

# PXE Awareness

*National Association for Pseudoxanthoma Elasticum (NAPE, Inc.)*

Volume 11, Issue 1 . January 2005



## **2004 Kansas City Conference**

Stem Cell Therapy, p7

Intermittent Claudication, p15

Swallowing Difficulties, p19

Web Access Tutorial, p24

# **National Association for Pseudoxanthoma Elasticum (NAPE, Inc.)**

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## President's Letter

Dear Friends,

NAPE's annual meeting at the Radisson Kansas City Airport Hotel on November 12 and 13 was very successful according to participant evaluations and

our own interactions and observations. It was good to greet old friends from across the nation and Canada and a very special pleasure to welcome new friends from the Midwestern United States.



Dr. Frances Benham

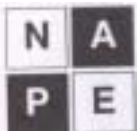
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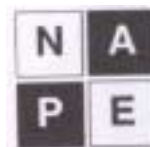
Dr. Kenneth Neldner

Dr. Kenneth Neldner provided a brief history of NAPE, presented several ideas for our organizational structure, and updated us about popular herbal supplements, which may cause increased blood pressure and internal bleeding. He reported on the importance of reading nutrition labels on processed foods such as baked and fried foods. Calcium, fats, vitamins and the role of exercise were discussed. The questions regarding nutrition and routine personal care reminded long-term members of the importance of NAPE's educational mission until the cure for PXE is available.

Dr. Gregory Clark reported on stem cell research and its potential. Many questions followed and Dr. Clark agreed to follow the rapid developments in stem cell



work and to keep us informed. His talk is presented in this issue.



R.N. Mary Krieger presented talks on two problems about which NAPE receives many inquiries: difficult swallowing and intermittent claudication. NAPE members shared coping strategies and raised many questions. Articles about both issues can be found in this issue.

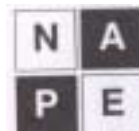
Rosemary Atallian and I presented updates of our experiences with feeder vessel treatment. Rosemary continues to enjoy excellent vision three years after treatment that was provided very quickly after bleeding was detected in her eye. My eyes are now stable after a year of treatment initiated after several months of bleeding. My vision has gradually improved, but I still read with magnification.

Deborah Clark followed with a report on a study underway regarding possible reduction of calcium in the tissue of those who have PXE. A report will be included in a forthcoming newsletter.

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Sally Dawoud demonstrated a new feature in preparation for the NAPE website. This is the addition of natural human speech which reads items found on the website so that readers who have a computer with speakers can listen to website information. The most current newsletter was recorded and soon will be available to website readers. Board member Claudia McCallister called for a vote of those present as to their preference for an audible newsletter







issue over a print copy. The vote was decisive in favor of audible access. The newsletter will continue to be made available both in print and audibly as the Board considers how best to meet individual NAPE member needs.

Finally, the Board met immediately prior to the conference and after the conference ended to hold business meetings. The Board has become quite active and it is listening to NAPE members for program and article ideas to help us cope with PXE. The Board invited Linda Zeug and Ron Dore to join for three-year terms. Planning for next summer's annual meeting is underway and will be announced early in 2005.

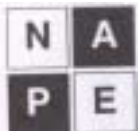
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From the Board, I wish you a joyful holiday season. Have a wonderful time and remember that it is alright to splurge occasionally - so enjoy those holiday goodies! Then, back to the veggies, fruit, and daily exercise.

All the best in the New Year,  
Frances Benham



Kansas City Conference



# Stem Cell Therapy



*By Gregg Clark, Ph.D.*



## **What are stem cells?**

Stem cells are cells in the body that continually renew themselves by producing daughter cells. Some of these daughter cells go on to change and become cells that are specific to different tissues in the body. So stem cells are the source cells from

which all other cells in our body come from. Their ability to give rise to a variety of different types of cells (e.g. lung, heart, skin, retina, etc.) is what makes them so valuable to the biomedical world today.

## **Are they all the same?**

No. There is a variety of stem cells that are in our bodies that give rise to various tissue specific cells. Some can give rise to more tissue cell types than others and that is actively being studied in labs around the world. The stem cells present in our bodies are known as adult stem cells while another type of stem cell also can be found in developing embryos of animals and humans. These are called embryonic stem cells. These can be isolated from embryos only days after fertilization of the egg occurs. They are easier to isolate in large numbers relative to adult stem cells and tend to give rise to a greater number of cell

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types than do adult stem cells. Their ability to develop into a wide variety of cell types is a key reason for the current enhanced interest in studying their biology.

There has been much discussion over the ethics of human embryonic stem cell research and I will not touch on that topic here, as I want only to explain the scientific basis of stem cell research so you have a better understanding of what it involves. While embryonic stem cell research is a hot political topic, much is being done with adult stem cell research/therapy that is quite exciting.

### **Why are they important?**

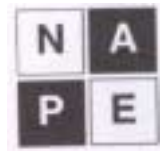
Because they are able to give rise to a variety of new cell types, stem cell use in tissue regeneration is of great potential. The idea would be to replace damaged tissues by the addition of stem cells that then would grow and generate new undamaged tissue, for example, in the brain or heart. In almost any situation where tissue damage has occurred, stem cell therapy could be applied. This potential has caused great excitement in the field of medicine, as well as other fields.

