THE MEDULLOBLASTOMA INITIATIVE

Harnessing the Power of Collaboration to Defeat Medulloblastoma

MB INITIATIVE REPORT

June - December 2021

Whoever saves one life saves the world entire.

- The Talmud

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Children's National

FOREWORD

The notion that research in pediatric cancer, and particularly pediatric brain cancer, is lagging behind the research done on any type of adult cancer seems to be common knowledge, recognized across the board by the media and governmental institutes alike. "The medical knowledge of how to treat brain cancer in children is far behind other adult cancers," states a CNN article[1] published last September as part of National Childhood Cancer Awareness Month — and, according to the National Cancer Institute (NCI), "clearly, more research is needed to develop new, more-effective, and safer treatments for childhood cancer."[2]

The pediatric cancer research gap results, to a large extent, from the limited funding available for what is considered a "rare" disease — pediatric cancer affects far fewer people as compared to adult cancers. Less pediatric cancer research means more toxic treatments, more adverse effects, and in some cases — like relapsed medulloblastoma — no standard treatment protocol at all. Thus, in the case of relapsed medulloblastoma, the survival of children is very poor.

In what could be a disheartening scenario, collaboration can be a game changer. And indeed, changing the game through collaboration is the gist of The Medulloblastoma Initiative: a unique, direct collaboration between a fundraising effort and the researchers striving to find a cure. The inspired vision that made The Medulloblastoma Initiative position itself as an active player in the research undertaking, with the research team and institutions acknowledging and facilitating that role, has translated into greater hope for all, with impressive results that are outlined in this brief report.

The content provided here refers to the first six months of collaboration between The Medulloblastoma Initiative and the Cure Group Four Consortium. It serves to thank and inform those who have already donated, keeping their enthusiasm about the many achievements over such a short period of time; and it also serves to inspire others to come aboard and keep up the good work, driving the research forward. There is no limit to what we can do together.

“Changing the game through collaboration is the gist of The Medulloblastoma Initiative”

[1] LaMotte L. All parents should be 'outraged' in fight against pediatric brain cancer, say two CNN correspondents. CNN Health. Available at: https://edition.cnn.com/2021/09/07/health/childhood-brain-cancer-wellness/index.html Accessed 10 March 2022.
[2] National Cancer Institute. Research on Childhood Cancers. Why Research is Critical to Progress against Childhood Cancer. Available at https://www.cancer.gov/research/areas/childhood#why-research-is-critical-to-progress-against-childhood-cancer Accessed 10 March 2022.

REMARKABLE PROGRESS, WITH A SENSE OF URGENCY

Fernando Goldsztein
Founder, The Medulloblastoma Initiative

It was with great hope that we at The Medulloblastoma Initiative received the first scientific follow-up report on the research activities carried out from July to December 2021 by the Cure Group Four Consortium. At the beginning of this period, the Consortium led by Dr. Roger J. Packer included seven laboratories housed in four institutions. Since then, the group has integrated additional researchers with synergistic activities. There are now nine laboratories in the United States and Canada, bringing together some of the most renowned researchers on the planet in this area of medicine.

As evidence of the effectiveness of the research model adopted, Dr. Packer was already able to report remarkable achievements in the first six months of operation. So far, the pace of work indicates that it will be possible to achieve the goals proposed for the end of the first year. As a highlight, the group made a fundamental discovery: the identification of the probable mechanism of medulloblastoma development. A scientific paper reporting this finding, which will acknowledge the support provided by the Medulloblastoma Initiative, which made this work possible, was recently submitted and awaits the result of peer review for publication.

Even in these first 6 months, the Cure Group Four Consortium worked very swiftly and managed to engage two labs that have been essential in creating synergy between the various institutions. According to Dr. Packer, we are successfully accelerating research and today we already have four tangible paths to develop new treatments.

I would also like to note that a Cure Group Four Consortium arm is being structured in Brazil. Hopefully, the clinical trials may be extended to Brazil in the near future.

