

MBI Achieves \$5.6 Million for Brain Tumor Research in Recent Fundraising Rounds

New investments strengthen MBI's coordinated research pipeline, accelerating promising therapies for children with brain tumors.

WASHINGTON, D.C., September 25, 2026 — The Medulloblastoma Initiative (MBI) today announced **\$2.2 million in new research funding, its latest investment in a coordinated portfolio of programs focused on developing new treatments for medulloblastoma, the most common malignant brain tumor in children.**

The new funding builds on \$3.4 million awarded in MBI's previous funding round, bringing the combined investment across the organization's two most recent rounds to \$5.6 million. Since its founding 5 years ago, MBI has invested approximately \$15 million in medulloblastoma research, accelerating the pace of scientific discovery and its translation to clinical treatment.

The investments are part of MBI's broader research network led by **Dr. Roger J. Packer**, one of the world's leading pediatric neuro-oncologists, who directs the Brain Tumor Institute at Children's National in Washington, D.C. MBI brings together 16 world-class laboratories across the United States, Canada and Germany around a shared scientific strategy. Rather than funding isolated projects, MBI has built a coordinated bench-to-bedside pipeline that identifies and validates new targets, translates promising discoveries into therapies, advances treatments through regulatory development and supports clinical trials.

The model is based on a simple principle: when research funding is scarce, every dollar must be deployed strategically and with a clear path toward impact. MBI brings leading scientists together around common priorities. It matches scientific questions with teams best positioned to address them, reduces unnecessary duplication and promotes collaboration and data sharing across institutions.

MBI places particular emphasis on promising science that has moved beyond early discovery but needs focused funding and coordination to reach the clinic. A critical push can move scientifically compelling opportunities toward patients faster.

The model is already producing clinical progress: two MBI-funded clinical trials are treating patients. Additional therapeutic programs are moving toward FDA submission and clinical testing, and new approaches continue to advance through the pipeline. MIT has highlighted MBI's approach as a potential "new playbook for curing rare childhood diseases," recognizing the potential of strategic funding, scientific collaboration and urgency to accelerate progress.

US\$ 15 million

invested in medulloblastoma research over five years

US\$ 5.6 million

invested across MBI's two most recent funding rounds

US\$ 2.2 million

new research funding announced in this round

Independent Scientific Oversight

An independent External Advisory Board (EAB) composed of internationally recognized experts in pediatric brain cancer reviews and validates MBI's research strategy and funding decisions:

- **Dr. Scott Pomeroy**, Harvard Medical School and Boston Children's Hospital;
- **Dr. Paul Northcott**, St. Jude Children's Research Hospital;
- **Dr. William Weiss**, University of California, San Francisco.

The EAB helps ensure resources are directed toward the most scientifically compelling and translationally promising opportunities.

New Research Investments

- **PRECISION - Dr. Duane Mitchell**
University of Florida
PRECISION is the next generation of MBI-supported RNA-based immunotherapy for medulloblastoma, designed to identify and validate a defined set of tumor antigens for a more precise therapeutic approach. MBI funding will support antigen selection and validation, safety and toxicity studies, and regulatory preparation required to move the program toward FDA submission and clinical testing.
- **CARVengers - Dr. Dalia Haydar, Dr. Nicholas A. Vitanza, Dr. Myron Evans & Dr. Jessica Foster**
Children's National Hospital | Seattle Children's | Children's Hospital of Philadelphia
CARVengers brings together leading CAR T-cell researchers to collaboratively develop and compare therapeutic strategies targeting medulloblastoma, including approaches involving B7-H3, GPC2 and CD2. By testing different CAR designs and strategies across institutions and sharing results, the program aims to identify the most promising approaches and accelerate their path toward patients.
- **PRTC CAR T - Dr. Mohammad Abu-Arja & Dr. Michael Taylor**
Texas Children's Hospital & Baylor College of Medicine
This program is developing a CAR T-cell therapy directed against Protogenin (PRTC), a promising target associated with a progenitor-like population of cells implicated in Group 3 and Group 4 medulloblastoma. Building on encouraging preclinical research, MBI funding will support the translational and regulatory work required to move PRTC CAR T toward clinical readiness and ultimately a first-in-human trial.
- **Dr. Vijay Ramaswamy**
The Hospital for Sick Children (SickKids), Toronto
Dr. Vijay Ramaswamy's laboratory is investigating biological and metabolic vulnerabilities that could lead to new treatments for medulloblastoma, including the mTOR pathway and next-generation compounds designed to disrupt signaling essential to tumor growth and survival. MBI funding will support continued validation of promising therapeutic opportunities and help determine which approaches could progress toward clinical development.
- **Dr. Robert Wechsler-Reya**
Columbia University
Dr. Robert Wechsler-Reya's laboratory uses advanced disease models, genetic screening and therapeutic studies to identify biological dependencies that medulloblastoma cells require for survival and translate those discoveries into potential treatments. MBI funding will support continued investigation of promising vulnerabilities, including approaches aimed at making tumors more susceptible to existing and emerging therapies.
- **Dr. Lena Kutscher**
German Cancer Research Center, Heidelberg, Germany
Dr. Lena Kutscher's laboratory develops advanced models of medulloblastoma to understand how the disease develops, identify therapeutic vulnerabilities and test potential treatments in systems that closely reproduce human tumor biology. MBI is supporting this work to provide the rigorous disease models needed to identify and validate promising therapeutic opportunities for the next generation of treatments.