### **Do they have therapeutic uses?**

Yes they certainly do. For years now people with leukemia and other blood diseases have been getting bone marrow transplants for successful treatment of their disease. The bone marrow is the source of stem cells that give rise to new blood cells to replace the diseased ones in a patient. And now, new uses are being tested in animal models and in some cases with humans that go beyond the treatment of blood diseases alone. In addition, cord blood cells isolated from the umbilical cords of newborn babies are also a rich







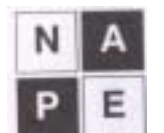
source of blood stem cells and have been used in treatment of various blood diseases. New uses of these cells are also being tested for treatment of disease other than that of the blood.

### **Stem cell applications**

I would like to talk about stem cell application in the areas of heart disease, diabetes, arthritis, brain disease, and eye applications. Then I will briefly discuss some of their limitations and future directions of the research.

### **Heart disease**

Studies have been performed with mice that have had induced heart attacks and then received bone marrow-derived cells by direct injection into the heart. Cardiac function improved in those mice receiving the bone marrow cells, implying that the cells were able to help aid heart function in some way. The exact reason for this improvement is still a matter of discussion, but the results have encouraged doctors to test for such effects in humans. In one trial, some patients who had suffered acute myocardial infarction and had been treated with angioplasty were treated with bone marrow-derived or blood-derived stem cells by intracoronary injection. In 20 patients there was an improvement in heart function (left ventricular function) regardless of the type of stem cell they received. This has been repeated in several different trials with similar results and few side effects have been observed. The actual mechanism behind the improvement remains to be determined, but with larger trials and continued study this should be discovered eventually. For a review on this subject see: Lee, M.S. and Makkar, R.R. 2004. Stem-Cell Transplantation in Myocardial Infarction: A Status Report. *Annals of Internal*





Medicine, vol. 140, pp. 729-737.

### **Diabetes**

In another application of stem cell therapy for a disease state, diabetic rats have been injected with embryonic stem cells that express insulin. Improvement in glucose control was observed. Transplantation of bone marrow-derived cells were found to normalize glucose and insulin levels in diabetic mice and their survival rate was better. Studies are ongoing to further define the fate of donor bone marrow cells. There is also an autoimmune component to diabetes that complicates treatment success, but that is under investigation. For a review see: Hussain, M.A. and Thiese, N.D. Stem-cell therapy for diabetes mellitus. 2004 Lancet, vol. 364 (July 10), pp. 203-205.

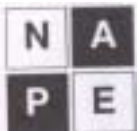
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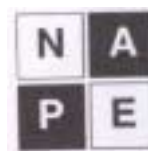
### **Arthritis**

I attended a seminar recently, where scientists at the University of Florida are trying stem cell therapy to treat arthritis in animal models. They are hoping to relieve inflammation and to be able to develop new cartilage in affected joints with this treatment. The work is in the beginning stages, but it is promising.

### **Brain Disease**

Embryonic stem cells and adult brain stem cells have been shown to possess plasticity with their ability to convert to different cell types in the lab. Continued research into the control of this conversion is ongoing for use in cell transplant applications for diseases like Parkinson's. In this situation dopamine production in the brain has declined and the idea would be to enhance it through the addition of new functional dopamine producing brain cells. Although adult brain cells have been found in both rodents and humans,





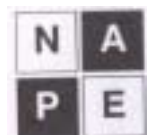
their numbers are small but potentially very powerful in their potential for treatment of neurological disease. Also the application of bone marrow stem cells in mice with a Parkinson-like disease has showed encouraging results. For a review in this area of research see: Rice, C.M., Halfpenny, C.A. and Scolding, N.J. 2003. Stem cells for the treatment of neurological disease. *Transfusion Medicine*, vol. 13, pp. 351–361.

Treatment of stroke using human bone marrow derived stem cells is another application that is showing promise in animal models. In addition, just recently a report has come out that human cord blood specific cells can promote the growth of blood vessels in the areas of brain damage in mice with induced stroke. This treatment may promote brain cell regeneration through the development of new blood vessels and the recruitment of brain stem cells to the injury site. This was taken from: Taguchi, A. et al. Administration of CD34+ cells after stroke enhances neurogenesis via angiogenesis in a mouse model. 2003. *The Journal of Clinical Investigation*, vol. 114, Number 3 August, pp 330-338

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### **Eye Applications**

Progress is being made in the area of treating retinal diseases with stem cell therapy, but the task is a little more complicated because of the variety of cells that make up the retina. The retina is a multi-layered tissue that consists of about 7 classes of cells with some 50 different types making up those classes. These classes appear at different times during embryo development and are fitted together in a specific pattern to give a functioning retina. For stem cell therapy to be effective for the retina, the cells will have to find their way into the retina and develop into the correct cell type.





