Fall 2025

NEUROtransmitter

Communicating our message.

Resilience and Survival: Awesome Alicia

Featured Article

In many ways, CBTF changed my life. I started fundraising for CBTF in 2009, soon after being diagnosed with a brain tumor, juvenile pilocytic astrocytoma (JPA), at the beginning of the third grade. That October, my family and friends formed a team for our first CBTF Superheroes 5k. As a nine-year-old who'd discovered the fun of alliteration, I loved the name "Team Awesome Alicia", and it became official. We picked out bandanas in my favorite colors, pink or purple, depending on the year, for Team Awesome Alicia supporters to wear at the race. For the next ten years, Team Awesome Alicia, including members of my soccer teams, classmates, neighbors, friends, and family, came together at the annual run to support CBTF. We raised over $60,000 thanks to their incredible generosity.

Fundraising and rallying Team Awesome Alicia to support CBTF as I underwent chemotherapy helped me to feel the support of my community and to fight back against brain tumors by raising funds for groundbreaking research. I am forever grateful for the dedicated volunteers who led such a meaningful event and to the caring community who came out to support me and all CBTF Superheroes each year.

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Grant programs receiving funding 2026

Second-Year Funding

The Rector and Visitors of the University of Virginia, **Roger Abounader, MD, Ph.D.,** *Transcribed Ultra Conserved Regions in Medulloblastoma*

Children's Hospital of Philadelphia, Division of Oncology, **Simone S. Riedel, MD,** *Investigating the role of MN1 fusions in the newly recognized subgroup "Astroblastoma, MN1 –altered"*

First-Year Funding

The University of Texas Health Science Center at San Antono, **Luis O. Penalva, Ph.D.,** *Targeting Dyskerin with peptide inhibitors, a new treatment strategy against aggressive medulloblastoma*

Grants: CBTF will open our grants application process for 2026 in the winter of 2025. A limited number of number of applications will be funded.

CBTF RAISES FUNDS FOR RESEARCH

Funds raised benefit pediatric brain tumor research and other *CBTF* programs

The Childhood Brain Tumor Foundation

Our mission is to support and fund basic science or clinical research for childhood brain tumors. We are dedicated to heightening public awareness of this devastating disease. and improving the quality of life for those that it affects by funding vital research initiatives.

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Online: Experts video sessions are posted on our website.

Grant Summary

First-year Funding—Summary written by **Luis O. Penalva, Ph.D.**
The University of Texas Health Science Center at San Antono

Targeting Dyskerin with peptide inhibitors, a new treatment strategy against aggressive medulloblastoma

The Penalva lab has been studying the contribution of RNA binding proteins and RNA-mediated processes to medulloblastoma initiation and growth. Ultimately, we hope to use this information to develop novel therapeutic approaches. Our CBTF project focuses on ribosome biogenesis, the complex process to generate components and assemble ribosomes. Cancer cells require increased and specialized protein production to attend to their growth demands and therefore, they are highly dependent on ribosome biogenesis. This dependence creates an opportunity for targeted therapy.

We determined that in aggressive medulloblastoma, the oncogene MYC promotes the expression of a gene network implicated in ribosome biogenesis. We propose that this activation modulates different steps of ribosome biogenesis to produce more efficient and specialized ribosome—**Fig. 1**. A critical step is ribosomal RNA (rRNA) modification, which modulates ribosome assembly and selection of ribosomal proteins. This process is coordinated by small nucleolar RNAs (snoRNAs) and the enzyme Dyskerin (DKC1). We determined that both are altered in aggressive medulloblastoma – **Fig. 2**. We are pursuing the idea of targeting snoRNAs and DKC1 as therapeutic strategies. Initial in vitro studies using antisense oligo nucleotides to target snoRA71 showed positive results with a disruption in the growth of medulloblastoma cells. In the case of DKC1, we developed peptide inhibitors in collaboration with Dr. Andrew Beekman (UEA. UK) and successfully tested in MB cells.

