondary malignant neoplasm and death. Primary institutional information on the cost of investment and Medicare data regarding the cost of management of the various adverse health conditions, in addition to peer-reviewed publications analyzing incidence of side effects were used in the simulation model to perform a cost-effectiveness analysis comparing proton and photon therapy from the societal perspective. Outcomes were

American Society for Radiation Oncology
8280 Willow Oaks Corporate Drive • Suite 500 • Fairfax • VA • 22031
phone 703-502-1550 • fax 703-502-7852 • Web www.astro.org

measured in incremental cost-effectiveness ratios, with costs measured in 2012 U.S. dollars (USD), and effectiveness measured in quality-adjusted life years (QALYs). A societal willingness-to-pay (WTP) threshold of \$50,000/QALY was the benchmark.

The clinical benefits of proton therapy have been recognized in reducing side effects when compared to photon therapy, but the significant expense of building and maintaining proton facilities and the high treatment costs have been areas of concern. The study's results demonstrate that by avoiding years of costly side effects, proton therapy can be cost-effective for children with medulloblastoma. Using current risk estimates and data on required capital investments, proton therapy for pediatric medulloblastoma treatment was not only cost-effective compared to standard photon radiation, but also found to be cost-saving in many simulations.

Results from the base case analysis showed that due to the prevention of side effects, proton therapy was cost-saving. In sensitivity analyses, proton therapy strongly remained the more appealing treatment, in part due to decreased risks of hearing loss, secondary malignancy and heart failure, resulting in cost-savings in more than 95 percent of simulations.

"We believed that proton therapy might prove to be cost-effective in treating pediatric brain tumors, and we were intrigued that it also proved to be cost-saving in the base case and in almost all of the sensitivity analysis simulations," said Raymond Mailhot Vega, MD, MPH, the presenting author of the study; a resident at Mount Auburn Hospital, the teaching hospital of Harvard Medical School; and a 2014 radiation oncology resident at New York University's Langone Medical Center. "Proton therapy might prove to be both cost-effective and cost-saving for other malignancies, too, and consequently, more cancer patients may benefit from proton therapy."

The abstract, "Cost-Effectiveness of Proton Therapy Compared to Photon Therapy in the Management of Pediatric Medulloblastoma," will be presented in detail during a scientific session at ASTRO's 55th Annual Meeting at 1:45 p.m. Eastern time on Sunday, September 23, 2013. To speak with Dr. MailhotVega, please call Michelle Kirkwood on September 22-25, 2013, in the ASTRO Press Office at the Georgia World Congress Center at 404-222-5303 or 404-222-5304, or email michellek@astro.org.

American Society for Radiation Oncology
8280 Willow Oaks Corporate Drive • Suite 500 • Fairfax • VA • 22031
phone 703-502-1550 • fax 703-502-7852 • Web www.astro.org

ASTRO's 55th Annual Meeting, held in Atlanta, September 22-25, 2013, is the premier scientific meeting in radiation oncology and brings together more than 11,000 attendees including oncologists from all disciplines, medical physicists, dosimetrists, radiation therapists, radiation oncology nurses and nurse practitioners, biologists, physician assistants, practice administrators, industry representatives and other health care professionals from around the world. The theme of the 2013 meeting is "Patients: Hope • Guide • Heal" and will focus on patient-centered care and the importance of the physician's role in improving patient-reported outcomes and the quality and safety of patient care. The four-day scientific meeting includes presentation of four plenary papers, 363 oral presentations, 1,460 posters and 144 digital posters in 70 educational sessions and scientific panels for 19 disease sites/tracks. Keynote speakers include: William B. Munier, MD, Director of the Center for Quality Improvement and Patient Safety at the Agency for Healthcare Research and Quality; Darrell G. Kirch, MD, President and CEO of the Association of American Medical Colleges; James Cosgrove, PhD, Director, the U.S. Government Accountability Office; Otis W. Brawley, MD, Chief Medical Officer of the American Cancer Society; and Peter Friedl, MD, PhD, of St. Radboud University Nijmegen Medical Centre at the University of Nijmegen and MD Anderson Cancer Center.