The news is really very positive. It fills us with optimism, and at the same time with a sense of urgency for results, strengthening our motivation and conviction about the path we have chosen. Very soon, with the help of our donors and partners at The Medulloblastoma Initiative, and together with this true “dream team” of scientists led by Dr. Packer, we will find a cure for this terrible disease, and save the lives of tens of thousands of children.

Fernando Goldsztein

“The news fills us with optimism and at the same time a sense of urgency for results, strengthening our motivation and conviction about the path we have chosen.”

TOGETHER WE CAN SOLVE THE MEDULLOBLASTOMA PUZZLE.

LEARN MORE:
WWW.MBINITIATIVE.ORG

ABOUT THE MEDULLOBLASTOMA INITIATIVE

The Medulloblastoma Initiative is a project designed to raise funds from private donors for investment in accelerating research to find a cure for medulloblastoma, the form of brain cancer that kills the most children around the world. After initial conventional treatment, relapse or disease recurrence imposes a devastating prognosis, as no standard treatment protocol is available.

In a scenario of lacking resources to fund research in all types of pediatric cancers — which for many reasons (as discussed elsewhere in this report) are not prioritized for funding — the Medulloblastoma Initiative was founded in 2021 by Fernando Goldsztein, father of a boy who has faced recurrent medulloblastoma. Despite all the struggles they have been through, their readiness to participate in very initial experimental research procedures has translated into good results so far. The goal of the Medulloblastoma Initiative is to develop a protocol for cure and make it available to affected children all over the world, saving thousands of lives every year.

Since its inception in July 2021, the Medulloblastoma Initiative has raised and dispersed significant financial support to a consortium of laboratories with a tradition in medulloblastoma research, installed in renowned institutions in the United States and Canada — the Cure Group Four Consortium. Together, brain tumor laboratories in these countries are working synergistically to unravel Group 4 medulloblastoma and its molecular and immunologic underpinnings. Consortium collaborators share a bold vision: to harness and galvanize extensive research and clinical expertise to accelerate the development of a novel therapeutic treatment for children and teens with Group 4 MB.

The Consortium aims to do this within 18 to 24 months — not in the traditional 3 to 5-year timeline.

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LEGAL SUPPORT AND EXTERNAL ADVISORY BOARD

In the conceptualizing of The Medulloblastoma Initiative, several steps were taken to ensure security for donors. In addition to legal support (through Kalbian Hagerty LLP, an international firm based in Washington D.C.), an External Advisory Board (EAB) was established, consisting of three renowned researchers. The role of the EAB 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, that is, several research endpoints aimed at accelerating the development of a cure protocol for relapsed Group 4 medulloblastoma. All funds raised by The Medulloblastoma Initiative are managed at the Children's National Hospital Foundation in Washington D.C., and distributed to participating laboratories according to established research priorities. A preliminary audit has already been carried out at the beginning of the project. In addition, research milestones will be audited every three months. Research workshops are also scheduled to occur every six months for the presentation of results and decision-making by the laboratories involved.

MBI External Technical Advisor:

André Fay
School of Medicine, Pontifícia Universidade Católica do Rio Grande do Sul (PUCRS), Porto Alegre, Brazil

EAB members are world-class medical researchers:

Ian F. Pollack
UPMC Children's Hospital, Pittsburgh, USA

Scott Pomeroy
Harvard School of Medicine, Boston, USA

William A. Weiss
UCSF Helen Diller Family Comprehensive Cancer Center, San Francisco, USA

NEWS FROM THE CURE GROUP FOUR CONSORTIUM

A letter from Dr. Roger J. Packer, principal investigator, to The Medulloblastoma Initiative founder Fernando Goldzstein

"Thank you once again for your generosity and trust, allowing us to create the Cure Group Four Consortium. The five laboratories initially chosen to be part of the Consortium are all working towards meeting their Year 1 Milestones.

