THE MEDULLOBLASTOMA INITIATIVE

Clinical trials
just around the corner

The Medulloblastoma Initiative Report

August 2023


EVERY CHILD DESERVES A FUTURE
THE
MEDULLOBLASTOMA
INITIATIVE
Give Now!

CONTENTS

We are pleased to share the MBI August 2023 report along with the Children's National Impact Report! Enjoy the exciting developments in the quest for a cure.

OUR JOURNEY: HIGHLIGHTS

Based on Dr. Roger J. Packer's vision, The Medulloblastoma Initiative (MBI), is created with support from a founding donation of USD 3 million.

"Our work will culminate in novel therapeutic approach, not in 3-5 years, but in 18-24 months."

The Cure Group 4 Consortium is established with Dr. Roger J. Packer as principal investigator. Three institutions in the US and one in Canada constituted the initial Consortium group.

Children's National · SickKids · SBP Medical Discovery Institute · UFHealth University of Florida Health

JUNE **JULY**

2021

AUG

By August 2021, MBI's consistent fundraising efforts had secured the first million USD from non-founder donations.

The Consortium constitutes its independent advisory board, formed by three renowned researchers — Drs. Ian F. Pollack (UPMC Children's Hospital, Pittsburgh); Scott Pomeroy (Harvard School of Medicine); and William A. Weiss, (UCSF Helen Diller Family Comprehensive Cancer Center, San Francisco). Their role is to monitor the development of research in accordance with ethical and scientific standards, as well as the allocation of resources to the activities for which they were raised.

SEPT

McMaster University

Dr. Sheila Singh joins the Consortium to work on the development of human stem cell lines capable of generating Group 4 medulloblastoma tumors in laboratory models for testing of new drugs.

DEC

Dr. Tobey McDonald joins the Consortium to work with molecularly targeted therapies.

Emory University School of Medicine

The year 2021 ends with USD 5 million raised.

US$ 5 mi

JAN

2022

A **major breakthrough** is achieved: Dr. Sheila Singh establishes the first line of human stem cells capable of generating Group 4 medulloblastoma in a laboratory model — a critical basis for multiple investigations aimed at discovering new drug candidates for clinical trials.

"If the problem is going to be solved, it's going to be solved by this group."

MAR

Dr. Javad Nazarian joins the Consortium to develop a platform for liquid biopsy — a simple blood draw that among others may enable doctors to monitor how a child's tumor is responding to treatment.

Children's National

APR

Work on PARP protein inhibitors — potent but non-toxic drugs that have shown promise in the treatment of brain tumors — starts as Drs. Lena M. Kutscher and Carl Koschmann join the Consortium.

KiTZ Heidelberg · Michigan Medicine

MAY

The first face to face Consortium workshop is was held at the Children's National Research & Innovation Campus, in Washington DC.

JULY

The MBI is featured in the MIT Sloan alumni website. The story told by MBI founder Fernando Goldsztein was chosen one of the top 10 MIT Sloan alumni stories in 2022.

MIT Management Sloan School

SEPT

Three Consortium members co-author an article published in the prestigious journal Nature, describing a groundbreaking discovery that traces the origins of medulloblastoma. The MBI support is acknowledged in the article.

nature

THE MEDULLOBLASTOMA INITIATIVE

Dr. Dalia Haydar, from Children's National, joins the Consortium to expand research on CAR-T cells that harness the body's own immune system to seek and destroy tumor cells.

"Bringing all of the investigators together - people who see things from different angles enchances our focus on how we make a therapy work."

Texas Children's Hospital

Dr. Michael Taylor, a world-renowned physician, neurosurgeon and researcher and a member of the Consortium since the early days in 2021, takes his work with medulloblastoma to Texas Children's Hospital, thus expanding the Consortium reach.

The MBI is covered in a story in newspaper O Globo — a powerful daily engaging readers in Brazil and abroad. Because the technological advances of many fields have not resulted in better cancer treatments for children, the impact of the MBI has been incredible, engaging institutions, researchers, and donors to focus on curing medulloblastoma, which accounts for nearly 50% of pediatric brain tumors.

O GLOBO


Saúde

LUTA PELA CURA

'A doença do meu filho mudou a minha vida', relata empresário

The MBI is presented at MIT to a packed Wong Auditorium as part of the Ideas Made to Matter Talks in the 2023 MIT Sloan Alumni Reunion.

MIT MANAGEMENT SLOAN SCHOOL

The year 2022 ends with USD 8 million raised.