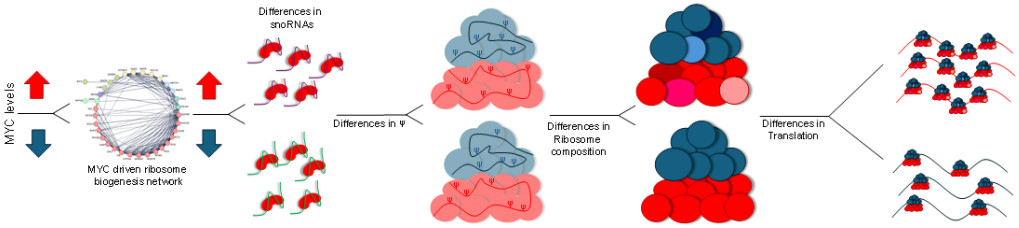

Figure 1. Our proposed model. MYC regulates a network of ribosome biogenesis factors. Activation of this network will produce alterations in snoRNA expression, rRNA modification and ribosome composition that will ultimately cause…

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Please support CBTF: Your monetary contribution is always meaningful to CBTF. We hope we can count on your continued support.

CBTF has posted a series of videos on many relevant topics.

Childhood Brain Tumor Foundation
Visit our GIVE ONLINE donation button:
https://www.givedirect.org/donate/?cid=1605

Be part of the solution in helping fund vital research initiatives cure childhood brain tumors!

2009 - Awesome Alicia's team

I underwent chemotherapy from third to fifth grade and have been in long-term follow-up care in the years since with an amazing team of doctors and medical providers led by Dr. Roger Packer. Finding community and support with CBTF helped me to develop a passion for advocacy and an appreciation for the transformative power of research. I graduated from Cornell University in Spring 2024 with a B.A. in sociology and a minor in public policy. I now work as a research assistant at the Urban Institute, a think tank in Washington, DC.

Written by Alicia Gonzalez, survivor
CBTF thanks Alicia for sharing her update.

CBTF Sponsorships

CBTF is delighted to provide a Silver sponsorship for the **2026 International Symposium on Pediatric Neuro-oncology, 22nd.** The symposium will be held from June 29th—July 2nd in Sydney, Australia.

As in past years, we are pleased to provide support in 2026 to the **Society for Neuro-Oncology** for the international program in **Sub-Saharan Africa.**

It is through the dedicated support CBTF receives from friends, families, constituents, and businesses that we are able to continue supporting these important programs. Thank you so much!

Funding, Luis O. Penalva *(continued from page 2)*

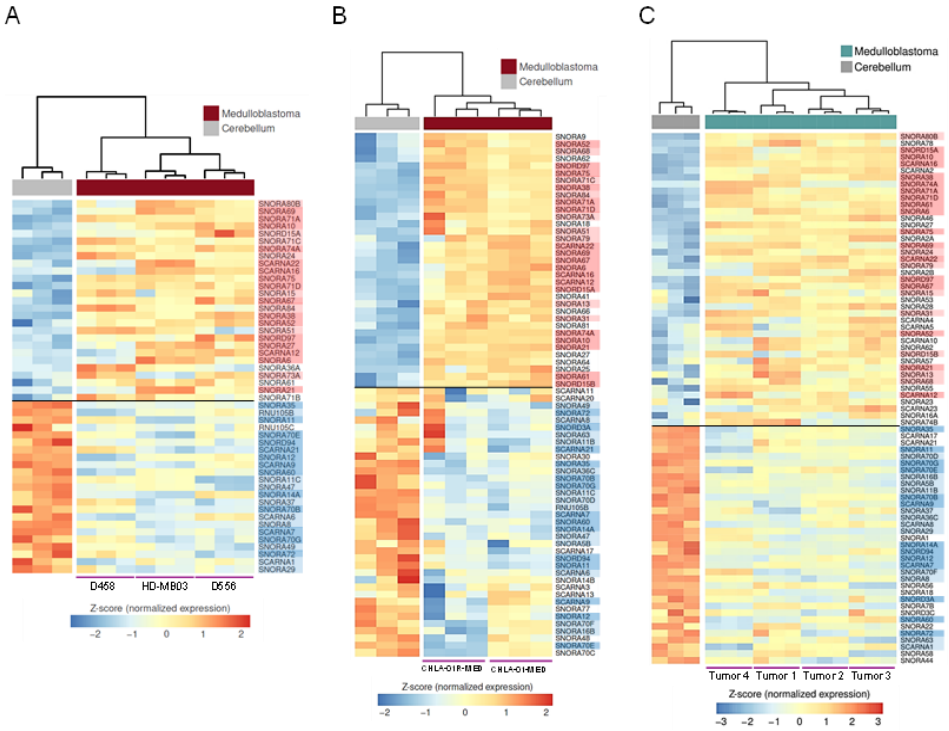


Figure 2. snoRNA expression analysis. We have employed an Ampliseq platform to specifically evaluate the expression profile of snoRNAs/scaRNAs. This platform was used to identify snoRNAs/scaRNAs altered in medulloblastoma. We have conducted three studies. A) Comparison between normal cerebellum and MYC amplified Group 3 MB lines. **B)** Comparison between normal cerebellum and MYC amplified Group 4 MB lines. **C)** Comparison between normal cerebellum and MB tumors. snoRNAs/scaRNAs that were identified in two out of three analyses are indicated in light red/blue while dark red/blue highlight snoRNAs/scaRNAs identified in all three analyses. Red shows snoRNAs/scaRNAs up regulated in MB and blue the down regulated ones.

