The North American Brain Tumor Coalition:
An Agenda for Improving Brain Tumor Treatments

The North American Brain Tumor Coalition (NABTC) seeks to enhance the access of individuals with brain tumors to quality brain tumor care. Achieving this goal means ensuring that all brain tumor patients have access to the best treatments currently available while at the same time fostering, encouraging, and demanding the development of better treatments.

Although there have been incremental improvements in brain tumor treatment, the survival rates for many brain tumors have not changed in the last 40 years. The five-year survival rate for all brain tumors is 35 percent, and for some types of brain tumors the rates are even lower. For those with brain tumors and their families and friends, there is a desperate need for more research into better treatments.

Ten years ago, the National Cancer Institute (NCI) and the National Institute of Neurological Disorders and Stroke (NINDS), the two institutes leading the federal brain tumor research program, convened a panel of brain tumor experts to develop a plan for brain tumor research. Unfortunately, the Brain Tumor Progress Review Group report that emerged from that planning process remains almost entirely relevant today, as the goals of that report have not yet been realized through research advances. The speed of research progress is much too slow for brain tumor patients, and the NABTC proposes a concrete and specific plan of action to improve brain tumor research and care.

The NABTC proposes the following agenda to advance brain tumor care and research:

- Congress should boost funding for the National Institutes of Health (NIH) by at least 10 percent in FY 2010. This funding increase, which builds on the infusion of funds through the economic stimulus package, will sustain ongoing research and permit initiation of innovative new projects.
- The National Cancer Institute should restore funding for the Adult Brain Tumor Consortium for the five-year period beginning in 2008.
- Congress should define a plan for revitalizing and reinvigorating the brain tumor research effort by enacting the Brain Tumor Care and Cures Act.
- Congress should enact legislation to eliminate the Medicare waiting period (H.R. 1708/S. 700). This bill would terminate the unreasonable and unjustified two-year waiting period for Medicare benefits that faces brain tumor patients and others with serious illnesses from the time they are eligible for Social Security Disability payments.

A Strong Investment in NIH Is Necessary For Improvements in Brain Tumor Treatment

The North American Brain Tumor Coalition urges that National Institutes of Health (NIH) funding be increased by ten percent in fiscal year 2010. We also recommend that ongoing brain tumor clinical research programs, critical to answering questions about new brain tumor treatments, be protected from deep funding cuts.

Brain tumors present a daunting research challenge because of their complexity and diversity. Moreover, because of their location in the closed cavity of the skull, treatment and research are complicated and may be accompanied by significant side effects. Although we have made progress in the fundamental understanding of brain tumors and some incremental advances in their treatment, we have been unable to change the mortality rate from brain tumors in the last 40 years.

For a disease with an overall five-year survival rate of 35 percent, much more research must be supported, ranging from basic research to clinical trials testing new therapies. Although there are aggressive efforts to raise private funds for brain tumor research, these resources are not adequate to support the kind of brain tumor research program that will be required for real progress. Therefore, brain tumor research efforts will require a strong federal investment.

The North American Brain Tumor Coalition applauds President Obama and the 111th Congress for their leadership in including $8.2 billion in extramural research funding through the National Institutes of Health (NIH) as part of the American Recovery and Reinvestment Act (ARRA). This critical infusion of funds will support quality research that would otherwise have gone unfunded and will also permit researchers to expand and intensify ongoing research efforts. Brain tumor patients are among those who will reap the benefits of research support by ARRA funds.

It is critically important that Congress and the Obama Administration sustain the commitment they have made to biomedical research, so that progress is not interrupted and those with serious illnesses are not denied new treatments. We have observed the problems created by static funding for biomedical research and are not willing to suffer that problem again.

The North American Brain Tumor Coalition urges that Congress:

- Boost funding for NIH by at least 10 percent in fiscal year 2010. An increase of this magnitude will ensure that outstanding research projects can be sustained and that promising new brain tumor research efforts can be initiated.
- Protect the work of the Adult Brain Tumor Consortium in conducting early phase trials of brain tumor treatments by requesting that the National Cancer Institute restore funding for the Consortium for its five-year grant, which began in 2008.

