2026

PFIC Family & Scientific Conference Impact Report

123
TOTAL IN-PERSON

38
VIRTUAL ATTENDEES

26
UNIQUE FAMILIES

5
PFIC SUBTYPES REPRESENTED











What Brought Us Together: PFIC

If you're a person or family living with PFIC, isolation is part of the diagnosis in every day life. PFIC is a disease most people have never heard of, surfaces difficult questions that your local healthcare providers can't always answer. It often forces you to make care decisions without support or guidance from anyone nearby who's faced them before themselves. But once a year, PFIC is what brings us together. The PFIC Family & Scientific Conference exists to close these gaps: it is an event that connects people living with PFIC with one another, and with those who care for and study the disease, to exchange knowledge, compare perspectives, and identify shared priorities.

Shaped by lessons learned from three years of PFIC-specific convenings, last year's inaugural Cholestatic Liver Disease Summit, and the feedback our attendees and advisors have given us along the way, this year's conference was sharpened to deliver maximum value and provide more opportunities for new ideas to emerge and solutions to take shape for people and families navigating PFIC. For two days in Chicago, 123 people showed up to participate in a focused, independent forum built by and for this community.

Our Impact

Our 2026 Conference brought together the most people living with PFIC and their families in the event's history, to provide more access to trusted information and time with experienced peers, clinicians, and researchers than ever before. Conversations extended past the formal sessions and Q&A: facilitated breakouts, topic-based discussion tables, and networking activities like the welcome reception speed meeting activity gave attendees more ways to ask questions, compare notes, and meet others managing the same disease.

PFIC patients and their families weren't the only ones learning at the event. Clinicians, researchers, and industry partners who care for and/or study PFIC heard directly from those living with the disease about the day to day demands,, where current care falls short, and what outcomes matter most. That understanding can help shape improvements in how care is delivered, how research studies are designed, and how new treatments are developed.

> As a family new to this diagnosis, we arrived feeling nervous, overwhelmed, and scared. We left with hope, knowledge, and a much deeper understanding of our daughter's condition. We also left knowing we are not alone, in the words of my husband, actually slept last night with a calm peace of mind.
>
> - PFIC Parent, 2026 Conference Attendee

Thanks to scholarship and committee stipends,
in person attendance was made possible for many attendees and that access mattered. Survey respondents left feeling more informed, better connected, and hopeful about the future. By bringing together the PFIC community in one place, the conference strengthened connections between families, healthcare professionals, industry partners, and researchers.

None of this - the event, the resources, the impact - would have been possible without the individuals and organizations who helped plan and support it. We extend our sincere thanks to everyone who contributed.







Session Highlights

Sessions in full can be found on PFIC Network's website.
www.pfic.org/learn-about-pfic-disease/pfic-webinar-series

Keynote Address: The Evolution of the PFIC Diagnosis and Landscape

Presented by: Dr. Ron Sokol

Key Takeaways

The keynote traced how PFIC research has evolved, from early biology and symptom recognition to today's remaining knowledge gaps and priorities.

- Spanned from 1960s discovery in the Amish population, to 1998 genetic classification of PFIC 1, 2, and 3, to today's understanding of up to 13 different genetic subtypes
- Demonstrated how prior PFIC biology research now enables gene-specific individualized therapies.
- Emphasized that understanding disease mechanisms and discovering new treatments requires collaboration across the field.

Managing PFIC Today

Moderated by Dr. Emily Perito

Presented by: Dr. Paula Hertel, Dr. Alyssa Kriegermeyer, and Dr. Lee Bass

Key Takeaways

The panel addressed current approaches to PFIC care, including genetic testing and diagnosis, monitoring and prevention of complications, treatment approaches, and nutrition.

- Described how genetic testing increasingly informs both diagnosis and individualized treatment decisions for people with PFIC
- Explained that tracking fibrosis and other progression markers guide ongoing disease monitoring
- Reviewed how IBAT inhibitors control symptoms for many, but some people still see limited or no response

PFIC Research Advancements

Moderated by Dr. Chunyue Yin

Presented by: Dr. Henkjan Verkade, Dr. Richard Thompson, and Dr. Aki Asai

Key Takeaways

The panel covered the latest PFIC research, from advances in genetic and clinical diagnostics to novel therapies in development.

