

# NF2 Compass

A Quarterly Advocure Online-Newsletter

Formerly known as 'Flutterby'



Advocure NF2 Inc. is a Working Advocacy Group, Liaison, and 501(c)(3) Public Charity for the NF2 International Community & NF2 Crew.

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## Our Mission:

**Advocure NF2 is dedicated to advocacy, and to strengthening efforts that expedite research contributing to systemic therapies to treat and eventually cure NF2.**



Email: [contact@advocurenf2.org](mailto:contact@advocurenf2.org)

Web: [www.advocurenf2.org](http://www.advocurenf2.org)

[RSS Feeds](#)

**How to Help** - Advocure NF2 Inc. is a 501(c)(3) public charity.

All contributions to Advocure NF2 Inc. are tax-deductible




## NF2 donations, and in memory of those we lost:

- Roland Thoms (Halloween fundraiser, see pg 9)
- Mary Davis
- John and Linda Manth
- anonymous
- Julie Pittman Bogard
- Johnna Somerville


- Jennifer S Schwartz
- Beth Humpert
- Tom and Lori Nolan
- Steve Lee
- Catherine Hawkins Farnsworth
- Sheryn Helgason
- Carol Ellis
- Beth Catharine
- Glenn Barnier
- Sylvia and Shanna Byrd
- The Orr Family
- Roberto da Silva

**Thank you for your support!**

## NF2 in the News:

-  **Advocure NF2** attends the **NF Midwest 2010 Symposium** held in Chicago, on Oct 16, 2010. Synopses of Dr. Vijaya Ramesh's presentation: '[Understanding the Meningioma and Schwannoma Growth in NF2](#)'.
- **Advocure NF2** attends the **2010 NF2 Ohio Gathering** held in Columbus, on Oct 8-10, 2010. [Transcript](#) of the doctors' presentations on the new NF2 Clinic in Columbus OH, and on *Avastin* for NF2.



-  **Advocure** Inc. [Advocure Raises \\$20,000 for NF2 Research at OSU!!](#)  
See "Sixth Annual Thoms Halloween Bash" page 9.



- [2011 Strategic Plan: Request for Ideas](#) (RFI). Please complete this short RFI on NF, and return to: Dr. Kim Hunter-Schaedle, Chief Scientific Officer: [khs@ctf.org](mailto:khs@ctf.org) or FAX to 212-747-0004 - Deadline: December 20, 2010.

The RFI can be [downloaded](#) as a MS document.

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## [The Children's Tumor Foundation Names](#)

[new President](#) - Advocure NF2 Inc. would like to express our sincere appreciation to Mr. John Risner for his service and a productive tenure as President of the Children's Tumor Foundation (CTF). We wish him every success in future endeavors. At this time, we would like to extend a warm welcome to CTF's new President, Mr. George Orfanakos. We look forward (as well as remain hopeful) in continuing our mutual aim of expediting treatments and a cure for NF2.

Mr. Orfanakos is currently the Executive Director of CTF



Advocure raises NF2 awareness at the recent [Communication Access & Technology Expo](#) in FL, on November 20, 2010.



## [Advocure NF2 is on Facebook!](#)

Help support our Cause!!



## [Request for Applications \(RFA\) for a second phase of the 'NF Preclinical Consortium' \(NFPC\) totaling \\$3.5M](#)

Phase 2 of the NFPC will provide up to two extended years of funding. Current phase 1 NFPC Centers may apply for a continuation of funding, and new applications are invited from other centers that wish to join the Consortium. Funding (at ~\$1/4M ea. per yr) is available for UP TO five representing NF1 tumors and UP TO two representing NF2 tumors. Applications deadline is 5:00PM EST Monday January 10, 2011.



## ["NF Preclinical Consortium" brochure](#) -

An informative brochure on the NF Preclinical Consortium program, so far, at CTF. Summary list of preclinical drugs:

### SUMMARY OF DRUGS TESTED AND STATUS

MODELS	DRUG	TARGET	AGREEMENTS	PILOT STUDY	PRE-CLINICAL TRIAL
NF1, NF2	BEZ-235	PI3K/MTOR	SIGNED	COMPLETE	COMPLETE
NF1, NF2	Lapatinib	EGFR/Her2	n/a	COMPLETE	COMPLETE
NF1	GDC-0941	PI3K	SIGNED	COMPLETE	IN PROCESS
NF1, NF2	GNX-222	Pan ErbB	SIGNED	COMPLETE	IN PROCESS
NF1	PD-0325901	MEK	SIGNED	IN PROCESS	
NF1	GDC-0941/ PD-0325901	PI3K/MEK	SIGNED	IN PROCESS	
NF2	anti-ErbB3	ErbB3	IN PROCESS		
NF1	MK-2206	Akt	IN PROCESS		



## [Phase 2 Study of Bevacizumab in Children and Adults With Neurofibromatosis Type 2 and Symptomatic Vestibular Schwannoma](#) *ClinicalTrials.gov*

Identifier: NCT01207687. - Following on from the promising results that have emerged to date from bevacizumab (Avastin) for the treatment of tumor growth and resulting hearing loss in NF2, a new Avastin NF2 trial will open shortly. The trial aims to improve hearing and decrease the size of vestibular tumors and is being opened on October 18, 2010. This study is collaboration between Johns Hopkins Hospital (serving as the primary coordinating site), Massachusetts General Hospital and the National Cancer Institute. People with NF2 who are 12 years of age or older and who have recently experiences hearing loss due to their vestibular tumors may be eligible for this study. People with NF2 who have worsening hearing in their only hearing ear are considered particularly appropriate study candidates. If you are interested in participating in this study or learning more about it, please contact Amanda Bergner in the Central Study Office at Johns Hopkins at 410-502-6732 or [abergne1@jhmi.edu](mailto:abergne1@jhmi.edu)



## [A Phase 2 Study of Nilotinib in Growing Vestibular Schwannomas](#). (in Canada).

ClinicalTrials.gov Identifier: NCT01201538.

