

April 16, 2012

The Honorable Tom Harkin  
Chairman  
Senate Committee on Health, Education, Labor &  
Pensions  
428 Senate Dirksen Office Building  
Washington, D.C. 20510

The Honorable Michael B. Enzi  
Ranking Member  
Senate Committee on Health, Education, Labor &  
Pensions  
835 Senate Hart Office Building  
Washington, D.C. 20510

Dear Chairman Harkin & Senator Enzi:

The under signed organizations, representing patients, advocates, physicians, caregivers, consumers and researchers strongly support the establishment of a Breakthrough Therapy designation for new drugs that show a high magnitude of clinical activity early in their development.

**This idea was included in the bipartisan “Advancing Breakthrough Therapies for Patients Act of 2012” that was introduced on March 26, 2012, by Senators Bennet (D-CO), Hatch (R-UT), and Burr (R-NC).**

We support legislation on this important issue and commend the bipartisan effort that will help ensure the most promising new treatments reach patients as safely and efficiently as possible.

The Breakthrough designation would initiate an “all hands on deck” approach for FDA to collaboratively work with the sponsor to design the most efficient development program for the very promising therapy. Designation as a Breakthrough Therapy would encourage use of strategies to expedite getting new, more targeted drugs to patients who desperately need them, as well as avoid giving large numbers of patients potentially harmful or ineffective drugs as part of a control arm. This approach that could include the use of a variety of innovative clinical trial designs, or specific strategies for accumulating further evidence on the breakthrough drug after it is approved.

**The under signed groups respectfully ask that a Breakthrough Therapy designation be included as part of the upcoming reauthorization of the Prescription Drug User Fee Act (PDUFA).**

Sincerely,

Accelerate Brain Cancer Cure (ABC2)  
Accelerate Cure/Treatments for Alzheimer’s Disease  
Adenoid Cystic Carcinoma Research Foundation (ACCRF)  
AHAF Alzheimer’s Disease Research  
Alliance for Aging Research  
ALPHA-1 ASSOCIATION

ALPHA-1 FOUNDATION

Alzheimer's Association  
American Association for Cancer Research  
American Association for Respiratory Care (AARC)  
American Society of Clinical Oncology  
Association of American Cancer Institutes  
Cancer Support Community  
C-Change

COPD FOUNDATION

Facing Our Risk of Cancer Empowered (FORCE)  
FasterCures  
Fight Colorectal Cancer  
Friends of Cancer Research  
Intercultural Cancer Caucus (ICCC)  
JDRF  
Kidney Cancer Association  
Lung Cancer Alliance  
LUNGeivity Foundation  
Lupus Research Institute National Coalition  
Mental Health America  
National Alliance for Caregiving  
National Alliance on Mental Illness  
National Brain Tumor Society  
National Coalition for Cancer Research (NCCR)  
National Comprehensive Cancer Network  
National Organization Against Rare Cancers (NOARC)  
National Organization for Rare Disorders  
National Patient Advocate Foundation  
Oncology Nursing Society  
Ovarian Cancer National Alliance  
Prevent Cancer Foundation  
Sarcoma Foundation of America  
Sjögren's Syndrome Foundation  
Society for Women's Health Research  
Society of Gynecologic Oncology  
U.S. Pain Foundation  
United Cerebral Palsy  
USAgainstAlzheimer's

VITAL OPTIONS INTERNATIONAL