US$ 8 mi

OCT NOV DEC JAN MAR JUNE JULY AUG

2023

Dr. Wechsler-Reya's lab moves to Columbia University, where the team will continue the Consortium's essential work.

COLUMBIA

"Children should not have cancer," stated MBI founder in a video interview with EXAME, a major Brazilian news and business magazine, with massive repercussions.

exame.

MBI launches an effort to engage scientific partners in Brazil for the planning of clinical trials.

Children's National produces and launches the No Time to Lose video campaign, sealing the partnership between the MBI and Children's National, where Dr. Roger Packer, Cure Group 4 Consortium Principal Investigator, is based.

The most important highlight of the second Cure Group 4 Consortium Workshop was the announcement that the Consortium's advances **have enabled the planning of two clinical trials to be launched over the next 6 to 12 months.**

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FOREWORD: CLINICAL TRIALS ARE HERE

In 2021, we began our efforts at The Medulloblastoma Initiative (MBI) with the promise of finding new therapeutic approaches for medulloblastoma, the most common brain tumor in children, in the shortest possible time. Each year, thousands and thousands of children are diagnosed with this terrible disease.

The treatment available worldwide for medulloblastoma was created over three decades ago, around the same time when fax machines and VCRs were in use. Since then, we have witnessed a real technological revolution across all imaginable fields. Medicine has evolved significantly. Sadly, however, this progress has not translated adequately for children with brain tumors. In addition to the fact that a high percentage of children diagnosed with medulloblastoma perish, the survivors often suffer serious long-term side effects due to the high level of toxicity from the current treatments.

Thanks to our generous donors, who so far have contributed more than 8 million dollars for research — and also thanks to the tireless work of Dr. Roger J. Packer, who coordinates our spectacular group of scientists spread across 13 laboratories in the United States, Canada, and Germany — we are advancing rapidly towards a cure. And I am thrilled to announce that we expect two clinical trials in patients to begin within only 6 to 12 months! Additionally, we have two more clinical trials in the pipeline for the near future! All of this is the result of an enormous effort from our donors and scientists who decided to change the sad situation for children with brain tumors, children who have been overlooked in research investments. And now, with the trials, we are getting closer and closer to saving thousands of lives! However, the much awaited clinical trial phase, which is the final stretch to find a cure for medulloblastoma, requires considerable investments to recruit, treat, and follow the patients. We hope we can continue to count on the support received so far and to gain new partners in this endeavor. We know that only research can find a cure, and that, as written in the Talmud, “whoever saves one life, saves the world entire.”

Fernando Goldsztein
Founder, The Medulloblastoma Initiative

www.linkedin.com/in/fernando-goldsztein

OUR ECOSYSTEM

SUPPORTERS / DONORS / MEDIA

THE MEDULLOBLASTOMA INITIATIVE

- **Leading the process**
- **Fundraising**
- **Awareness raising**

Children's National Hospital

- **Operational support**
- **Fundraising**
- **Scientific leadership**

The Cure Group 4 Consortium

13 labs in the US, Canada and Germany

- **Research**
- **Development**
- **Clinical trials**

THE CURE

To fulfill its mission of finding a cure for medulloblastoma, the most common pediatric brain cancer, the MBI operates in an ecosystem that is fueled by the support from private donors, individuals who contribute through networking and awareness building, and individuals and media outlets that disseminate our message. All these assets are channeled by the MBI to Children's National in Washington DC, a fundraising partner and the institution in charge of managing, overseeing and linking financial resources to the researchers, laboratories, and institutions that make up the Cure Group 4 Consortium. The Consortium then focuses on research and development and coordinates the clinical trials that will translate into new treatments, and hopefully a cure for medulloblastoma.

Praise for the MBI

"Whoever saves one life saves the world entire," twitted Organization of American States (OAS) Secretary General Luis Almagro, about medulloblastoma and the MBI. Almagro was introduced to the MBI during an event attended by around 20 ambassadors from several countries.

OPAS
Organização Pan-Americana da Saúde
Organização Mundial da Saúde Américas

"Some rare diseases, such as medulloblastoma, receive very little attention from developers of new medications. Therefore, the work of MBI is highly important as it pioneers in mobilizing resources, originally from Brazil, to support the development of new therapies."

Jarbas Barbosa
Director at the Pan American Health Organization/World Health Organization (PAHO/WHO).