April/May 2009

DRAFT LETTER FROM CONGRESS TO NCI DIRECTOR NIEDERHUBER

Dr. John Niederhuber
Director
National Cancer Institute
Building 31, Room 11A48
Bethesda, Maryland 20892

Dear Dr. Niederhuber:

I am writing to recommend immediate action to restore funding for the Adult Brain Tumor Consortium. The brain tumor clinical trials network plays a pivotal role in conducting early phase clinical trials on brain tumor therapies, and deeply cutting federal support will cripple this research program.

A small portion of the substantial new funds available to the National Cancer Institute (NCI) through the American Recovery and Reinvestment Act should be promptly committed to the Adult Brain Tumor Consortium. The award made to the consortium was $2 million per year for a term of five years, a substantial reduction from the $3.5 million annual funding level set aside in the request for proposals. A cutback of this magnitude cannot be absorbed without threatening the clinical research work of the consortium.

A well-organized and stable system with multiple trial sites is essential for conducting clinical trials, as the disease is really more than 120 diseases, each of limited incidence. Multiple research sites organized in a network are necessary to conduct a diversity of trials and to ensure efficient patient accrual in all trials. Funding cuts of the size proposed by NCI will undermine the stability and viability of the system if it forces a reduction in the number of trial sites or hinders the research effort at any trial site. Now is not the time to slow the accrual of patients in brain tumor trials or hamper the overall clinical research effort.

The Cancer Genome Atlas Project and the Glioma Molecular Diagnostic Initiative may be important elements of the NCI brain tumor research program, but they do not eliminate the need for an early phase clinical trials network that is adequately funded. Such a network is necessary for the translation of basic research findings into new treatments.

One of the basic goals of the American Recovery and Reinvestment Act is to support wise investment in “shovel ready” projects. Restoration of funding for the Adult Brain Tumor Consortium -- boosting annual funding from $2 million to $3.5 million for a five-year term, as anticipated in the announcement of the grants competition -- is consistent with the intent and goals of the economic stimulus effort.

Sincerely,

cc: James H. Dorshow, MD
Director
Division of Cancer Treatment and Diagnosis

April/May 2009

Improve Brain Tumor Therapies and Care
Through a Revitalization of the National Institutes of Health
Brain Tumor Research Program

Brain tumors have an overall five-year survival rate of 35%, and for some types of brain tumors the survival rates are even lower. Although there have been important advances in our understanding of brain tumors and some incremental improvements in treatment, overall the situation is only modestly improved over the last 40 years.

Almost a decade ago, the National Institutes of Health evaluated the status of brain tumor research and issued a series of recommendations for strengthening the research effort. The recommendations of that report, despite the decade of research that has intervened, still provide a valid roadmap for brain tumor research. This excruciatingly slow pace of progress must be addressed. The research report of 2000 must be rendered meaningless by virtue of research advances.

To ensure that the pace of brain tumor research accelerates and intensifies and that another decade does not pass with such limited progress in improving brain tumor treatment, the North American Brain Tumor Coalition proposes a specific legislative plan for brain tumor research.

The NABTC recommends that Congress enact legislation to:

- Authorize the brain tumor consortia program to ensure efficient completion of Phase I and Phase II therapeutic trials;
- Establish a Brain Tumor Research Coordinating Committee;
- Establish a new public-private partnership to accelerate brain tumor therapeutic development;
- Call for a conference to assess the potential for a collaborative brain tumor tissue bank;
- Authorize a research program focused on the neuro-rehabilitation needs of brain tumor patients; and
- Establish a program to test methods for providing survivorship care to brain tumor patients.

111th Congress

1st Session

A DRAFT BILL

To strengthen and intensify the federal effort to develop better treatments for brain tumors and to improve the quality of care for brain tumor patients.