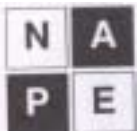
Experiments where brain stem cells from rats were injected into the vitreous of adult rat eyes resulted in migration and incorporation of the cells into the rat retina. Unfortunately, development of retinal cell characteristics did not occur. However, more recently mammalian retinal stem cells have been isolated and tested in a similar way and they do seem to be able to incorporate and to develop into retinal cells. This is work that has been done by groups at Harvard, Iowa State University and Children's Hospital of Orange County, California.

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This fall a report was published by a group from the Scripps Institute in San Diego that tested bone marrow stem cell therapy on mice with degenerating retinas. They found that when bone marrow cells from human or mouse origin were injected into the vitreous of the defective mice, a stop in retina cell degeneration was observed and new cells were generated. In this case the stem cells may have helped preserve important blood vessels that led to an enhancement in retinal cell generation. Some functional activity of the retina via electroretinogram recordings was observed although they were abnormal. However, these results are very exciting in their application perhaps to slowing retinal degeneration in diseases like retinitis pigmentosum. Another potential application of this type would be to target stem cells to the retina to prevent formation of unwanted blood vessels in eye diseases where this is a problem.

## **Are there limitations to the uses of stem cells?**

Yes. The range of different types of cells that stem cells can change into may be limited to a set of cells which may not be useful for certain diseases. The complexity of individual diseases will govern whether



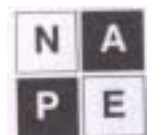


stem cell therapy is applicable. Currently, we are in the beginnings of the field and therefore are limited by our lack of knowledge about the processes involved in cell differentiation, tissue generation and organogenesis, the process whereby organs develop into fully functional units. Another possible restriction is the problem of tissue rejection when stem cells from other sources are used to treat a person who is tissue-incompatible. This is a situation where adult stem cells from the patient could have great use, since they would not be recognized as foreign by their body. However, adult stem cells seem to be limited in their range of development and in the small amounts present in the body. Continued research into the control of stem cell differentiation will extend our ability to stimulate cell differentiation in a manner useful to fighting various diseases. Also it will enable us to understand how to enhance the growth of larger quantities of adult stem cells for the same purpose. Embryonic stem cells can be isolated in greater numbers and they are less limited in the number of cell types they can generate, which makes them attractive for study, but their current use has funding and ethical constraints. So the potential application of stem cell therapy to disease treatment is broad, but not without its limits.

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### **What does the future hold?**

From what I said today, you can see that the potential for stem cell therapy is immense and much of this potential is applicable to the use of adult stem cells which makes things less complicated from the ethics point of view. However, with efforts from individual organizations and states underway to fund human embryonic stem cell research, this field will also move forward rapidly. I think in the next fifteen years amazing things will be achieved in the field, as research moves







along at a quickening pace. Thus the future is bright for the field of stem cell research, its application to the treatment of a variety of diseases and the resulting enhancement to the quality of life for many. 

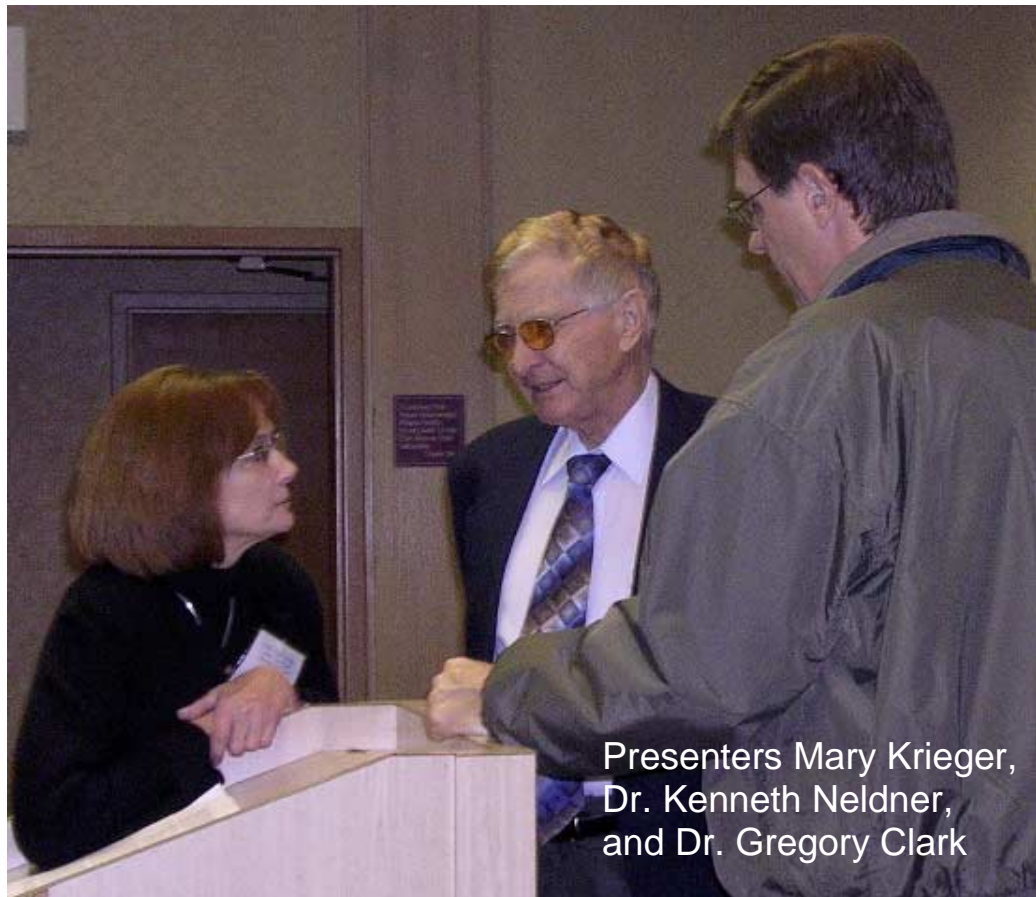
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**Useful websites:**