The year 2023 brought an unexpected addition to my public activities — I met my friend Fernando Goldsztein and the MBI. I have been a career diplomat since 1991. I am used to the long cycles of my profession: it takes time for concrete results to be perceived by society. Thus, making a contribution to the MBI meant a paradigm shift. Contributing to networking and building international awareness for the MBI, with its amazing purpose and its ability to combine the best of science and with unprecedented modeling, focused on results that will change the future of humanity, has been the most rewarding experience of my professional life. Thank you, Fernando, for the opportunity.

Ambassador Otávio Brandelli
Brazil Representative to the
Organization of American States (OAS)

OAS More rights for more people

Jarbas Barbosa, Fernando Goldsztein and Otavio Brandelli


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Put in a good word

Telling the MBI story continues to be a major form of support for our project. Since April 2023, two interviews have greatly contributed to disseminating the MBI milestones and vision.

In June, the MBI founder was interviewed by Lucas Amorim, Executive Editor of EXAME, an important business and economic Brazilian media outlet. The article focused on the upcoming clinical trials while providing context for the creation of MBI and the funding gap for pediatric research. As stated, even in the US, for every dollar the government invests in cancer research, only 4 cents go towards pediatrics; if the upcoming trials are successful the funding landscape is likely to change. Quoting Goldsztein, Amorim also highlighted the power of the MBI idea to produce action: ***"We didn't join a project that was already in motion. We took the initiative to create a project that mobilizes scientists from around the world. And to move on with clinical trials, we need more resources."***

exame. Check out the full interview at Exame's Website

Saúde

I LIVED TO TELL

THE FIGHT FOR A CURE

'My son's disease changed my life', says enterpreneur

A family united. Fernando with his wife Barbara and their two sons, Frederico and Henrique

FERNANDO GOLDSZTEIN*

Nineteen years ago, I had sarcoma, a very serious cancer. I had to remove 25% of my bod underwent

or Boston, they are all still using a treatment created 40 years ago. Governments invest very little in research. In a ranking of scientists who publish the most about the disease worldwide we realized that wasn't

children in the same situation and are supporting us. If families come together, they can overcome anything. We treated Frederico at world-class centers. When

