HLH Podcast Transcript

Support for this program is provided by CSL Behring, Takeda, and Grifols.

Emma Mertens:
Alright. It appears that we're at the top of the hour, so we're going to go ahead and get the webinar started. Good afternoon and welcome. Today, we are so excited to be joined by Dr. Michael Jordan from the Cincinnati Children's Hospital HLH Center. My name is **Emma Mertens**, and I'm the Program Manager for Education here at the **Immune Deficiency Foundation**.

On behalf of IDF, we thank you for joining us and tuning into this virtual event. IDF's lunch and learn events explore some of the rarer types of primary immunodeficiency, providing diagnosis-specific information and support to the PI community. In offering these programs, we aim to move forward in our vision of a healthier day every day for every person living with immune deficiency.

Today, we will learn about HLH, a rare type of hyperinflammatory PI. Today's program is sponsored by **Sobi**. It is due to their partnership and support that we can offer programs like this for the individuals and families we serve. And with that, I am pleased to turn it over to **Brittney Franklin**, Associate Director of Advocacy Relations at **Sobi**. Welcome, Brittney.

Brittney Franklin:
Thanks, Emma, for the introduction. My name is **Brittney Franklin**. I am the immunology lead for the advocacy team at Sobi. For those who are not familiar with Sobi, we are a biopharmaceutical company, and our mission is to make innovative care available for people living with rare and severe diseases such as HLH.

This mission hits home for me personally, as I am a mom to a now thirteen-year-old who has been living with primary HLH for the last twelve years. Over the last several years, there have been many advancements in understanding the mechanism behind HLH and how best to treat it. And I believe this is a really great opportunity for you all to learn not just about the science of the disease, but also to have the opportunity to ask questions and learn.

Thank you to the **Immune Deficiency Foundation** for hosting, and most importantly, thank you all for attending.

Emma Mertens:
Thanks so much, Brittney. Alright, before we get started, a brief disclaimer. Please remember that information presented during this meeting is not intended to be a substitute for medical advice, diagnosis, or treatment. We are here today as a trusted source and friend to provide you with information. Always seek the advice of your physician or other qualified health provider with questions concerning a medical condition. Never disregard professional medical advice or delay seeking it based on information presented during an educational event.

And now, I'm so pleased to introduce our presenter for today. **Dr. Michael Jordan** is a renowned pediatric immunologist and hematologist at Cincinnati Children's. He is part of a team of experts at the Cincinnati Children's HLH Center of Excellence and specializes in treating young patients with rare immune deficiencies. Thank you for joining us and welcome, Dr. Jordan.

Dr. Michael Jordan:
Thank you, Emma. And thank you to the **Immune Deficiency Foundation** for this invitation, and to **Sobi** for sponsorship.

[Presentation covers:]

- HLH overview, causes, and classifications (familial and secondary)
- Pathophysiology and immune mechanisms
- Diagnostic markers including **sCD25**, ferritin, and **HLA-DR**
- Treatment strategies such as dexamethasone, etoposide, emapalumab, and **nivolumab**
- EBV-associated HLH (corrected from GBDHLH)
- Advances in stem cell transplant and registry data

Q&A Session

Emma Mertens:
Would you recommend pediatricians to order **sCD25** and ferritin levels on suspected Kawasaki patients that are inpatient?

Dr. Michael Jordan:
There are a number of patients that are ultimately diagnosed with HLH whose initial diagnosis is Kawasaki's disease. These are very different disorders — they are completely unrelated from a developmental perspective — but because they're both inflammatory disorders, they can look somewhat similar. So in general, yes, it's good practice to at least consider HLH when someone is considering Kawasaki disease. Sending off specialized inflammatory markers like ferritin and sCD25 is a good first step, even though those values may not always be elevated in Kawasaki cases.

Emma Mertens:
You mentioned during your talk that the general awareness of HLH within the medical community has really come a long way. How common is HLH, really?

Dr. Michael Jordan:
One widely used estimate based on the Swedish health registry is about one in 150,000 children, though this varies by ethnicity. In the U.S., the numbers may be slightly different. Familial HLH is probably diagnosed in the hundreds of cases per year, while secondary forms — particularly cancer-associated HLH — may involve thousands, though recognition is still evolving.

Emma Mertens:
In addition to the HLH registry you mentioned, are there other opportunities for future research?

Dr. Michael Jordan:
There are ongoing efforts in the U.S., Europe, China, and Japan. Some clinical trials have closed, such as those for emapalumab and ruxolitinib, but there may still be opportunities — for example, a ruxolitinib trial for adults at a center in Michigan. Clinicaltrials.gov is the best place to check for updates.

Emma Mertens:
Final question: Do you know of any patients who had a primary HLH flare and survived for several years without undergoing stem cell transplant?

Dr. Michael Jordan:
Yes, there are cases where patients have remained stable for years without relapse.

However, for classic familial HLH, we generally recommend transplant, as the risk of relapse remains significant over time.

Emma Mertens:
Thank you so much, Dr. Jordan. I don't see any more questions in our Q&A box, so that's going to wrap up our Q&A. Thank you so much, Dr. Jordan, for all this incredible information, and thank you to our audience for asking these great questions.

Before we wrap up, I'm going to share some information on IDF resources and upcoming events. Dr. Jordan, you're free to stick around, or if you need to get back to your day, thank you again for sharing your time and expertise with us.

Closing Remarks

- Visit **primaryimmune.org** for resources, events, and materials — all free to access or request.
- Contact IDF’s **Ask IDF** navigator for personal support.
- Check out IDF podcasts, including *Bold Conversations*, *Undiagnosed*, and *Chronic Twenties*.
- Watch recorded educational events on the IDF YouTube channel.
- Join the **Walk for PI** to help fund IDF’s research grant program.
- Explore virtual **Get Connected Groups** for peer support.

Emma Mertens:
Thank you again to **Sobi** for their generous support of IDF educational programming, and to Dr. Jordan for this incredible presentation. And to our audience — thank you for joining us.