MUSELLA
FOUNDATION

BRAIN TUMOR RESEARCH
AND INFORMATION

Greetings,

I am writing to you today to thank you again for your previous support of our organization, and to kindly ask you to consider making another donation. I am attaching a list of our accomplishments so far this year. We are planning many exciting projects that depend upon your generosity to continue propelling us forward. We are only limited by the amount of money we can raise.

Our mission is to accelerate the search for the cure of brain tumors as well as help families dealing with the devastating diagnosis of a brain tumor.

In addition to funding innovative brain tumor research, patient educational events and materials, the patient help line, and our copayment assistance program, we have launched our new website which is mobile friendly and easier to navigate.

We started a new program to help patients find the best treatment options, and to help them get access to them. We initiated a new study to track these patients and learn from every patient!

We have been very active in advocacy: we helped write a proposed bill that could speed up the search for the cure in time to help people who are already diagnosed with a brain tumor. Visit our website and click on ACTIVISM ALERT to learn about it and to easily contact your senators!

If you use Amazon, consider using their charity arm, smile.amazon.com and select the Musella Foundation as your charity. A small percentage of your purchase will go to our organization and it doesn't cost you anything!

To make a Tax Deductible donation, go to Virtualtrials.org/donate

Or send a check in the attached envelope.

Happy and Healthy Holidays!

Al Musella DPM

Al Musella, DPM

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Musella Foundation For Brain Tumor Research & Information, Inc
2020 Highlights

The Covid-19 pandemic, as well as monthly disasters all over the country, has rendered 2020 a most challenging year for everyone. This had a huge impact on brain tumor patients as well as all charities, including ours. We had a slower year than we would have liked, but still managed to create some highlights!

Copayment Assistance Program:

Due to the pandemic, donations to all of the assistance programs slowed down. We had our busiest year ever - awarding over $1.3 million to 267 brain tumor patients so far this year, which completely eradicated all of our reserves for the program. We had to close our program to new patients for almost half the year; had funding been available, we would have been able to help so many more families.

Many of these patients went without their treatments because they could not afford them. While we were closed, we heard heartbreaking stories every day. Hopefully, conditions will return to normal next year, but for now, this program is a priority for us. It helps patients immediately – it is literally life- saving. The research programs, on the other hand, are all about helping people in the future. Both programs are obviously needed, but the copay assistance program is most urgent at this time.

Research:

Again, due to the pandemic, donations were slow. All of our "National Walk To End Brain Tumor" 5k event fundraisers were cancelled and converted to virtual events, which did not perform as well as the live events. So far this year we gave out $455,292 in brain tumor research grants, and we hope to give out more by year's end. This is down approximately 20% compared to last year at this time. As always, we made the most of the money we had, and this was an especially exciting year for research projects. We were approached by many more researchers than usual with great projects, who were unable to find funding elsewhere, leading to a much wider selection of projects for us to choose. Unfortunately, that also meant we had to reject a record number of applications for promising projects.

We donated another $250,000 installment for the Onc-201 program for children and young adults with diffuse midline glioma and DIPG, which was our largest grant this year. This is the most successful program we ever supported – we treated over 120 children and young adults with this over the last three years and had some absolutely miraculous responses. I am attaching a copy of a letter one mother sent about her experiences with this program. Results like these are what give us the hope and impetus to move forward with our fight to find the cure. This grant was a collaborative effort among the Musella Foundation, The Cure Starts Now Foundation, Dragon Master Foundation and the Finn Family Foundation.

Unfortunately, the project ran out of funding and was terminated. After the project ended, we frequently received frantic calls from parents looking to get their children access to this amazing

--over--

drug, but we had to tell them we could no longer help. We currently are working with many other organizations to try to reopen the program, and have donated an additional $25,000 to the cause. We are hopeful it will open again soon.

This year, we also funded seven other exciting brain tumor research projects, which are all important and may make a huge impact. You can find details on our website at https://virtualtrials.org/Grants.cfm.

Advocacy:

We, along with a few other organizations, helped author a bill titled the "Promising Pathway Act", which was introduced into Congress by Senator Braun. This bill will drastically speed up the search for the cure for not only brain tumors, but most serious uncurable diseases. The bill should also hold down the cost of new drugs, make access faster and easier, and drastically increase the amount of research being done to find treatments! This is one of the most important bills for you to help support, as it can produce results to help people who are already diagnosed with these diseases as well as those who will be diagnosed in the future. For more information, go to virtualtrials.org and click on the "**ACTIVISM ALERT**" for a quick and easy way to write your representatives!

We launched a new program to help patients find the best treatment options for their individual cases. We collaborated with Cancer Commons and XCures to provide this service. For more information, go to virtualtrials.org and click on "**Find Treatments**".