As noted, each lab has made progress, and as expected, progress has been made to a different degree, depending on the project. Some notable accomplishments (already early in operations of the Consortium) include:

• The Taylor/Ramaswamy Laboratory from Toronto has recently made a seminal discovery, pinpointing for the first time the likely developmental origin of Group 4 medulloblastoma. The group is targeting the unipolar brush cell (UBC) to identify novel cellular targets that can be used for therapy, focusing on the oncofetal antigens expressed in Group 4 medulloblastomas. A paper outlining these discoveries has just been submitted to a leading journal with an acknowledgement to Mr. Fernando Goldsztein's and the Medulloblastoma Initiative's support making this work possible.

• The Taylor Lab has begun, with the supplemental grant awarded, to sequence the largest number of Group 4 medulloblastoma samples assembled. This will confirm findings from their seminal studies and also allow a better clarification of what oncofetal antigens, other antigens and other biological processes are best therapeutically targeted.

• The Wechsler-Reya Laboratory has begun the CRISPR screening and will perform high throughput drug screening to identify chromatin modifying drugs that may augment immunogenicity in Group 4 medulloblastomas. This will be critical work to enhance the efficacy of the immunotherapeutic approaches being developed in the Bollard/Cruz and the Mitchell laboratories.

• Work ongoing in the Bollard/Cruz and Mitchell laboratories is utilizing somewhat different techniques to develop T-cell approaches, including CAR-T for Group 4 medulloblastoma. As outlined in their reports, the lack of neoantigens to target has been a limitation in medulloblastoma research and that is why the studies in the Taylor/Ramaswamy and Mitchell laboratories to better identify neoantigens and in the Rood laboratory to use proteomics to develop another way to identify targetable antigens are so important and synergistic.

• A major limitation for all of these labs has been the lack of Group 4 medulloblastoma cell lines and mouse models. The work being collaborated upon by Drs. Pei and Wechsler-Reya is moving steadily to generate better transgenic models of Group 4 medulloblastoma. It will result in more informative ways to preclinically evaluate proposed means of therapy. This work will, in addition, aid the identification of novel targetable neoantigens.

As noted previously, one of the obstacles in making progress in the therapy of Group 4 medulloblastoma has been the lack of informative models specifically for the Group 4 subtype. The work being done both in the Wechsler-Reya and Pei laboratories will hopefully partially overcome this obstacle and both labs are working together, sharing cell lines and PDX models and also sending models to other labs in the Cure Group Four Consortium to expedite the development of therapeutic approaches. A major development has been the identification of a novel Group 4 cell line by Dr. Sheila Singh's laboratory, which has already been shared with other laboratories in the Consortium. The Consortium labs will interrogate the cell line to better understand its properties and how it specifically mimics Group 4 medulloblastoma. If this is found to be as informative a cell line as Dr. Singh believes, it will greatly accelerate the work of probably all of the laboratories involved in the Consortium.

It was truly fortuitous that with your help we added Dr. Singh's laboratory to our Consortium in the latter half of 2021. We initially added Dr. Singh because of her work with this stem cell drug, BMI1, which seemed a very good candidate for therapy of Group 4 medulloblastoma. Dr. Singh agreed to investigate the BMI1 and drug combinations including BMI1 in Group 4 medulloblastoma models that were available from the Consortium. The simultaneous development of another, possibly more informative, Group 4 model in Dr. Singh's laboratory was an added bonus.

A more recent addition to the Consortium has been the inclusion of Dr. Tobey MacDonald's lab at Emory in Atlanta. Dr. MacDonald is already working on a novel drug ONC206, which seems to have excellent brain penetrance and ability, in cell lines, to effectively stop medulloblastoma growth. His lab had not tested the drug in Group 4 medulloblastoma, but with his addition to the Cure Group Four Consortium (made possible by the additional funds made available to the Consortium in later 2021), a project was rapidly developed using the laboratory expertise in Dr. MacDonald's laboratory and those in Drs. Wechsler-Reya's and Pei's labs to study ONC206 in Group 4 medulloblastoma (including using Dr. Singh's cell lines), while also moving towards combination therapy with ONC206 and other potentially effective drugs including PI3 kinase pathway inhibitors.

