



REGISTRY NEWSLETTER

Welcome!

Welcome to the latest issue of the International PPB/*DICER1* and OTST Registries newsletter! This past year included exciting developments in research and opportunities to share those new findings with those who made it possible and need it most. Since our last newsletter in May 2022, we have hosted the annual *DICER1* Scientific Symposium bringing together more than 100 clinicians and researchers from around the world. We also hosted the 2022 virtual PPB/*DICER1*/ OTST Family Meeting. Following the Family Meeting, we also gathered with patients and families for an outdoor summer picnic at the Pine Tree Apple Tennis Classic. Thanks to all who participated in these events! It was fun to spend the day and evening with you!



Publications in 2022 included an analysis of treatment and outcomes for Types II and III PPB (see update below) as well as an analysis of Type I and Ir PPB. Additionally, we published new findings in health-related quality of life after PPB therapy as well as new discoveries related to Sertoli-Leydig cell tumor.

In 2022, we initiated the first meetings of the International *DICER1* Laboratory Consortium to collaborate with other researchers and broaden our network of laboratory and translational research.

As we start 2023, we look forward to a new year of collaboration and continuing to learn together as we work toward our mission of improving outcomes for children and adults with pleuropulmonary blastoma (PPB) and other *DICER1*-related cancers as well as ovarian and testicular stromal tumors.

Thank you for all that each of you do to advance this shared mission!

With warmest regards,

The Registry Team

2022 Family Meeting Recap

Thank you to all of the patients and families who were able to join us for the 2022 Family Meeting. We appreciate the time you took to join us and thoughtful questions you asked.

If you were unable to join us for the meeting or would like to review some of the presented information, please use the links below to watch select presentations from the event.

[Watch Now](#)

DICER1 Testing: ABCs by Shari Baldinger

[Watch Now](#)

Clinical and Research Updates from the International PPB/*DICER1* Registry by Dr. Kris Ann Schultz

Watch Now

Updates from the International Ovarian and Testicular Stromal Tumor Registry by Dr. Kris Ann Schultz

Save the Date: 2023 Family Meeting

Mark your calendars for Thursday, August 3rd, 2023 for the 8th PPB/*DICER1*/OTST Family Meeting.

In preparation for this meeting, we would be grateful to receive your recommendations and ideas.

Click the link below to access the planning survey. Your time and ideas are greatly appreciated!

Click for Survey



Outcomes for Children with Type II and Type III Pleuropulmonary Blastoma Following Chemotherapy

From the founding of the International PPB/*DICER1* Registry in 1987 to 2021, 314 children with centrally reviewed Type II or III PPB enrolled in the Registry and received chemotherapy as initial treatment. Of these, 132 children received a regimen called IVADo, consisting of ifosfamide, vincristine, actinomycin D and doxorubicin.

On September 22, 2022, the Registry published a report in the *Journal of Clinical Oncology* analyzing the outcomes of children with Type II and III PPB who received IVADo chemotherapy or other regimens. An adjusted analysis was used to account for differences in metastatic status and radiation therapy between these groups.

This analysis, made possible by these 314 children and their families, provides key data and benchmarks guiding development of future treatment protocols for children with advanced PPB. We are so grateful to the kids and families contributing to this research and endeavor to continue to improve outcomes for children with PPB in their honor.

Sunglasses

By: Whittney Jones, Mom of Indy Jones

Something I have never asked another cancer parent, but feel like I can safely assume, is whether or not they have a picture of their child that they consider the last picture they took before their diagnosis. It's a picture they didn't think anything of until that moment they were told their child had cancer, and then it suddenly became a very important picture—the defining line between everything life was before and everything it then becomes. The image that will somehow come to represent all of the hopes and dreams you had for your baby when they were first born. Every time you look at it, all you can think is: I wish I could go back to that day. I wish I could go back to that life. Why did this happen to us?

That picture for me was of Indy on a sunny day, when the weather was just starting to get warm, lying on her belly on the porch step next to me. Her hair was in a ponytail, but still noticeably windblown, and she was wearing what would become her trademark pair of glasses: a pair with round shades, one side of the frame was yellow and the other side was pink. After her diagnosis, my parents made Team Indy shirts to sell for her, and, for whatever reason, chose those sunglasses to represent her, further emblazoning that picture of her with an easy, open-mouth smile into my memories and that place in them known as the “before”. The place we were always fighting to get back to. That pair of sunglasses now sits on her shelf, next to her urn, and is possibly the most difficult item on that shelf to look at, because that pair of sunglasses embodied so much of what Indy was: quirky, fun, colorful, and impossible to forget.



Though PPB Type 3 took a lot away from Indy physically, it never took away that spunk or eagerness to play or sense of adventure. One of the hardest things about being without her now is how the mundane has returned to the mundane without her beautiful imagination to invigorate our lives. Indy was five-years-old, her favorite color was green, she loved to collect sparkly rocks and long sticks that could be swords or wands, her favorite bedtime song was what she called The Kite Song from Mary Poppins. She loved Trolls so much that it was the theme of her last birthday party. She loved to sing and dance.

