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.

In this Issue:

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

Web: www.advocurenf2.org

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Thank you for your support!

NF2 in the News:

- ***Advocure*** **sends reps with the** ***NF Coalition*** **group to lobby in Washington DC to lobby Congress on Jan 31 and Feb 1 for further funding of NF research**

- ***Phase 2 of the NF Preclinical Consortium is set to Proceed*** CTF is delighted to announce a Request for Applications (RFA) for a second phase of the 'Neurofibromatosis Preclinical Consortium' (NFPC), totaling $3.5M, which will provide up to two years of funding (July 1, 2011 through June 30, 2013). Funding is available for UP TO five (5) NFPC Centers representing NF1 tumors, and UP TO two (2) NFPC Centers representing NF2 tumors, at approximately $250k (each) per year.

- **NF2 Newsletter Winter 2010** 'NF2 News' winter 2010 newsletter by the NFA, in the UK. Interesting opening article on the NFA's 5 year plan. Also, a very interesting article on Avastin for NF2, on page 7.

- ***Inside the "Orphanage" at the FDA*.**

A concisely-written, open-access article published in the *Journal of Cardiovascular Translational Research* entitled the 'Orphanage at the FDA', outlines the role and responsibilities of the FDA's Office of Orphan Product Development (OOPD), established in 1982. The OOPD does not determine which products should receive marketing authorization; rather, it promotes *"the development of products that demonstrate promise for the diagnosis, prevention, and/or treatment of rare diseases or conditions".* The article also describes the Orphan Products Grants Program available for clinical trials involving drugs, biologics, devices and medical foods for rare conditions, and through which, *"since 1983 more than 500 funded studies have yielded data that have brought 45 products to market". A Pediatric Device Consortia Grant Program was also established in 2007, which funds "nonprofit consortia that create new pediatric medical devices linking innovators and manufacturers".* Currently, four such consortia receive funding.

- **USA seeks to consolidate its research programme for rare diseases and orphan drugs on eve of workshop with Europe**.

A comprehensive new study, entitled *Rare Diseases and Orphan Products: Accelerating Research and Development* has been produced by the independent, non-profit Institute of Medicine of the National Academies at the request of the US National Institutes of Health. Offering a detailed overview of the rare disease and orphan drug situation in the United States, with frequent comparisons to Europe and other countries, the document encompasses epidemiology, cause, prevention, diagnostics, treatment, and the impact of rare diseases. The regulatory framework for orphan drugs is delineated, with comparions between the US approach and other countries. In the field of research, the report evaluates target discovery, therapeutics discovery, the infrastructure for basic research and drug discovery for rare diseases, and innovation platforms for target and drug discovery. The authors – comprising a committee of experts from diverse institutions and organizations – consider the development of new therapeutic drugs and biologics, medical devices, and explore issues relating to coverage, reimbursement and various incentives and disincentives for rare disease product development.

- ***Opening Options for Neurofibromatosis 2.*** - The Galloway's generous gift made possible the *phase II Avastin trial for NF2* at John Hopkins, MGH, and NCI (ClinicalTrials.gov Identifier: NCT01207687).

- **FDA declares its commitment to accelerating orphan drug development** A comment by members of the US FDA's Office of Orphan Products Development and Office of New Drugs, appearing in *Nature Reviews Drug Discovery*, highlights new policy initiatives that aim to enhance progress in the developmental process of medicinal products for rare diseases in the USA. Accelerating Orphan Drug Development details the achievements of the Orphan Drug Act (1983) in the USA: over 2,250 orphan drug designations, of which 361 have received marketing approval. Furthermore, "*in 2009, orphan drugs constituted 38% of the 29 new therapies that the US FDA approved for marketing".* Of particular interest are initiatives to speed up the process of bringing products to market for rare conditions, which include the recently founded Rare Diseases Program within the Office of New Drugs of the FDA's Center for Drug Evaluation and Research; the recently published report by the Institute of Medicine on accelerating rare disease drug development (learn more); and the FDA's internal Rare Disease Review Committee (mandated by the Section 740 Amendment), which is "currently performing a comprehensive analysis of current and projected practices for rare disease review and regulation at the FDA. The report describing the findings and recommendations of the review group is due to be presented to the US Congress in March 2011". The article sums up by stating that the *"FDA is committed to accelerating orphan drug development through a regulatory system built on integrity, consistency and transparency; a system that has delivered benefits to people who desperately need them and promises to deliver much more".* Consult the PubMed abstract.