Section 1. Short Title.

This Act may be cited as the Brain Tumor Care and Cures Act of 2009.

Section 2. Findings.

Congress makes the following findings:

(1) More than 50,000 cases of primary malignant and non-malignant brain and central nervous system tumors will be diagnosed in 2009.

(2) Of the total number of primary brain tumors diagnosed, more than 20,000 are malignant.

(3) Worldwide, more than 185,000 primary brain tumors will be diagnosed in 2009.

(4) More than 3750 children in the United States will be diagnosed with a primary brain tumor in 2009, and this total includes more than 2800 tumors in children under the age of 15 years.

(5) Brain tumors are the second most common childhood cancer and account for 25% of all pediatric cancer cases.

(6) Childhood brain tumors are the leading cause of solid tumor death in children and the third leading cause of cancer death in young adults from 20 to 39.

(7) The five-year survival rate after diagnosis with a primary malignant brain and central nervous system tumor is 28.8% for men and 31.6% for women.

(8) It is estimated that as many as 100,000 Americans will be diagnosed with metastatic brain tumors, which begin as cancer elsewhere in the body and spread, or metastasize, to the brain.

(9) "Brain tumor" is a misleading term, because there are more than 120 different brain tumors, according to the World Health Organization.

(10) The report of the National Cancer Institute Brain Tumor Progress Review Group (2000) described the special problems of treating brain tumors. Surgical removal of the tumor or entire organ with a generous surround of normal tissue cannot be easily accomplished in the case of brain tumors, unlike most tumors elsewhere in the body. Moreover, because most brain tumors are also relatively insensitive to radiation and chemotherapy, treatment of brain tumors is especially difficult.

(11) The Progress Review Group report captures the fundamental problem of brain tumor research and care with this statement: "Brain tumors represent a unique challenge in that they affect the organ that is the essence of the 'self'."

(12) All forms of brain tumor treatment – surgery, chemotherapy, and radiation – hold the potential to cause lasting side effects. Radiation and chemotherapy may have especially severe effects on children's developing brains.

(13) Children treated for brain tumors may face educational and employment challenges, difficulties in living independently, and obstacles to obtaining health insurance coverage.

(14) There have been modest improvements in the treatment of malignant and non-malignant brain tumors, but patients, families, and researchers agree that progress in improving the length and quality of life for those diagnosed with primary brain tumors is inadequate and that research must forge new approaches to brain tumor research, acute care, and neuro-rehabilitation and survivorship care.

(15) Brain tumors are diagnosed in the youngest and the oldest Americans, affecting patients at all stages of development and stage of life.

(16) Advances in understanding the biology of brain tumor and the migration of glial cells may contribute to development of better treatments and may also improve the overall pace of therapeutic development.

(17) A stronger federal investment is necessary to realize the research aims and goals of the Brain Tumor Progress Review Group report of 2000 and to build upon new insights about the complex biology of these tumors.

(18) Coordination and communication among brain tumor researchers holds the promise of stimulating creative approaches to research challenges and can create synergies among brain tumor experts to derive novel therapies.

(19) Because of the diversity of brain tumors and their relatively low incidence, special clinical research structures are necessary to ensure an aggressive pace of research on treatment and survivorship care.

(20) Coordination of all elements of brain tumor care will ensure a higher quality of care for brain tumor patients.

Section 3. Brain Tumor Clinical Research Consortia Program

(a) Establishment of Brain Tumor Consortia Program-- The Secretary of Health and Human Services shall establish a program of brain tumor clinical research consortia to accelerate the translation of basic research findings into new brain tumor treatments. The program shall --

(1) Include at least one adult brain tumor consortium and at least one pediatric brain tumor consortium;

(2) Sponsor and coordinate, through the separate adult and pediatric consortia, Phase I and Phase II clinical trials to evaluate new brain tumor treatments; and

(3) Provide adequate funding to support participation of leading brain tumor research sites as clinical trial sites in the consortia; the funding for the consortia shall be adequate to support a sufficient number of sites for efficient accrual and completion of trials.