Continued Investment in Existing Programs

MBI will also renew funding for programs it has supported through earlier stages of development. These are not new program launches, but continued commitments to therapies already progressing toward or through clinical testing. This reflects a central element of MBI's model: supporting promising programs not only until they reach the clinic, but through the critical stages of regulatory development, patient enrollment and generation of clinical evidence.

- **MATCHPOINT - Dr. Duane Mitchell**

University of Florida & Children's National Hospital

MATCHPOINT is an MBI-funded clinical trial for recurrent medulloblastoma combining tumor-reactive T cells, a personalized dendritic-cell vaccine and immune checkpoint inhibition to attack cancer through multiple mechanisms. MBI's renewed funding will support continued patient enrollment and treatment as the trial generates the clinical evidence needed to evaluate this approach.

- **mRNA Vaccine - Dr. Elias Sayour**

University of Florida & Children's National Hospital

Dr. Elias Sayour's mRNA-based immunotherapy uses lipid nanoparticles to deliver tumor-derived RNA and stimulate a broad immune response against pediatric brain tumors, including medulloblastoma. MBI helped advance the platform from preclinical development into clinical testing, where patients are now being treated. Renewed funding will support continued treatment and evaluation of its therapeutic potential.

- **DEFEAT-MB - Dr. Dalia Haydar and Dr. Roger Packer**

Children's National Hospital, Seattle Children's & Children's Hospital of Philadelphia

DEFEAT-MB is developing a dual-target GPC2/B7-H3 CAR T-cell therapy designed to reduce the ability of medulloblastoma cells to escape treatment. Following the evaluation and selection of promising CAR designs, MBI's continued funding will support IND-enabling studies, manufacturing preparation and regulatory work needed to move the therapy toward a first-in-human clinical trial.

About the Medulloblastoma Initiative

The Medulloblastoma Initiative (MBI) is a nonprofit philanthropic organization dedicated to accelerating the development of new treatments and cures for medulloblastoma.

Founded by Fernando Goldsztein in collaboration with **Dr. Roger J. Packer** of Children's National Hospital, MBI brings together leading scientists and institutions in a coordinated research network focused on translating scientific discoveries into treatments for children as quickly as possible.

MBI was created following the experience of Fernando's son, Frederico, who was diagnosed with medulloblastoma at age nine and later experienced a recurrence of the disease. Faced with extremely limited treatment options, Fernando and Dr. Packer set out to build a different model for advancing research - one centered on collaboration, strategic funding, scientific accountability and urgency.

In just five years, MBI has demonstrated what is possible when scientists, institutions, families and donors come together around a shared mission and a new model for accelerating research. We are deeply grateful to everyone who has believed in and supported this effort. **While we have made meaningful progress, the road ahead remains long. Continued collaboration, commitment and support will be essential to achieving what ultimately matters most: bringing better treatments, and one day, cures to children with medulloblastoma.**

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