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- ***Give NIH Your Feedback On Proposed Translational Research Center*** *The National Institutes of Health (NIH) proposal to create a new National Center for Advancing Translational Sciences (NCATS) - to be dedicated to accelerating the identification of drug treatments - has promoted media interest as well as questions on whether this will result in cannibalization of other centers at NIH. NCATS could actually offer significant advantages for neurofibromatosis: unlike e.g. cancer, because its a multi-system disorder (affecting many tissues) NF doesn't have one focused NIH institute supporting NF research. Instead NF research funding is spread out across 9 or 10 institutes ranging from NINDS (neurological disorders) to NIAMS (muscular and skeletal diseases) which can have a diluting effect on the impact of any funds invested in NF. In short - a focused translational institute could really help advance the development of NF drug therapies. Now NIH is soliciting feedback on NCATS. We encourage you to take a look at the proposal and offer your comments on the Web site.*

- ***Back to the future: Proceedings from the 2010 NF Conference***. (excerpts) "*…..Recently, however, an overall consensus has been reached by the NF2 community to pursue a strategy Phase 0 and pilot Phase II clinical trials [Evans et al., 2009]…..*

NF2 Clinical Trials update:

• Dr. D. Bradley Welling (The Ohio State University) reviewed the status and outlook for NF2 clinical trials.....

• Dr. Matthias Karajannis (New York University Langone Medical Center) described a new Phase II Clinical Trial of Lapatinib in children and adults with NF2-related tumors....

• Dr. Scott Plotkin (Harvard Medical School/Massachusetts General Hospital) reported on the extended follow up of 29 NF2 patients treated with Bevacizumab……."

- ***PTC Therapeutics Announces Achievement of Milestone in BMI1 Collaboration with the Wellcome Trust - PTC Receives $2.2 Million Payment*** *- PTC Therapeutics, Inc. (PTC) today announced that it has identified a chemical series of molecules that penetrates the blood-brain barrier in animal models and reduces levels of BMI1, a protein linked to drug-resistant cancers. The research marks a milestone in PTC's collaboration with the Wellcome Trust and triggers drawdown of a $2.2 million tranche of their $5.4 million Seeding Drug Discovery award.*

- **CDMRP's FY10 Annual Report -** CDMRP's vision is to find and fund the best research to eradicate diseases and support the war-fighter for the benefit of the American public. Consumer involvement with CDMRP has exceeded 500 organizations since it began in 1992. CDMRP continues to value the experiences of our consumer advocates.

- **Request for Information (RFI) for the NIH Director's Early Independence Award Program-** A major concern among young researchers is the gloomy prospect that when they are done with their Ph.D or MD training, they won't be able to get funding to set up their own lab. This might include Children's Tumor Foundation Young Investigator Award recipients ready to become independent neurofibromatosis researchers. It is a worrying situation as young researchers are the 'seed corn' of science, ensuring future continued research progress. Faced with the current financial situation, though, many young scientists leave scientific research for other career paths. To address this, and especially to ensure support for the brightest young stars, the National Institutes of Health is planning an Early Independence Award (EIA) that would allow researchers to move directly from completing their Ph.D or MD, and on to running an independent lab. At this time the NIH is asking for YOUR feedback and input on this proposed program. Deadline for feedback: March 18th. For more information and to offer your comments.

- ***Orphan drug discounts for children's hospitals in the USA saved by Medicare and Medicaid Extenders Act***
  Discounts on orphan medicinal products for children's hospitals were menaced by a provision of the sweeping federal health care legislation overhaul enacted in the USA earlier this year. The provision prohibited independent children's hospitals from accessing discounts to orphan drugs. However, the Medicare and Medicaid Extenders Act 2010 signed in by President Barack Obama on 15 December removes children's hospitals from the orphan drug exclusion found in section 340B(e) of the Public Health Service Act, rendering the discounts once again accessible.