(b) Standards for institutions participating in consortia. -- Institutions applying for a consortium grant shall evaluate institutions for participation in the consortium according to the following standards: existence of interdisciplinary research and clinical team, number of brain tumor patients evaluated and treated, number of clinical trial participants enrolled, and number of clinical trials undertaken.

(c) Authorization of Appropriations -- For fiscal years 2010 through 2014, such sums as may be necessary are authorized to the appropriated for the brain tumor research consortia program.

Section 4. Brain Tumor Research Coordinating Committee

(a) Establishment of Coordinating Committee -- Not later than 90 days after the enactment of this section, the Secretary, in consultation with the Director of the National Institutes of Health, shall establish a committee to be known as the Brain Tumor Research Coordinating Committee.

(b) Membership -- The Secretary and Director of NIH shall appoint members of the Committee to ensure active participation by the broad cross-section of brain tumor researchers. The Committee shall include:

(1) 5 members representing the National Cancer Institute, National Institute of Neurological Disorders and Stroke, National Institute of Biomedical

Imaging & Bioengineering, National Institute of Nursing Research, and National Institute of Child Health and Human Development;

(2) 10 researchers who represent disciplines involved in brain tumor research and who have received grant support from one of the institutes at NIH;

(3) 4 brain tumor advocates, representing adult and pediatric patient perspectives, including individuals diagnosed with brain tumors or family members of brain tumor patients.

(c) Chair -- The Secretary shall designate the chair of the Coordinating Committee from among its members.

(d) Terms of Members -- The members of the Committee will be appointed for three year terms but may be re-appointed for a second three-year term.

(e) Responsibilities - The Committee shall --

(1) Evaluate the components of brain tumor research supported by NIH, including the major categories of research supported by NIH, the objectives of the program, promising areas of research that may not be adequately supported by NIH, and opportunities for greater coordination and communication among intramural and extramural brain tumor researchers supported by NIH.

(2) Make recommendations for activities and initiatives, including a standing brain tumor research working group, which would facilitate innovative brain tumor research initiatives.

(3) Submit an annual report to the NIH Director regarding the strengths and weaknesses of the brain tumor research program and recommendations for enhancing the overall research effort.

(f) Authorization of Appropriations -- For fiscal years 2010 through 2014, there are authorized to be appropriated such sums as may be necessary to support the activities of the Brain Tumor Research Coordinating Committee.

Section 5. Brain Tumor Therapeutics Development Acceleration Initiative

(a) Establishment -- The NIH Director will establish a therapeutics development program to accelerate the development of new brain tumor therapies.

(b) In general -- The program shall support grants to researchers to support partnerships with public and private entities to accelerate development of new brain tumor treatments.

(c) Standards for grants -- Grants shall be --

(1) Awarded to nonprofit research entities for projects aimed at therapeutics development;

(2) Awarded to entities for projects that complement and support the research initiatives of the brain tumor research consortia, specialized programs of research excellence (SPOREs) and cancer cooperative groups; and

(3) Awarded to grantees with a well-defined plan to move therapies through the development process.

(d) Authorization of appropriations -- To carry out the Brain Tumor Therapeutics Development Acceleration Initiative, there are authorized to be appropriated such sums as may be necessary for fiscal years 2010 through 2014.

Section 6. Conference on Collaborative Brain Tumor Biospecimen Repositories.

(a) Purpose of Meeting. -- The Secretary shall sponsor a meeting of experts to investigate models of collaborative and multi-institutional brain tumor biospecimen repositories and the feasibility of a coordinated and collaborative repository to be utilized by brain tumor researchers supported by NIH grants.

(b) Establishment. -- The Secretary shall announce the date for the meting not more than 120 days after the date of enactment of this Act.