In conclusion, because of your generosity and the generosity of the other donors you were able to recruit, the Cure Group Four Consortium was able to move quickly and add two additional laboratories with synergistic capabilities. There are multiple collaborations between the other laboratories in the Consortium which did not exist prior to the formation of the Consortium. This is in no doubt accelerating research. Remarkably there are already four tangible potential therapeutic avenues to explore including:

• The use of the BMI drug, possibly in combination, for a relapsed Group 4 medulloblastoma.

• The use of ONC206, likely coupled with another pathway inhibitor (possibly a PI3 kinase inhibitor) in relapsed Group 4 medulloblastoma.

• The development of a T cell or a CAR-T cell vaccine targeting Group 4 medulloblastoma possibly utilizing novel neoantigens identified in the Rood laboratory, the oncofetal antigens identified in the Taylor laboratory or those identified by Mitchell's laboratory. (Please note the expertise in the Bollard/Cruz and the Mitchell laboratory will greatly facilitate the development of such a T cell approach.)

• Coupling chromatin remodeling drugs, as being studied in the Wechsler-Reya laboratory, with the immunotherapeutic approaches to make therapy immunotherapy more effective for relapsed Group 4 medulloblastoma.

I and the laboratory chiefs are very aware of our timelines and are working carefully, but with a sense of urgency. I will be sharing this report with our External Advisory Committee (Drs. Pomeroy, Pollack and Weiss). The Cure Group Four Consortium has involved colleagues in Brazil to extend the reach and impact of the Consortium. Clinical monthly meetings with Dr. Epelman, who is developing a clinical consortium in Brazil, are ongoing and this report will be shared with Dr. Epelman. He has already been invited to the Consortium meeting in May in Washington. I hope you and your advisors will be able to join us.

Once again, thank you for this great opportunity to make a real difference for those with Group 4 relapsed disease."

Roger J. Packer, MD

Senior Vice President, Center for Neuroscience and Behavioral Medicine
Endowed Distinguished Professor & Director, Gilbert Family Neurofibromatosis Institute
Director, Brain Tumor Institute, Children's National Hospital
Professor of Neurology and Pediatrics, The George Washington University Medical Center

CURE GROUP FOUR CONSORTIUM LEAD SCIENTISTS

DECEMBER 2021

Dr. Roger J. Packer, Principal Investigator
Children's National Hospital, Washington D.C., USA

Dr. Eugene Hwang, Dr. Brian Rood, Dr. Conrad Cruz, Dr. Yanxin Pei
Children's National Hospital, Washington D.C., USA