ABOUT ASTRO

ASTRO is the premier radiation oncology society in the world, with more than 10,000 members who are physicians, nurses, biologists, physicists, radiation therapists, dosimetrists and other health care professionals that specialize in treating patients with radiation therapies. As the leading organization in radiation oncology, the Society is dedicated to improving patient care through professional education and training, support for clinical practice and health policy standards, advancement of science and research, and advocacy. ASTRO publishes two medical journals, International Journal of Radiation Oncology • Biology • Physics (www.redjournal.org) and Practical Radiation Oncology (www.practicalradonc.org); developed and maintains an extensive patient website, www.rtanswers.org; and created the Radiation Oncology Institute (www.roinstitute.org), a non-profit foundation to support research and education efforts around the world that

American Society for Radiation Oncology
8280 Willow Oaks Corporate Drive • Suite 500 • Fairfax • VA • 22031
phone 703-502-1550 • fax 703-502-7852 • Web www.astro.org

enhance and confirm the critical role of radiation therapy in improving cancer treatment. To learn more about ASTRO, visit www.astro.org.

###

**2013 American Society for Radiation Oncology (ASTRO) 55th Annual Meeting
News Briefing, Tuesday, September 24, 2013, 2:45 p.m., Eastern time**

Clinical Trial Session: Sunday, September 22, 2013, 1:45 - 3:15 p.m., ET, Georgia World Congress Center

10 Cost-Effectiveness of Proton Therapy Compared to Photon Therapy in the Management of Pediatric Medulloblastoma

R. Mailhot Vega¹, J. Kim², M. Bussière³, J. Hattangadi⁴, A. Hollander⁵, J. Michalski⁶, N. Tarbell⁷, T. Yock⁷, S. MacDonald⁷,
¹Washington University in St. Louis School of Medicine, Saint Louis, MO, ²Harvard School of Public Health, Boston, MA,
³Massachusetts General Hospital, Boston, MA, ⁴University of California, San Diego, San Diego, CA, ⁵Washington University in Saint Louis School of Medicine, Saint Louis Children's Hospital, Saint Louis, MO, ⁶Washington University in St. Louis School of Medicine, Siteman Cancer Center, Saint Louis, MO, ⁷Harvard University School of Medicine, Massachusetts General Hospital, Boston, MA

Purpose/Objective(s): Proton therapy has been a hotly contested issue in both scientific publications and lay media. Proponents cite the modality's ability to spare healthy tissue, but critics claim the small benefit gained from its use does not validate its high cost compared to photon therapy. We aimed to evaluate the cost-effectiveness of proton therapy versus photon therapy in the management of pediatric medulloblastoma.

Materials/Methods: We performed a cost-effective analysis comparing proton and photon therapy from the societal perspective using a first-order Monte Carlo simulation model. We used a population of 18-year old survivors of pediatric medulloblastoma who were treated at age 5 and at risk of developing ten adverse events including growth hormone deficiency, hypothyroidism, gonadotropin deficiency, adrenocorticotrophic hormone deficiency, coronary artery disease, congestive heart failure, ototoxicity, secondary malignant neoplasm, and death. Costing data captured cost of investment and the diagnosis and management of adverse health states through use of primary institutional and Medicare data. Longitudinal outcomes data and recent modeling studies informed risk parameters for the model. With costs in 2012 USD and effectiveness measured in quality-adjusted life years (QALYs), incremental cost-effectiveness ratios were used to measure outcomes. We assumed a societal willingness-to-pay threshold (WTP) of \$50,000/QALY.

Results: Results from the base case analysis demonstrated that proton therapy was associated with higher QALYs and lower costs, and therefore dominated photon therapy. In one-way sensitivity analyses, proton therapy robustly remained the more attractive strategy, either dominating photon therapy or having a very low cost per QALY gained (<\$5,000/QALY). In the sensitivity analysis, risk of hearing loss, risk of secondary malignancy, and risk of heart failure were most influential on the incremental effectiveness of proton therapy. Cost of capital investment and risk of GHD were most influential on the incremental cost of therapy. These five parameters were selected for further testing in a probabilistic sensitivity analysis. PSA results illustrated domination of proton therapy over photon therapy in 96.4% of simulations and ICERs below WTP in 100% of simulations.

Conclusions: Using current risk estimates and data on required capital investments, proton therapy is a cost-effective strategy for the management of pediatric patients with medulloblastoma compared to standard of care photon therapy. The results of this analysis would be improved with further outcomes data.

Author Disclosure Block: R. Mailhot Vega: None. J. Kim: None. M. Bussière: None. J. Hattangadi: None. A. Hollander: None. J. Michalski: None. N. Tarbell: Q. Leadership; Spouse sits on the Medical Advisory Board of Procure. T. Yock: None. S. MacDonald: None.