- ***Excellent Captioning Rsource!***
  20/20 Captioning & Reporting provides open and closed captioning services, onsite and remote CART services, many types of webstreaming/ webcasting, as well as court reporting and transcription services. Our teams of Nationally Certified realtime writers are experienced at projecting captions to the Jumbotron as well as displaying captions to a projection screen in smaller venues. Client testimonial: *"It's a company in Kansas that can do remote captioning. Besides meetings, they can be made available to hospitals, doctors and companies. In fact, companies use this for meetings where there is a deaf/hard of hearing employee or client - and the meeting is captioned on a computer screen. I believe they can do this with up to 20 people in a room. Excellent resource!"*

- ***FDA Offers New Guidelines to Speed Combo Drug Programs*** The Food and Drug Administration have announced the intention to accelerate drug approvals, issuing new draft guidelines aimed at speeding development work by encouraging joint applications for two drugs simultaneously. Currently, drugs must be approved singly and if joint use is proposed this use must then be separately approved. "We expect increasing use of combinations," noted Janet Woodcock, director of the FDA's Center for Drug Evaluation and Research. "This isn't just true of cancer. It's going to be true of many serious diseases."

- **Proposed '*National Center for Advancing Translational Sciences'* at NIH.** *The National Institutes of Health (NIH) Scientific Management Review Board has voted to establish a 'National Center for Advancing Translational Sciences'. The role of this proposed new center would be to establish a focused, integrated, and systematic approach to link basic science research with therapeutics development and clinical care. This has been a missing bridge at the NIH, so this announcement is being very well received.*

- ***FDA Approves Avastin; Serious Side Effects***
  Almost from the beginning of interest in the role of anti-angiogenesis (the prevention of new blood vessel formation) in cancer, there have been suggestions that natural substances might achieve the same ends as these patented products, and at a fraction of the cost.

- **CTF Present the '*NFPC*' as "Innovators" at *Partnering for Cures* Meeting.** *The 2010 Partnering for Cures meeting was the second annual meeting organized by the Milken Institute which brings together medical research leaders and decision-makers, innovators, and advocates, from across sectors to find workable solutions that will expedite the medical research and development process. Over 800 attendees participated. Following a competitive application and review process, CTF was selected for the second year in a row to present as an 'Innovator.' Dr. Kim Hunter-Schaedle presented 'The Neurofibromatosis Preclinical Consortium' as an example of an innovative collaboration between foundations, universities and industry*

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- Medical News TODAY ***Two Acceleration Methods Lead To Major Advance In MRI, Allowing Much Faster Brain Scans.*** An international team of physicists and neuroscientists has reported a breakthrough in magnetic resonance imaging that allows brain scans more than seven times faster than currently possible.

- YouTube ***Pole Pole "The Slow Walk"-*** Highlights & preview of the upcoming movie, entitled *"Perseverance Factor".* Story of Rebecca Dufek's (NF2er) recent attempt to summit *Mount Kilimanjaro*, the highest peak in Africa on Oct 10, 2010.

- ***Changing Lives One Smile at a Time*** - *KUSI* - A local news story in San Diego, CA of KC's successful facial reanimation surgery and outcome. She has **neurofibromatosis type 2.** – "*Whether it's a smile or a smirk, facial expressions are something most of us make hundreds of times a day without even thinking about it. But for the 150-thousand people who suffer from facial paralysis each year, this important form of non-verbal communication is impossible….."*

- ***Inventing with Legos*** - *Woodbury Bulletin*. – "*…Jacobson was recently diagnosed with* ***neurofibromatosis Type II****, which causes tumor cells develop on nerve endings in the brain. …"*

- YouTube ***A Struggle for Hope*** - Featuring Courtney Gale. This film has been entered in the *2011 Neuro Film Festival* from the American Academy of Neurology at www.neurofilmfestival.com . Let's put our brains together to support brain research. Directed by Debbie Wolfe (dpwolfe@gmail.com). Reported by Donna Winchester. Please vote for this documentary! Here's how:
  1. Visit this URL: http://patients.aan.com/go/about/neurofilmfestival
  2. Next, click on the "Vote Now" button.
  3. Then scroll down as *'A Struggle for Hope'* is near the bottom.

  Thank you.