Indy passed away on October 16th, 2020, but two days before that, she celebrated my birthday. She baked the most beautiful two-layered birthday cake literally covered top to bottom with sprinkles. We went through an entire tub and two cylinders of sprinkles. She was looking forward to her dad arriving home that evening from work so that we could laugh at his surprise when he cut into the cake for a slice, and a mound of sprinkles poured out from a hole cut into the middle. She thought this was the best prank anyone had ever invented, and she laughed a lot that night. I didn't realize at the time, taking a picture of her blowing out my candles, that I was taking another “last” or “before” photo. Just like the day before her original diagnosis, everything seemed okay. We went to bed that night thinking everything was going to be okay.

Indy had just started kindergarten. She was looking forward to her favorite holiday: Halloween. She was already making a Christmas list. She wanted all of the same things most normal kids want. But she also dreamed of one day having long, curly hair again that she could pull up into a ponytail. She dreamed of being a super hero. Despite everything she went through, she dreamed of becoming a doctor one day to help other little kids like her. What her father and I want to do by sharing her story is to help make sure no one else has to take a last photo.

Project Spotlight: What is the Registry working on?

What are the blood samples used for?

The mission of the International PPB/*DICER1* Registry is to improve outcomes for children and adults with PPB and other *DICER1*-related cancers through [four strategic pillars](#). One of the pillars is to develop new ways to diagnose and follow children with *DICER1*-related cancers.

Imaging is the most often recommended form of tumor surveillance for *DICER1*-related cancers. The goal of surveillance is to detect tumors in their earliest, most curable form and assess tumor status and response. Unfortunately, even cross-sectional imaging methods such as CT and MRI have limited sensitivity for small tumors or early recurrence. CT also entails radiation, and MRI or CT may require sedation in young children.

Our current work includes efforts to develop better ways to detect and monitor *DICER1*-related cancers. As an optional part of the Registry study, blood samples are collected at certain time points in an individual's journey, when blood is already being collected for clinical reasons (no extra pokes).

The collected blood samples are analyzed in an effort to detect *DICER1*-related circulating tumor DNA (ctDNA). We plan to use these results to determine if this test may be clinically useful in the care of children and adults with *DICER1*-related tumors.

Thank you to all who are participating in the biomarker portion of our study. We are grateful for your help with this ongoing research!



What are the QoL questionnaire responses used for?

At the time of PPB/*DICER1* Registry consent, each participant and/or guardian may opt to receive a Quality of Life (QoL) questionnaire in the mail every two years. These questionnaires ask age-specific questions about fatigue, physical and psychosocial function.

The Registry compiles participant responses to better understand how different aspects of medical care, such as surveillance, diagnosis, and treatment, affect health-related quality of life (HR-QoL).

On November 24, 2022, the Registry published a report in *Pediatric Blood & Cancer* that highlights our findings from the compiled QoL data from participants with PPB and discusses the goals for continued data collection.

All individuals enrolled in the PPB/*DICER1* Registry are eligible to participate in the Registry's QoL project. Please reach out to us if you are interested in participating or if you'd like to update your mailing address or other contact information.



Ways to Get Involved

Together, we can find a cure for PPB, Sertoli-Leydig cell tumor, and other *DICER1*-related cancers as well as non-*DICER1* ovarian and testicular stromal tumors. Much progress has been made, but there is still much to learn. Each shared journey helps improve outcomes for children and adults with or at risk for these rare tumors.

Please consider following the Registries on Facebook ([PPB/DICER1](#) and/or [OTST](#)) and [Twitter](#). Additionally, click here to visit the [PPB/DICER1 Registry website](#) and here for the [OTST Registry Website](#).

We also invite you as a patient or family member to share your story.

We look forward to hearing from you!



Click to Contact the Registry About:

[PPB/DICER1 Enrollment](#)

[Update Contact Info](#)

[OTST Enrollment](#)

[Share My Story](#)

Celebrating Dr. Messenger!

Many of you know Dr. Messenger, pediatric oncologist, researcher, mentor and friend. Dr. Messenger exemplifies wisdom, kindness, compassion, wit, integrity and humility. In addition to his renown leukemia and lymphoma work, he has been involved in decades of PPB/*DICER1* clinical care and Registry research, starting with the historic PPB Registry protocol in 1987 and continuing through the Registry Treatment and Biology protocol and the latest version of the protocol, initiated in 2016. His contributions to PPB and *DICER1* research are immense and his legacy incredibly broad.

I've had the great honor of learning from Dr. Messenger and also the joy of watching him care for kids and families in the clinic and hospital. I'm smiling even as I write this, thinking of his lighthearted jokes and kindness to all.

Many of you have your own Dr. Messenger memories. As Dr. Messenger transitions toward retirement, we invite you to share your stories with us at DICER1@childrensmn.org, ideally prior to March 1st. We plan to compile these stories and well wishes along with our own to share with him in March.

Thank you!

Kris Ann Schultz, MD



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