Second-year, Summary written by **Simone S. Riedel, MD**
Children's Hospital of Philadelphia, Division of Oncology

Investigating the role of MN1 fusions in the newly recognized subgroup "Astroblastoma, MN1 –altered"

Astroblastoma, Meningioma 1 (MN1)-altered is a distinct subgroup among the tumors of the central nervous system (CNS). These tumors are defined by the presence of MN1-fusions. Morphologically, these tumors present variably. However, as evidenced in recent research their DNA methylation and gene expression profile sets them distinctly apart from other CNS tumors. Diagnosis frequently occurs at a median age of 14 and currently the treatment options are limited to resection and radio/chemotherapy.

Nothing is known about the mechanistic function of these MN1-fusions that define this disease. Our aim is to identify the oncogenic function of MN1-fusions in Astroblastomas which is an important step in potentially identifying specific therapeutic targets in the future. MN1 is a transcriptional co-activator with no sequence homology to any other protein. Therefore, it has no annotated classical domains with associated functions that could indicate this proteins' role.

We previously shown that MN1 acts as an oncogenic driver in MN-1 translocated acute myloid lekemia (AML). Here, we showed that MN1-fusions aberrantly stabilize the nucleosome remodeling complex BAF on chromatin. This leaves the associated gene loci open and in an active state preventing their necessary shutdown which ultimately impedes normal cellular development.

We will now use our experience in MN1-fusions to investigate their role in CNS tumors. The genomic loci bound by MN1-fusions in MN1-altered astroblastoma are undefined. Currently, we are performing Chromatin Immunoprecipitation (ChIP) followed by sequencing (ChIP-seq) to identify the MN1-fusion binding sites, By integrating ChIP-Seq and RNA-Seq data we will identify direct target genes of MN1-fusions. Initially, we are using the model cell line KS-1 with confirmed MN1-PATZ1 fusion to perform experiments and generate a first dataset. In the next step we will perform these analyses on two patient-derived samples to identify differences or similarities. In the future we will also investigate the growth, differentiation status, and the expression of specific genes are dependent on the MN1-fusion in MN1-altered Astroblastomas.

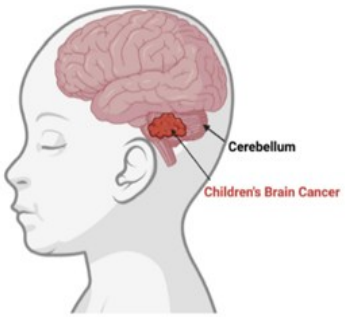
These experiments will provide a basic understanding about the role of MN1-fusions in these Astroblastomas and hopefully be the first step towards a targeted therapy.

Research

Second-year Funding—Summary written by **Roger Abounader, MD, Ph.D.**
The Rector and Visitors of the University of Virginia

Transcribed Ultra Conserved Regions in Medulloblastoma

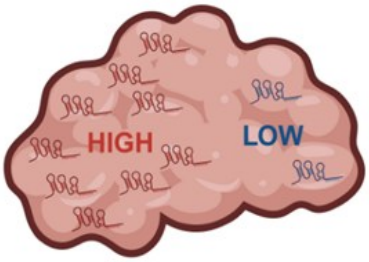
Why are we doing this research?



One of the most common and most deadly types of children's brain cancer is called "medulloblastoma". This cancer comes from a specific part of the brain called the "cerebellum". If the cancer is left untreated, it can spread into the spine. We need better treatments to help children with this brain cancer. If we can understand where medulloblastoma comes from, how it grows, and how it moves, then we can create better treatments.

What are we doing?

We are trying to better understand medulloblastoma by studying what a new group of molecules called Transcribed Ultraconserved Regions "TUCRs" do in this brain cancer. There are 481 TUCRs in humans and we think that they have play important roles in medulloblastoma and that their deregulation can affect medulloblastoma growth. We use molecular information derived from biopsies of human medulloblastoma to learn about TUCRs and their roles in MB.



