

One-Page Summary:

Testimony of Ruth Hoffman, Executive Director, Candlelighters Childhood Cancer Foundation before the House Committee on Energy and Commerce Subcommittee on Health May 2, 2007.

Members of the Committee,

I am the Executive Director of Candlelighters Childhood Cancer Foundation, representing 100,00 members nationwide including children and adolescents with cancer, survivors, and their families.

I am also the mother of a 20-year survivor of childhood cancer. My daughter Naomi was fortunate to have survived the Acute Myelogenous Leukemia she was diagnosed with at age 7. But like so many other children subjected to toxic chemotherapies and radiation treatments, she has suffered from serious physical disabilities including being recently diagnosed with secondary cancer caused by her childhood exposure to radiation therapy.

Current toxic therapies that have proven effective for adults aren't a solution for kids with cancer – in fact, these treatments are causing secondary cancers in some of the children who survive to adulthood. The best hope for children with cancer rests in the research and development of new and targeted biologics.

I am here today to explain to the committee how important it is that you not deprive children with cancer of future cures by depriving the biotech industry of incentives to innovate. What kids need is increased incentives for industry to develop new types of targeted therapies to treat children with cancer.

Treatment for children with cancer hasn't really changed much since Naomi was originally diagnosed in 1987. Today, ALL children with cancer continue to be treated solely with highly toxic cancer drugs that were developed 20 to 30 years ago. We have reached a plateau with survivorship rates, and we have reached the limit of toxicity for current chemotherapy drugs and radiation treatments.

Children with cancer need treatment breakthroughs. They need new molecular-based therapies that will “kill the cancer, not the kid.” Biologic drugs have proven to be an effective weapon in the war on cancer for adults, and one of the most promising treatments for the future. Because conventional chemotherapy and radiation treatments are so dangerous to children, young cancer patients are depending on innovative biotech companies to continue to develop more effective and targeted treatments in the future.

As you work to cut costs and increase access to biologic therapies, please take care to find a balance between the need for cost-saving, and the need to encourage development of new life-saving biologics for children with cancer. Biotech innovation is the best hope for a true cure to childhood cancers. Kids can't fight cancer alone. They're depending on you to keep the pipeline of innovative biologic drug development alive and well.

Testimony of Ruth Hoffman, Executive Director,  
Candlelighters Childhood Cancer Foundation

Before the

House Committee on Energy and Commerce  
Subcommittee on Health

**“Assessing the Impact of a Safe and Equitable  
Biosimilar Policy in the United States”**

May 2, 2007

Chairman Pallone, Ranking Member Deal, and Members of the Committee, I sincerely thank you for giving me the opportunity to testify before you today on an issue of great importance to the childhood cancer community, and to me personally.

I'm referring to the crucial role of biologics in the ongoing war on childhood cancers. Children with cancer have unique needs. They are not simply "little adults;" children have their own biologic systems and unique tumor characterizations. Current toxic therapies that have proven effective for adults aren't a solution for kids with cancer – in fact, these treatments are causing *secondary* cancers in some of the children who survive to adulthood. *The best hope for children with cancer rests in the research and development of new and targeted biologics.* I am here today to explain to the committee how important it is that you not deprive children with cancer of future cures by depriving the biotech industry of incentives to innovate.

My name is Ruth Hoffman, and I am the Executive Director of the National office of Candlelighters Childhood Cancer Foundation. Candlelighters was founded in 1970 by concerned parents of children with cancer. Our mission, then and now, is to provide information and awareness for children and adolescents with cancer and their families, to advocate for their needs, and to support research so every child has the opportunity to survive and lead a long and healthy life. Today we have a membership of over 50,000 members of the national office and more than 100,000 members across the country linked to our 37 affiliate offices in 28 states.

Advocating for children with cancer is my job as Director of Candlelighters. But I am also the mother of a 20-year survivor of childhood cancer.

Twenty years ago, on July 10<sup>th</sup>, 1987, my world changed forever. I was 31 years old, had a 9 yr. old son, a 7 yr. old daughter (Naomi), a 1 yr. old son -- and I was 5 months pregnant with identical twin boys. My daughter Naomi had been sick for 9 months. She had been diagnosed by our family physician as having a bug bite, virus (put on antibiotics), ear infections (had tubes put in), tonsillitis (tonsils and adenoids removed),

and a neurotic mother – me! Despite these attempts to explain her “failure to thrive,” Naomi continued to deteriorate to a mere 32 pounds. She was no longer able to walk. So in sheer desperation, I carried her to the emergency ward of our local Children’s hospital. It was on that day in July that I heard the words that seared my heart and my soul forever: “Your daughter has cancer.”

Naomi was diagnosed with Acute Myelogenous Leukemia (M5) – the “bad” leukemia. Her prognosis was poor. Few survived AML in 1987. Fortunately, bone marrow transplants were just beginning as a potential therapy to treat children with AML, and her nine-year-old brother was a perfect donor match for Naomi. Her treatment included I.V. chemotherapy for 5 days on, 24 hours a day, followed by 3 weeks off, for 5 months. This was followed by high dose chemotherapy and total body radiation. After 9 months of living in a complete bubble environment, Naomi was considered “cured.”

What I didn’t know then, that I sadly know now, is that childhood cancer is for life. The five-year survival rate used to determine adult cancers as “cured” has little meaning to children who complete treatment at 8 years of age. We are treating children with intensive toxic therapies at a time in their lives when they have growing bodies and developing brains. You can’t treat a child’s young body with these kinds of invasive therapies and not impact their overall health for the rest of their life.

