STATEMENT OF JENNY MOSIER
MEETING OF THE FDA'S PEDIATRIC ONCOLOGY SUBCOMMITTEE
OF THE ONCOLOGIC DRUGS ADVISORY COMMITTEE
JUNE 20, 2019

Good afternoon. My name is Jenny Mosier. I am here today as a parent whose child died from a diffuse intrinsic pontine glioma (DIPG) tumor, and as the Executive Director of Michael Mosier Defeat DIPG Foundation, a nonprofit that funds medical research to find a cure for DIPG. Our Foundation has made grants to support development of ONC201 as a potential therapy for DIPG patients. I appreciate the opportunity to speak to the Pediatric Oncology Subcommittee today.

As a parent and a DIPG advocate, I would like to highlight three important points for the subcommittee's consideration.

First, development of ONC201 should include the population of children fighting DIPG. There is an urgent need for therapies for DIPG, as illustrated by the story of my own son, Michael.

On September 4, 2014, one week after Michael's sixth birthday and the start of kindergarten, we learned he had a brainstem tumor. In shock, we were told that surgery was not an option and that he probably would not live to see his 7th birthday. We quickly learned that DIPG had no viable treatments and near 0% survival.

Tragically, DIPG does not only lead to the death of way too many children, it also inflicts substantial suffering on these kids as they fight the disease. Around six weeks after my son's diagnosis, he was unable to walk on his own. The tumor paralyzed Michael's body over a period of months. It gave him double vision. It made him constantly nauseated so he would often vomit and could only sleep if elevated. The tumor stole his ability to smile. It stole his voice. It made it difficult to chew and swallow. It eliminated his bladder control. The steroids he took doubled his weight over a period of months.

At the end of Michael's life, he could not move any part of his body or speak. The last thing he could do was blink in response to yes/no questions. And then that went away too. Michael fought for 8.5 months, and he suffered tremendously. He did not make it to his 7th birthday.

It is essential that decisions surrounding ONC201 as a potential treatment for this vulnerable patient population are viewed through a lens that takes account of the terminal nature of DIPG as well as the toll it takes on children during their fight.

Second, FDA should utilize all tools available to expedite development and approval of ONC201 for DIPG. The median survival from diagnosis for a child diagnosed with DIPG is only 9 months. DIPG epitomizes a serious condition with unmet medical need.

Children who are fighting DIPG right now cannot afford delay and desperately need options. Any steps that advance approval by months could give these kids access to a treatment that has the potential to extend survival.

There is also a need for therapies for DIPG with a better side effect profile.

Our son took an experimental drug coupled with an FDA-approved chemotherapy that was given one week per month. I was supposed to wear gloves just to handle the medication. For a few weeks after he took the drugs, Michael took anti-nausea medicine around the clock. If we missed a dose, Michael would typically vomit right away. For a few weeks, Michael would be very tired, hardly speak, and lose interest in activities and school. He would have around a week when his energy would rebound and then it was time to do it all over again.

While further study is needed, ONC201 shows promise to offer improved tolerability to DIPG patients. I have spoken with a number of parents whose children have taken ONC201, through the clinical trial or compassionate use. I have also interacted personally with the children while they are on a treatment protocol. Families have shared that their children need some anti-nausea medicine on the day they take the drug but that they typically feel better within a day or two and can quickly resume normal activities. We urge FDA to use all tools at its disposal to expedite development and approval of ONC201 for DIPG.

<u>Third</u>, while ONC201 continues to be studied through clinical trials, FDA should take steps within the trial context, or through expanded access, to enable as many patients as possible to gain access to the drug.

My role allows me to regularly interact with DIPG families, and many are frustrated because they have been unable to access ONC201 or other treatments for their kids. When a child is facing a terminal disease, we should be eliminating impediments that block access to experimental treatments, especially when they offer potential benefit with limited side effects.

We need progress for children with DIPG. These kids deserve a chance for a future.

Thank you to the FDA, and specifically this subcommittee, for allowing me to speak today.