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## The Laryngoscope

### [Facial Reanimation Surgery in NF2](#)

**[Restores Smile](#)** - Facial paralysis can be a concern for many persons living with NF2. Though not often top of the list of clinical issues for the care provider, who is principally focused on managing NF2 tumors, facial paralysis can cause a number of issues. Now a team from MGH has presented exciting results restoring the ability of individuals with NF2 to smile following muscle implant surgery and follow up physiotherapy. The MGH team is headed by Dr. Tessa Hadlock and includes Dr. Scott Plotkin who directs the CTF NF Clinic Network Affiliate Clinic at MGH. In NF2, facial paralysis occurs if vestibular schwannoma tumor growth pressing on the facial nerve (which lies close by the vestibular nerve on which the tumor grows), or if the facial nerve is damaged at the time of vestibular schwannoma surgery. In the MGH study, five patients were treated between 2006 and 2009, aged 13-50, with a single stage free gracilis muscle transfer for smile reanimation. The result was a significant increase in the ability to smile. Management of facial paralysis is often overlooked when defining a care plan for NF2 patients that typically have multiple brain and spine tumors. However the loss of facial expression can have great psychological and social impact on the individual with NF2, and this new technique offers the first hope that it may be reversed. The preliminary findings are reported in the journal **Laryngoscope**. Vakharia K, Henstrom D, Plotkin SR, Cheney M, Hadlock TA. (2010). [Facial Reanimation of Patients with Neurofibromatosis Type 2](#). **Laryngoscope**. Oct;120(S3):S108.

## PBS NEWSHOUR [For Scientists, Collaborative Efforts](#)

**[Could Speed Medical Advances](#)** - Special correspondent Dave Iverson reports on a new push to get scientists to work together to find medical cures sooner.



### [Hand-crafted, Beautiful Beaded NF](#)

**[Bracelets for Sale](#)** - Erica, who designs and makes her own hand-crafted beautiful beaded bracelets for awareness of various different causes, including NF! You can order a bracelet at the website listed. For each bracelet purchased \$10 of it will go to Children's Tumor Foundation! For no extra charge, you can order a custom message like HOPE, CURE, NF, RACE, CURE NF, or if you have a special request contact Erica and she will do her best to make it happen! It makes great gifts too.

## Annals of Oncology

### [New Report: Avastin NF2 Tumor](#)

**[Shrinkage Needs Continuous Drug](#)** - "There is continued interest in the use of bevacizumab (Avastin) for treatment of NF2 vestibular schwannomas (VS) due to reports indicating that the drug can reduce the size of these tumors, in some cases sufficient to restore hearing ability. A recent report\* on 2 treated individuals from Dr. Victor-Felix Mautner and colleagues in Hamburg, Germany suggests that these effects of Avastin on NF2 VS relies on continued use of the drug; and that once drug treatment is stopped the tumor will return to its original size. However, it may be possible to obtain continued benefits from Avastin over long periods by i) lowering the dose used and by ii) dosing with the drug at less frequent intervals....." \*Mautner VF, Nguyen R, Knecht R, Bokemeyer C. "[Radiographic regression of vestibular schwannomas induced by bevacizumab treatment: sustain under continuous drug application and rebound after drug discontinuation](#)". *Ann Oncol*. 2010 Sep 22.



### [Is Wi-Fi Safe?](#) - "Reports of parents concerned over

the health risks of Wi-Fi dominate the headlines, but are their concerns valid? We asked two experts on opposing sides of the argument to help you determine if Wi-Fi is safe for you and your family....." By: Lesley Young at BestHealth.

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## [Use this Common Device and](#)

[Double Your Risk of Brain Cancer...](#) - An epidemiologist and toxicologist who is an expert in environmental health has found evidence linking cell phone usage to an increased rate of certain kinds of brain tumors in young people who were heavy cell phone users.....

**My Own Opinion (私的意見)** Blog: [Fibromas of NF1 Patients Disappear in A Month at Last!](#) Note: Would this also be relevant to NF2 too? - "Fibromas (skin tumors) from NF1 patients have never been successfully grafted in nude (immuno-deficient) mice. Thus, it is impossible to test the therapeutic effect of a given drug on the growth of human fibroma xenografts in mice. In other words, NF1 patients carrying a limited number of fibromas would be the best possible "in vivo model" for testing the anti-fibroma property of natural anti-PAK1 products such as Bio 30 (NZ propolis extract) and GPE (Brazilian green propolis extract)....."



## [Considerations for Rare Disease Clinical](#)

[Trials](#) - "A major consideration for a rare disease clinical trial is that it may take a very long time to recruit all of the patients you need. During the time it takes to do a trial, you run the risk of the trial losing momentum; staff turnover, which is inevitable in any clinical research center; or depletion of your own eligible patient population.



[Centers of Excellence for Research on CAM](#) - These Centers support three or four major, synergistic research projects each, and are focused on elucidating mechanisms of action of Complimentary and Alternative Medicine (CAM). (P01 mechanism; R01-level projects)



[CTF announces a call for Letters of Intent for the new NF Clinical Research Awards of up to \\$100,000.](#)

[Dr. Roland to Receive 2010 Wholeness of Life Award at Ceremony on October 28](#) - J. Thomas



Roland Jr., MD, is this year's recipient of the Wholeness of Life Award from HealthCare Chaplaincy, a nonprofit organization that specializes in multifaith, spiritually centered healthcare. Every year, HealthCare Chaplaincy, in coordination with the Department of Pastoral Care, honors a select number of healthcare professionals in metropolitan New York who embody the chaplaincy's commitment to care for the whole person, encompassing spirit, body, and mind. The honorees have all earned a reputation among their colleagues for caring holistically for patients and co-workers by showing respect for the dignity of people, demonstrating a commitment to excellence and caring, and exhibiting compassion and understanding of the spiritual needs of individuals regardless of cultural and religious differences....."



