Shortening the Diagnostic Odyssey for Children and Families:

The Medulloblastoma Initiative

National Press Foundation:

Rare Disease Reporting Fellowship

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Duane A. Mitchell, MD, PhD

Co-director, Preston A. Wells Jr. Center for Brain Tumor Therapy
University of Florida

UFHealth

"The ultimate goal is we want to cure kids with brain cancers and see immunotherapy treatments move into frontline treatments to perhaps avoid or diminish some of the toxicities of standard treatments we use now."

Clinical Trial 1:

The Immunotherapy Protocol

MATCHPOINT*

FDA IND 17298

- Phase 1 clinical trials approved by the FDA on February 29, 2024.
- Enrollment will include patients from UF Health Shands Children 's Hospital /Gainesville, University of Florida and Children' s National Hospital /Washington, DC.
- **Clinical trial are already underway, with the first children receiving treatment**.

MATCHPOINT* is a personalized immunotherapy that leverages the body's immune response to **target cancer cells more effectively and safely** than traditional therapies.

It uses the patient's own T cells in combination with a drug that breaks down the tumor's resistance to treatment. The mRNA extracted from the patient's tumor is used to create the vaccine.

*Medulloblastoma Adoptive T Cell Therapy, DC vaccines, and Hematopoietic stem cells combined with immune checkPOINT blockade - MATCHPOINT

Elias Sayour, MD, PhD

Pediatric Oncologist and Scientist
University of Florida

UFHealth

"Recurrent medulloblastoma in nearly every form is fatal. We are in this to provide hope that we can move the needle."

Clinical Trial 2:

The Medulloblastoma Vaccine

mRNA Vaccine

FDA IND 19304

APPROVED BY FDA

- Enrollment will include patients from UF Health Shands Children 's Hospital /Gainesville, University of Florida and Children' s National Hospital /Washington, DC.
- Patients with recurrent group 4 medulloblastoma will be recruited for the study after the vaccine—previously tested in adults—is tested in a pediatric patient with glioma.

The vaccine developed by Dr. Sayour adapts similar methodologies used in COVID-19 vaccine development. **This approach aims to provide a versatile and mobile treatment that could be distributed globally**.

The vaccine uses mRNA and lipid nanoparticles, similar to COVID-19 vaccines, but with two key differences: first, it uses the patient's own tumor cells to create a personalized vaccine; second, it employs nanotechnology to build a unique delivery mechanism within the vaccine. **This combination holds the promise of reprogramming the patient's immune system to attack the cancer**.

Recruiting

Adoptive T Cell Therapy, DC Vaccines, and Hematopoietic Stem Cells Combined With Immune checkPOINT Blockade in Patients With Medulloblastoma (MATCHPOINT)

ClinicalTrials.gov ID NCT06514898

Sponsor University of Florida

Information provided by University of Florida (Responsible Party)

Last Update Posted 2025-03-13

This is a pilot study in a small number of children and young adults with relapsed/progressive medulloblastoma (MB) looking at the feasibility and safety of adoptive cell therapy plus PD-1 blockade.

Duane A. Mitchell, MD, PhD

Co-director, Preston A. Wells Jr. Center for Brain Tumor Therapy
University of Florida

UFHealth

"I'm hopeful that we're not far from the day of being able to tell a parent with a high-risk medulloblastoma that we're going to treat their child differently. We'll be able to say, based on their risk assessment, that we have a very, very high probability of being able to defeat this disease in the newly diagnosed setting."

Clinical Trial 3:

Precision Adoptive Cellular Therapy

- Close to filing an Investigational New Drug (IND) application with the U.S. Food & Drug Administration.
- Clinical trials will begin after obtaining IND approval.

Precision adoptive cellular therapy uses computer prediction capabilities to identify the real, unique components in each patient's tumor that their immune system could recognize as foreign. This makes it possible to select the specific T cells that **respond effectively to the antigens expressed in the patient's tumor**.

This advancement enables the development of a highly specific vaccine or T-cell therapy targeting those unique antigens in each patient's tumor. It represents **a much more potent approach**, whether through an RNA liposome vaccine or T-cell therapy, propelling science from personalized to precision immunotherapy.

Dr. Nicholas A. Vitanza

Associate Professor,
Hematology/Oncology
Principal Investigator, Ben Towne Center for Childhood Cancer and Blood Disorders Research

Seattle Children's
HOSPITAL • RESEARCH • FOUNDATION

"My goal is to find safer and more effective therapies for children with central nervous system tumors."

Clinical Trial 4:

CAR T Cell Therapy

- Significant advances have already been achieved in non-solid tumors.
- There are also prospects for progress in solid tumors.
- The goal is to initiate a series of clinical trials within the next two years.

CAR T-cell therapy (chimeric antigen receptor) **modifies a patient's T cells so that the T cells can recognize and destroy cancer cells**. This approach has revolutionized the treatment of certain blood cancers, halting disease progression in patients with multiple myeloma and several forms of lymphoma.