[www.medlineplus.gov](http://www.medlineplus.gov) -- for information on all aspects of health, medical research and the latest news on medical breakthroughs

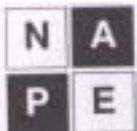
[www.pubmed.gov](http://www.pubmed.gov) -- to search biomedical literature

<http://stemcells.nih.gov/index.asp> -- stem cell information

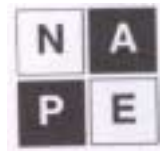
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Presenters Mary Krieger,  
Dr. Kenneth Neldner,  
and Dr. Gregory Clark



# PXE and Intermittent Claudication



*By Mary Krieger, R.N.*

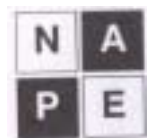
One of the major systems in the body affected by PXE is the cardiovascular system-- the heart and blood



vessels. Arteries carry oxygen-rich blood from the left side of the heart throughout the entire body. Normally, arteries are strong and flexible, and the inner walls are smooth allowing blood to flow freely to muscles, skin, tissues, and organs. As a person ages, the walls of the arteries may become clogged with clumps of fat and other materials called plaques. The arteries become hardened and narrow, and this slows down the flow of blood. This condition is known as atherosclerosis, and individuals with PXE may develop clogged, hardened arteries in their late 20's or early 30's.

While atherosclerosis can occur in arteries anywhere in the body, it most commonly affects the peripheral arteries (arteries to the arms and legs) in PXE. The pulses in the wrists and feet may be weak or absent. When the flow of blood to leg muscles is decreased, it can cause pain, cramping, or heaviness in the legs which is called intermittent claudication. The word *claudication* in Latin means "to limp." The leg discomfort is "intermittent" because it begins with exercise (like fast walking or climbing stairs) and

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subsides when exercise stops. The reason for this is that the muscles in the legs need more oxygen when they are working. When blood flow is decreased, the oxygen supply to the muscles is also decreased. So if the demand for oxygen is greater than the supply, pain or cramping in the muscle occurs.

If the narrowing of the arteries is severe enough, leg pain may also occur while at rest. The toes may appear pale or bluish, and they may be cool to the touch. In severe cases, sores or ulcers can develop on the lower legs, ankles, or feet.

### **How is intermittent claudication diagnosed?**

To diagnose the cause of leg pain or cramping, the physician may perform a few simple tests:

- 1) ask about diet, smoking habits, and medical conditions that occur in the family
- 2) check the pulses in the arms, groin, knees, and feet
- 3) check the arms and legs for coolness of the skin, hair loss, or non-healing sores
- 4) a simple test called the Ankle-Brachial Index can be performed which measures the blood pressure in the ankle and arm and compares the two.
- 5) treadmill walking at a slowly increasing speed to determine when the leg pain first occurs
- 6) other tests such as an ultrasound may also be used.

### **What can be done about intermittent claudication?**

- 1) Eat a balanced, low fat diet with plenty of fruits and vegetables to prevent any further buildup of fatty deposits in the blood vessels.
- 2) Keep your blood cholesterol level within the normal range.
- 3) Do NOT smoke.






- 4) If you have high blood pressure or diabetes, see your physician regularly to treat these conditions.
- 5) A regular, supervised walking program, under the direction of your physician, is one of the **MOST** effective treatments to reduce the symptoms of claudication, and to increase the distance and time that you are able to walk comfortably.
- 6) Certain medications can increase blood flow in the legs and decrease leg pain caused by poor circulation. If you have PXE, be sure that your treating physician is aware of your condition.

### **More about an exercise program that helps with claudication...**

These are some of the general guidelines for exercise rehabilitation programs for persons with claudication.

**You should consult your personal physician before starting any exercise program.**

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- 1) Warm up for 5-10 minutes prior to exercise and cool down for 5-10 minutes afterwards.
- 2) Walking on a treadmill or around a track is considered to be the most effective form of exercise for claudication.
- 3) Walk at a speed that produces mild claudication symptoms within 3-5 minutes. When the symptoms become moderately severe, rest for a short time, either sitting or standing, until the symptoms subside.
- 4) Continue the walk-rest-walk pattern throughout the session. The first session will optimally include 35 minutes of intermittent walking. Then the amount of walking is increased by 5 minutes each session until 50 minutes of intermittent walking is achieved.
- 5) This exercise should be done 3 to 5 times a week. 





