



# Year in Review

## 2024

HAPPY  
NEW  
YEAR!!

2024 has been an impactful and productive year for PXE International as we continue our mission to support research, education, and care for individuals with pseudoxanthoma elasticum (PXE). Here are the highlights of our efforts and achievements over the past year:

### **Advancing Research: DS-1211 Phase II Clinical Trial**

PXE International played a critical role in the U.S. arm of the Phase II clinical trial for DS-1211. Our organization led the recruitment efforts, ensuring strong participation from the PXE community. Sharon Terry, CEO of PXE International, contributed as a co-investigator, exemplifying our deep engagement in the research process.

## **Collaboration with the PXE International Center of Excellence**

We continued to fund and collaborate with the PXE International Center of Excellence in Research and Clinical Care at Jefferson University. Under the leadership of Koen van de Wetering and Qiaoli Li, this center has made significant strides, publishing several important papers this year. Their research provides critical insights into PXE's underlying mechanisms and potential pathways for treatment, fostering hope for the future.

[Dr. Li's Papers](#)

[Dr. van de Wetering's Papers](#)

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## **International Patient Engagement**

We connected with the PXE community through various platforms, including:

- A patient meeting in Ireland, fostering global solidarity and sharing vital updates.
- 11 webinars attended by more than 300 live participants and viewed over 1,000 times, offering updates on clinical care and research developments.
- Responding to 256 emails from PXEers, addressing specific concerns about symptoms, finding specialized care, and providing tailored advice.

[PXE International's YouTube Channel](#)

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## **Support for New Studies**

PXE International also contributed to the recruitment efforts for Kristina and Maxamilian Pfau's study. Their recent publication

adds to the growing body of knowledge that drives the development of effective treatments.

## Drs. Kristina and Max Pfau Study

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### **Engaging Industry and Regulatory Partners**

This year, we held discussions with six companies actively exploring PXE treatments, demonstrating increasing interest in the field. Additionally, we met with the FDA to clarify the path forward for trial treatments, reinforcing our commitment to accelerating the approval process.

### **Global Advocacy and Education**

Our advocacy efforts spanned the globe, with 30 talks delivered worldwide in person and virtually. Highlights included presentations in Geneva, Tokyo, Kyoto, New York, and Nashville, sharing PXE International's work and inspiring broader engagement in the PXE community.

### **Looking Ahead**

The milestones achieved this year reflect the dedication of the PXE International team, researchers, clinicians, and our global community. As we look to 2025, we remain committed to advancing research, enhancing care, and empowering PXEers worldwide. Together, we will continue to drive meaningful progress toward treatments and, ultimately, a cure for PXE.

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**As we close out the year, we thank you for your support! All of our work is funded by your generous donations.**

**Double your donation! Matching Campaign is in full swing.**

- [What do your donations pay for?](#)
- [Make a donation](#)

\$148,285.57 of \$350,000.00



Donate

## Raising Awareness for PXE

Do you want to educate your community members, co-workers, friends, and family about PXE? In response to numerous requests, we've created an informative infographic to help you share what PXE is, who PXE International is, and how others can contribute!

Attached, you'll find a full-sized, printable flyer for your convenience. Let's join forces to raise awareness about PXE!



Help those suffering with Pseudoxanthoma Elasticum (PXE)

### What is PXE?

A rare genetic disorder causing:

- severe legal blindness
- skin issues
- vascular complications

### Who is PXE International?

Since 1995, we have been the vanguard against PXE by:

- pioneering research
- bringing together a global network of laboratories
- managing an extensive tissue bank/registry
- leading charge with clinical trials

### Support



A responsive lifeline to affected individuals, providing crucial information to those newly diagnosed.

### Our Mission?

#### Educate



Equip medical professionals with knowledge and resources to recognize and understand PXE.

#### Research



A collaborative research consortium that's the powerhouse for most of the world's research on PXE.

### How can YOU help?

As a non-profit, we rely on donations and your contribution directly fuels research for treatment development and supports clinical trials.

- **Credit Card:** Secure and convenient - Scan the QR Code for more info
- **Zelle:** Quick transfer via [mary@pxe.org](mailto:mary@pxe.org), under PXE International.
- **Check:** Send to PXE International, 26400 Woodfield Road #189, Damascus MD 20872-7509



[Click Here to Download the PXE Infographic](#)

## Increasing Your Impact: a Message from our Dear and Famous Board Member



Our most well-known PXE supporter, Jessica Harper, is dedicated to advancing PXE research and will match donations. Please listen to her call for us to step up! We are very grateful to her and her family!

[Donate to our Matching Campaign Here](#)

[PXE International](#)



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