

Remarks by CPRIT CEO Wayne Roberts  
Rare Disease Day – February 28, 2015 – Texas State Capitol

Thank you, Barbra, for that introduction. And thank you all for being here on Rare Disease Day.

In 6 days we'll recognize the 179<sup>th</sup> anniversary of the battle for the Alamo.

In his memorable letter of February 24, 1836, Colonel Travis begins with these three words: **I AM BESIEGED**. 179 years later, we are still besieged, but in ways not understood in Travis' time.

Today we're besieged by 7,000 rare diseases that impact the lives of 30 million Americans. **That's 1 out of every 10 of us here in Texas.**

Unfortunately, help arrived too late for Colonel Travis and the defenders of the Alamo. But that history isn't one we should accept in our defense against rare diseases.

Rare diseases are difficult to diagnose...the symptoms can linger for many years and be misdiagnosed...often there is no existing effective treatment or cure.

When you look at research funding from all sources, a smaller percentage of dollars are directed at rare diseases—and there are fewer medical experts dedicated to their study and treatment.

Half of all rare diseases affect our kids—and it's especially heartbreaking when children die too early of diseases with no treatments or cures.

At the Cancer Prevention and Research Institute of Texas, CPRIT, priority is given to prevention and research of **rare** and hard-to-treat cancers—including pediatric and adolescent cancers.

I won't go into all projects, but let me provide a few highlights:

- CPRIT funding seeks new treatments for a highly malignant brain tumor in children—treatments without the many severe neurological and long-lasting side effects that exist now.
- CPRIT funding seeks to identify the genetic links that lead to bone cancer in children and adolescents.
- And one of CPRIT's grantees is seeking ways to increase the survival rate of infants suffering from a rare form of leukemia—

A survival rate under 20 percent for babies is unacceptable.

How do we get to advancements that offer a treatment or possibly even a cure where none previously existed?

CPRIT funding has made it possible to recruit nearly 90 of the top cancer researchers in the world to Texas research institutions. We're well on our way to having the finest cluster of cancer expertise in the world right here in the Lone Star State.

AND...the end game of CPRIT's product development program is to take new drugs and devices from the research lab to the doctor's office to treat people with cancer now.

From the development of the integrated circuit at Texas Instruments in the 1950s...

...through the space race at NASA's Johnson Space Center...

...and Jim Allison's groundbreaking work at MD Anderson to unleash our body's immune system against cancer...

Texas has always been home to innovative people determined to accomplish what hasn't been done—or supposedly can't be done.

The people of this state have the can-do spirit.

As Texans, we must do everything we can to ensure that advancements in rare cancers are not only found more quickly, but found right here in Texas.

Your passion and advocacy in the fight against rare diseases inspires us at CPRIT to work on your behalf.

Keep pressing and keep the faith.