



Biomarkers Innocentive Challenge

Announced January 15, 2013: Biomarker Development for Plexiform Neurofibroma

Overview:

There is great variability in the clinical behavior of pNF (variable growth rates, locations, symptoms). In addition, diverse cell populations (Schwann cells, mast cells, macrophages, endothelial cells and neural tissue) are seen within these tumors, each with variable prevalence and activity that may dictate a given tumor's response to targeted therapies. Finally, there is a growing body of evidence that pNF have their maximal growth rate in childhood and that there may be a natural senescence in adolescence or adulthood. If this is confirmed, this would suggest that successful early intervention in high risk tumors would protect patients during the period of maximal growth and thereby potentially permanently control tumor size and symptoms with a relative short, early drug exposure. In order to justify treatment of small, pre-symptomatic tumors in young children, a biomarker that reliably predicts tumor growth is needed. There are currently no biomarkers that inform about the current or future behavior of a pNF or its likelihood to respond to treatment. The goal of the biomarker initiative is to identify or develop biomarkers that will both predict tumor behavior as well as predict response to therapy.

Funded Solutions:

- A serum exosomal RNA signature as a biomarker for pNF.
- Potential metabolic biomarkers for Plexiform Neurofibroma Growth through Systems Biology Approaches.
- Serum Human Telomerase Reverse Transcriptase Messenger RNA has diagnostic implications for Plexiform Neurofibroma Growth.
- Serum MicroRNAs: Potential Biomarkers for Plexiform Neurofibroma Growth
- Imaging solution: Properties of a stable isotope tracer ^{13}C .
- Abnormally hyperactive c-kit pathway detection in type 1 neurofibromatosis patients to determine risk of tumor growth.