- ***Ronnie Polaneczky: Generous readers came to the rescue*** - *Philadelphia Daily News*. - "'….*and I thought they said 'stink bugs,' laughs Kristen, 20, ever upbeat despite having* ***Neurofibromatosis Type 2*** *- a genetic disorder that threatens her hearing ...*"

- ***Catching up with people who made news in 2010*** - *Sheboygan Press*. – "*…Jackson Kloes, 9, has* ***neurofibromatosis type 2****, or NF2, a genetic disorder in which tumors form unabated in the body, particularly on the nerves of the ear ..."*

- ***Second of two parts*** (page 3) - *Philadelphia Daily News*. - *"Meghan Kingsley, 26, of Gaithersburg, Md.: Liver transplant. At 16, she was diagnosed with* ***neurofibromatosis type 2****, characterized by the growth of non-cancerous tumors along the nerve that transmits information from the inner ear to the brain. An exceptional competitive swimmer who had dreamed one day of going to the Olympic Games, she underwent surgery in June 2001 for the removal of a tumor and was left deaf in one ear In October 2007, she had decompression surgery on another tumor that doctors chose not to remove. In an effort to preserve what remained of her hearing, they instead carved away some bone that would allow the tumor room to grow. However, she began experiencing significant hearing loss and in September 2009 enrolled in a study for the experimental drug PTC299. By Nov, she was in the throes of liver failure. .."*

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 is to purchase a ***magazine subscription***. Forty percent (40%) of the proceeds will be donated to Advocure.

For a list of magazines, please click here

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- Find us on Facebook Another easy way to contribute to NF2 is to visit us and donate on **Facebook**.

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

http://www.prweb.com/releases/2006/06/prweb398339.htm

(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 *Congressionally 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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NF2 Natural History Study at NIH:

A Patient's Perspective.

by: John & Linda Manth

When our daughter Leah was diagnosed in 2007 with Neurofibromatosis Type 2 we were completely overwhelmed. Any patient or parent of someone with NF2 has experienced that original shock and feeling of *"WOW"* this is serious.

After educating ourselves on NF2 by reading, visiting doctors in our home town of Buffalo, NY, getting expert opinions from the NF Clinic at Children's Hospital in Boston and attending a couple of NF symposiums we began to grasp the complexities of having and living with NF2. We came to the conclusion that finding a cure or course of treatment would be very difficult and it would take many different entities to accomplish this goal. These entities consist of scientists, doctors, the US Government (politics), pharmaceutical companies and of course money (fundraising). The often forgotten piece to this puzzle is the patient. If a cure is to be found it will take brave patients to enter clinical trials and possible inherent risks so that a drug or treatment can be explored.

Due to our daughter's age she was not a candidate for any of the drug studies like *Lapatinib* and *Avastin*™. One day while looking at http://clinicaltrials.gov, a government website that lists all the current and ongoing clinical trials, we came across the NIH *Natural History Study for NF2*. We asked Leah how she felt about being part of a study and she thought it would be fun, it gave us a sense of doing something when at the time there was not a lot being done in NF2. We applied and were accepted into the study in June of 2008.

A natural history study tries to gain insight about a particular disease/diagnosis, to learn about it from the beginning in the hopes of appropriately steering further studies into effective treatments. The objective of the NIH sponsored NF2 Natural History Study is to *"…gain clinical and molecular insight into the effects of this tumor suppressor syndrome on tumor development and progression and to identify factors linked to symptom evolution."* The scientists hope to learn how fast the tumors grow, if certain factors might affect their growth and also examine effects of tumors on patient's activities of daily living. There are different specialists that each patient sees, some yearly some twice a year. Rovella Hyman schedules all the appointments based on who you are expected to see that visit. Research nurse specialists Hetty DeVroom and Rene Smith oversee that every appointment is completed, labs are drawn and paperwork signed (along with many behind the scenes activities). The study is headed up by Dr. Ashok Asthagiri who meets with you at the end of every visit. Visits are every 6 months, but more often if needed. Labs and MRI's are done every visit, then oncologists, ophthalmologists, audiologists, speech therapists, ENT's, neurologists, vestibular (and more if needed) testing are all completed if scheduled.