CAR T-cell therapies are being developed for pediatric patients with solid tumors, such as medulloblastoma. **This precision attack can preserve healthy tissue and reduce side effects**, offering long-term tumor control — and possibly a cure.


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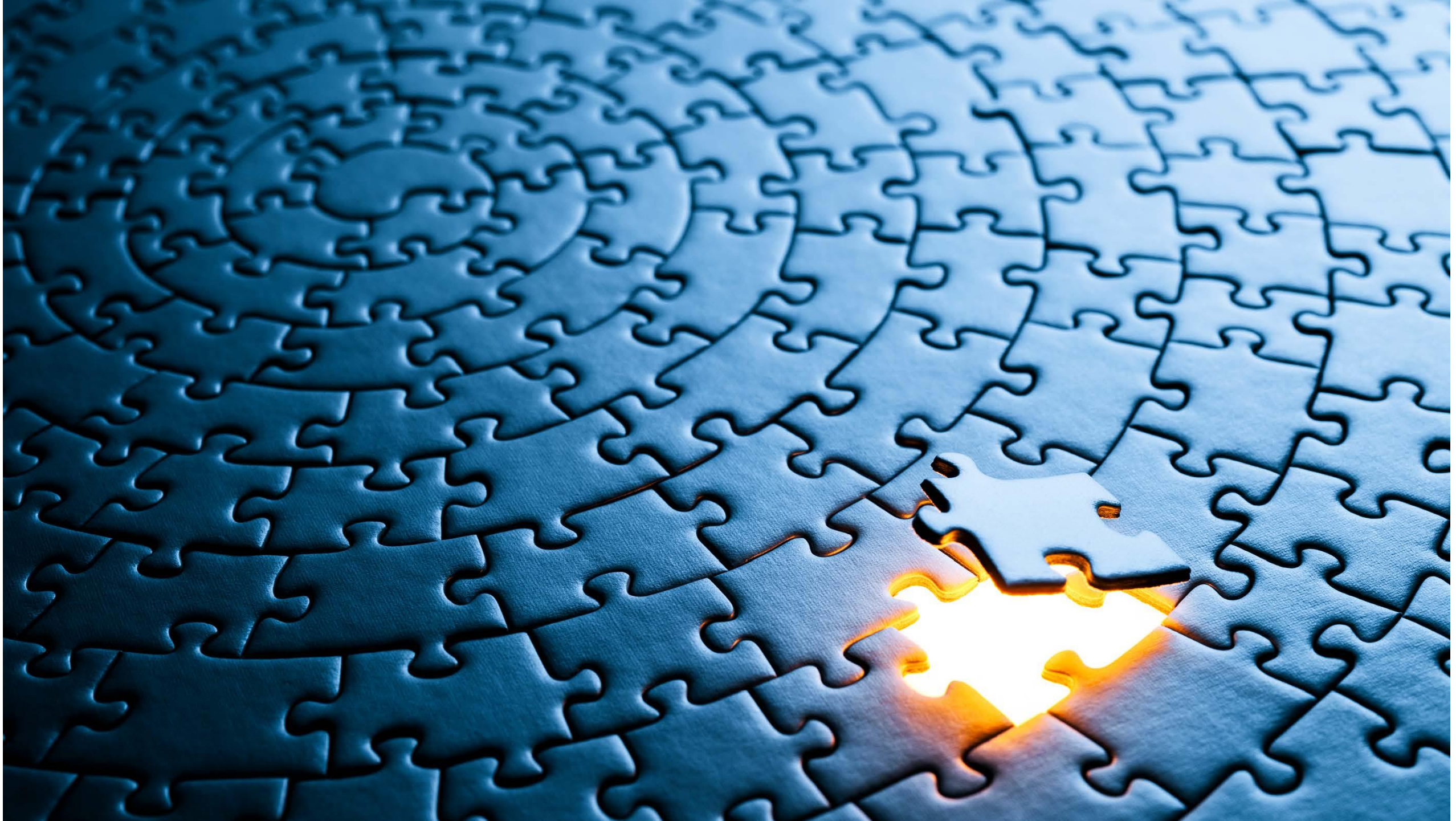