Dr. Michael D. Taylor, Dr. Vijay Ramaswamy
SickKids, Toronto, Canada

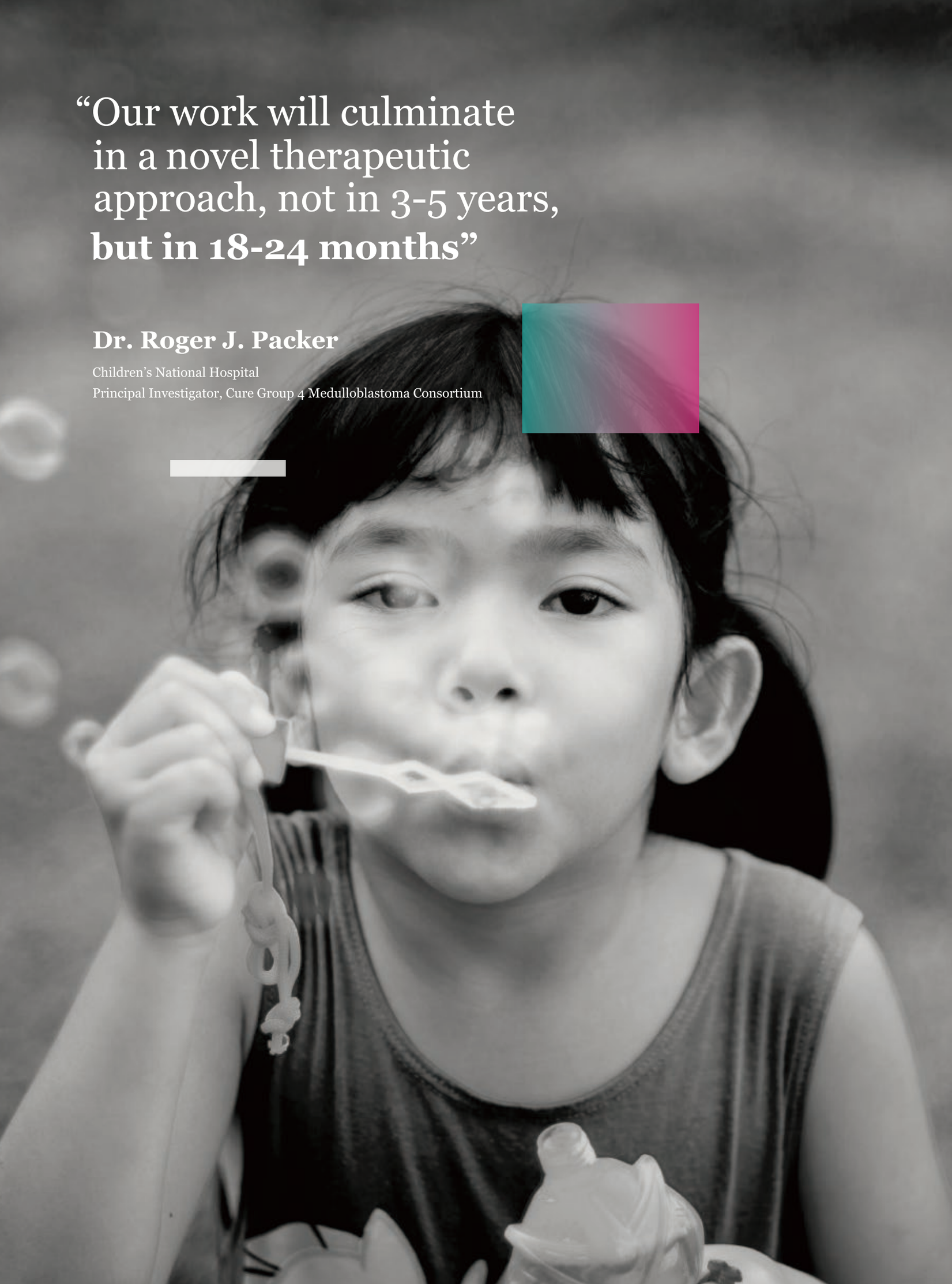
Dr. Robert Wechsler-Reya
Sanford Burnham Prebys Medical Discovery Institute, La Jolla, USA

Dr. Duane A. Mitchell Laboratory
University of Florida, Gainesville, USA

NEW PARTNERS

Dr. Sheila Singh
McMaster Children's Hospital, Hamilton, Canada

Dr. Tobey MacDonald
Emory University School of Medicine, Atlanta, USA



“Our work will culminate in a novel therapeutic approach, not in 3-5 years, **but in 18-24 months**”

Dr. Roger J. Packer

Children’s National Hospital

Principal Investigator, Cure Group 4 Medulloblastoma Consortium

CURE GROUP FOUR CONSORTIUM BIANNUAL WORKSHOP

The biannual Cure Group Four Consortium workshop has been scheduled for May 23rd, 2022. This full day meeting will be attended in person by the lead researchers from all the laboratories. The focus of the May 23rd meeting will be to assess and if possible finalize one or more therapeutic protocols for relapsed Group 4 medulloblastoma.

The workshop will take place at the Children's National Hospital in Washington, D.C.. Members of the External Advisory Committee will also attend, as well as representatives from The Medulloblastoma Initiative.