[FDA Funds Could Support Neurofibromatosis Clinical Trials](#) - "New Food and Drug Administration funding could provide an opportunity for neurofibromatosis clinical trial applications to be supported. FDA Orphan Products Development Grants support the clinical development of products for use in rare diseases or conditions for which no current therapy exists or when the proposed product would be superior to the existing therapy. Phase 1 clinical trials are eligible for grants of up to \$200,000 per year for up to 3 years. Phase 2 and 3 clinical trials are eligible for grants up to \$400,000 per year for 4 years. Dollar caps represent ALL costs (i.e. direct plus indirect). Next application deadline is February 2, 2011. Questions: contact [Katherine Needleman](#) or phone 301-796-8660.

To read the Request For Applications (RFA), pls see:

<http://grants.nih.gov/grants/guide/rfa-files/RFA-FD-11-001.html>

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### Outstanding NIH Funding Opportunity for

**Neurofibromatosis Researchers!** - The National Institutes of Health have announced a potential commitment of up to \$50 million OVER 5 YEARS to form a Tumor Microenvironment Network (TMEN). This U54 opportunity proposes to assemble up to TWELVE Centers to 1) generate a comprehensive understanding of composition of the stroma in normal tissues as well as its roles in tumor initiation, progression, and metastasis; 2) delineate mechanisms of tumor-stroma interactions in human cancer; and 3) identify the mechanism(s) by which tumor stroma may affect responses to treatment. Primary brain tumors are of interest as well as peripheral nervous system tumors. The program is co-funded by National Cancer Institute and National institute for Neurological Disorders and Stroke. An applicant may request a project period of up to 5 years and a budget up to \$700,000 per year (direct costs). Given the established role of the microenvironment in NF tumors, this program offers a terrific opportunity for neurofibromatosis research centers to apply. Letters of Intent are due December 20, 2010 and if invited full applications by January 20, 2011. Anticipated earliest start date of funding: September 2011. For more information see: <http://grants.nih.gov/grants/guide/rfa-files/RFA-CA-10-021.html> (Request for Applications RFA-CA-021).



**NIH Announces Five Botanical Research Centers** - *Studies of the safety, effectiveness, and biological action of botanical products are major focuses for the five dietary supplement research centers selected to be jointly funded by the Office of Dietary Supplements (ODS) and the National Center for Complementary and Alternative Medicine (NCCAM), two components of the National Institutes of Health. The NIH's National Cancer Institute is co-supporting two of the five centers.*



### Rare Diseases Clinical Research

**Network (RDCRN), funded by NIH** - The Rare Diseases Clinical Research Network (RDCRN) is made up of 19 distinctive consortia (funded by the NIH) that are working in concert to improve availability of rare disease information, treatment, clinical studies, and general awareness for both patients and the medical community. The RDCRN also aims to provide up-to-date information for patients and to assist in connecting patients with advocacy groups, expert doctors, and clinical research opportunities..... RDCRN is basically a coalition of patient advocacy groups representing these rare diseases as vital partners in the network, in helping set the agenda and providing a link to the affected families. Unfortunately, NF is not currently part of RDCRN yet.



### From Military Device to Life-Saving

**Surgery Tool** - A new tool that allows doctors to use laser surgery in complex operations has been hailed as a breakthrough in minimally invasive laser technology. Originally designed as a military tool for the United States Department of Defense (DOD), the fiber-optic invention is revolutionizing the ways surgeons carry out brain surgeries.



THE NEUROFIBROMATOSIS ASSOCIATION

### Voice Recognition Software

Rosemary Abbott explains about voice recognition software now in trial at Manchester's NF2 Clinic (UK).... If used alot by the same doctors repeatedly within a NF2 clinic, it could be practical, easier, more dignified, and may provide a more cost effective alternative to meeting their traditional 'accommodation' cost obligations, such as sign language interpreters and/or CART.



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- **The Washington Times** [How to Widen the Hunt for Targeted Cancer Therapy](#) - NIH To Address Cancer's 'Big Questions' - Cancer is a tale of two sets of genetic code, your own and your tumor's — and tracing the unique areas of damage makes for a way to target treatment. Fifty years after the discovery of the first direct genetic link to cancer, scientists are assessing the state of so-called targeted therapy — with nearly 30 treatments on the market and a dozen or so more under study.



- **NIH Rapid Access to Interventional Development (NIH-RAID)** - NIH-RAID is intended for use by academic discovery laboratories, not-for-profit organizations, and SBIR-eligible businesses.



- **Weitz & Luxenberg Files Lawsuits Against Cochlear Implants Manufacturer Advanced Bionics** - If you believe that you may have received an "Important Notification" letter from Advanced Bionics about your HiRes90k or Clarion II cochlear implant in either 2004 or 2006, we urge you to contact us to receive important legal information - even if your CI is working. Get a free cochlear implants lawsuit review when you complete the form below, and/or view an important Vlog by attorney Teresa Curtin about our cochlear implants lawsuits



- **The New Nationally Commissioned NF2 Service (UK)** – Article also lists the major NF2 Clinics in the UK, by Dr. Gareth Evans.



- **Using Positron Emission Tomography (PET) to Predict Intracranial Tumor Growth in Neurofibromatosis Type II (NF2) Patients.** ClinicalTrials.gov Identifier: NCT01222728

- **mysuburbanlife.com** [Raising Money for Genetic-Disorder Research](#) - Glen Ellyn resident Sally Richards is working to raise money to benefit research for a genetic disorder she has lived with for 25 years.