## Sources:

*Walk this way: Managing the pain of intermittent claudication.* Mayo Foundation for Medical Education and Research <http://www.mayoclinic.com/>

Stewart KJ, Hiatt WR, Regensteiner JG, Hirsch AT. Exercise training for claudication. *N Engl J Med.* 2002 Dec 12;347(24):1941-51)

For reliable information on atherosclerosis, peripheral arterial disease, and other medical conditions, visit the *MedlinePlus* web site: <http://medlineplus.gov/>

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At the conference...

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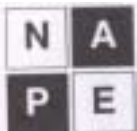


Top Left: Dr. Kenneth Neldner giving an update on PXE



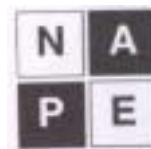
Above: Mary Sigler, Lori Swiler, and Marilyn Cheek

Left: Ken and Shirley Vanderwall with Linda Zeug at coffee break





# PXE and Swallowing Difficulties



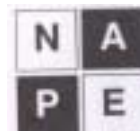
*By Mary Krieger, R.N.*

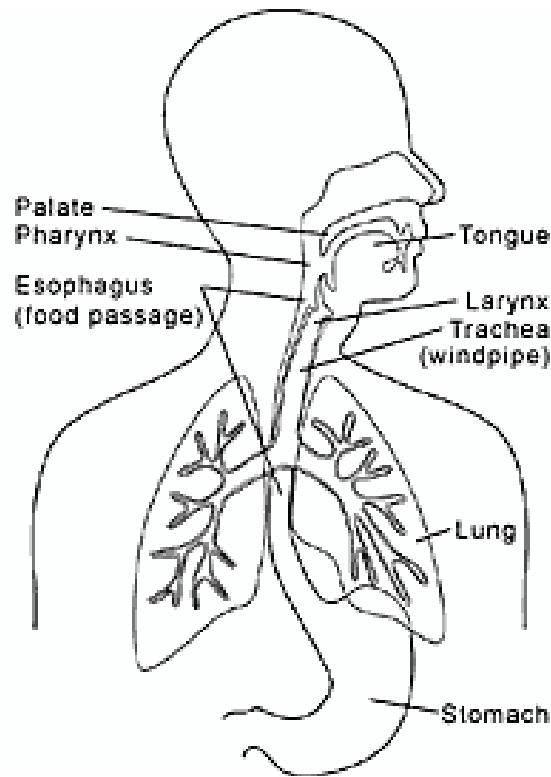


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Swallowing is a basic human function that most of us take for granted. But swallowing is actually a complex process in which 50 pairs of muscles and many different nerves move food from the mouth down into the stomach. It is an essential function because we must take in food and liquids to survive. The medical term for difficulty with swallowing is **dysphagia**. This term comes from the Greek words **dys-** (with difficulty) and **phagia** (to eat).

There are no studies to date (November 2004) in the medical literature which clearly link dysphagia with PXE. However, many individuals who have PXE also report difficulties with swallowing. It may be helpful to review the events that occur in “normal” swallowing.





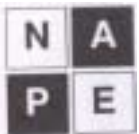
**NIDCD** National Institute on Deafness and Other Communication Disorders  
*Improving the lives of people who have communication disorders*  
ONE OF THE NATIONAL INSTITUTES OF HEALTH

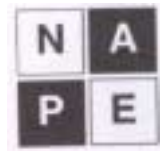
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## Normal Swallowing

There are three stages in the process of swallowing:

- 1)** The tongue moves food around in the mouth and 32 adult teeth grind the food into an appropriate size for swallowing. Saliva is mixed in to soften and moisten the food to make swallowing easier. Then the tongue collects this soft round mass of food, called a bolus, and moves it to the back of the throat (**pharynx**). During this time, the lips seal and the tongue pushes up to the roof of the mouth in a front to back motion.
- 2)** The second phase of swallowing is involuntary and happens in approximately 1 second. But it consists of a series of intricate, coordinated events. As the food bolus moves through the pharynx, the passage that leads upward to the nasal cavity (**nasopharynx**) closes off to prevent food from moving up into the nose.





Similarly, the voice box (**larynx**) closes tightly and breathing stops to keep food and liquids from entering the lungs. Finally, a ring-like band of muscle fibers (**sphincter**) at the opening to the **esophagus** relaxes and allows the bolus to pass through.

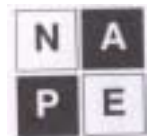
**3) The esophagus** is a hollow, muscular tube about 10 inches in length that connects the pharynx to the stomach. It has sphincters at the upper and lower borders. The upper sphincter prevents air from entering the esophagus during breathing and the lower sphincter prevents food and acid in the stomach backing up into the esophagus. Once the food enters the esophagus, it is passed along to the stomach by **peristalsis**, a wave-like series of muscle contractions and relaxation.

### **Difficult Swallowing**

Dysphagia or difficulty in swallowing can occur at any age, but it seems to be more common in older adults. It occurs if there is a problem with any of the structures or organs involved in the swallowing process: teeth, tongue, mouth, throat, or esophagus. The problems may be mechanical (anatomic) like a tumor or a narrowing of the esophagus by scar tissue (stricture). Or, the problems could be due to impaired motor (neuromuscular) function, as seen in Parkinson disease, multiple sclerosis, or stroke.

