



# BIOINFORMATICS SEMINAR

## Anders Kolb

*Director, Blood & Bone Marrow Transplant*

NEMOURS, A.I. DUPONT HOSPITAL FOR CHILDREN

### The Promise of Precision Medicine in Pediatric Oncology: New Challenges and Hope

Acute myeloid leukemia (AML) of childhood remains incurable for 40% of children, and only after therapy that pushes the limits of toxicity and tolerability resulting in life-altering and life-limiting short and long-term side effects. Precision medicine promises to bring less toxic therapies that are specifically targeted. However, it is clear that we will not be able to link a single mutation to a single targeted therapy for most children with AML. For years, we have hoped to identify molecular targets that are “druggable”. We have also hoped that lessons learned from adults with AML would more rapidly inform therapy for children with AML. Neither of these approaches have resulted in rapid progress for children. Childhood AML genomics are confounding and challenging our traditional precision medicine models. Not only have we recently identified mutations not seen in adult AML, but we are beginning to understand that since AML in children geminates from a different developmental context than AML in adults, even shared mutations may impact cells differently. We need novel computational approaches that are biased neither by known somatic events that define AML in adults or by available druggable targets.

#### BIOGRAPHY

The children we treat need the most powerful, effective therapies right now – not days, weeks, or months from now. So I’m deeply involved in clinical and translational research, actively seeking to learn as much as possible from each child in our care. Our physicians stay up-to-date on the latest research strategies for kids with cancer, because everything we learn will help others in the future.

The focus of our lab is to identify novel therapies for children with cancer. We recognize the challenges that we face: 1) childhood cancer is a rare and orphan disease; 2) it is difficult to complete clinical trials for rare diseases; 3) drug development is focused on malignancies in adults; 4) we need better treatments for children now. To address all these problems, my lab works with a team of investigators at other institutions around the world to identify those therapies most likely to succeed in children and to prioritize them for clinical development. With more than 60 publications resulting in dozens of clinical trials, we have made great progress. Team science is the model that works. We don’t compete, we cooperate and share.

As a member of the Children’s Oncology Group Scientific Council, Chair of the Myeloid Disease committee, member of the Bone Tumor Committee and member of the Young Investigator Committee, I am in a position to maintain a cutting edge and relevant focus in my lab. We are continually taking results from the bench to bedside and back again; asking those research questions that will have the greatest impact on patient care. Our mission is, simply stated, to find better treatments for childhood cancer. Cure is not impossible with the right teams asking the right questions.



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03/19/2018**

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