

Brain Cancer Patients Enrolling in Phase 3 Clinical Trial of Promising Drug for Rare Brain Tumor

First Viable Option to Treat Rare Brain Tumor, most often found in children

SANTA MONICA, CALIFORNIA, UNITED STATES, June 15, 2023 /EINPresswire.com/ -- Early this year, the Saint John's Cancer Institute at Providence Saint John's Health Center enrolled the first patient in a Phase 3 global clinical trial for a promising drug to treat a rare, aggressive and often terminal form of brain cancer.



We are very hopeful as we proceed that this trial will be pivotal in establishing a new treatment for patients with this rare brain tumor."

Neuro-oncologist Akanksha Sharma, M.D.

The Santa Monica, Calif., hospital, which participated in the first two phases of the drug's clinical development, was the first of 120 sites across 15 countries to begin enrolling patients in the trial's final phase. Three more patients have enrolled, and two others now are being screened.

The cancer, called [H3 K27M-mutant diffuse midline glioma](#), is known as DMG and typically is found in children and young adults. But because Providence Saint John's doesn't specialize in pediatric cancer, it is recruiting young adults and taking referrals to enroll patients in the clinical trial, said neuro-oncologist [Akanksha Sharma, M.D.](#), a palliative care specialist and principal investigator for the trial. Dr. Sharma said the hospital typically treats four to five DMG patients a year.

The goal of the placebo-controlled, randomized trial is to determine if a drug known as ONC201 is effective, following initial radiation therapy, in extending a patient's survival and halting the cancer's progress.

"We are very hopeful as we proceed that this trial will be pivotal in establishing a new treatment for patients with this rare brain tumor, which, thus far, has had no other proven and evidence-based option," Dr. Sharma said.

Manufactured and developed by North Carolina-based Chimerix, Inc., the experimental drug aims to induce cell death – without harming healthy cells – by shutting down signaling pathways that help tumors grow. ONC201 has shown promising early results for patients with brain

tumors that have the H3 K27M-mutation, including significant tumor shrinkage in some patients, according to Chimerix. In addition, few serious side effects related to ONC201 were reported in previous phases of the trial.

One patient, 36-year-old Benjamin, who asked not to use his last name, has been able to take the drug for more than three years through an expanded access trial and now is beating the odds, even showing some disease regression.

For Benjamin, the first sign something wasn't right came just before his 32nd birthday, when his tongue went numb. Other symptoms later followed – his migraines started increasing and then there was the day at work when he misjudged a corner and walked into a wall. Benjamin's stepfather, a family practice doctor, suggested he see a neurologist. The specialist couldn't find a problem, but Benjamin pressed, and an MRI was ordered. The diagnostic test revealed what was later determined to be a DMG tumor on his brainstem.

He chose to enroll in the clinical trial in the hopes of finding an innovative option that could safely treat his tumor. In the more than four years since, Benjamin has continued to take ONC201 and he and his wife have had two daughters, 3 and 1 years old. He counts Cow, his goldendoodle, among his best friends.

"I have my health issues, but I'm alive," said Benjamin, who had to leave his job as a computer programmer for a tech company due to his illness. "I'm living day-by-day, minute-by-minute, and I'm so thankful.

"I'm young. I'm alive. I'm going on five years. That's success."

Benjamin is among patients who participated in a podcast titled "What's the Best Way to Help Someone with Cancer?"

Dr. Sharma is encouraged by stories like Benjamin's and continues to follow the journey of this experimental cancer drug as it undergoes clinical trials. She hopes that additional data from this Phase III study will be positive and eventually lead to approval of the drug from the U.S. Food and Drug Administration.

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Providence is a national, not-for-profit Catholic health system comprising a diverse family of organizations and driven by a belief that health is a human right. With 52 hospitals, over 1,000 physician clinics, senior services, supportive housing, and much other health and educational services, the health system and its partners employ more than 120,000 caregivers serving communities across seven states – Alaska, California, Montana, New Mexico, Oregon, Texas, and Washington, with system offices in Renton, Wash., and Irvine, Calif. Learn about our vision of health for a better world at [Providence.org](https://www.providence.org).

About Providence Southern California

Providence Southern California is the region's largest health system with 11 hospitals, more than 100 clinics, outpatient centers, TrinityCare Hospice and its TrinityKids Care pediatric hospice, Providence High School, home health care services, eight wellness centers, telehealth and numerous physician groups in its Southern California Region. Providence is committed to an enduring mission of outreach to the poor and vulnerable, and last year contributed \$485 million in services, programs and charity care to those in need.

About ONC201

ONC201 is the founding member of the imipridone class of anti-cancer small molecules which selectively targets Dopamine Receptor D2 (DRD2) and ClpP. ONC201-mediated cell death occurs via induction of the integrated stress response and upregulation of apoptotic factors, such as tumor necrosis factor (TNF)-related apoptosis-inducing ligand (TRAIL). This investigational agent is dosed orally and has been well-tolerated and shown clinical activity in Phase I and II trials for specific advanced cancers. Based on clinical trials, the most common adverse events are mild to moderate headache, fatigue, nausea and vomiting. The most common adverse event considered related to ONC201 is fatigue.

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