- **CTF's Annual New York City Benefit Dinner raised over \$1 million** - Monies raised will help fund clinical trials and preclinical testing to advance future clinical trials. Actor Alec Baldwin was the evening's host and the Master of Ceremonies.



- **Improving Access to Clinical Trials Act** - American Brain Tumor Association (ABTA) - Please contact your congressional representative and ask him/her to sponsor the 'Improving Access to Clinical Trials Act' - H.R. 2866. It would change current Social Security Supplemental Income (SSI) guidelines so that clinical trial participants can receive up to \$2,000 in compensation without affecting SSI eligibility.



- **Curry cooking ingredient could provide recipe for fight against cancer** - Scientists at the University of Leicester, funded by Hope Against Cancer, are pioneering the use of a common curry cooking ingredient to target cancer cells.



- **Anne Shigley Runs NYC 2010 to cure NF!** -NYC Marathon on November 7, 2010.

- **The New York Times** [Children's Hospitals Lose Some Drug Discounts](#) - Children's hospitals have been told by drug companies that they will no longer get discounts for medications, [such as Avastin™, etcetc], that are known as "orphan drugs." The change is an unintended consequence of the new health law, and it is costing hospitals millions of dollars.

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## NF ENDURANCE

- [Sally Richards's Fundraising Page](#) - Race Walk for NF research - "My name is Sally Richards, and I have Neurofibromatosis 2 (NF2). This is a genetic disorder in which tumors form on the nerves of the brain and spine (the central nervous system). I've lived with the uncertainties of NF2 for 25 years. Early after diagnosis, my challenges seemed insurmountable, but they proved to be stepping stones to a stronger future. I went from a shy, quiet individual to an articulate, outspoken professional....."
- [Orofino teen battling rare disease, still finds time to give back](#) - KLEW TV - By Stephanie Smith - 19-year-old Jacob Walk has type-2 neurofibromatosis, a rare genetic disorder where tumors grow on his nerves. Walk said he was battling ...
- [Beautiful in My Eyes](#) - Manila Bulletin - Name: Geraldine J. Oliva Account Number: 5890-12991-1 (The author is suffering from a rare genetic disorder called Neurofibromatosis Type 2 (NF2). ...
- [Canteen Helps Make Life More Normal, says Teen](#) - Northern Advocate - He was diagnosed with a rare genetic disorder known as neurofibromatosis type 2. The lumps grow on nerve endings and can push on the brain and spinal cord ...
- [Personal Tragedy Hits Woman with Rare Genetic Disorder](#) - AsiaOne - She will be sponsored under a programme called the Natural History Study of Patients With Neurofibromatosis (NF) Type 2 conducted by the National Institute ...
- [Tumorigenesis: Merlin, the Liver Wizard](#) - Nature.com - One exception is mice that are heterozygous for the tumour suppressor neurofibromatosis type 2 (Nf2), which develop both types of tumours.
- [Kcat's Dream: To Live in Spite of Brain Tumors](#) - ABS CBN News - Kcat is suffering from NF2, or Neurofibromatosis Type II, a

neurological disease characterized by the growth of non-malignant tumors in the brain. ...

- [Bittersweet Symphony](#) - Pueblo Chieftain - The cause of these seizures: neurofibromatosis type 2, a rare genetic disorder of the central nervous system that creates tumors on the body's acoustic ...
- [Youth: Living with Cancer](#) - Cherokee Tribune - ...Lauren O'Prey's Story: She and her family have done so since 2006 after she was diagnosed with neurofibromatosis type 2 during her senior year at Etowah High School. ...
- [Researchers Link Protein to Tumor Growth](#) - Ascribe - The protein, named YAP, is linked to the NF2 tumor suppressor gene via a relay of ... The team discovered the YAP-NF2 link by studying mice that had been ...
- [Kulin Digs Deep for a Good Cause](#) - Farm Weekly - The day was organised to raise money for 23-year-old Ashleigh Arundel, who was diagnosed with Neurofibromatosis Type 2 seven years ago, an invasive disease ..

## Fundraising:



### Zazzle™ - [Cups & Stuff](#)

Let us customize a product for you; T-shirts, cups, hats with logos, family photos, pet photos, etc.

An easy way to contribute to NF2 this holiday season is to purchase a **magazine subscription**. Forty percent (40%) of the proceeds will be donated to Advocare.

For a list of magazines, please [click here](#).



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## Group Self-Organizing to Advocate for Cures to NF2

[Advocure is lobbying government and putting a more prominent face on this rare, but devastating, genetic disease.](#)

(PRWEB) June 16, 2006 - Advocure - Advocates for a Cure for NF2, a parent/patient advocacy project, recently launched a web site, <http://www.advocurenf2.org>, designed to help raise the profile of this devastating genetic disease.

Neurofibromatosis Type II (NF2) is one of a pair of historically linked, but genetically distinct, disorders. NF2 causes tumors to grow throughout the brain and spinal cord (Central Nervous System - CNS), threatening hearing, vision, mobility and even basic life functions like swallowing. While these are mostly benign tumors, they are 'malignant' by location and number. In its mildest form, the disease is characterized by bilateral acoustic neuroma -- tumors on both sides of the head. Eventually surgery or radiation becomes necessary, risking immediate deafness and facial paralysis. In almost all cases, deafness is the final outcome regardless. NF2 affects 1 in 35,000 newborns. Despite the fact that the NF2 gene was identified in 1993 and much has been learned about how the gene works, there is still no systemic drug therapy for NF2

And therein lies the reason Advocure was formed.