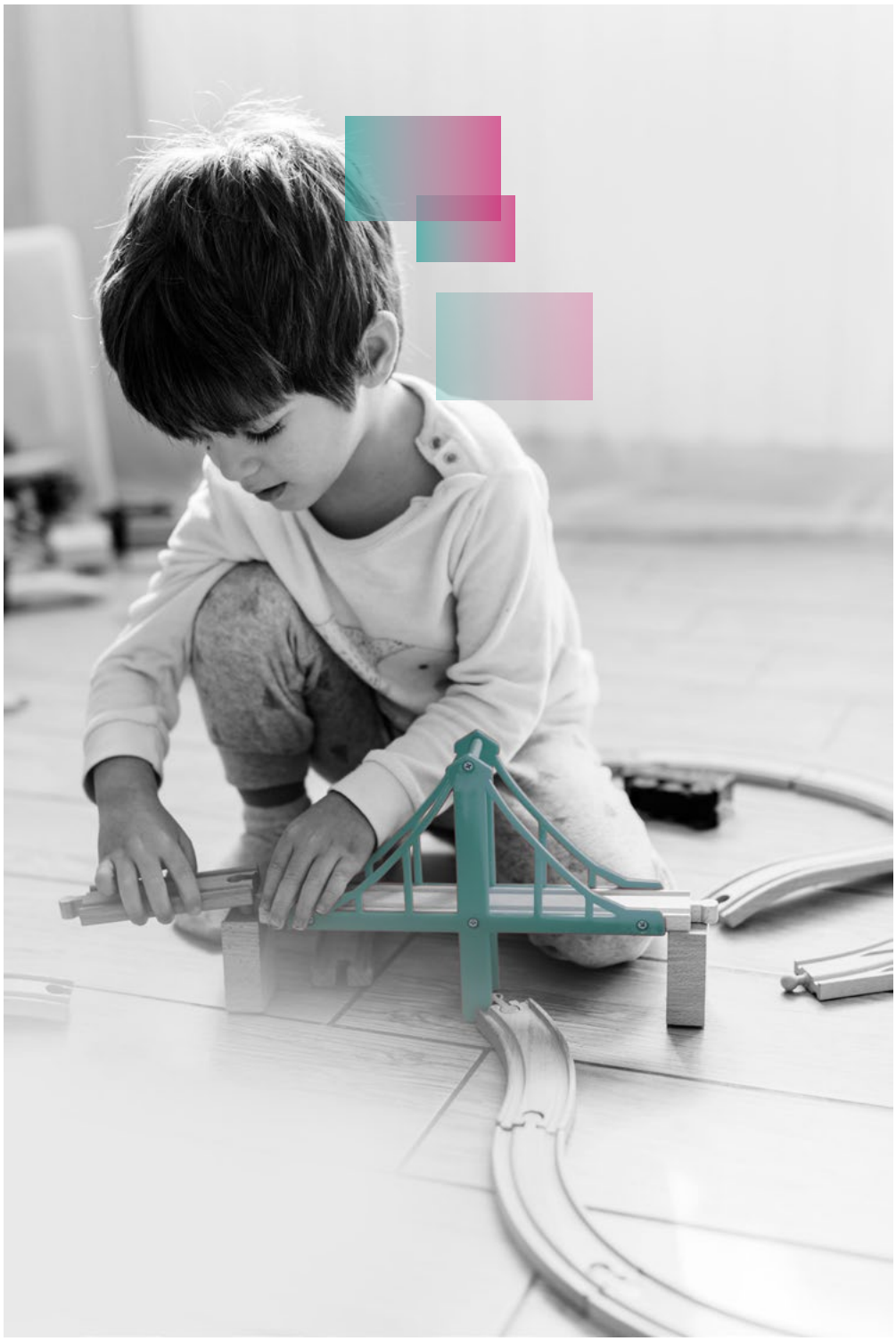
O GLOBO

Check out the full interview at O Globo Website

In the National daily O Globo newspaper — one of the most prestigious newspapers in Brazil — an article by special reporter Janaína Figueiredo dedicated a whole page to the MBI. The interview with the MBI founder shows how illness has no borders, and how fast the word is being spread about the MBI:

"I have been contacted by people from Israel, Iran, the Netherlands, the USA, people who have children in the same situation and are supporting us. If families come together, they can overcome anything," said Goldsztein. *"We treated our son at world-class centers. When we realized that wasn't enough, we decided to get more involved and ensure that science reaches all patients."*

You can read these and other stories at www.mbinitiative.org



Children's National Impact Report

Check the next pages for the Impact Report produced by Children's National, MBI's strategic partner.

Leah makes a sign to celebrate her family's success raising more than $50,000 for The Medulloblastoma Initiative (see last page for story).

Potential Cures in Sight

In late 2021, The Medulloblastoma Initiative (MBI) set an ambitious two-year goal. It aimed to launch clinical trials to advance new treatments for medulloblastoma – the most common malignant brain cancer in children – with a focus on relapsed Group 4 tumors. Today, less than two years later, we can celebrate that our goal is in sight.

Duane Mitchell, M.D., Ph.D., of the University of Florida will embark on three distinct immunotherapy trials within the next six to 18 months (see feature on the next page). The trials will test promising approaches that enable doctors to use a patient's own cells to create personalized tumor vaccines and therapies.

Dr. Mitchell will work alongside fellow investigators in MBI's global scientific dream team – the Cure Group 4 Consortium. The forthcoming trials are just one aspect of the Consortium's progress made possible by your support through MBI.

We present this report with heartfelt gratitude for your generosity or for your interest in philanthropic partnership. Gifts to MBI directly support research, which provides new hope for patients and families – particularly those facing relapsed tumors with no established care protocols.

Thank you for joining our global quest, which unites 13 laboratories worldwide in pursuit of one goal: to find cures that save lives.

Fast Forward

Many children with brain tumors may run out of time if we leave them behind. I have met many patients and families confronted with medulloblastoma. Despite great recent advances in biomedicine, the treatment protocol has remained largely the same for over three decades. There is no established treatment for patients like my son, whose tumor has relapsed. Group 4 tumors like his are the most common type of medulloblastoma, but we still lack the ability to treat them consistently.

My son has received the gold standard treatment for the disease: a protocol approved in the 1980s! That's right – the same time that the CD, the fax machine and the VCR were released. Blockbuster had just opened its doors. Internet, email and cell phones were largely things of the future. A situation like this is not acceptable. We can do more for children – much more!

There are not enough clinical trials for children. More than one out of every four children diagnosed with medulloblastoma still perish. Those who survive often face lifelong impairments from standard treatment. I founded The Medulloblastoma Initiative in 2021 with the aim of changing this picture and saving thousands of children every year.