Children's National Hospital
Washington, DC

FINANCIAL HIGHLIGHTS

TO DATE

THE MEDULLOBLASTOMA INITIATIVE HAS ALREADY SECURED SIGNIFICANT FUNDS TO ADVANCE RESEARCH

US$ 6.1 MI

Amount committed by donors to The Medulloblastoma Initiative

US$ 1.5 MI

Funds already received and applied to research

US$ 4.6 MI

Funds to be received and applied to planned milestones

THE MEDULLOBLASTOMA INITIATIVE

THERE IS NO LIMIT TO WHAT WE CAN DO

Additional donations will serve to:

- Engage additional experts and laboratories
- Accelerate research on the most promising approaches
- Develop multiple clinical trials
- Test novel strategies
- Resolve the complex Group 4 medulloblastoma puzzle and save lives

TESTIMONIALS

Uncovering new treatments and a cure for medulloblastoma will only be possible through the strength and work of many hands and minds. The trust and generosity of the donors empower the Cure Group Four Consortium to collaborate in new, promising ways. Thanks to the MB Initiative for partnering alongside us as we push closer to answers for the children and their families from all over the world.

The Children's National Hospital's Impact Report, USA

Your drive, energy and passion towards raising money for Group 4 medulloblastoma is simply remarkable. Your comment about not being a donor, but rather a fellow fundraiser and teammate really stuck with me. We are honored to have your partnership.

Jessica L. Miley, COO - Children's National Hospital Foundation, USA

I never doubted that you would transform pain into a reason to make a difference. It was quite clear to me that all that motivation to make the impossible for your son would serve as the impulse to do good for hundreds, and why not thousands, of children.

Francine Hehn de Oliveira, MD, Chief of Pathology,
Hospital de Clínicas de Porto Alegre, Brazil

Children should have every possibility to thrive and succeed in life, both physically and mentally. I have always focused on helping children with their mental well-being. But the physical needs of children are equally as important. This organization is the only chance of survival for many children faced with this illness. I try to live my life in an empathic manner and try to have a positive impact in the world.
I receive many receipts for charitable donations, each of which are very worthwhile. However, when I look at the receipt from the Medulloblastoma Initiative, a donation that gives kids a chance at life, I am certain that there can be no greater cause than this.

Jonathan Solnicki, USA - Donor

The Medulloblastoma Initiative is backed by outstanding institutions, but much more importantly by great human beings that have the courage and the capabilities to lead this great cause.
If the opportunity comes to you to contribute to the Medulloblastoma Initiative, don't let it go. If you have not donated yet, then to the same as me, take your first step. Do not miss the chance.

Mariano Garguilo, Argentina - Donor

Stand with other supporters:

Baptista family

Brochman family

Garguilo family

Goldsztein family

Krakoviak family

Ling family

Safra family

Solnicki family

The Medulloblastoma Initiative and all children who will benefit from the Group 4 research projects are immensely grateful for the support.

AN INTERVIEW WITH DR. ROGER J. PACKER

Medulloblastoma Initiative Interview with Dr. Roger J. Packer, Senior Vice President of the Center for Neuroscience and Behavioral Medicine, Gilbert Endowed Distinguished Professor in Neurofibromatosis, Director of Gilbert Family Neurofibromatosis Institute, and Director of Brain Tumor Institute.

How long have you been involved in medulloblastoma treatment and research?

I began my research career in 1981. Over the last 40 years i have been actively involved in research in medulloblastoma including identifying the cause of the tumor, its pattern of spread, and discovery of better ways to treat medulloblastoma and other aggressive pediatric brain tumors.

Since you were first engaged in medulloblastoma research, what major advances have been achieved?