While a number of drug trials have been in place for NF1 for some time, there are no promising trials for the sufferers of NF2. For 10 years, a US Army's [Congressional Directed Medical Research Programs](#) (CDMRP-NFRP) has driven investigations into both of these debilitating diseases in a non-discriminatory basis. NF1 is much more common than NF2, affecting 1 in 3,500, and is characterized by dark skin markings (café-au-lait spots), multiple tumors on or just below the skin, and learning disabilities. Only a minority develop more severe CNS tumors.

However, in 2004, the CDMRP's Neurofibromatosis Research Program (NFRP), took the controversial step of announcing a new NF Consortium Award, to develop the infrastructure and resources for investigators to conceive, develop, and conduct collaborative clinical trials - **but for NF1 only.**

Families dealing with NF2 were appalled at being left out in the cold in this new push towards clinical trials, and well aware of how not including NF2 right from the early set-up stages would impact future research directions. It seems the NFRP expected the NF2 community to be satisfied with vague promises to include NF2 at some stage in the future. It is one thing to live with a debilitating disease where patients often develop countless CNS tumors, but to live without hope is a much crueler proposition. The NF2 community found this blatant exclusion from the Consortium Award particularly disturbing. Advocure hopes to change that.

Advocure has undertaken to work towards ensuring NF2 sufferers will not be left behind. In the short term, the group will lobby relevant governing bodies and the medical establishment for the immediate inclusion of NF2 in this new award mechanism. Over the longer term, they will ensure that NF2 interests are represented on the boards and committees directing both research and research funding, to raise awareness about the devastation of the disease, and to advocate for the urgent implementation of drug therapy.

Their new website (<http://www.advocurenf2.org>) is a key part of that mission. The site features updated information on relevant research, how current scientific understanding can lead to therapies aimed at stopping NF2 tumor growth, helpful links in understanding and dealing with NF2, and information on where and how to donate to NF2-related charities. Advocure's site also features the personal stories, blogs and photography of many living with this destructive genetic anomaly.

As Advocure mobilizes, members hope to contribute to push therapies from theory into reality.

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## Sixth Annual Thoms Halloween Bash

By: Roland Thoms  
[Advocure NF2, Inc.](http://Advocure NF2, Inc.)



The 6<sup>th</sup> annual Thoms Halloween Bash benefit Children's Hospital Oakland and find a cure for Neurofibromatosis 2 took place on Friday, October 22<sup>nd</sup> in Walnut Creek, California. Roland and Nicole have been throwing this Halloween since 2003, a year after their daughter, Camille (then 7 years old) was taken to

the hospital for emergency surgery to remove a tumor the size of a tangerine from her brain. It was at that time that she was diagnosed with Neurofibromatosis 2. Since then, she has had MRIs every 6 months to monitor the growth of the other tumors growing in her body. She had Middle Fossa surgery on her left ear at the House Ear institute last year (November 2009) with Dr. Derald Brackmann. While the surgery was successful in removing the tumor and preserving her cochlear nerve, she was rendered deaf in that ear. She is a candidate for a future cochlear implant.

The party started in the Thoms backyard under the banner "Celebrate Life". It became a fundraiser for the hospital in year 3 to benefit the hospital that saved her life. This year was the first time that the majority of funds raised are going to NF2 through ADVOCURE. \$28,000 was raised altogether through entrance fees, a silent auction, corporate sponsorships and by auctioning off 4 paintings executed by performance painter, David Garibaldi. His performance knocked everybody's socks off as he painted 5 foot x 6 foot portraits of Mick Jagger, Bob

Marley, a San Francisco Giants batter, and Albert Einstein (which he painted upside down...the painting, not him). These 4 paintings fetched \$10,000 for ADVOCURE. Another \$10,000 was earmarked for ADVOCURE so that \$20,000 can be sent to Dr. Brad Welling, of Ohio State University. Dr. Welling is currently investigating two lines of drug development that hope to suppress tumor growth. One involves a study into naturally occurring compounds, while the other is focused on traditional drugs.

Next year's Bash will be held on Friday, October 21<sup>st</sup> with goals of taking it to the next level. Information is at: [www.varsitypainting.com/halloween](http://www.varsitypainting.com/halloween) and [www.varsitypainting.com/camille](http://www.varsitypainting.com/camille) (Camille's story)



## Forward to a Friend?

Know someone who would like to read this online newsletter?  
Why not forward it to them? Thanks.

[http://www.advocurenf2.org/newsletter/NF2-compass\\_2010winter.pdf](http://www.advocurenf2.org/newsletter/NF2-compass_2010winter.pdf)



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## Advocating for NF Federal Research Funding

by: [John and Linda Manth](#)  
[Advocure NF2, Inc.](#)



### How is NF Federally Funded?

Since 1996, NF research has been funded by Congress through the [Congressionally Directed Medical Research Programs](#) (CDMRP-NFRP) (from the Department of Defense (DOD)), and through the [National Institutes of Health](#) (NIH).

### Government 101

#### **United States Congress**

The United States Congress is the bicameral legislature of the federal government of the United States of America, consisting of the *Senate* and the *House of Representatives*. The Congress meets in the United States Capitol in Washington, D.C. Both *Senators* and *Representatives* are chosen through direct election. Each of the 435 members of the *House of Representatives* (Congressmen) represents a district and serves a two-year term. House seats are apportioned among the states by population. Meanwhile, the 100 *Senators* serve staggered six-year terms. Each state has two senators, regardless of population.

These are the links to finding your *senator* and/or *representative*:

- **U.S. Senate:** <http://www.senate.gov/>
- **U.S. House of Representatives:** <http://www.house.gov/>

Current US Federal legislative Information. Bills, Laws, Congressional Record, reports, and links to further information can be found at: <http://thomas.loc.gov/>

### **Committees**

Both the House of Representatives and the Senate have committees that draft laws and determine funding levels for various appropriations. The following are the committees which are most important for NF federal funding.