What have we learned so far?

We discovered that TUCRs are frequently deregulated in medulloblastoma. There were too many of some TUCRs and not enough of other TUCRs in the tumors. We developed and used molecular and computational approaches to find the four most important TUCRs in medulloblastoma. We discovered that one of these TUCRs changes how fast medulloblastoma grows and moves. We are now doing experiments to figure out how the TUCRs work.

Why is this important?

This is the first time anyone has ever studied TUCRs in medulloblastoma. We think TUCRs are important for keeping the brain healthy and that their deregulation can promote tumor formation and growth. We hope that understanding TUCRs will lead to better treatments for children's brain cancer in general and medulloblastoma in particular.

More information:

Please visit https://www.abounaderlab.org/ to learn more about this work and other projects studying children's and adult brain cancers.

In Honor of

Our dedicated scientific advisors who have dedicated their time and compassionate care for the patients and families. We also honor them for their commitment of voluntary service to review grants and assist CBTF as needed.

Fae Daniels
Roger Packer
Michael Schoenfeld

Special thanks to Drs. Kenneth Aldape, Roger Packer, Kristina Hardy, Tobey MacDonald, and Gilbert Vezina for working with our videographer, Neil Rubino, to tape presentations on various topics. No charge to view, register for log-in to access the videos: ***cbtf@childhoodbraintumor.org***

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BY CHECK: Make out to CBTF or Childhood Brain Tumor FDN.
Send to: CBTF
20312 Watkins Meadow Drive
Germantown, MD 20874

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Your contributions make it possible for CBTF to fulfill its mission.

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Be sure to add your message if you would like one included with the acknowledgment. Thank you!

If you would like to share a story in our newsletter, contact us via e-mail.

The Childhood Brain Tumor Foundation is dedicated to funding research for all pediatric brain tumor types. We also provide resources, information, and consult names upon request.

Visit our secure website:
www.childhoodbraintumor.org
Go to our Support page and click on

Give2Charity button.
(Accepts Discover, MC,VISA, and AE).
Please contact us if you have any questions:
cbtf@childhoodbraintumor.org

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Gift Matching Opportunities

Many companies offer a matching gifts program to support charitable organizations.

Your human resources department can tell you if such a program exists at your company. Ask them about the form that can be sent to the Childhood Brain Tumor Foundation reporting a contribution (donation or event contribution). The form states that they will match your contribution.

We return the form to the employer with the proper acknowledgment and information required.

Thank you to our friends who donated through workplace charitable giving campaigns this year, inclusive of the **CFC**.

The Childhood Brain Tumor Foundation, friends and families are very appreciative of your support. (National) CFC **12035**

Charity Campaign and other independent campaigns.

Campaign donations may be made for the United Way (**UW**) through the "donor option" or "donor choice." Please check with your employer in reference to **UW** campaigns. You may write us in.

Thank you donors!

Maryland Charity Campaign

The Childhood Brain Tumor Foundation participates in the **Combined Federal Campaign (CFC) and Maryland Charities.**

Stock Donations

If you would like to make a stock donation, contact us:
cbtf@childhoodbraintumor.org

Our treasurer will provide you with the necessary details to proceed with your donation.

Thank you.

The Childhood Brain Tumor Foundation
20312 Watkins Meadow Drive
Germantown, MD 20876

Address change requested

together, reaching for a cure!

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Online: Expert video sessions are posted on our website.

Happy Holidays

Publication: *Neurotransmitter*
Editor: Jeanne P. Young
Contributing Editors: Colleen Snyder. Bridgette Wood
Contributors: Roger Abounader, MD, Ph.D., Alicia Gonzalez, Luis O. Penalva, Ph.D., Simone Riedel, MD
CBTF Logo®: Michael Young
Photography: Alicia Gonzalez
Graphic Design: Bridgette Wood/Wood Web Worx

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Their generous support is deeply appreciated.

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Please let us know if your address has changed.

We welcome any donations in the way of financial support, commentary or information for this newsletter, or volunteer services to help with our operations.

CBTF is a tax-exempt, not for profit foundation IRS 501(c)(3) whose mission is to raise money to improve prognosis and quality of life for children suffering from brain tumors. Every year the requests continue to grow,

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Help children with brain tumors.

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Email preferred
cbtf@childhoodbraintumor.org
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