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
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