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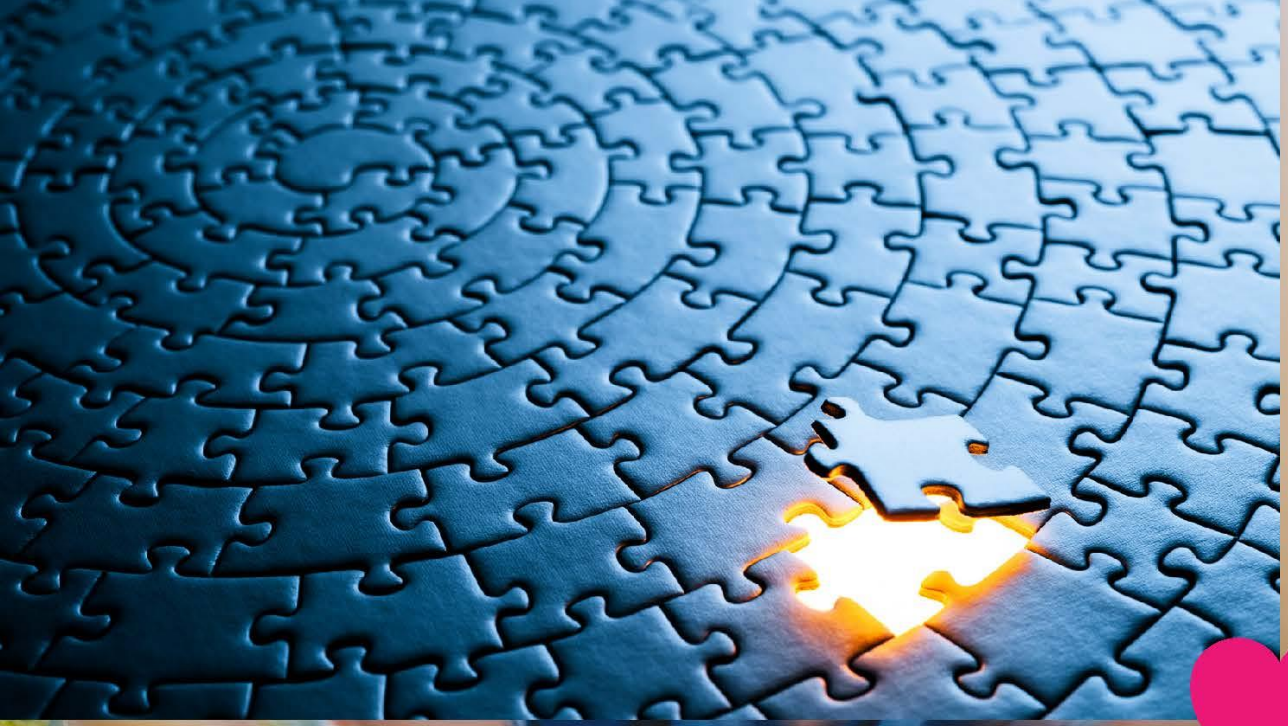

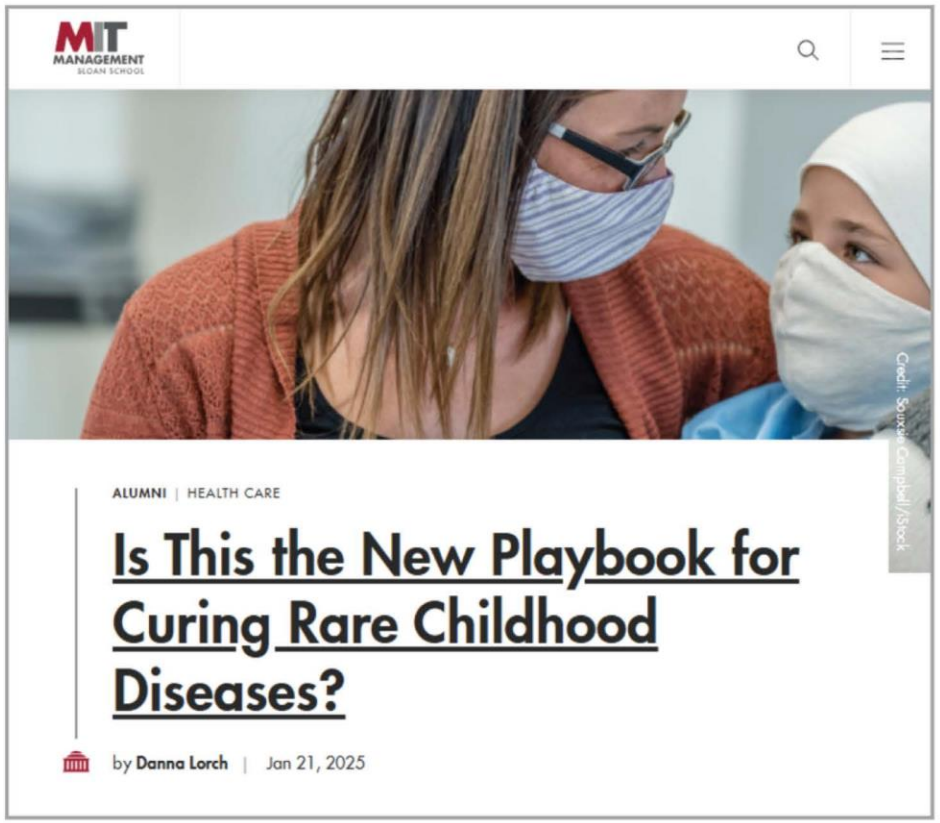

"The MBI blueprint for funding cures for rare diseases is replicable and likely to disrupt the standard way health care research is funded and carried out by radically shortening the timeline."

MBI as featured by MIT Management / Sloan School on January 21st, 2025

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TEAM CAPTAIN

Thank you

Learn more at

mbinitiative.org

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