Some of the signs and symptoms of dysphagia are:

- pain or pressure in the chest,
- coughing or choking while eating,
- frequent heartburn,
- feeling of food getting stuck in the throat or chest,
- food or stomach acid backing up into the throat (regurgitation).





## Tests to Diagnose Swallowing Problems:

**1) Standard or modified barium swallow.** This test involves drinking a barium solution which makes the throat and esophagus more visible on x-ray. It can help to pinpoint the site of the swallowing problem.

**2) Upper GI endoscopy.** A thin, flexible lighted instrument is passed down the throat to allow the physician to directly view the esophagus.

**3) Esophageal manometry.** This highly sensitive test measures the pressure of the muscle contractions in the esophagus during swallowing.

**4) Chest CT or MRI** may also be used.

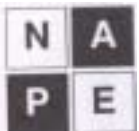
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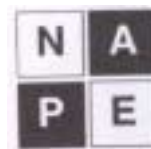
## General Guidelines for Managing Swallowing Difficulties

- Chew food very well.
- Eat slowly.
- Take small sips of fluid with meals.
- Eat smaller, more frequent meals.
- Remain upright while eating and for 30 minutes afterward.
- Avoid foods that trigger swallowing problems.
- Sleep with the head of the bed elevated 6-8 inches on blocks--using pillows to elevate the head during sleep are not as effective.

## A Special Note on Gastroesophageal Reflux Disease


Gastroesophageal reflux disease (GERD) occurs when the lower esophageal sphincter does not close properly and stomach contents or acid leak back up into the





esophagus. When stomach acid touches the lining of the esophagus, it can cause a burning sensation, known as heartburn. No one knows the exact cause of GERD, but several factors may contribute to this condition such as obesity, alcohol use, smoking, pregnancy, and/or hiatal hernia.

Certain foods may trigger reflux events: citrus fruits (like oranges), chocolate, caffeine drinks, fatty and fried foods, garlic, onions, mint flavorings (like peppermint), spicy foods, and tomato-based foods (like spaghetti with tomato sauce).

Occasional heartburn may not be serious, but if you have heartburn more than twice a week, you should consult your physician. 

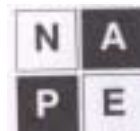
# 23

### **Sources:**

*Dysphagia*. National Institute on Deafness and Other Communication Disorders, National Institutes of Health.  
<http://www.nidcd.nih.gov/health/voice/dysph.asp>

*Heartburn, Hiatal Hernia, and Gastroesophageal Reflux Disease (GERD.)* National Digestive Diseases Information Clearinghouse, National Institute of Diabetes and Digestive and Kidney Diseases, National Institutes of Health.  
<http://digestive.niddk.nih.gov/ddiseases/pubs/gerd/index.htm>

For reliable information on these and other medical conditions, visit the *MedlinePlus* web site:  
<http://medlineplus.gov/>







## NAPE Website Access



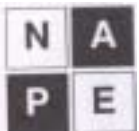
# 24

In an on-going effort to increase access to PXE information and educational resources, we at NAPE continuously make efforts to update and improve our website. Recently, we've added voice files which will allow the visually impaired to listen to certain contents by simply clicking the red lips icon as depicted below.



However, it has been brought to our attention by Dr. Neldner that a step-by-step guide on how to access the NAPE website may be useful to many of our members.

In response, this technical note is intended as a technology guide for individuals who have limited or no experience with internet access. We assume that if you are visually impaired you may need to call on a sighted friend to help guide you through the 2-minute process once until you are able to navigate on your own.





**Step 1** – Open up a browser window. This is typically either Internet Explorer or Netscape, although there are other browsers out there. To open, for example, Internet Explorer you will need to do one of 2 things:

1. Find the Explorer icon (typically a blue **e** as depicted) on your desktop and double click on it to open a browser window.
2. If you cannot see it on your desktop, click on **Start** (bottom left of your screen), choose **Programs**, locate **Internet Explorer** and double click on it to open a browser window.



**Step 2** – In the Address bar (top left of browser window, beneath the menu bar), erase whatever is there and type the following web address:

[www.napxe.org](http://www.napxe.org)

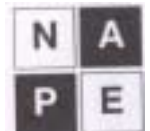
Then hit **Enter/Return**.

This should take you to the website in a second or two (or 6 depending on the speeds of your computer processor and internet connection). If you do NOT get to the NAPE website, repeat Step 2, you may have made a mistake in typing the address.

**Step 3** – If you are visually impaired, ask a friend to help you add the NAPE website to your favorites so you do not have to re-enter the address every time you wish to visit the website. To add the website to Favorites, go to the menu bar of the browser window and click on **Favorites**. Choose **Add Page to Favorites**.

**Step 4** – if you got this far, give yourself a pat on the back. You are finished.

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Once you are at the website, click links to articles, Q&A, conference information, etc. at your leisure.

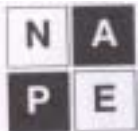
For example, under the NAPE logo, you will find a link to the latest edition of the newsletter, “read PXE Awareness newsletter”. To access the contents (through the table of contents), double click this link.