Over the past 40 years we have learned a great deal about medulloblastoma. When I entered the field less than 50% of children lived 5 years from diagnosis and late relapses, occurring after 3 to 5 years of disease control, were not infrequent. I was involved in the first study that demonstrated clearly that the addition of chemotherapy benefited children with medulloblastoma. This study, which I was fortunate to lead at the Children's Hospital of Philadelphia, and later at Children's National Hospital here in Washington DC, resulted in nearly 80% of children with medulloblastoma which was localized in the brain at the time of the diagnosis surviving; the majority not only survived but maintained disease control and were cured of disease. We were able to also show in subsequent studies that the amount of radiation required for disease control could be safely reduced in the majority of children with medulloblastoma as long as patients were carefully evaluated prior to the initiation of treatment and had excellent surgery. However, there are still some significant gaps in the care of children with medulloblastoma.

These gaps include:

- There are subgroups of children with medulloblastoma, including subgroups of children with Group 4 (non-SHH/non-WNT tumors) that have a much poorer survival rate. After standard treatment with radiation and chemotherapy less than 40% can be cured of their disease.

- The treatment required for disease control causes significant long-term difficulties including learning disabilities, growth failure and in some groups of children, secondary tumors.

- For the subgroups of children with medulloblastoma resistant to present forms of therapy, there has been no real improvement in treatment over the past three decades.

- If medulloblastoma recurs after initial treatment it is very difficult to put the child in a continuous remission and cure is infrequent.

- Younger children with medulloblastoma are very difficult to treat and survival rates for those less than three years of age are usually less than 40% at 5 years.

What major barriers or challenges have stalled progress or research?

There have been great advances in our understanding of medulloblastoma, including the realization that medulloblastoma is not one disease but rather different diseases that look similar under the microscope but respond differently to treatment. These great advances have been achieved by cooperation between multiple laboratories across the world in better understanding the molecular changes associated with some forms of medulloblastoma. However, these new understandings have not yet been turned into better treatment for children with a more resistant form of medulloblastoma.

There are daunting challenges for the treatment of those with the more resistant forms of medulloblastoma. Our understanding of the origins of resistant tumors and what drives them is incomplete. We do not have good animal models for some groups of these tumors, especially resistant Group 4 tumors; these models are crucial to try new forms of treatment so we can avoid needlessly experimenting on children until we better understand the likelihood of any drug or immunotherapy working.

Funding is a major limitation for medulloblastoma research. It is hard to fund when there are limited models to begin with; it is a chicken and egg situation, because without the models and without the understanding of the basis of the disease, it is hard to get government funders, such as the National Institutes of Health or the National Cancer Institute, to support research. However, without research we will not understand this molecular underpinning of the disease.

Without funding, research laboratories cannot be convinced to study some forms of medulloblastoma and will study other forms of medulloblastoma about which more knowledge is available, and where it is easier to set up successful experiments. Also, because of this molecular variability in medulloblastoma at the time of relapse, children with a specific subgroup of medulloblastoma cannot easily access therapy if they are in a geographic region or socioeconomic status where it is difficult to access innovative care. We have to overcome all of those additional barriers to get newer therapies to children who are in developing countries that have limited access to very detailed, innovative forms of therapy.

The only way to make progress is for multiple laboratories to work on a specific form of medulloblastoma, such as Group 4 medulloblastoma together, and to make it a priority of these laboratories to focus on these resistant medulloblastoma types. Then we must disperse such treatment as widely as possible.

What is the impact of medulloblastoma for children in terms of morbidity and mortality worldwide?

Cancer remains one of the leading causes of death in childhood. There have been great advances in the treatment of some forms of cancer such as acute lymphocytic leukemia but advances in the treatment of children with primary brain tumors has lagged behind other cancers. There has been less funding aimed at childhood brain tumors than other pediatric tumor

types. Now, unfortunately childhood brain tumors are the leading cause of cancer related deaths and the leading cause of severe medical, cognitive (learning) and psychological complications from cancer in children. Medulloblastoma is the most common of the primitive childhood brain tumors. Worldwide, it comprises almost one-fifth of all childhood brain tumors. It affects children of all ethnicities, and it is a worldwide problem. For some of the more common forms of medulloblastoma, nearly 80% of children can now be cured with present forms of therapy. However, for some subtypes including resistant Group 4 medulloblastomas less than half of children can be cured with present treatment. In addition, there are some children who are apparently initially successfully treated who relapse years after diagnosis.