Some of our visits have been 1-1 ½ days, while others are 4-5 days. All expenses are paid for as part of the study. One plane ticket for adults, two for a minor and an adult are paid for or reimbursement for mileage based on federal guidelines is provided if you drive. $30 in meals per person per day is also provided for study participants, which while not covering everything, certainly helps. Hotels are also paid for, or in our case we stay on campus for free in the wonderful Children's Inn, which is truly an oasis, a home–away-from-home. Every detail in the facility has been thought of and is beautifully carried out. Volunteers pour in and provide meals some nights, music, massages, and special surprises in your family's mail boxes. You name it they think of it. There is also a Safra Family Lodge for adults receiving medical care at NIH. If you fly shuttle busses pick you up at the airport and drive you to NIH. Once on campus the metro has a stop in the NIH campus so if time allows you can go into DC for a quick tour/visit.

One thing is clear when you are at NIH, your tax dollars are at work. You are surrounded by incredibly bright people from every nationality you can imagine. There is state-of-the-art equipment and personnel

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and top notch researchers, who in our case are genuinely interested in NF2 and want to figure it out. We have met wonderful people from every walk of life at both the Children's Inn and in the various waiting rooms. Some have NF2, some NF1, some have diseases we have never heard of. One thing we have also come to appreciate is no matter how bad we may think we have it, a visit to NIH puts that all in perspective, someone else always has it worse. We are just so grateful there is a place like NIH where they are truly trying to find some answers. Again we cannot reiterate enough that we want to be part of the solution with NF2. While we do not want to expose our daughter to unnecessary risks, we need to have as good of an understanding as possible as lay people with the complexities of NF, so that when an appropriate trial does come up - we can make an informed decision about participation.

If this sounds like something you may be interested in, it is easy to begin the process. For more information you can go to http://clinicaltrials.gov website and look up the NF2 study NCT00598351 *(Natural History Study of Patients With Neurofibromatosis Type 2)*, or simply contact patient recruitment by email at cc-prpl@cc.nih.gov, or by toll free at 1-800-366-5165 (TTY 1-866-411-1010). After answering some simple eligibility questions, someone will contact you from there. After that you give them some dates to work with and your first visit will be scheduled.

You have to be part of the solution if you want an answer to a problem, if you are affected by NF2, this is one possible solution.

NF Symposiums or Conferences, etc

With an NF2 component!

- April 15-16, 2011 • Los Angeles, CA, USA
  UCLA Eleventh Annual Brain Tumor Conference
  Hosted by the UCLA Neuro-Oncology Program.

- May 20-22, 2011 • Houston, TX, USA
  Together in Hope: A Conference for Brain Tumor Patients and Their Families. Hosted by the National Brain Tumor Society, and MD Anderson Cancer Center.

- June 11-14, 2011 • Jackson Hole, WY, USA
  2011 NF Conference
  Hosted by the Children's Tumor Foundation (CTF).

- June 17-19, 2011 • Cincinnati, OH, USA
  ANA 20th National Symposium *- partly captioned*
  Hosted by the Acoustic Neuroma Association (ANA).

- June 28-July 1, 2011 • Los Angeles, CA, USA
  Sixth International Conference on Acoustic Neuroma
  Hosted by the House Ear Institute (HEI).

- July 29-31, 2011 • Minneapolis, MN, USA
  2011 NF Forum *- captioned*
  Hosted by the Children's Tumor Foundation (CTF).

- Oct 1-6, 2011 • Washington, DC, USA
  2011 CNS Annual Meeting*: Congress of Neurological Surgeons*

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Why not forward it to them? Thanks.

www.advocurenf2.org/newsletter/NF2-compass_2011spring.pdf

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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 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 phase 0 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

- ***Phase II Study of Lapatinib Study for Children and Adults With Neurofibromatosis Type 2 (NF2) and NF2-Related Tumors*** - ClinicalTrials.gov Identifier: NCT00973739
  This study is currently recruiting participants.
  The purpose of this study is to determine if Lapatinib has any effect on tumors found in patients with **Neurofibromatosis 2**.

- ***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

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.
Issue 9; spring 2011, page 11

- ***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.

- ***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.

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
- dasatinib

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

For more trials and/or studies, please see: *www.clinicaltrials.gov/ct2/results?term=nf2*

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