

Spotlight on Pediatric Precision Medicine: Vision, Expectations, and Reality

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Precision Medicine in Pediatric Oncology

- Precision medicine is an emerging approach for disease treatment and prevention that takes into account individual variability in genes, environment, and lifestyle for each person. (NIH)
- Precision medicine is the use of genomic, epigenomic, exposure and other data to define individual patterns of disease, potentially leading to better individual treatment. (NAS)
- Precision medicine principles in pediatric oncology (prognosis) for more than two decades:
 - MYCN (neuroblastoma)
 - Chromosome number (ALL)
- Precision medicine principles in pediatric oncology (treatment):
 - BCR-ABL1 (Ph+ ALL) treated with imatinib
 - Anaplastic large cell lymphoma treated with crizotinib

Some Key Questions

- How to translate an enormous, ever-expanding quantity of genomic/ epigenomic/ clinical data into clinically useful findings (and avoid spurious conclusions)?
 - For diagnosis and prognosis
 - For treatment
 - For genetic counseling
- How to expand *therapeutic* applications of precision medicine beyond the small proportion of children who currently benefit?
 - Additional discovery research to match subsets of children to available targeted agents
 - Pediatric-specific targeted agents and the challenges of drug development for small patient populations

Panels

Panel 1

- ***Stephanie Devaney, Ph.D.***
 - Project Manager of the Precision Medicine Initiative, White House Office of the Chief of Staff
- ***Dr. Katie Janeway***
 - Asst. Professor of Pediatrics, Harvard Medical School and Pediatric Oncology, Dana-Farber/Boston Children's Cancer and Blood Disorder Center
- ***Dr. Will Parsons***
 - Director, Pediatric Center for Personal Cancer Genomics and Therapeutics, Texas Children's Cancer Center and Assoc. Professor of Pediatrics-Oncology, Baylor College of Medicine
- ***Dr. Steven Joffe***
 - Children's Oncology Group Bioethics Committee and Attending Physician, CHOP Cancer Center and the Blood and Marrow Transplant Program

Panel 2

- ***Dr. Nita Seibel***
 - Head of the Pediatric Solid Tumor Therapeutics, Clinical Investigations Branch, CTEP (NCI) and Co-chair, NCI Pediatric MATCH study
- ***Dr. Martha Donoghue***
 - Pediatric Solid Tumors Scientific Liaison Clinical Reviewer for Pediatric Oncology, Neuro-Oncology, and Rare Tumors Office of Hematology and Oncology Products, U.S. Food and Drug Administration
- ***Michael Cox, PharmD***
 - Loxo Oncology
- ***George Kirk, Ph.D.***
 - AstraZeneca



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