CYSTIC FIBROSIS FOUNDATION
ADDING TOMORROWS

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Written Testimony for the Hearing: "Improving the Medicaid Program for Beneficiaries"
Committee on Energy and Commerce, Subcommittee on Health

Summary:

- The Cystic Fibrosis Foundation offers its full support for the Ensuring Access to Clinical Trials Act, HR 209.
- The law the Ensuring Access to Clinical Trials Act makes permanent, the Improving Access to Clinical Trials Act, allows people with rare diseases to receive up to $2,000 annually in compensation for participation in clinical trials without that compensation counting toward their income eligibility limits for SSI and Medicaid.
- Unless Congress acts, it will expire on October 5, 2015. We urge swift Congressional action on the Ensuring Access to Clinical Trials Act.
- The Senate passed identical legislation on July 16, 2015.
- The Improving Access to Clinical Trials Act has enabled people to participate in clinical trials who would otherwise have not for fear of losing critical benefits.
- Rare disease researchers face a serious challenge in recruiting participants for clinical trials, especially for those diseases or subtypes of disease with very small populations.
- CF is an expensive disease, and Medicaid is critical for many to afford care.
- A broad coalition of health care organizations support the Ensuring Access to Clinical Trials Act, including NORD, BIO, MDA, Research!America, and over 70 other groups.
- The Congressional Budget Office (CBO) has given HR 209 a minimal preliminary score.

Written Statement:

On behalf of the Cystic Fibrosis Foundation, representing the approximately 30,000 people with cystic fibrosis (CF) in the United States, I am pleased to offer this testimony in support of H.R. 209, the Ensuring Access to Clinical Trials Act of 2015.

We are deeply grateful to Health Subcommittee Chairman Pitts and Ranking Member Green, as well as full Committee Chairman Upton and Ranking Member Pallone, for convening this discussion on an issue that is so important to the rare disease community. As I will discuss in greater detail later in my testimony, the Improving Access to Clinical Trials Act expires on October 5, and swift Congressional action on HR 209 is vital to ensure the continued viability of this critical law.

The Ensuring Access to Clinical Trials Act will permanently remove a barrier to clinical research for rare diseases and allow Supplemental Security Income (SSI) and Medicaid recipients to participate in and benefit from clinical trials. We greatly appreciate the efforts of the bill's sponsor, Congressman Lloyd Doggett (D-TX), and all of those working to pass this important legislation.

Cystic fibrosis is a rare, genetic disease that primarily affects the lungs. It causes the body to produce thick, sticky mucus that clogs the lungs and other parts of body and leads to life-threatening infections and serious digestive complications. In the 1950s, few children with CF lived to attend elementary school. Since then, tremendous progress in understanding and treating CF has led to dramatic improvements in length and quality of life for those with cystic fibrosis. Many people with the disease can now expect to live into their 30s, 40s and beyond.

As a physician, professor, and clinical investigator at the Johns Hopkins Division of Pulmonary and Critical Care Medicine, I have observed the devastating impact of this disease and the importance of clinical research in developing treatments that can change the lives of

individuals with CF. It is for this reason that I am here today to ask that the Ensuring Access to Clinical Trials Act be passed without delay.

The Ensuring Access to Clinical Trials Act of 2015 eliminates the five-year sunset clause from a current law — the Improving Access to Clinical Trials Act (IACT). Signed into law in 2010, IACT allows people with rare diseases to receive up to $2,000 annually in compensation for participating in clinical trials without that compensation counting toward their income eligibility limits for SSI and Medicaid. Unless Congress acts, this critical law will expire on October 5, 2015.

The Senate has already passed identical legislation by unanimous consent, and we urge similar swift consideration of this bill in the House.

Through my work with the Cystic Fibrosis Therapeutics Development Network and in my own clinical research at Johns Hopkins as Director of the Johns Hopkins Adult Cystic Fibrosis Program, I have seen firsthand how the Improving Access to Clinical Trials Act has enabled people to participate in clinical trials who would otherwise have not for fear of losing critical benefits.