The presence of a picture of red lips indicates there is a voice file attached to the contents that will read contents to you. To listen to available voice files, make sure the volume on your computer is turned up and double click the red lips. Again, depending on your computer processor speed and internet connection, downloading the sound files may take a minute or two depending on the size of the file.

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As we continue to make improvements to our website, we will keep you informed. In the meantime, check the website every little while to see what has been added. For example, the newsletter will always be added as it becomes ready for publishing.

We hope this brief tutorial has been helpful and will aim to include more information in the future regarding tools to simplify your life and assist you in coping with PXE.



# NAPE Q & A



*By Gregg Clark, Ph.D.*

**Q** Is human embryonic stem cell (HESC) research illegal?


**A** No it is not, but no federal funding will be given to a researcher to do HESC research on anything but the approved HESC lines. The generation of new HESC lines is not to be funded by any federal governmental agency; however, private, state and local funding could be used for HESC research. In fact, Harvard has set up a stem cell research institute to pursue HESC research through funding from individuals and private agencies while California recently passed a bond issue to raise \$3 billion for HESC research which should aid Stanford University's Institute of Cancer/ Stem Cell Biology and Medicine in its efforts in HESC research.

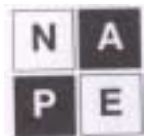
27

**Q** Will stem cell therapy be available soon for people suffering loss of sight from PXE?

**A** Unfortunately, the use of stem cells for treating retinal disease is probably 10-15 years off in the future. Currently, tremendous strides are being made in animal models and hopefully this will translate to humans. Careful testing will be done with any promising therapies that are developed and this will take some time before they become available for public use.

**Q** What do you think will be the first area of application of stem cell therapy (SCT) to humans?

**A** Based on my literature review, it seems that some impressive results are being achieved in treating heart disease in a number of small clinical trials and therefore this seems a likely first application. 



# PXE Pals

If you would like a PXE friend with whom you can correspond and talk, these NAPE members would like to hear from you:

**Joan Bittner**

111 Willis Drive  
Tonganoxie, KS 66086  
Phone: 913-845-2554

**Steve Boyle**

828 N. 2<sup>nd</sup> Street  
Montrose, CO 81401  
Phone: 970-249-8731  
E-mail: sboyle@independence.net

**Lydia Chang**

77-11 35<sup>th</sup> Avenue, Apt. 2P  
Jackson Heights, NY 11372  
Phone: 718-898-1762  
E-mail: stockchang@msn.com

**Ron Dore**

282 Ole Road  
St. Augustine, FL 32080  
Phone: 904-471-4662

**Anita Fekkers**

P.O. Box 40  
Wauneta, NE 69045  
Phone: 308-394-5917  
E-mail: aef@bwtelcom.net

**Patricia Gadziala**

5359 Ashleigh Road  
Fairfax, VA 27030  
Phone: 703-263-7949

(had translocation surgery)

Please notify the NAPE office if you have trouble contacting any of these PXE Pals so we can update our information and keep it accurate.

**Susan Golasz**

2604 Nassau Bend, Apt. A2  
Coconut Creek, FL 33066  
Phone: 954-974-7630  
E-mail: susang2604@bellsouth.net

**Marilyn Grobeson**

11256 Garfield Avenue  
Culver City, CA 90230-4810  
Phone: 310-390-6087  
E-mail: pxeladymg@webtv.net

**Lindy Humphreys**

2845 Cambridge Lane  
Mound, MN 55364  
Phone: 952-239-9495  
E-mail:  
jay\_lindy\_humphreys@hotmail.com

**Rosalie (Lea) McMurtry**

1019 W. Pioneer Avenue, Apt. 2  
Puyallup, WA 98371  
Phone: 253-845-2527

**Jeff Parnell**

1817 Primrose Avenue  
Granite City, IL 62040  
Phone: 618-931-3831  
E-mail: par3jcn@forteisp.net





# N A P E, Inc.

## The Three Amigos Lo32w-Vision Aid Program — Request Form

NAME \_\_\_\_\_ APT. \_\_\_\_\_

ADDRESS \_\_\_\_\_ CITY \_\_\_\_\_

STATE \_\_\_\_\_ ZIP \_\_\_\_\_ COUNTRY \_\_\_\_\_

PHONE \_\_\_\_\_ EMAIL or FAX \_\_\_\_\_

*An invoice or receipt must accompany each request*

**Description: Doctor Visit/Other — Name/Address/Phone#**

\_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_

(Invoice enclosed , Receipt enclosed ) Cost \$ \_\_\_\_\_

**Item 1: Company/Address/Phone#**

**Description of Purchase**

\_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_

(Invoice enclosed , Receipt enclosed ) Price \$ \_\_\_\_\_

**Item 2: Company/Address/Phone#**

**Description of Purchase**

\_\_\_\_\_  
\_\_\_\_\_  
\_\_\_\_\_

(Invoice enclosed , Receipt enclosed ) Price \$ \_\_\_\_\_

- I am a N A P E member who does not qualify for this assistance from another source.
- In order to continue leading an active life, I am in need of the above vision help.
- I am attaching a physician's statement of visual acuity and a diagnosis of PXE.