#### [Senate Committee on Appropriations](#)

- Labor, Health and Human Services, Education Subcommittee
- Defense Subcommittee

#### [House Appropriations Committee](#)

- Labor, Health and Human Services, Education Subcommittee
- Defense Subcommittee

### Why do We Need to Advocate

Advocacy is an area that many NF2 patients and families are unfamiliar with and as a result may or may not be interested in participating in. The United States Government funds research for NF1, NF2 and Schwannomatosis through the [Congressional Directed Medical Research Program](#) (CDMRP-NFRP) and the [National Institutes of Health](#) (NIH).

These two agencies currently fund millions of dollars toward research. We need to advocate for not only the continuation of this funding, but for funding to increase, specifically in the area of NF2 research.

### How to Advocate

#### **At Your Home District:**

Everyone in the United States has a local *Congressman* from their district and two *Senators* from their state. You can make an appointment and meet with one of their staffers anytime you would like to. It is more difficult to get a face-to-face meeting with the actual *Senator* or *Congressman*, but it can be done if you are flexible and persistent. Both your local *Congressman* and State *Senators* will have offices within a close proximity to your home town or city. Meeting at your home district is definitely easier than traveling to Washington, DC and can be equally effective if you meet with a



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staffer that relays your message to *the Congressman/Senator* if a face-to-face cannot be arranged.

### Going to Washington DC:

Going to DC to lobby is a much more extensive effort. Appointments have to be made ahead of time and it can have more impact if it is timed correctly. The following year's appropriations begin getting discussed in February of the current year (e.g. in Feb. 2010 fiscal year 2011's appropriations begin to be discussed) with negotiations usually lasting into August/September. Those discussions all begin with a 'Dear Colleague Letter' that must be circulated in February and needs enough signatures on it. A 'Dear Colleague Letter' is a letter written by one *Congressman/Senator* asking a committee to consider a certain level of funding. The more signatures on a letter, the more influence it is likely to have. While meeting with *Congressmen/Senators* at anytime is valuable, it definitely has more impact when your cause is fresh in the minds of the people who are actually signing on.

### Letter Writing:

Writing letters can also be effective. It takes approximately 30 letters about the same subject to have an impact on a *Congressman/Senator*. This is best done with a concerted effort through NF organizations like *NF Inc.* and *CTF* who each have lobbyists assisting their organizations and supplying them with up-to-date information. *ADVOCURE* will supply links to those organizations along with sample letters, which again may have more of an effect at certain times of the year. Letters are sent by e-mailing not postal service because of security screening. Here are the links for *NF Inc* and *CTF's* advocacy information and sample letters:

- [http://nfnetwork.org/get\\_involved/advocacy/](http://nfnetwork.org/get_involved/advocacy/) (*NF Inc*)
- <http://www.ctf.org/How-You-Can-Help/advocacy.html> (*CTF*)

**How to Lobby Your Legislator** - Even as a single person, you can have an impact on how your legislator votes, and what bills he or she decides to sponsor:

[http://www.ehow.com/how\\_4783812\\_lobby-your-legislator.html](http://www.ehow.com/how_4783812_lobby-your-legislator.html)

### Tell Your Personal Story:

To leave a positive impression on a *Congressman/Senator* you should think about leaving something with them to help make a personal connection. Some people have photos, picture books made up, wrist bands related to fundraising they do. Think of something that the staffer you are most likely meeting with can take to the *Congressman/Senator* and really relay your story and why funding for NF is so important.

### What to Ask for:

If you are going to meet with a staffer the first thing they will ask you is "What can we do for you?" You want to have an answer, and a specific one would be best. The reason why timing is more important here is because of the 'Dear Colleague Letter'. Asking for the *Congressman/Senator's* signature on that letter is a great achievement.

If the *Congressman/Senator* you are meeting with is on one of the above mentioned committees, they are NOT allowed to sign a 'Dear Colleague Letter', since they are a member of the committee determining the appropriations. By meeting with an actual committee member, you may actually yield more power. You simply need to ask them to not only continue NF funding, but increase NF's allocation. These members change each year and again. *ADVOCURE* can post links to sites where this information is available. Keep in mind members of both branches of government and committee members have all been contacted by an NF representative, the "squeaky wheel gets the oil!"

### How Can ADVOCURE NF2 Assist?

*ADVOCURE* would like to be the coordinators and assist NF2 patients and families with their advocacy efforts. If we are going to have a voice, having a unified voice would be best. Consider donating or directing your fundraising efforts to advocacy. This is where thousands of dollars in supporting lobbying can turn into millions of dollars of federal research funding. Money donated to *ADVOCURE* could be used to assist in the cost of lobbyists associated with NF groups. For further information and assistance, please contact us at: [contact@advocurenf2.org](mailto:contact@advocurenf2.org)

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## Advocacy Appeal from



Reprinted from: <http://www.ctf.org/How-You-Can-Help/advocacy.html>

### Update December 1, 2010

Congress came back for a lame duck session and has extended the Continuing Resolution (CR) expiration from December 3 to December 18th. This is a good sign, as it bodes well that Congress intends to pass full appropriation bills rather than another CR that would expire next year.

The CDMRP-NFRP is funded through the defense appropriations bill as part of our military's healthcare program. The House completed their work on the bill over the summer and we expect that their bill included the \$20 million requested for FY2011. The Senate is currently working on their version of the defense appropriations bill, and we expect a conference committee composed of House and Senate members to meet quickly to work out the differences in the two versions and pass a joint bill.