- Explored how new sequencing technology improves precision in diagnosing PFIC subtypes
- Reviewed advances in gene therapy for other rare diseases and shared how these are accelerating the timeline for similar treatments being developed for PFIC
- Walked through examples of how combining diagnostics, animal studies, and genetics are helping researchers predict how a person will respond to treatment

Research Experience Roundtables

Led by Melissa Kochanowsky & Dr. Gitta Lubke

The session shared updates on PFIC Network's PCORI-funded project to establish a Consortium for Patient-Centered Research on PFIC, followed by facilitated breakout discussions on what helps or hinders participation in research studies.

A summary of the breakout findings will be published soon and will inform methodology recommendations in the Consortium's research agenda.

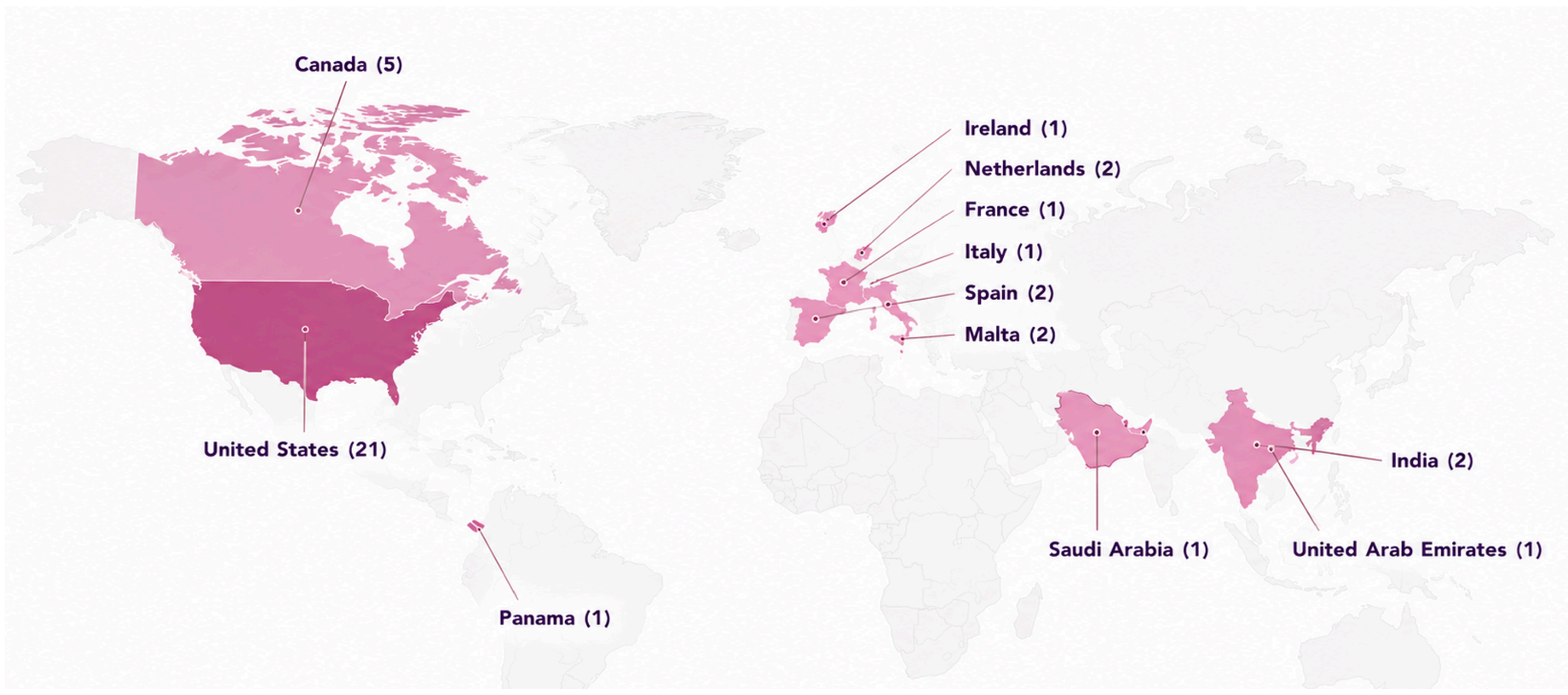
Note: this session was audio recorded for project team use only and will not be shared via YouTube.

Who Attended

78	21	17	26	30
INDIVIDUALS & FAMILIES W/PFIC	SCIENTISTS & PROVIDERS	INDUSTRY REPRESENTATIVES	UNIQUE FAMILIES	ATTENDEES UNDER 18

This year's conference drew 123 in-person attendees, along with 38 participants through the virtual platform. Specifically, in-person attendees represented a wide range of individuals; with PFIC, their families, and other stakeholders from around the world.