–Fernando Goldsztein, Founder, MBI

"The Consortium has accomplished a lot over the last 12 months. It is clear that soon we will have tangible progress due to MBI and the team's efforts. These include an immunotherapeutic approach to Group 4 medulloblastoma and progress toward molecularly targeted therapies."

–Roger J. Packer, M.D., Director, Brain Tumor Institute, Children's National Hospital; Principal Investigator, Cure Group 4 Consortium

Upcoming Clinical Trials – An Interview with Dr. Duane Mitchell

Dr. Duane Mitchell is a pioneer within the emerging field of personalized cellular therapies – or immunotherapies – for brain tumor patients. He co-directs the Preston A. Wells Jr. Center for Brain Tumor Therapy and leads the Brain Tumor Immunotherapy Program at the University of Florida. He brings to these positions – and to his work with the Cure Group 4 Consortium – a lifetime fascination with the potential of curing cancer using a patient's own cells. His research includes

trailblazing clinical trials for medulloblastoma (Re-MATCH) and gliomas (PEACH) that established the approach's clear potential as a platform for cures. This has helped make the University of Florida a global destination for brain tumor immunotherapy.

Dr. Mitchell shared his excitement about three upcoming clinical trials made possible by MBI's support.

How could immunotherapy cure Group 4 medulloblastoma?

While two patients may share the same diagnosis, say of medulloblastoma, we can observe that the actual cancers between those two patients can be quite different biologically. This complexity is one reason we have had uneven success treating medulloblastoma. Immunotherapy may solve this dilemma. We can tailor cellular therapies to each patient's own immune system and their unique tumor.

Our lab creates precision immunotherapies using RNA, genetic material derived from a patient's own cancer. RNA provides a blueprint of all of the proteins that make up the cancer. This information enables us to design vaccines and to train T cells (a type of white blood cell that plays a key role in the human immune system) to attack tumors as if they were foreign invaders to the body.

We generate T cells from the patient's own immune cells. These T cells are like soldiers that, if instructed properly, seek and kill whatever they've been trained to recognize as their target. We train the T cells to recognize the unique pattern of antigens and the unique pattern of proteins that are expressed in the tumor.

Injecting the vaccines and the T cells stimulates immune response, and we can strengthen the response. It's a very individualized therapy. We believe that it holds the best potential to deal with some of the complexity that has thwarted our attempts to control these tumors with standard drugs.

How have your previous trials set the stage for the three clinical trials you propose for Group 4 medulloblastoma?

I call our research approach "bench to bedside and back to bench." We took observations from earlier trials and went back to the laboratory to improve the approach.

The Group 4 medulloblastoma trials we will pursue absolutely build on this foundation. They represent the third generation of adoptive cell therapy. We have learned how to generate, expand and activate

T cells much more efficiently than we did in the past. We have learned how to drive them towards a longer-lived type of T cell called a memory T cell.

In the current research, we're focusing on how to tailor those to the best targets expressed within the cancer. We believe this will lead to a much higher probability of achieving successful control. Our ability to launch these trials is expedited because of these foundational trials and our clinical experience.

Could you please describe the three clinical trials?

CLINICAL TRIAL 1: The first clinical trial is of a personalized immunotherapy treatment. The personalized adoptive cell therapy approach takes the patient's genetic material — the RNA derived from their own tumor — to load dendritic cells, which are really the generals of the immune system that instruct the T cells what to attack. We use dendritic cells both as a vaccine and as a platform for expanding a large number of T cells that are activated against the patient's own tumor.

In addition to injecting these cells as a vaccine, we also grow billions of these personalized T cells outside the patient in a clinical grade laboratory using growth factors. This produces much larger numbers of cells than we can achieve inside the body. These additional cells are injected into the patient, who we continue to boost with vaccines. Those T cells then circulate through the body. They traffic to the brain and attack the brain tumor cells wherever they see them. That is the goal.

We are the only lab in the world doing this particular approach.

The first trial for Group 4 medulloblastoma will also include an immune checkpoint blockade. We learned from our earlier studies that we need to help the activated T cells persist long term. The checkpoint blockade prevents the T cells that we're infusing from becoming exhausted or deactivating when they reach the tumor. With it, they persist, expand and remain in a much stronger activated state, leading to better tumor clearance.

