

Brazil to take part in studies to treat brain cancer in children

Hospital in São Paulo joins international consortium working to develop new therapies for young patients with medulloblastoma.

By [Larissa Beani](#)

Updated Nov. 10, 2025, 4:39 p.m. - Published Nov. 10, 2025, 3:31 p.m.



Ten years ago, businessman Fernando Goldsztein received devastating news from doctors: his 9-year-old son, Frederico, had a brain tumor.

A rare disease, medulloblastoma is a malignant tumor that affects the central nervous system and causes symptoms such as severe headaches, loss of balance, dizziness, nausea, and vomiting. Challenges range from diagnosis to treatment, as therapeutic options remain limited.

The Goldszteins traveled from Brazil to the United States in search of experimental therapies. For a decade, Frederico has successfully managed the disease, while his father has turned his personal battle into a broader effort to expand access to cutting-edge treatments for more patients.

In 2021, Mr. Goldsztein founded the Medulloblastoma Initiative (MBI), a consortium that brings together scientists around the world with the goal of raising funds for clinical trials of new therapies against medulloblastoma.

Since then, the initiative has raised US\$ 11 million and secured Food and Drug Administration approval for two research studies. Sixteen laboratories and hospitals across several countries now participate in the effort.

“In the United States, we were initially told there was nothing more to do for Frederico,” Mr. Goldsztein said. “But I found people who believed we should invest in clinical research for innovative treatments against medulloblastoma. More than 100 families have already reached out and been impacted by the project.”

Strong scientific partners

Throughout this journey, his main scientific partner has been Dr. Roger Packer, a pediatric oncologist who directs both the Gilbert Neurofibromatosis Institute and the Brain Tumor Institute at Children’s National Hospital in Washington, D.C.

Dr. Packer developed the standard treatment protocol for medulloblastoma and is considered one of the world’s leading authorities on pediatric and brain cancers. He has mentored several prominent Brazilian physicians, including Dr. Sidnei Epelman, founder of the Association for Children and Adolescents with Cancer (Tucca) and a pediatric oncologist at Oncoclínicas.

“Today, up to 80% of children diagnosed with medulloblastoma are cured using existing treatments,” Dr. Packer said. “But for children who relapse and whose tumors grow back, our options remain limited, and we must explore new alternatives.”

Last week, Mr. Goldsztein and Dr. Packer met in Brazil to formalize MBI’s first agreement with a national institution. The Einstein Hospital Israelita in São Paulo signed a memorandum of understanding with the consortium on Wednesday, Nov. 5.

The agreement lays the groundwork for scientific cooperation, knowledge-sharing, and priority access to future MBI-sponsored clinical trials. With the partnership, the hospital now becomes a “friend institution” of the nonprofit organization.

“Thanks to the efforts of Fernando and his colleagues, we will be able to offer Brazilian patients the opportunity to access experimental treatments through clinical studies,” said Dr. Luiz Vicente Rizzo, an immunologist and research director at the Einstein Hospital Israelita. “We will combine our expertise with that of institutions facing different realities around the world.”



The future of treatment

The leading hope for treating relapsed medulloblastoma lies in immunotherapies.

“The real breakthrough we have seen in laboratories — and now in humans — is the ability to activate the patient’s own immune system against the tumor, which led to the record-time approval of two MBI-supported clinical trials,” Dr. Packer explained.

Securing approval for such trials typically takes 7 to 10 years. But thanks to the MBI consortium’s mobilization and expertise, the green light came only 6 months after its creation.

“We brought together researchers from across the country [USA] with different approaches and asked them not to compete but to collaborate, so we could identify the best ideas and develop them. That is clinical research,” Dr. Packer emphasized. “And now, a Brazilian hospital is part of the network and can contribute its experience and excellence in the field.”

No timeline has been set for launching clinical trials in Brazil, but the consortium is exploring two major avenues: a personalized RNA vaccine for patients with the disease and the use of genetically engineered immune cells, known as CAR-T cells.

“The vaccine is not like the ones we are used to, which prevent disease,” Mr. Goldsztein explained. “They are drugs for people who already have the disease and work by stimulating the immune system to recognize one of the proteins present in the tumor.”

CAR-T therapy reengineers a patient’s own immune cells in the laboratory and reinfuses them to hunt and destroy tumor cells. The technology is already widely used for blood cancers, such as leukemia and lymphoma, and is now beginning to be explored for solid tumors.

“These are tissues that T cells do not normally attack,” Dr. Packer said. “There are additional challenges in directing these agents to places like the brain — and we must stay alert to potential adverse effects that the treatment may cause.”

With coordinated efforts from teams around the world, families and specialists hope to soon bring better news to children and adolescents facing a recurrence of the disease.

Read more at: <https://saude.abril.com.br/medicina/brasil-passara-a-participar-de-ensaios-clinicos-para-tratar-cancer-cerebral-em-criancas/>