Please email your Senators as soon as possible seeking their support to include \$20 million of funding for the Congressionally Directed Medical Research Program-Nf Research Program (CDMRP-NFRP) in FY 2011. This program has been vital to the advances we have seen in NF research, and its continued funding is critical in our drive to translate the progress made in the lab to actual FDA approved treatments for NF. You can reach your Senators by visiting <http://www.senate.gov/> and clicking on "Find your Senators" in the upper right corner of the site.

While all outreach is important, if you live in **AL, CA, NH, IL, KS, KY, MS, MO, OH, PA, TX, UT, VT, WA, or WI** your voice is particularly important. The Senators in these states (listed on webpage at left) are members of the defense subcommittee of the appropriations committee which ultimately determines the success or failure of this program.

It is important to note that this funding is not an earmark – it has been in existence since 1996 and none of the funding is directed to any particular company or institution until after going through a strict, two-tier peer review process.

Please take 3 minutes now to email both of your Senators now to help preserve this vital program. In addition to any personal stories you would like to add to emphasize the vital importance of this program, here is some suggested text:

*I am writing to ask for your support for \$20 million of funding for the Congressionally Directed Medical Research Program- NF Research Program (CDMRP-NFRP). This program has been funded through the Department of Defense since 1996 and has been vital to the advances made in NF research. Critical work in nerve regeneration, brain and spinal trauma and pain management have led to important advances in research, and it is the current clinical trials consortium funded through program that offer promise of actual treatments in the very near future.*

As the Senate considers the appropriations bill for FY2011 I hope you will support the \$20 million requested for the CDMRP-NFRP. Thank you.



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## NF2 Clinical Trials & Studies:

- [Phase 2 Study of Bevacizumab \(Avastin™\) in Children and Adults With Neurofibromatosis Type 2 and Symptomatic Vestibular Schwannoma](#)

ClinicalTrials.gov Identifier: NCT01207687

**This study is NOT yet open for participant recruitment.**

This study is exploring whether a drug that is approved by the FDA and is currently used to treat other tumors might also work to treat VSs. Based on people who have taken this drug to treat VSs already, there is some reason to think that it might be helpful to certain people with **NF2**. People enrolled in this study will receive the drug one time every three weeks for one year by infusion. This study will follow subjects over the course of the year that the person is taking the drug and for six months after the drug is stopped. This study is recruiting people who have **NF2** and are currently having symptoms of tinnitus, dizziness, and/or hearing loss from their VSs. If you have **NF2** and are currently having symptoms caused by your VSs, you may be eligible to participate.

- [A Phase II Trial of the Combination of Bevacizumab \(Avastin™\) and Everolimus in Patients with Refractory, Progressive Intracranial Meningioma](#)

ClinicalTrials.gov Identifier: NCT00598351

**This study is currently recruiting participants.**

*In this multi-center, Phase II trial, the investigators plan to evaluate the activity of the combination of bevacizumab (Avastin™) and everolimus in patients with recurrent, progressive meningioma following maximal treatment with surgical resection and local radiation therapy. Although these patients are relatively rare, there is currently no established standard of treatment for a disease that causes a great deal of morbidity, and that is eventually fatal.*

- [Natural History Study of Patients with Neurofibromatosis Type 2 \(NF2\)](#)

ClinicalTrials.gov Identifier: NCT00598351

**This study is currently recruiting participants.**

*This study will examine over the long-term the progress of patients with neurofibromatosis Type 2 (NF2), a condition associated with tumors of the nerves, brain and spinal cord. It will study patients' tumors to learn how fast they can grow and if certain factors might affect their growth. It will also examine the effects of the tumors on patients' abilities to carry out activities of daily living. People between 8 and 75 years of age with **NF2** may be eligible for this study, (**most expenses are reimbursed**).*

- [Phase II Study of Nilotinib in Growing Vestibular Schwannomas](#)

ClinicalTrials.gov Identifier: NCT01201538

**This study is NOT yet open for participant recruitment.**

*The primary objective of this study is to evaluate the efficacy of Nilotinib in the treatment of patients with progressing sporadic and **NF2** VS. Secondary objectives of this study is to evaluate the toxicity profile, quality of life and symptom management of Nilotinib in the treatment of patients with progressing VS.*

- [Using Positron Emission Tomography \(PET\) to Predict Intracranial Tumor Growth in Neurofibromatosis Type II \(NF2\) Patients](#)

ClinicalTrials.gov Identifier: NCT01222728

**This study is currently recruiting participants.**

**Objectives** - To use magnetic resonance imaging and positron emission tomography to better understand the growth of brain tumors in people with **neurofibromatosis type II**.

**Eligibility** - Individuals between 18 and 50 years of age who have been diagnosed with **NF2** and have at least three untreated intracranial tumors.

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- [Neurofibromatosis Type 2 Associated Color Vision Anomalies and Birth Defects: Incidence and Insights](#)

**This study is currently recruiting participants**

What is the purpose of the study? 1/ Determine the frequency of the birth defects and miscarriages in patients with **NF2**. 2/ Determine the frequency of color blindness in **NF2** patients

- [Phase II Trial of Bevacizumab \(Avastin™\) in Patients With Recurrent or Progressive Meningiomas](#)

ClinicalTrials.gov Identifier: NCT01125046

**This study is NOT yet open for participant recruitment.**

**RATIONALE:** Monoclonal antibodies, such as bevacizumab (Avastin™), can block tumor growth in different ways. Some block the ability of tumor cells to grow and spread. Others find tumor cells and help kill them or carry tumor-killing substances to them.  
**PURPOSE:** This phase II trial is studying how well bevacizumab (Avastin™) works in treating patients with recurrent or progression meningiomas.

