CoQ10 or Vitamin E that only addresses one aspect of oxidative stress at a central and critical point. This would be in contrast to selecting NAC because it supplements the supply of glutathione in cells and directly addresses the issue of increased oxidative stress. Dr. Porter notes that these oxysterols have been identified as important markers and a drug with a good safety profile. That is why we pushed to get this trial running simultaneously with the animal studies. The data obtained from the NAC trial will also help us in designing future trials, evaluation of the efficacy of NAC.

Patients in the trial will have four visits to the NIH over 12 months. Subjects will be on NAC for 8 weeks and a placebo for 8 weeks. Patients in the trial will have four visits to the NIH over 5 months. This drug trial is focused on biomarkers rather than clinical symptoms in an attempt to get answers in a shorter time frame, even your worst-case scenarios. Dr. Porter commented that “NAC will not be a cure by itself. We need to have some external way to put a diagnostic test on the hypothalamus and hippocampus and make a specific pathological process. The ongoing animal research will give us clues that we are possibly measuring in vivo and this is the most important type of good markers and a drug with a good safety profile. That is why we pushed to get this trial running simultaneously with the animal studies.”

If you would like to participate in the Natural History Study, to qualify, NP-C patients are not allowed. If you would like information about participating in the trial contact Nicole Yanjanin, CPNP, at 301-594-1765 or email her at: nyanjanin@mail.nih.gov. Ms. Yanjanin has played a key role in this trial. Furthermore, Ms. Yanjanin’s position is funded by the APMRF, and has made great progress in understanding the NP-C disease process. Since starting the NIH Natural History Study in August of 2006, 45 patients with NP-C have been enrolled from the United States, as well as from the United Kingdom, Canada, Israel, and Japan. The team of researchers at the NIH, working with Dr. Denis Callahan (Washington University in St. Louis), identified a pair of oxysterols in the blood of NP-C patients that may be the basis of a diagnostic test and may provide some outcome measure for this fatal disease. Oxysterols are produced from cholesterol in the brain and have a characteristic and identifiable signature. Further studies have identified increased oxidized cholesterol. Dr. Porter notes that these oxysterols may be biomarkers and then toxic, and eventually the hypothesis is that these oxysterols are linked by pathways. The oxidized cholesterol is potentially making our neurons sicker. By correcting the oxidative stress, he hopes to protect the neurons as much as possible. Hopefully, if some of the stress is eliminated, the cell can better deal with the primary insult.

Identification of oxysterols and oxidized cholesterol in the blood of NP-C patients has lead to the initiation of this therapeutic trial which will test the efficacy and safety of NAC in a small number of NP-C patients. NAC is a cholesterol-specific antioxidant that absorbs and removes the body from its toxic effects on the central nervous system. CoQ10 is then converted to a chemical called glutathione. Glutathione is a critical element that the cell uses to fight oxidized stress. Then NAC is a proven for glutathione. Glutathione itself does not get absorbed or reach the cerebrospinal fluid (CSF). The CSF is a clear liquid that surrounds the brain and spinal column. This leads to the CSF reaching the brain where NAC is cell-directly addressing the issues of increased oxidized cholesterol in a central nervous point. This would be in contrast to CoQ10 or Vitamin E, that only address one aspect of oxidized stress. NAC has a very good safety profile and has been used in children for other purposes.

In addition to the animal studies, researchers found that an increase of mitochondrial toxicity indicates increased oxidized stress in patients with NP-C. Patients with NP-C have differences in their mitochondrial function. The researchers from the NIH and their counterparts at the University of Utah identified a significant difference in the level of mitochondrial activity in NP-C patients. Patients with NP-C have been enrolled from the United States as well as from the United Kingdom, Canada, Israel, and Japan. The team of researchers at the NIH, working with Dr. Denis Callahan (Washington University in St. Louis), identified a pair of oxysterols in the blood of NP-C patients that may be the basis of a diagnostic test and may provide some outcome measure for this fatal disease. Oxysterols are produced from cholesterol in the brain and have a characteristic and identifiable signature. Further studies have identified increased oxidized cholesterol. Dr. Porter notes that these oxysterols may be biomarkers and then toxic, and eventually the hypothesis is that these oxysterols are linked by pathways. The oxidized cholesterol is potentially making our neurons sicker. By correcting the oxidative stress, he hopes to protect the neurons as much as possible. Hopefully, if some of the stress is eliminated, the cell can better deal with the primary insult.

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This drug trial is focused on biomarkers rather than clinical symptoms in an attempt to get answers in a shorter time frame, even your worst-case scenarios. Dr. Porter commented that “NAC will not be a cure by itself. We need to have some external way to put a diagnostic test on the hypothalamus and hippocampus and make a specific pathological process. The ongoing animal research will give us clues that we are possibly measuring in vivo and this is the most important type of good markers and a drug with a good safety profile. That is why we pushed to get this trial running simultaneously with the animal studies.” The data obtained from the NAC trial will also help us in designing future trials, evaluation of the efficacy of NAC.

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Amanda Ashton is a registered nurse and was diagnosed with Niemann-Pick Type C (NP-C) disease at age three. Her disease rapidly progressed to the point where she needed a cane to walk and has since spent time at a nursing home. She was taken out of the program last year when an entirely new team of doctors started treating her. Ashton is now much happier, laughing and playing games with nurses.

Amanda Ashton with David Smith and the late Keaton Barton.

**Heartfelt Thanks...**

**What do you get when you put together one of the most giving companies in the country with a dedicated leader dedicated to fighting for a worthy cause?**

A winning combination that supports many worthwhile causes, including the race to find a cure for NP-C.

The event’s title, “I cones Is Pink” evolved from a comment that Ashton made when faced with the prospect of needing a cane to aid her walking. Her “Nana” feeling saddened over the image of the traditional cold gray metal variety was assured by Ashton that this clearly was not going to be a problem; with a big grin and a commitment to the prospect of needing a cane to aid her walking. Her “Nana” feeling saddened over the image of the traditional cold gray metal variety was assured by Ashton that this clearly was not going to be a problem; with a big grin and a...