YOUR SIGNATURE \_\_\_\_\_ DATE \_\_\_\_\_

**N A P E APPROVES:**  payment to doctor and/or vendor(s) for above expense(s)  
 reimbursement to member for above expense(s)

**N A P E AUTHORIZATION** \_\_\_\_\_ **DATE** \_\_\_\_\_

(Complete other side if your purchases exceed \$200 or if you have any comments or suggestions.)

8764 Manchester Rd., Suite 200, St. Louis, MO 63144

Phone 314-822-6273 Fax 314-962-0100



# NAPE, Inc.

## The Three Amigos Lo32w-Vision Aid Program — Request Form

**NAPE now reimburses 50% for the cost of purchases to a lifetime total of \$1,000**

Please complete this portion of the form if the vision aids you need exceed \$200 and explain the circumstances that prevent you from making these purchases without financial assistance:

I have \_\_\_\_ dependents;      I share my home with \_\_\_\_ adults;      I live alone

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Please use this portion of the form for comments or suggestions on how we can improve services to our members:

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**THIS SECTION RESERVED FOR NAPE RECORD KEEPING**

1. Date \_\_\_\_\_ Ck# \_\_\_\_\_ To: \_\_\_\_\_

Description \_\_\_\_\_

Amount \$ \_\_\_\_\_ Reimbursement Balance \$ \_\_\_\_\_

2. Date \_\_\_\_\_ Ck# \_\_\_\_\_ To: \_\_\_\_\_

Description \_\_\_\_\_

Amount \$ \_\_\_\_\_ Reimbursement Balance \$ \_\_\_\_\_

3. Date \_\_\_\_\_ Ck# \_\_\_\_\_ To: \_\_\_\_\_

Description \_\_\_\_\_

Amount \$ \_\_\_\_\_ Reimbursement Balance \$ \_\_\_\_\_

Please cut out, complete, and send this form to the NAPE office. Upon approval NAPE will return this form to you with an authorized signature insuring reimbursement upon receiving proof of service or purchase; or, a check can be issued to the doctor/vendor if you have submitted an invoice.

# National Association for Pseudoxanthoma Elasticum

8764 Manchester Rd., Suite 200, St. Louis, MO 63144-2724

## Donations, Subscriptions and Membership for 2005

***One part of your contribution is for helping yourself; the other part is for helping others, but no one will be turned down for membership if no donation is received.***

PLEASE PRINT LEGIBLY & MAKE DONATION IN U.S. CURRENCY

- Friend of NAPE Membership     Professional Membership     New     Renewal  
 \$1,000 or more     \$100—\$500    Other: \_\_\_\_\_  
 \$ 500—\$1,000     \$ 25—\$100    \$ \_\_\_\_\_

Donations can be made in Honor or Memory of a loved one, and also for the Research Fund and/or the Low-Vision Fund. All donations are tax deductible in the USA.

- Honor     Memory     General Fund     Low-Vision Fund

Name of Loved One: \_\_\_\_\_

Address for Acknowledgement: \_\_\_\_\_

↓ PLEASE COMPLETE THE SECTION BELOW IF YOU HAVE PXE, THINK YOU HAVE PXE, OR ↓  
ARE FILLING THIS OUT FOR SOMEONE ELSE

Name: \_\_\_\_\_ Phone: \_\_\_\_\_

Fax: \_\_\_\_\_ Email: \_\_\_\_\_

Address: \_\_\_\_\_

City: \_\_\_\_\_ State: \_\_\_\_\_ Zip: \_\_\_\_\_ Country: \_\_\_\_\_

Male  Female  Birthdate: \_\_\_\_\_ Age: \_\_\_\_\_

I am diagnosed with PXE  Yes  No Occupation: \_\_\_\_\_

Are you legally blind?  Yes  No Request Newsletter:  Printed  Taped

Do others in your family have PXE?  Yes  No If so, whom? (Mother, Father, Sibling, etc. & Name) \_\_\_\_\_

Please list the medical problems you are experiencing; e.g., eyes, skin lesions, gastric bleeding, etc., and comments/questions: \_\_\_\_\_

Please list any medical problem(s) you are experiencing: e.g., eye involvement, skin lesions, gastric bleeding, etc., and comments/questions (use another page if required): \_\_\_\_\_

Are you willing to be contacted, for example, by someone in your area who wishes to talk with someone else who has PXE?  Yes  No

*Thank you for your contribution. It is deeply appreciated.*

## Have You Changed Your Address?

Please help by letting us know. Please be sure to print your new zip code number, including the extra four digits, if possible. When we use the full zip code, our costs of mailing in the United States are lower. Please help.

### *New Address*

Name: \_\_\_\_\_

Street: \_\_\_\_\_

City, State, Zip \_\_\_\_\_

### *Old Address*

Name, if different: \_\_\_\_\_

Street: \_\_\_\_\_

City, State, Zip \_\_\_\_\_

***PLEASE PRINT NEATLY***

National Association for Pseudoxanthoma Elasticum  
**NAPE, Inc.**  
8764 Manchester Road, Suite 200  
St. Louis, MO 63144-2724

**ADDRESS SERVICE REQUESTED**