Website:

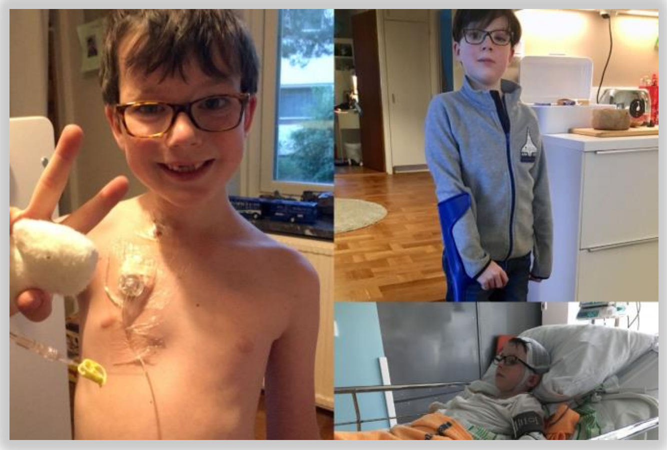
We have launched our new and updated website: Clinical Trials and Noteworthy Treatments For Brain Tumors, at virtualtrials.org. We finally made the switch from .com to .org! The new website is mobile friendly and easier to read and navigate. We removed older material and updated many of the articles. I would like to thank Linda Singer at LCS Production (lcsproductiondesign.com) for her help in designing the new website. We could not have done it without her!

About Donations:

We understand that some people prefer their donations to go solely to research, and do not want them to be used for salaries, overhead, educational programs and materials. Therefore, when donating online or with the attached donation form, we allow you to select how your donation will be used. ***If you select "Research Only", 100% of your donation will go to research.*** If you select "Co-pay program", 91% of your donation will go to patients who need assistance, and the remaining 9% goes to the cost of running the program. If you select "Unrestricted", you afford us the most flexibility to use the money where it is most needed.

This is a testimonial from the mother of a patient who we helped get access to Onc201.

Our Brain Tumor Journey



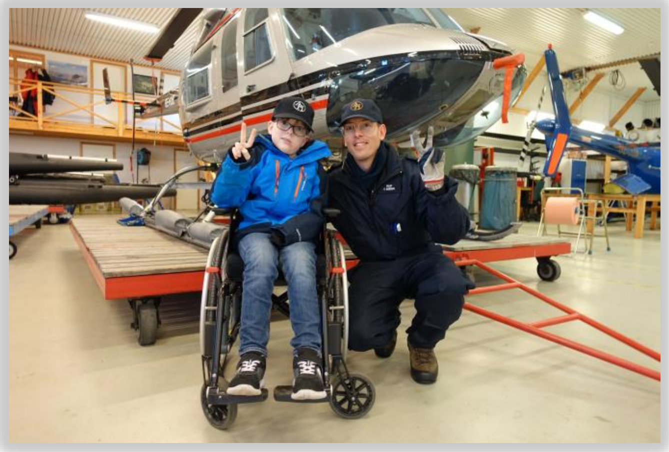
Winter 2018

Our son *A* was diagnosed with a Diffuse Midline Glioma on the thalamus with a H3K27M mutation in May of 2018, shortly before his eighth birthday. He was treated over the summer with proton therapy, followed by chemotherapy, and then was prescribed a new protocol in October. But in January of 2019, the tumor was still growing. Surgery was not an option and the most advanced chemo available in Europe had failed. We were told that nothing could be done for our son but palliative care.

Unwilling to give up, my husband and I sought information on websites such as the Musella Foundation and Cancer Commons. We learned of a new promising drug, ONC201, aimed at *A*'s kind of tumor and mutation, which was only available in clinical trials in the US. At the end of January, we received expert confirmation that ONC201 was the most—and only—promising option we had.

Diffuse Midline Glioma are classified as lethal high-grade pediatric brain tumors that are inoperable and without cure. Despite numerous clinical trials, the prognosis remains poor, with a median survival of ~1 year from diagnosis.

We contacted the four US cancer centers that were offering the clinical trial to children. We gathered all our son’s medical files, the molecular status of the tumor, description of care, and medication. We catalogued all his blood test results and sent reports of the MRIs, CT scans, PET scans, radiotherapy, and operations (translated mostly by us). For each center, we had to repeat the process: make contact, upload all the documents and images into their system, and complete specific questionnaires. This was so time-consuming, but we had no other options. Concurrently, we contacted Al Musella for further information on ONC201 and he suggested that his team and Cancer Commons assess our son’s information and records.