CLINICAL TRIAL 2: The second trial is an RNA nanoparticle vaccine. This is a newer strategy developed within our research program. When we looked at our platform for using tumor-derived RNA to load dendritic cells to stimulate the immune system, we asked whether we could package the RNA into a carrier that can deliver dendritic cells in the body. We want to test whether we can deliver the RNA directly as a vaccine.

Today, we're all pretty familiar with RNA vaccines due to COVID-19. These vaccines use m-RNA packaged into a courier which we call a liposome to stimulate the immune response. We actually began this work years before the pandemic. The approach is to use a personalized RNA extracted from the patient's own tumor, but this time the liposome can deliver the RNA directly to the cells of the immune system in the body. It's injected intravenously. We have shown that this can stimulate a very potent immune response.

This approach is currently being tested as a new strategy for treating glioblastoma in adults and soon will be explored for high-grade brain tumors in children. We think that the approach can be adapted to treat medulloblastomas as well. It could be a very innovative and exciting strategy for Group 4.

CLINICAL TRIAL 3: The third trial involves elements of the first two trials. We call it a precision adoptive cellular therapy treatment. It uses a computer algorithm and prediction capability that we have developed called Open Reading Frame Antigen Analysis. This can study the patient's unique immune system alongside the specific genes being expressed in the patient's tumor compared to information about every protein that is normally expressed in the human body.

We anticipate this will enable us to identify the real, unique components in each patient's tumor that their immune system could recognize as foreign. We then can develop an RNA-based vaccine that exquisitely targets those tumor-specific antigens, whether they are mutations or uniquely expressed proteins.

Once we've profiled tens of thousands of genes, we may narrow it down to 50, 100 or even 300 genes. We then can make a very specific, very tailored vaccine or T cell therapy that homes in on those unique antigens of the patients.

We have also worked on a process to identify which of the T cells are actually responding. Instead of expanding large numbers of T cells to great capacity, we can really select the very specific T cells that are responding well to the very specific antigens that are being expressed in the patient's own tumor. We believe we'll get hundreds, if not thousands-fold enrichment of the right T cells targeting the right antigens. This is a much more potent approach — either for an RNA liposome vaccine or for a T cell therapy approach. It will bring us from personalized to precision immunotherapy.

How quickly can we launch these trials?

The first two trials take approaches that have been in the clinic or are already starting in clinical trials. We will likely need about six to 12 months to start clinical trials. For each, we are proposing a pilot clinical trial of between six and nine patients. That is enough to understand the safety profile, the immune response and whether we've seen clinical responses in any of the patients that were treated. We believe that's the right amount of information to then go for a much larger trial, should those initial findings really generate the enthusiasm to go forward.

The third trial must go through what's called an investigational new drug (IND) application. The first two trials will run under existing INDs. For the third, we need to apply to the U.S. Food & Drug Administration. This will likely take 12 to 18 months.

How close do you think we may be to saving lives among patients with the relapsed Group 4 tumors?

As a scientist, I know the reality that we currently don't have a standard of care that can lead to long-term cures in that relapse setting. That said, in our own clinical trials, we have some long-term survivors who've been treated with immunotherapy. The largest number of our longest-term survivors were treated prior to subtyping some of these tumors, but they were mostly Group 3 relapses.

I'm cautious but very optimistic that in the next five years we're going to see different outcomes for patients with recurrent Group 4 medulloblastoma. This will be based on biologically driven, molecularly targeted combination approaches with immunotherapy.

It may turn out that combinations of targeted therapy and immunotherapy give us a different kind of synergy than we were expecting — which is why the synergistic approach of the Cure Group 4 Consortium is important.

I honestly think that in the next five years we're going to see the benchmarks in this field changed significantly. 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. For those that do have a relapse, we'll be able to say the we have effective treatments that we think have a high probability of controlling the disease.

I've not seen the field accelerate and change as dramatically as the field of immunotherapy has over the last 10 years. I know that we haven't had the home runs in brain cancer because we're not curing 100% of patients. But I do think the advances are there, and the field is really building quite a bit of momentum towards positive outcomes.

On a personal note, what motivates you to pursue this work?

1993 is literally 30 years ago. Right?

Three decades ago, I was thinking about whether I was going to study pre-med or computer engineering. I like computers and I like biology. I read Stephen Rosenberg's book "The Transformed Cell" about curing patients with melanoma using immunotherapy. I was transfixed by the idea that you could cure cancer with the immune system. It just made sense to me. Cancer is such a dreaded disease that people fear so much. I'm transfixed by the notion that your own body could fight this off — with help, obviously.