The particular individual that comes to mind when I think of the Ensuring Access to Clinical Trials Act is a young man with cystic fibrosis by the name of Michael that I was caring for in 2009 prior to the original passage of this law. Mike had significant lung disease from CF, but for many years had made time to participate in clinical trials to help speed the development of desperately needed new therapies. Yet in 2009, when a trial of a very promising new therapy called ivacaftor started and was looking for CF clinical trial participants, Mike did not participate. Not because he didn’t want to – in fact, he desperately wanted to enroll in the trial of a drug which was later found to be the most effective drug that has ever developed for his type of

CF - but because he had evaluated his finances and was afraid that the modest payment of approximately $750 associated with participation in the trial would put the Medicaid and SSI support on which he was completely reliant in jeopardy. Mike even volunteered to participate in the trial without payment, but this is not allowed by Hospital Review Boards for the vast majority of clinical trials, including this one. Approximately 4 months after deciding not to enroll because of financial concerns, Mike died unexpectedly from complications of CF. To this day, I still wonder if his outcome may have been different if he had enrolled.

Rare disease researchers face a real challenge in recruiting participants to test new medications. Removing barriers to drug trial participation is particularly important, as recent advances in medical research and technology allow for the development of innovative and promising medications. Securing an adequate number of clinical trial participants is vital for therapies that treat rare conditions, but rare disease researchers in particular often have difficulty recruiting drug trial participants, simply because they have a smaller pool of patients.

If the Improving Access to Clinical Trials Act were allowed to expire and this barrier were reinstated, it would not only affect future trial enrollment, it could cause those with rare diseases who are participating in clinical trials to drop out of these trials for fear of losing their benefits. This would put vital clinical research at risk at a time when the medical needs of the majority of people with rare diseases are not being met.

With the advent of precision medicine, it is now possible to customize therapies to treat an individual's specific genetic makeup. As this new concept in drug development quickly becomes a reality, it opens the door for the advance of targeted therapies in many important areas of medicine, including cancer and rare diseases like CF.

For example, the Cystic Fibrosis Foundation has collaborated with pharmaceutical companies to develop targeted therapies that address the underlying cause of the disease in people with specific genetic mutations that cause CF. Two of these therapies are now available to patients; however, they are only effective for some groups of people with cystic fibrosis. Nearly half of those living with CF are still waiting for a therapy to treat their disease.

The mission of the CF Foundation is to find a cure for ALL people with cystic fibrosis — including those with the rarest CF mutations, which might be found in only a handful of people. In order to achieve this goal, we must ensure nothing stands in the way of carrying out this vital research; no one should be excluded from participation.

We are well on the way to our goal. There are currently 18 clinical trials under way for new cystic fibrosis therapies — more than ever before. Many in the CF community are calling 2015 the "Year of the Clinical Trial." Now is the time to ensure that all people with CF — and all rare diseases — have opportunities to participate in clinical trials for potentially life-saving treatments.

The necessity of ensuring an adequate number of clinical trial participants holds true not just for genetically targeted medications, but also for therapies that treat the symptoms of cystic fibrosis, including antibiotics and nutritional supplements like pancreatic enzymes.

It is also important to note that cystic fibrosis is an expensive disease, and individuals who rely on Medicaid for their coverage cannot afford to be without it due to their participation in a clinical trial. According to a 2014 Cystic Fibrosis Patient Survey, nearly 20 percent of those with CF receive Medicaid, and almost one quarter of CF patients delayed getting medical care or skipped treatment because they worried about the costs.

The Improving Access to Clinical Trials Act was the result of the rare disease community coming together five years ago in support of a policy that will help improve clinical research for devastating rare diseases and help people with those diseases keep vital health benefits.

Now, the Ensuring Access to Clinical Trials Act, which would remove the sunset clause from IACT, enjoys similar widespread support. This bill is supported by a diverse coalition of health care organizations, including the National Organization for Rare Disorders (NORD), the Muscular Dystrophy Association (MDA), the Biotechnology Industry Organization (BIO), Research!America, Faster Cures, the Children's Hospital of Philadelphia, the Massachusetts Medical Society, and more than 70 other groups.

This legislation will have minimal cost to American taxpayers. According to a preliminary estimate by the Congressional Budget Office, the Ensuring Access to Clinical Trials Act will cost less than $500,000 over ten years.

Again, I am deeply grateful to the Committee for this opportunity to offer testimony in favor of the Ensuring Access to Clinical Trials Act, and I ask for your support of it. The Cystic Fibrosis Foundation stands ready to work with this Committee and Congressional leadership to ensure passage of this bill to enable those with rare diseases to access life-sustaining treatments and enjoy the best health and quality of life possible.