



August 2016

# THE RESULTS ARE IN

## Find out which research question PXEers voted most critical

### Background

This summer PXE International asked you to weigh in on our research agenda by prioritizing research questions as *critical*, *important*, *would like to know*, and *not so important*. Now we want to share the results- check out the top four research questions you chose!

### Results

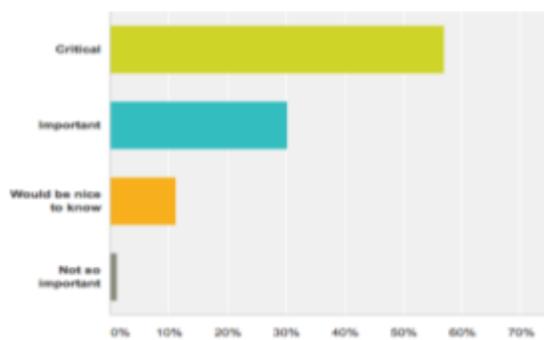
#### Priorities

**What does the ABCC6 protein do?** Where is the protein located? How does it function? Can the protein be repaired or its function restored?

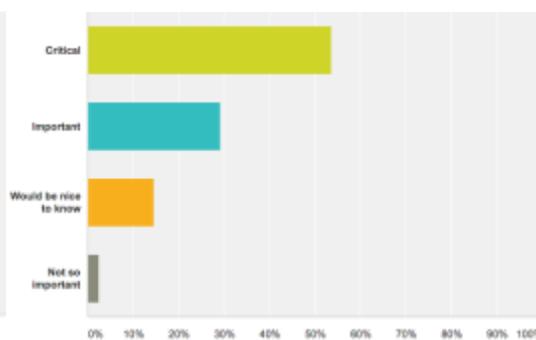
**Can eye complications of PXE be reversed?** Key hallmarks of PXE are peau d'orange and angioid streaks, the physical cracks in Bruch's membrane.

**Can we do stem cell experiments to alleviate the manifestations of PXE?**

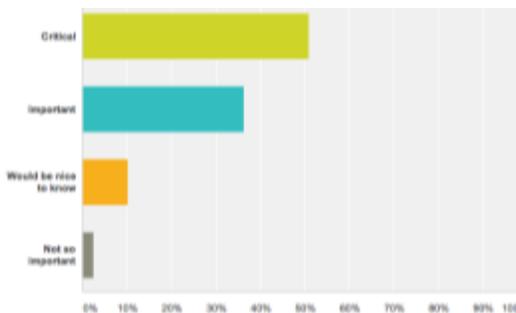
**Are there any vitamins or minerals that are deficient in PXEers?** Is too much vitamin E not good? Are there benefits of cutting down on high acid foods?



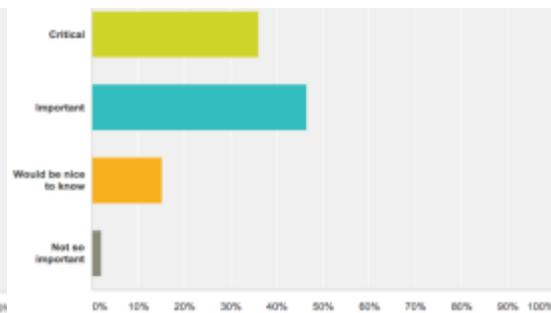
Abcc6 Protein



Stem Cells



Reversing Eye Manifestations



Vitamins and Minerals

*What now?*

You determined the priorities, what happens next? We developed plans to utilize our resources for practical research and effective strategies to share the results.

*ABCC6 Protein*

**What does the ABCC6 protein do? Where is the protein located? How does it function? Can the protein be repaired or its function restored?**

These are important questions that many researchers are currently investigating. This research will continue. PXE International will collect what is known and create a video and lay summary on the ABCC6 protein and its role in PXE. Our goal is to centralize what we know and what we're looking to discover in the near future.

*Reversing Eye Manifestations*

**Can eye complications of PXE be reversed? Key hallmarks of PXE are peau d'orange and angioid streaks, the physical crack in Bruch's membrane.**

It is unknown if these manifestations of PXE are reversible. Working together with patients and researchers, PXE International will develop a report on eye treatment options and current studies to be translated to a lay summary. The goal is to determine the effectiveness of various treatments for eye manifestations in PXE.

*Stem Cells*

**Can we do stem cell experiments to alleviate the manifestations of PXE?**

Stem cell research can be conducted for any disease, usually at the cost of millions of dollars. This research is far outside our ability to fund; however, research that other groups are conducting on retinal and liver systems will be beneficial to our understanding as well. PXE International will write a report and lay summary on what might be applicable to PXE. The goal is to determine the potential of stem cells as a method of treatment for PXE.

*Vitamins and Minerals*

**Are PXEers deficient in any vitamins or minerals? Is too much vitamin E not good? Are there benefits of cutting down on high acid foods?**

Diet and lifestyle are huge factors in your overall health. PXE International will summarize anything that is known about PXE and diet/nutrition in a report and lay summary. The goal is to determine if a change in diet could minimize or eliminate any PXE related symptoms.

**These reports will be shared with the PXE community in Fall 2016.**