



November 25, 2014

Re: Request to Review Appropriateness of Optune (Novocure) for Treatment of Glioblastoma

Dear National Comprehensive Cancer Network Central Nervous System Panel:

National Brain Tumor Society respectfully requests that National Comprehensive Cancer Network (NCCN) expedite review of Novocure's NovoTFF-100A system (now Optune) to determine whether there is sufficient evidence and consensus to declare it appropriate for the treatment of newly diagnosed in addition to recurrent glioblastoma multiforme (GBM) patients.

National Brain Tumor Society Advocates for Brain Tumor Patients To Have Access to Effective Treatments

National Brain Tumor Society is the largest nonprofit organization in the United States dedicated to the brain tumor community. We are fiercely committed to finding better treatments, and ultimately a cure, for people living with a brain tumor today and anyone who will be diagnosed tomorrow. Toward this end, we advocate for new treatments to be discovered, fully evaluated through well-controlled clinical trials, and ultimately for patients to have access to treatments that are both safe and effective.

Background

As you know, GBM is the most aggressive form of malignant brain tumor. There are approximately 10,000 patients diagnosed with glioblastoma each year.¹ There is no cure for glioblastoma and the five-year relative survival rate is only ~5%.² The current standard of care that includes a combination of temozolomide and radiation provides approximately fourteen months of survival. Patients want to live longer.

Until 2010, there were only 3 types of treatments available for GBM patients, including surgery, radiation, and chemotherapy. In April 2011, the U.S. Food and Drug Administration (FDA) granted marketing approval of NovoTFF-100A for the treatment of recurrent GBM.³ The FDA's approval of NovoTFF-100A marked the beginning of use of a medical device (using electric fields) for glioblastoma treatment. While some private health insurance companies cover the NovoTFF-100A device, the Centers for Medicare and Medicaid Services (CMS) does not.⁴ Lack of adequate health insurance coverage is one of the most well-documented barriers to cancer care.

¹ CBTRUS Statistical Report, Neuro Oncol (2014) 16 (suppl 4): iv1-iv63. doi: 10.1093/neuonc/nou223. http://neuro-oncology.oxfordjournals.org/content/16/suppl_4/iv1.full.pdf+html

² Ibid, Table 21.

³ FDA approval of NovoTFF-100A for treatment of recurrent GBM, http://www.accessdata.fda.gov/cdrh_docs/pdf10/p100034a.pdf

⁴ <http://www.medicarehnic.com/viewdoc.aspx?id=2518>

On November 15, 2014, phase III findings from the EF-14 clinical trial that investigated the efficacy of the NovoTFF-100A system in combination with standard of care temozolomide for the treatment of newly diagnosed GBM patients were reported at the Society for Neuro-Oncology conference. As principal investigator for the EF-14 trial, Dr. Roger Stupp reported that patients receiving the combination of NovoTFF-100A with temozolomide survived approximately 3 months longer than those receiving temozolomide alone.⁵ The trial's Independent Data Monitoring Committee recommended, on ethical grounds, that the trial be discontinued due to the efficacy findings.⁶ The official results of the EF-14 clinical trial have not been published. At this time we do not know if the FDA will grant approval of NovoTFF-100A for marketing for the treatment of newly diagnosed glioblastoma patients, as they did earlier for recurrence.

The NovoTFF-100A device (now marketed as Optune) is an expensive form of brain tumor treatment for many patients and their families. Anecdotal reports claim that that it can cost patients between \$15,000 to over \$20,000 per month. According to a durable medical equipment contractor for the Centers for Medicare Services (CMS), the NovoTFF-100A device will be denied (for coverage) as not reasonable and necessary.⁷

Brain Tumor Patients Deserve Access to Safe and Effective Treatments Without Delay

Based on the facts we know thus far, it is our understanding that Optune is considered to be a safe for the treatment for GBM patients. In addition, the device in combination with temozolomide has demonstrated prior to the publication of research results, effectiveness above the current standard of care. In light of the short survival rates of GBM patients, it is essential that treatments that demonstrate safety and effectiveness at prolonging survival be made available to patients as rapidly as possible.

Optune is not a cure to glioblastoma. It also does not so dramatically extend survival that it should deter or delay the aggressive pursuit of new treatments through clinical research. In addition, the ultimate regulatory approval of Optune for newly diagnosed glioblastoma patients is a separate matter from the immediate causes of determining appropriateness for treatment and ultimately improving insurance coverage. At issue is patient access, and the cost of care should not stand in the way of a brain tumor patient receiving treatment appropriate for extending survival or reducing tumor burden.

Expedite Review of NovoTFF-100A for “Appropriate” Treatment for Newly Diagnosed Glioblastoma Patients.

The Centers for Medicare and Medicaid Services (CMS) (part of the U.S. Dept of Health and Human Services) uses the appropriateness for treatment determination given by NCCN as a source of information and a basis for coverage decisions.

We respectfully urge NCCN to expedite review of the EF-14 clinical trial results and make a determination of appropriateness as soon as possible based on the science.

⁵ <http://www.novocure.com/~media/Files/N/Novocure/press-release/2014/201408-EF14-Trial-Results-Press-Release.pdf>

⁶ Ibid.

⁷ <http://www.medicarenhic.com/viewdoc.aspx?id=2518>

As experts in the treatment of GBM patients you, more than most, understand the hardships of a brain tumor diagnosis including those that are physical, mental and economic. It is our hope that NCCN's expert determination about Optune will provide clarity about the appropriateness for the treatment of newly diagnosed as well as recurrent GBM patients in light of the recent clinical trial results.

We thank you in advance for considering our request on behalf of brain tumor patients and their families.

Sincerely,

A handwritten signature in black ink that reads "David F. Arons". The signature is written in a cursive style and is centered within a light gray rectangular box.

David F. Arons, JD
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