How is The Medulloblastoma Initiative advancing childhood medulloblastoma research?

Over the past 20-25 years, the brain tumor research community has not made significant progress in advancing the understanding of Group 4 medulloblastoma. Once this subtype of tumor recurs the vast majority of children will die of their disease. The only way to make progress is to focus our efforts, working together as a collective, on this subtype of medulloblastoma.

The Cure Group Four Consortium aims to unravel the molecular and immunologic underpinnings of medulloblastoma and rapidly develop a new therapeutic treatment. The Medulloblastoma Initiative was created through the visionary leadership of philanthropists to fund the Consortium's research and accelerate the development of a new treatment and standard of care for Group Four medulloblastoma.

How does the approach taken by the Cure Group Four Consortium, which receives the funds raised by the Medulloblastoma Initiative, differ from the usual research approaches or from the research done in childhood medulloblastoma until this point?

The Cure Group Four Consortium has a laser-like focus on children with the most resistant form of Group four medulloblastoma, primarily tumors that have not only recurred but have disseminated. Leading laboratories across the world are banding together to focus on this disease, all looking at different potential ways to better understand and treat this tumor subtype.

The Consortium have all pledged to focus a significant part of their research efforts on this subtype of disease, efforts which were previously spent studying other forms of medulloblastoma and other types of brain tumors, or hiring staff to start new research investigations all on Group 4 disease.

This is an initiative that extends to the gamut of medulloblastoma research: from studies of the cell of origin of Group 4 medulloblastoma and its molecular and genetic drivers; to the development of new, more informative animal models on which to test new therapies; to the actual development of new therapies and their use in children with recurrent medulloblastoma. This kind of all-encompassing research has received very little in a way of funding. Also, the laboratories involved have pledged to work together, share data well before it is published and expedite the development of effective treatment protocols, aiming not to develop new therapies in 5 to 10- years (as is often the case), but rather in a 1 to 2-year timeline.

What is your assessment of the work done so far by the Consortium in terms of the proposed goals and projected achievements?

Over a 6-month period the Consortium is already starting to use novel models and make new discoveries of Group 4 medulloblastoma. The cell of origin of the tumor has been better defined and new animal models that are going to be more informative have been shared across all of the laboratories. Multiple drugs are presently being screened and immunotherapy is being looked at. One of the focuses of the Cure Group Four Consortium is to bridge the gap between our understanding of the molecular drivers of medulloblastoma and the tumor's immune environment so that we can use new molecular techniques and immunotherapy together to combat this disease. This is something which has not really been attempted previously. We have also added two new laboratories that are studying promising agents to expedite work even better. We also realize that we are going to need an early way to monitor whether therapy is effective, and we are developing a liquid biopsy platform for medulloblastoma, so we can more quickly and assuredly determine tumor response to treatment and disease control.

Thank you for your partnership and trust to advance this important research!

Dr. Roger J. Packer

Children's National

By Children's National
Hospital Foundation:

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www.mbinitiative.org/impact

TWELVE REASONS TO BECOME A PHILANTHROPIST

1. There are countless good causes, the world has endless needs
2. There is no time to spare — get up and do good things
3. Too many diseases remain incurable, which kill thousands of kids
4. Research (you better believe it) still lacks sufficient funds
5. Giving always makes a difference, no matter in what form
6. You can choose among your riches: donate money, time, a skill
7. It will inspire others
8. It will make you a better person
9. It will improve someone's life
10. It might improve thousands of lives
11. It will not resolve all problems, but it will help heal the world
12. As written in the Talmud, the Jewish book of laws, "Whoever saves one life, saves the world entire."

Fernando Goldsztein
Founder, The Medulloblastoma Initiative

THE MEDULLOBLASTOMA INITIATIVE

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