I will always remember being invited to an event to speak about the importance of philanthropic support for research. I met parents who had kids that were diagnosed with brain cancer at that event. Just the idea of being faced with a child battling cancer — I feel like no child should have to go through that.

Children have an optimism that they should never, never lose. I know how difficult the challenge is that we are facing. If we can contribute to a solution, I couldn't think of anything more worthwhile to do.

Additional Highlights from the Cure Group 4 Consortium

Cell-line development: The laboratory of Sheila Singh, M.D., Ph.D., at McMaster University in Ontario, Canada, continues to pioneer the world's first replicable human cell line capable of generating Group 4 medulloblastoma tumors in mice. Her lab provides Group 4 cells to investigators across the Consortium. These cells provide the basis for multiple investigations aimed at discovering new drug candidates for clinical trials. Consortium investigators using these cells include Drs. Kutscher, Mitchell, MacDonald, Wechsler-Reya, Haydar and Ramaswamy.

The team initially developed two human cell lines, but only one remains viable. However, the viable line is highly productive. "When we got home from the Consortium meeting in May, we were able to harvest cells from several large tumors," says Dr. Singh. "We now have 30 million cells to ship to all of our collaborators. Every investigator emailed us with great excitement hoping to receive a vial of cells. Also, Dr. Kutscher did methylation profiling and it not only confirmed the line as a true Group 4, but even suggested more possible drug targets to test."

Drug development: Much of the discussion at the Consortium's meeting focused on the ongoing search for molecularly targeted therapies. Candidate drugs and combined therapies under investigation include:

- **BMI1 in combination with PI3K inhibitors and PLK1 inhibitors (Singh lab)**
- **PI3K and HDAC inhibitors (Wechsler-Reya lab in collaboration with Singh lab)**
- **ONC206, likely in combination with other drugs (MacDonald lab)**
- **PARP inhibitors, likely in combination with other drugs (Kutscher lab)**

"I think great progress has been made as we work together systematically through the huge obstacles posed by the difficulty of modeling this tumor. Each time we make small incremental breakthroughs and they are all starting to add up and push us toward more transformative progress."

— Sheila Singh, M.D., Ph.D., Director, Centre for Discovery in Cancer Research, McMaster University

Genetic discoveries: Brian Rood, M.D. of Children's National used state-of-the-art proteomic technology to identify a gene that appears to function as an important driver in Group 4 medulloblastoma. Similarly, Michael Taylor, M.D., Ph.D., of Texas Children's Hospital has identified a novel genetic driver (ZIC1/ZIC4) for the majority of Group 4 tumors. These findings open the door to therapeutic targets.

The Consortium also developed the capacity to conduct experiments using genetic information from specific patients. The team can share the RNA and DNA molecular characterization of individuals online. "Coupling the cell line and animal model work with the molecular data obtained from individual patient tumors will expedite work and lead to more definitive experiments," says Dr. Packer.

Liquid biopsy: Javad Nazarian, Ph.D., of Children's National continues to develop a platform for liquid biopsy. Soon, a hassle-free blood test may enable doctors to monitor how a child's medulloblastoma is responding to treatment, detect tumor growth and provide an earlier indicator of recurrence. His team aims to have the platform operational by the end of 2023.