Naomi did not emerge from her treatments unscathed. She had cataracts, heart damage, endocrine dysfunction, and was sterile. But she had her life, and she was determined to live it to the fullest. Then, two and a half years ago, shortly after Naomi graduated from college, I received the call that made my life stand still once again. Naomi was diagnosed with papillary thyroid carcinoma, metastatic to her lymph and bones – a second cancer – but this one was caused directly by the total body radiation that she received to treat her first cancer.

Naomi just keeps living her life and doing her best to invest it with meaning. She currently works at Children’s National Medical Center here in Washington, DC, where

she's employed as a clinical trial coordinator for a study of boys with Duchenne Muscular Dystrophy. She volunteers as a camp counselor for children with special needs including cancer, and she recently attended the Lance Armstrong Summit in Texas, where she represented and advocated for survivors of childhood cancer.

Naomi lives every day with the fact that, in all likelihood, cancer will end her life prematurely. But she hasn't given up hope. On the contrary, she's more committed than ever to making her life matter – not just to herself, but to other young people with cancer. She's so committed to the search for new molecular-based therapies for children with cancer that she is organizing her own fundraiser this November. *Naomi's Hope for a Cure* will raise money for research towards a genomic characterization of pediatric AML.

Treatment for children with cancer hasn't really changed much since Naomi was originally diagnosed in 1987. Today, ALL children with cancer continue to be treated solely with highly toxic cancer drugs that were developed 20 to 30 years ago. Only one new drug has received marketing approval by the FDA for childhood cancer in the last decade. That drug was not a new class of "smart drugs." It too was another toxic chemotherapy agent.

These traditional chemotherapy drugs have not provided a cure for many childhood cancer tumors, and they leave those children who do survive facing lifelong late-effects, including severe drop in IQ, heart damage, sterility, deafness and -- most shockingly-- secondary cancers. As a result, cancer remains the number one disease killer of America's children – more children still die from cancer than Cystic Fibrosis, Muscular Dystrophy, Asthma, and AIDS combined.

Every day I get calls from frantic parents around the country, looking for guidance and for hope. Just last week, I got a call from a young father whose 10-day old son, Jack, had just been diagnosed with a brain stem tumor. My job was to tell him that all doctors can offer infants like Jack is chemotherapy – they can't radiate children under three. What I did not want to tell him was that even with treatment there's only a 10% chance that Jack

will survive to see his second birthday. Jack is not alone. Only half of children diagnosed with metastatic bone cancer will survive five years. Even today, half of children and teens diagnosed with Naomi's original cancer -- acute myelogenous leukemia -- will die within 5 years.

What is even more disheartening is that these mortality rates have not changed in the last decade! I want to repeat that: the toxic chemotherapy and radiation treatments that we are giving our children with cancer have NOT increased survivorship in the last ten years! We have reached a plateau with survivorship rates, and we have reached the limit of toxicity for current chemotherapy drugs and radiation treatments. I can't offer any more hope to families whose children are diagnosed with cancer today, than I could ten years ago.

As Director of Candlelighters, I've come here to tell you that the status quo is not good enough for children with cancer. As Naomi's mother, I'm asking you: "Can't we do better for our children?"

Is there hope for this youngest cancer patients? The answer is a resounding YES! We stand at the threshold of a new era in the genetic treatment of cancer. Large research initiatives are underway to identify the genetic fingerprints of many types of adult cancers -- but funding for targeted therapeutic research for childhood cancer is minimal, and lagging behind today's adult cancer research initiatives. What kids need is increased incentives for industry to develop new types of targeted therapies to treat children with cancer.

Children with cancer need treatment breakthroughs. They need new molecular-based therapies that will "kill the cancer, not the kid." Biologic drugs have proven to be an effective weapon in the war on cancer for adults, and one of the most promising treatments for the future. Because conventional chemotherapy and radiation treatments are so dangerous to children, young cancer patients are depending on innovative biotech companies to continue to develop more effective and targeted treatments in the future.

At this critical moment when targeted therapies are finally bearing the fruit of decades of research and providing new hope for cancer patients and their families, it is essential that we not undermine the development of these life-saving biologic agents.

Today you are considering the important issue of allowing for abbreviated approvals of biosimilar products. We fully support increasing access to affordable drugs. *But what kids with cancer need most is access to drugs that can treat and cure their disease.* A policy that produces *more copies* and *less innovation* will not help the children and their families living with cancer. Please don't create legislation that reduces costs by reducing incentives for biotech companies to develop targeted therapies for cancer. For me, and for the parents I represent, life-saving trumps cost-saving any day.

Elizabeth Edwards said in her statement to the press upon her relapse of breast cancer, "Femara didn't exist five years ago. I don't expect to get yesterday's medicine. If I can help it, I'd like to get tomorrow's medicine." Don't our children with cancer deserve the promise of tomorrow's drugs as well? The R&D pipeline for new biologics is a lifeline of hope for these kids and their families. Please don't shut it off.

My daughter Naomi draws her inspiration from something Ralph Waldo Emerson wrote: "Do not go where the path may lead, go instead where there is no path and leave a trail." We are a nation of trailblazers and innovators. I want to thank Representatives Inslee, Baldwin, and Green for introducing legislation that will enable this tradition of innovation to thrive in service to our nation's children with cancer. And I want to thank the committee for recognizing that the future of biologics can't be measured in dollars and cents alone – that the bottom line for patients and their families is the priceless currency of life, health, and hope.

Candlelighters' motto is "... because kids can't fight cancer alone!" I urge the members of this committee to think hard about the impact of your decisions on young lives. Kids can't fight cancer alone. They rely on adults like you and me to offer them hope, towards

a healthy adult future of their own.