Among the 26 unique individuals/families affected by PFIC who attended, 5 PFIC subtypes were represented, including: FIC 1 deficiency (PFIC1), BSEP deficiency (PFIC2), MDR3 deficiency (PFIC 3), TJP2 deficiency (PFIC 4), and MYO5B deficiency (PFIC 10). In-person attendees traveled from four countries (US, Canada, the UK, and the Netherlands), while virtual registrants represented 12 countries across North America, Europe, and Asia.







Attendee Feedback

Following the event, we distributed an evaluation survey to all attendees, both in-person and virtual, and received 28 responses. Results are summarized below.

What Worked

96% of respondents (26 of 27) said they were "very likely" to recommend the conference to another community member.

The share of respondents who agreed they learned something new was consistently high across every session:

- 100% at PFIC Research Advancements
- 95% at the Open Topic Tables (21 of 22)
- 92% at Managing PFIC Today (23 of 25)
- 86% at the Consortium Project Research Roundtables (19 of 22)

94% of respondents (15 of 16) agreed or strongly agreed the welcome hour speed meeting activity was a good use of their time.

In addition, members of our community were embedded in the design of the conference, through a conference advisory committee and consortium steering committee, which deepened engagement and ability to provide meaningful content.

Where There's Room to Grow

Time for Q&A and Discussion — was rated as the greatest area for improvement. Several open-text responses recommended an additional mixer or happy hour.

Event Schedule — multiple open-text responses described the main conference day as long and packed, with one suggesting splitting into two half-days.

Understandability of Research Presentations — PFIC Research Advancements was the only session which any survey respondents disagreed was easy to understand. Given it was considered the most valuable session, we will prioritize working more closely with research-related speakers in the future to improve understandability of their talks.

> Things were explained in an easy-to-understand manner without sacrificing the use of medical and technical terminology. The explanation of gene sequencing methods and the future possibilities and potential for genetic treatments was fascinating. It was like having a college-level course overview.
>
> - 2026 Conference Attendee

Suggested Future Topics

- Post-transplant management
- Relationship between hormones and PFIC
- ICP
- Genetic testing, including risks and barriers
- Updates on clinical advancements (eg., use of AI, updated care guidelines)
- Mechanisms of itch
- Nutrition, including specific examples of ideal food and beverages
- Symptom management strategies

Sponsor Highlights

Mirum Pharmaceuticals

Mirum Pharmaceuticals is a leading rare disease company with a global footprint of approved products and a pipeline of investigational medicines. Focused on rare liver and rare genetic diseases, Mirum brings forward breakthrough therapies for overlooked conditions through deep scientific expertise and strong connections with patient and family communities around the world. The company's portfolio includes LIVMARLI® (maralixibat), CHOLBAM® (cholic acid) and CTEXLI® (chenodiol), with pipeline programs across cholestatic liver diseases, chronic hepatitis delta virus, fibrodysplasia ossificans progressiva and Fragile X syndrome. Mirum's work is driven by purposeful collaboration and a commitment to advancing high-impact medicines. Learn more at www.mirumpharma.com.

"Being part of the PFIC Family Conference was incredibly impactful and motivating. Having the opportunity to hear directly from patients and families about their experiences, and seeing the resilience and hope within this community, was truly inspiring. We are so grateful to be welcomed into this community and to be part of such a meaningful event."

Ipsen Pharmaceutical

Ipsen is a global biopharmaceutical company focused on innovation and specialty care. At Ipsen, we work to provide hope for people whose lives are impacted by rare diseases, difficult-to-treat cancers and neurological conditions. Every day, our more than 5,000 employees worldwide, including nearly 800 in North America, work diligently to improve the lives of people around the world. For more information, please visit www.ipsen.com/us.

"For people living with PFIC and their families, the PFIC Network conference creates opportunities to learn and connect with one another. With education and support, families become more empowered to be their own best advocates. It's a privilege for Ipsen to attend and learn from the PFIC community."



















Sponsor Highlights *(continued)*

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Mirum Pharmaceuticals

- Patient Stories

Tanner Pharma Group

- Managing PFIC Today

Tanner Pharma GROUP



> One of the most meaningful aspects of the conference was seeing our daughter connect with other children living with PFIC. Having the opportunity to interact with peers who share similar experiences allowed them to openly discuss symptoms, exchange perspectives, and validate experiences that are often difficult to articulate or not widely understood. Most importantly, she left knowing she is not alone in her journey!
>
> — PFIC Parent, 2026 Conference Attendee

Thank you to all who make this experience possible for our community.

We look forward to seeing you next year in Kansas City!

– The PFIC Network Team