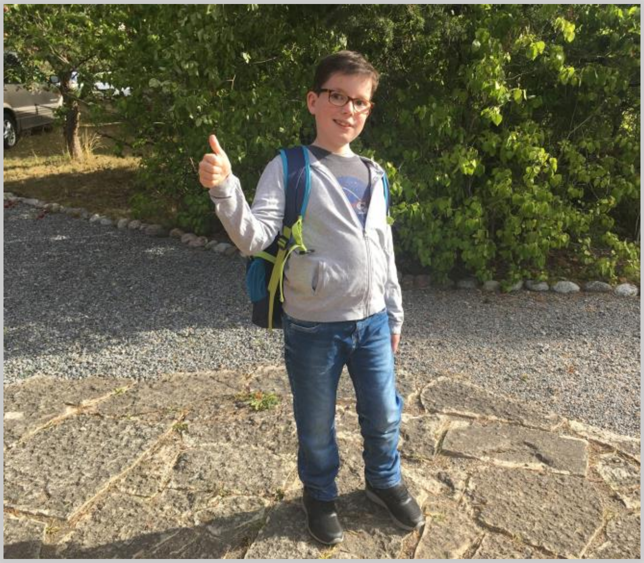
February 2019

Mid-February, we received answers. The first clinical trial was not open to international patients. The second emailed this heartbreaking message: “I’m sorry to hear about your son. Unfortunately I think the ONC201 is unlikely to work given the extent of his disease.” The third stated initially that *A* was a potential candidate, but then declined because exams would be too risky. The fourth invited us to come check *A*’s eligibility as soon as possible. We had begun fundraising in February and we travelled to the US in March of 2019 with our son and our oldest daughter, with no idea how long we would be gone. A friend who owns a travel agency paid for our plane tickets and the Chance for Hope Foundation provided us with an apartment near the hospital. Part of the money we raised was used to pay the financial guarantee of $56,000 required by the cancer center.

On March 13, 2019, we had our first appointment. On March 14, *A* had his MRI. And on March 18, he was denied. He was too weak, and the tumor too big. These days were more of an emotional roller coaster than even the previous nine months. We were exhausted. We were in despair. We had no idea what to do. We asked for help and Al Musella took things in hand with the help of Cancer Commons and xCures. They took the necessary steps to ask for expanded access to Oncoceutics and different trial sites. We applied to the Children's National Medical Center in Washington, DC.

A was accepted at Children's National and we were able to meet the medical team on March 28. A deposit of $102,000 was required by the hospital that, thanks again to our fundraising, we were able to pay. *A* started the ONC201 compassionate use on April 4, 2019. The first month after the beginning of the treatment, we stayed in DC for free in a donated apartment. *A* had an MRI and tests done before we could safely travel, and we finally returned home May 2, 2019. One month later we went back to assess whether the tumor was stable. After this visit, it was decided that we would need to come back every two months for *A* to receive the drug and to follow-up.

On our July 24 visit, we were told that the tumor was regressing 20%. *A*'s tumor was reacting as expected to the drug! The relief was indescribable. We made the journey every two months until March 2020, when the pandemic stopped all travel. *A*'s physician then managed to convince Oncoceutics to give us the drug without us having to go back and forth to Washington. We are now set until the beginning of December.



August 2020

More than two years after diagnosis, when our son should no longer be with us, *A* is a happy boy. He goes to school, has friends, goes to music class, and is a proud scout. He lives a normal life. At his worst, in February 2019, *A*'s tumor was the size of a lemon, 57x66 mm. Today it is 32x21 mm, even smaller than at diagnosis. He has no side effects. Only a better life with symptoms that tend to diminish. The compassionate use of ONC201 saved our son's life.

Never once did the Musella Foundation or Cancer Commons ask for payment or donations. Their help was priceless, their support invaluable. During this journey, we have been lucky. Lucky to be offered a compassionate use, lucky to be in contact with the Musella Foundation, Cancer Commons, and xCures, who helped us find our way even as we had less and less hope of helping our child. For all families who see the last hope for their children dashed by their ability to pay or to travel, facilitating access to compassionate use is the light they need to continue this journey.

Note from Al Musella, President, Musella Foundation: *This story shows how dysfunctional our medical system is. All of the hoops this family had to jump through to get access to an experimental medication that in reality was this patient's only hope. We have helped about 120 patients get this medication, but just as many people wanted it and could not get help. At the time I am writing this (10/27/2020), there is no way for any patient to get access to this outside of a clinical trial, and there are only a very few spots open in the trials. The majority of patients who need this can not get it now.*
We are working on a bill that could fix this problem. It is called the Promising Pathway Act. Go to https://virtualtrials.org/activism.cfm to learn more about it and to contact your representatives to fix this problem. If this law gets passed, this drug and many more would get approved quickly and any person who needs them could get them.