

Navigating Neuroblastoma and Myelodysplastic Syndrome



History (2010)

- ▶ March 26, 2010: Diagnosed with Stage 4, Neuroblastoma, unfavorable Histology, MYCN non-amplified (COG per ANBL 0532)
- ▶ Induction:
 - ▶ Complication: Hemorrhagic Cystitis from Cyclophosphamide
 - ▶ Complication: Moderate/Severe Hyperkalemia
 - ▶ Complication: Moderate/Severe Hearing loss.
 - ▶ Right nephrectomy

History(2010)

- ▶ Consolidation (BuMel) :
 - ▶ Complication: Moderate/Severe Veno-Occlusive Disease
 - ▶ Consequence: Declined primary tumor bed irradiation
 - ▶ Consequence: Fell off the protocol. (Do not proceed to ch 14.18)
- ▶ Declined from SKCMH due to too much tumor burden.
- ▶ First half of 2011: (Maintenance Chemo with Irinotecan and Temodal)

History(2011)

- ▶ Maintenance Chemo (Irenotecan and Temodal)
 - ▶ Complication: Severe neuropathic pain. Joints, finger tips, legs
- ▶ Enrolled in Personalized Medicine with Dr. Giselle Sholler.
- ▶ Declined by Ministry of Health for Clinical Trial at NMTRC, Grand Rapids, MI
- ▶ Subsequently, moved to NIH through referral from Dr. Sholler.
- ▶ Failed to enroll because we could not get a fresh tumor sample.
(Maintenance Chemo good? Bad?)
- ▶ Maya Kim: *God forgot about me... because I am still sick*

History(2012)

- ▶ Jan. 2012, St. Justine, Montreal (MIBG-131 Clinical Trial)
 - ▶ Found St. Justine, through another cancer family. Were considering CHOP
 - ▶ Complication: (We believe) Myelodysplastic Syndrome MDS. We would not find this out for another 6 – 7 months.
- ▶ July 2012: Enrolled in DFMO study with Dr. Sholler.
- ▶ Aug. 2012: Diagnosed with MDS. Translocation (1,7), (11,17)
 - ▶ Platelets down to 11, Blasts up to 25%.
- ▶ September 2012: Transferred Hematology/Oncology group. NB stable.
 - ▶ Started Azacitadine on compassionate grounds (Injection through CVL)

History(2013)

- ▶ Azacitadine therapy.
 - ▶ Very little literature on pediatric MDS/AML
 - ▶ By mid 2013, BMA clear. NB stable.
 - ▶ October, 2013 2 out of 70 cells found with Dysplasia
- ▶ December 2013 Accepted by St. Judes for KIR therapy (Killer Immunoglobulin like Receptor).
 - ▶ Importantly Non-myeloablative therapy.

History(2014)

- ▶ January 2014. KIR therapy start
 - ▶ Complication: Moderate/Severe Hemorrhagic Colitis. Due to MMF (Immunosuppressant)
 - ▶ Moderate GVHD.
- ▶ September, 2014:
 - ▶ Bronchiolitis Obliterans / Ground Glass Opacity
- ▶ Presently:
 - ▶ FEV1 approx. 40%
 - ▶ Endocrine issues starting to be seen
 - ▶ Celebrated 4 years post therapy in February

Example Description of C.T

- ▶ This phase II trial studies how well PI3K / mTOR inhibitor LY3023414 works in treating patients with solid tumors, non-Hodgkin lymphoma, or histiocytic disorders with TSC or PI3K / MTOR mutations that have spread to other places in the body and have come back or do not respond to treatment. PI3K / mTOR inhibitor LY3023414 may stop the growth of cancer cells by blocking some of the enzymes needed for cell growth.
- ▶ From: <https://www.cancer.gov/about-cancer/treatment/clinical-trials/search/v?q=neuroblastoma&loc=0&rl=1&id=NCI-2017-01249&pn=1&ni=10>