Goal Accomplished: A Community Rallies Around Leah

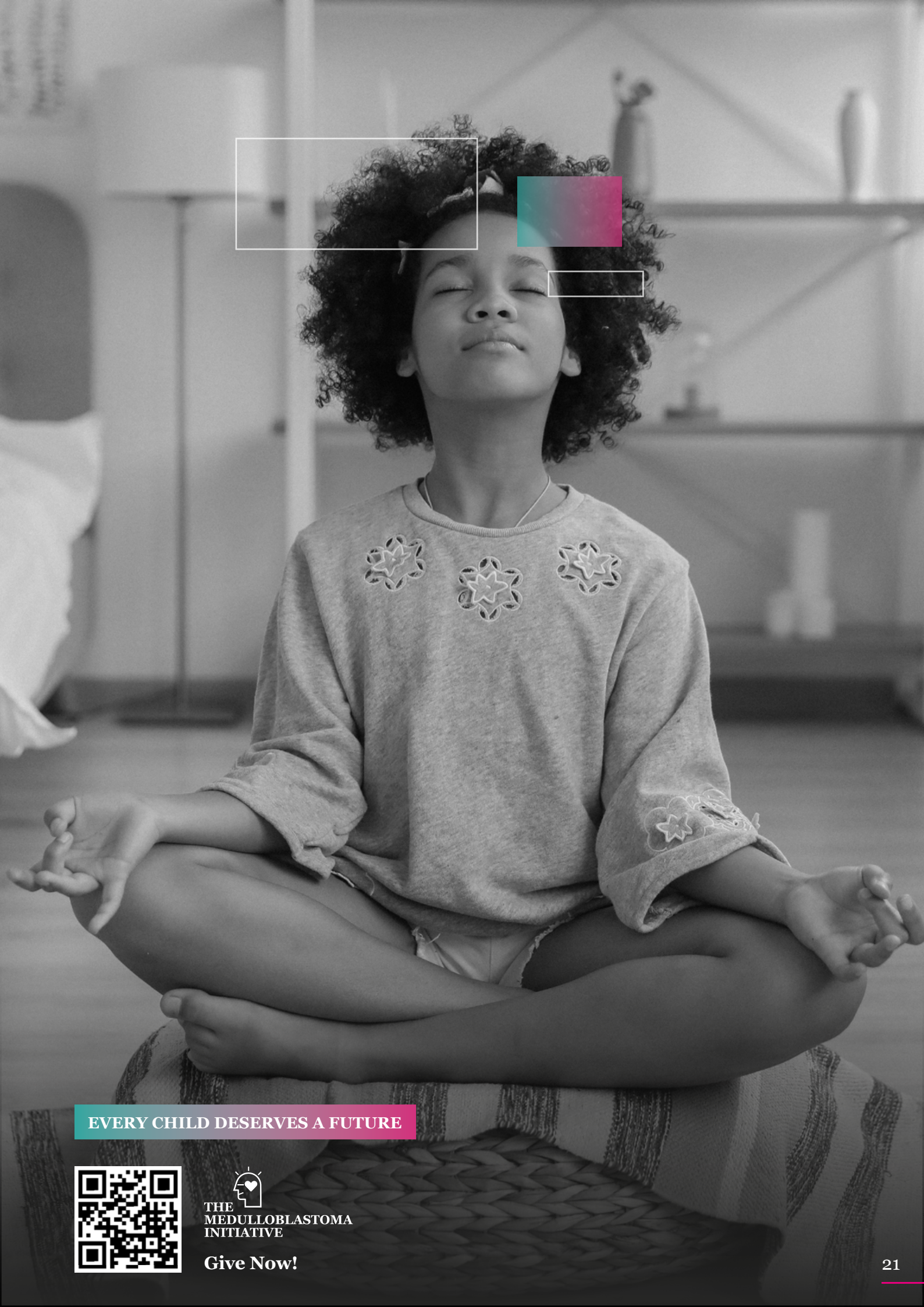
Leah, 11, is a bright and talented middle school student undergoing treatment for relapsed Group 4 medulloblastoma. In 2022, her parents Elena and Neal began supporting MBI philanthropically and encouraging family and friends to join them. They set a goal of raising at least $50,000, and their supporters responded overwhelmingly. This summer the family celebrated the accomplishment of their goal. They look forward to the progress that the donations will fuel for Leah and other patients like her.

A Global Stage

On June 2, 2023, MBI Founder Fernando Goldsztein spoke at a gathering of entrepreneurs hosted by MIT, his business school alma mater. He spoke of MBI's mission and shared a message: "Not if, but when we find the cure for this disease, I'm going to dedicate myself to the next one and the next one. So, for pediatric brain cancer, that's going to be the goal of my life. And I'd like to leave a message here that life is not all about accumulating assets. We all have to do good … We all have to do something to help other people."

Thank You for Your Partnership

MBI and its Cure Group 4 Consortium is driving unprecedented progress through the power of synergistic collaboration. Each MBI donor is a partner in our global effort. Your generosity – both past and prospective – increases hope for every medulloblastoma patient, particularly those with high-risk, relapsed tumors. Thank you for making a difference for them.



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Are you still using these technologies?

The 1980s: This is when the medulloblastoma treatment protocol was established. And it is still being used. In addition to not curing a high proportion of children affected by medulloblastoma, the kids who survive face severe side effects for the rest of their lives.

The MBI knows that research can change this scenario. And this is why we work to link private donors to leading scientists. Join us now — spread the word and make a donation for research that will find a cure for this brain cancer.

Help now!

THE MEDULLOBLASTOMA INITIATIVE

www.mbinitiative.org