(c) Standards for Meeting. -- In designing the meeting, the Secretary shall consider:

(1) Participants. -- Participants shall include intramural and extramural brain tumor researchers, researchers who administer biospecimen repositories, international researchers with successful biospecimen repository experience, and NIH Institute and Center personnel with experience administering biospecimen repository programs.

(2) Issues for Review. -- The meeting shall review standards for biospecimen donation and storage, best practices of successful repositories, obstacles to multi-institutional participation in a centralized repository, the benefits of a centralized repository model, and coordination of any new repository with ongoing federal biospecimen programs.

(d) Authorization of appropriations. -- For the conference on brain tumor biospecimen repositories, there are authorized to be appropriated such sums as may be necessary.

Section 7. Neuro-Rehabilitation Research Program.

(a) Establishment -- The NIH Director will establish a neuro-rehabilitation research program to focus on the wide range of disease and treatment wide effects experienced by brain tumor patients, including but not limited to loss of speech, loss of hearing, and seizure disorders.

(b) In general -- The program shall support grants to researchers to undertake basic and applied research to investigate and develop effective strategies for ameliorating the neurological side effects resulting from brain tumors and their treatment.

(c) Authorization of appropriations -- to carry out the Neuro-Rehabilitation Research Program, there are authorized to be appropriated such sums as may be necessary for fiscal years 2010 through 2014.

Section 8. Brain Tumor Survivorship Center Program.

(a) Establishment. -- The Secretary shall establish a program of Brain Tumor Survivorship Centers to meet the specific and diverse late and long-term effects that may be experienced by survivors of brain tumors.

(b) In general. -- The survivorship centers program shall focus on the optimal structure for services for brain tumor survivors and shall also include an evaluation component to permit assessment of best practices and facilitate the replication of model programs.

(c) Agencies -- The Institutes and Centers of NIH, the Centers for Disease Control and Prevention, and the Health Resources and Services Administration shall be involved in planning the survivorship center program, and the Secretary shall designate one of the agencies to administer the program.

(d) Authorization of appropriations. -- To carry out the Brain Tumor Survivorship Center Program, there are authorized to be appropriated such sums as may be necessary for fiscal years 2010 through 2014.

Eliminate the Medicare Waiting Period for Disabled Individuals

The North American Brain Tumor Coalition and brain tumor advocates nationwide urge their Senators and Representatives to join as cosponsors of the Ending the Medicare Disability Waiting Period Act of 2009 (S. 700/H.R. 1708). The legislation would end the two-year Medicare waiting period for disabled individuals with life-threatening illnesses and phase it out over a period of ten years for all others. This is an urgently needed reform to Medicare rules that currently prevent seriously disabled individuals from receiving Medicare benefits at the very time when they need desperately need health care.

Over 1.2 million Americans who are receiving Social Security Disability payments are caught in a two-year period before they will receive Medicare benefits. Of this total, 400,000 have no access to health insurance of any kind. In its report on this issue, *Too Sick to Work, Too Soon for Medicare: The Human Cost of the Two-Year Medicare Waiting Period for Americans with Disabilities,* The Commonwealth Fund found that nearly 39% of the individuals stuck in the waiting period do not have health insurance coverage at some point during this period, and 26% have no health insurance during the entire period. At a time when these disabled individuals are in great need of quality health care, they have no means to finance it and may be forced into bankruptcy to obtain appropriate care.

Counted among the seriously disabled who are "waiting" for health care are many brain tumor patients. For these patients, a delay in receiving care can be catastrophic. Brain tumor care often cannot be delayed for days and it certainly cannot be postponed for months or years.

The waiting period for Medicare benefits was implemented when the Medicare program was expanded in 1972 to include the disabled. The policy denies disabled individuals access to health care and can no longer be justified on cost-saving grounds or for any other reason.

The North American Brain Tumor Coalition requests that:

- Representatives cosponsor H.R. 1708.
- Senators cosponsor S. 700.
- All Members of Congress support the inclusion of the provisions of H.R. 1708/S. 700 as elements of health care reform. This reform of Medicare is an essential first step in expanding access to quality health care.

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