

# Groundbreaking Treatment for "Incurable" Brain Tumors Meet Dr. Michelle Monje, Revolutionary Neuro-Oncologist

Lucile Packard

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**Pediatric brain tumors are notoriously difficult to treat.** Visionary scientist and clinician Dr. Michelle Monje discovered that one such cancer, diffuse intrinsic pontine glioma (DIPG), is physically and electrically integrated, hijacking the brain's normal circuitry to fuel the cancer's rapid growth. After diagnosis, most children pass away within months. Dr. Monje's entirely new therapeutic approach is delivering hope to kids with DIPG for the first time.

## "I Couldn't Just Turn Away"

Dr. Monje first encountered DIPG as a medical student at Stanford School of Medicine. "I cared for a vibrant 8-year-old girl with the disease, and she passed away just six months after diagnosis," Dr. Monje recalls. "I was horrified that we understood so little about this tumor. I knew I had to do something to help."

She began by building a tumor bank at Stanford. Families who lost a child to DIPG donated their brain tissue to her lab, in hope of finding a cure.

After years of research, she had a breakthrough. Dr. Monje identified a sugar on the outside of the DIPG tumor that was vulnerable to CAR-T cell therapy, a treatment that engineers a child's own immune system to attack cancer cells.

brain cancer is the deadliest type of pediatric cancer

90%

20

of DIPG patients die within 2 years of diagnosis

labs have studied samples from Dr. Monje's tumor bank "As luck would have it, my colleague, immunologist Dr. Crystal Mackall, was working to seek and destroy the exact sugar we found on the surface of the tumor," she says. A serendipitous conversation in the office—between the world's leading DIPG and CAR-T experts—led to the long-awaited discovery that families had been hoping for.

The two scientists joined forces with a team of Stanford researchers, and in summer 2020, they launched the first-ever CAR-T cell clinical trial for children with DIPG. Initial results from patients were unprecedented, with most children's tumors shrinking significantly. Some children who entered the hospital in wheelchairs even regained the ability to walk. **Philanthropy will equip Dr. Monje to forge ahead in her quest to finally defeat this terrible disease.** 

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Michelle Monje, MD, PhD, professor of neurology and neurological sciences and, by courtesy, of neurosurgery, of pediatrics, of pathology and of psychiatry and behavioral sciences. She is a Howard Hughes Medical Institute Investigator, a MacArthur "Genius" Award winner, and a member of the National Academy of Medicine.

# Far-Reaching Hope, for the First Time

Dr. Monje is closer to an effective treatment for DIPG than ever before. One of her patients has even been tumor free for over two years an unprecedented result! This is a historic opportunity to change the future for countless patients with brain cancer. Philanthropy is crucial to making it possible.



#### Accelerating a Cure for DIPG

The scientific community is eagerly watching the progress of Stanford's clinical trial. Flexible donor support will fuel Dr. Monje's cuttingedge efforts and help her take learnings from the trial back to the lab for further refinement. Her findings could lead to effective treatment not just for DIPG, but for other cancers of the brain and spinal cord as well.



#### Helping Caregivers Navigate a Crisis

A DIPG diagnosis is a parent's worst nightmare, and it has long haunted cancer researchers, who have been unable to find any effective therapy. While this trial is exceptionally promising, Dr. Monje is acutely aware of the emotional toll that caring for a child with DIPG takes on families and researchers alike. Funding for psychological and social support is critical for families as they juggle managing their child's complex care with unimaginable grief. It will also support grief counseling for Dr. Monje's clinical team.

#### Long-Term Goal: A Center of Excellence

Dr. Monje's DIPG clinical trial has brought together more than two dozen of Stanford's brightest physician-scientists. Her long-term vision is to establish and direct a Center of Excellence in cancer neuroscience and neuro-immuno-oncology—fields Dr. Monje founded and continues to lead. With philanthropic support to fuel her leadership, she will train the next generation of researchers and clinicians and maximize the impact of breakthroughs to save as many lives as possible.



## HONORING JACE WARD, DIPG HERO

Jace Ward had been fighting DIPG for a year when he joined Dr. Monje's trial at Lucile Packard Children's Hospital Stanford. He was determined to help other children, who typically develop the disease between 5 to 10 years old. Jace told Dr. Monje, "I know that I'm going to die, and I know this therapy will one day be the thing that cures other kids. Figure it out with me."

At one point, Jace's condition worsened, leaving him unable to walk or open his mouth. Just two weeks after receiving an infusion of CAR-T cells, he walked out of the hospital and ate a hamburger. Not long after, he was able to walk four miles a day. He even fulfilled his lifelong dream to attend the Super Bowl (pictured above, center, with his brother and dad).

While Jace ultimately succumbed to his condition, his legacy lives on through Dr. Monje's search for a cure. "Throughout the trial, Dr. Monje was so compassionate, so accessible, so engaged, and really, so brave," says Jace's mother, Lisa. "She has given the DIPG community so much hope."

### PHILANTHROPY WILL CATALYZE NEW TREATMENTS

"For the first time in my career, I have been able to deliver good news to children with DIPG and their families," says Dr. Monje. "It is a moment of enormous hope, and philanthropy is essential to accelerating progress."

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