- [Sunitinib in Treating Patients with Recurrent or Unresectable Meningioma, Intracranial Hemangiopericytoma, or Intracranial Hemangioblastoma](#)

ClinicalTrials.gov Identifier: NCT00561665

**This study is currently recruiting participants.**

This phase II trial is studying sunitinib to see how well it works in treating patients with recurrent or unresectable meningioma, intracranial hemangiopericytoma, or intracranial hemangioblastoma

- [Phase II Trial of Sunitinib \(SU011248\) in Patients with Recurrent or Inoperable Meningioma](#)

ClinicalTrials.gov Identifier: NCT00589784.

**This study is currently recruiting participants.**

Sunitinib is a drug approved for advanced kidney cancer. Sunitinib is also being studied for other tumors. It may be useful in the treatment of brain tumors because it can prevent formation of new blood vessels that allow tumor cells to survive and grow.

- [Concentration and Activity of Lapatinib in Vestibular Schwannomas](#)

ClinicalTrials.gov Identifier: NCT00863122

**This study is currently recruiting participants.**

This study is exploring whether a drug that is approved by the FDA and is currently used to treat breast cancer might also work to treat VS. This study will measure the amount of drug that travels from the bloodstream and arrives at the tumor. This drug is safe and has few side effects. If this drug is shown to reach the tumor, it might be used in the future to treat VS without needing surgery or radiation. This study is recruiting people who are having surgery for VS. If you are going to have surgery to treat a VS, you may be eligible to participate

- [PTC299 for Treatment of Neurofibromatosis Type 2 \(NF2\)](#)

ClinicalTrials.gov Identifier: NCT00911248

**This study is currently recruiting participants.**

PTC299 is an oral drug that has been shown to decrease production of VEGF in animal models of human cancer. In these animal models, oral PTC299 administration decreases VEGF levels in the tumor and in the bloodstream, decreases blood vessel numbers in the tumor, and significantly slows or halts tumor growth. This Phase 2 study is designed to test the hypothesis that PTC299 will be tolerable and will show evidence of VEGF reduction, anti-tumor activity, and hearing improvement when administered orally to patients with **NF2**.

- [Oncology – PTC299](#)

- [Neurofibromatosis Type 2 Clinical Trial Overview](#)

- [Neurofibromatosis Type 2 Trial FAQ](#)

- [Auditory brainstem implant \(ABI\) patients needed for research study at MEEI and MGH.](#)

A clinical research study of patients who have an auditory brainstem implant (ABI) is being conducted jointly by researchers. We will use specialized hearing testing to better understand how your brain responds to stimulation from your ABI.



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• [Everolimus \(RAD001\) for the Treatment of Malignant Pleural Mesothelioma With Merlin/NF2 Loss as a Biomarker to Predict Sensitivity](#)

ClinicalTrials.gov Identifier: NCT01024946

**This study is currently recruiting participants.**

For patients with malignant pleural mesothelioma that has grown despite treatment with standard chemotherapy, no treatment has yet proven beneficial. The purpose of this study is to find out what effects, both good and bad, that everolimus has on the cancer. Everolimus works by blocking a protein that helps the cancer grow. The goal of this clinical research study is to learn if the study drug everolimus can shrink or slow the growth of mesothelioma. The safety of this drug will also be studied. The patients' physical state, changes in the size of the tumor, and laboratory findings taken during the study will help us decide if everolimus is safe and effective.

• [Monthly SOM230C for Recurrent or Progressive Meningioma](#)

ClinicalTrials.gov Identifier: NCT00859040

**This study is currently recruiting participants.**

The purpose of this research study is to evaluate the effectiveness and safety of SOM230C in treating recurrent meningiomas. SOM230C is a newly discovered drug that may stop meningioma cells from growing abnormally. This drug has been used in treatment of other tumors, and information from those other research studies suggests that SOM230C may help to stop the growth of meningiomas.

• [Corticosteroids in Prevention of Facial Palsy after Cranial Base Surgery](#)

ClinicalTrials.gov Identifier: NCT00438087

**This study is currently recruiting participants.**

Facial palsy after surgical removal of cranial base tumors adherent to the nerve can partly be explained by inflammation.

## SOME Pharmaceuticals of NF2 Interest:

*\*If you have any questions about these, please discuss with your primary caregiver and/or oncologist\*.*

• **PTC124** (Ataluren™), investigational new drug designed to enable the formation of a functioning protein in patients with genetic disorders due a nonsense mutation. [".....Though there may be applications in all forms of NF, it is considered that there may be most relevance initially to NF2 where nonsense mutations account for a significant proportion of sporadic cases."](#)

• bevacizumab (**Avastin**™) is a biologic antibody designed to specifically inhibit the VEGF protein that plays an important role in development and maintenance of blood vessels, a process known as angiogenesis.

- [sorafenib](#) (Nexavar™)
- [erlotinib](#) (Tarceva™)
- OSU-HDAC42 ([AR-42](#)™)
- OSU-03012 ([AR-12](#)™)
- [PTC299](#)
- [lapatinib](#)
- [propolis](#) ([BIO30](#)™ propolis)
- [RAD001](#)
- [valproic acid](#)
- [rapamycin](#)
- [cetuximab](#)
- [trastuzumab](#)
- [vandetanib](#) ([Zactima](#)™)
- [nilotinib](#)
- [curcumin](#)

If there is any other information about NF2 drugs or drug trials that you wish to bring to our attention, please contact us at: [contact@advocurenf2.org](mailto:contact@advocurenf2.org)

For more trials and/or studies, please see: [www.clinicaltrials.gov/ct2/results?term=nf2](http://www.clinicaltrials.gov/ct2/results?term=nf2)

When there, please try inputting "neurofibromatosis type 2", or a NF2 tissue type, such as; "Vestibular Schwannoma", "Schwannoma", "Meningioma", "Glioma", "Ependymoma", or "Astrocytoma", within the relevant search field.