Cancer Progress 25:
Day 2 Opening Remarks

Mike Rice, M.S., MBA, Senior Consultant, Defined Health

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CANCER PROGRESS by Defined Health

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Conrad New York

Cancer Progress: Day 1 Recap

- **Taking Immunotherapy Seriously: New Targets and Combinations** (Axel Hoos)
  - After decades of effort, Immunotherapy is a hot area – 2013 Breakthrough of the year
    - Checkpoint Inhibitors
    - Chimeric antigen Receptor (CAR) engineered T-Cells
  - Driven by advancement in both science and methods
  - Need for innovative clinical endpoints, clinical response may be delayed or absent even though immunotherapies may impact outcomes differently then conventional drugs.
  - Complementary to SOC
  - Immune regulation is complicated, need to study combinations of checkpoint inhibitors of with other cancer vaccine approaches.
  - Need for industry collaboration between biopharma companies
- **Brain Cancer - Tackling the Intractable: Novel Targets and Approaches** (Minesh Mehta)
  - Progress has been made, Chemotherapy and Antiangiogenic therapies
  - but unmet need is high, particularly for GBM, highlighting the limitations of current SOC
    - New developments with antiangiogenics
    - Tumors are heterogeneous necessitating advancement in molecular subgrouping (PTEN, PI3K, EGFR… mutations)
  - New Immunotherapeutic approaches are the next wave
    - Anti-CTLA 4, Anti-PD1, Vaccines: EGFR vIII, Sl-701
    - Brain Metastases may be one or the most prevalent tumors

Cancer Progress: Day 1 Recap

- **Big Data and Cancer: Transforming Patient Care by Turning Data into Decisions** (Colin Hill)
  - Modeling configuration of cancer scientifically and clinically feasible, costs coming down
  - In 5 years everyone's tumor may be sequenced?
  - Case studies demonstrate optimization of care (triple neg. BRCa)
  - Transition biomarkers to networks
  - Rx/Dx transition to Dx/Rx: Biomarker diagnostic may be developed with similar efficacy packages as a new drug – Will Dx be rewarded and priced accordingly?
  - Payers – Lacks evidence, not ready for prime time
  - Healthcare reform, moving accountability to physician
- **Evidentiary Standards for Diagnostics: When does a Biomarker Become a Diagnostic for Cancer Treatment? (**Steven Averbuch)
  - In both community and academic cancer centers there is a rapid adoption of NGS and other multiplex platforms to interrogate the biology of an individual's tumor.
  - For each individual patient, vast amounts of data are being generated, annotated with identification of "actionable" targets for therapeutic intervention.
  - Increasing number of CDx labeled drugs – level of evidence for target-directed therapies ranges from FDA approved therapy directed at that target in that patient population to anecdotal to hypothetical
  - Need for improved Analytical Validation, Clinical Validation and the Clinical Utility of NGS tests

Cancer Progress: Day 1 Recap

- **Oncology R&D: ROI and the New Expectations**
  - Cancer drug development is complex — while the science is highly innovative, and the pipeline is the largest of any therapeutic area, oncology drug development suffers the highest failure rate of any TA.
  - Next/best in class strategy leads to over concentration in MOA's - herd mentality in Innovation Center and Immunotherapy initiatives.
  - ROI benchmarks may lead to bad drug development portfolio decisions – Most oncology innovation has been serendipitous.
  - The focus at many players has been to run trials on populations that have been enriched with biomarkers, rare disorders, subsets
  - Patient advocacy taking an active role in improving oncology R&D: Regulatory input, data sharing, biobanking, and incentivized oncology R&D
- **New Treatment Options for Prostate Cancer**
  - Changing prostate cancer paradigm with newly approved androgen antagonists , Radium-223, chemotherapy and immunotherapy
  - Better insight into persistence of androgen signaling pathway in CRPC has been transformative
  - Multiple agents with varied mechanisms extend survival and delay progression
  - Cross resistance occurs between agents, active agents against similar targets (eg. Orterenol) have failed to meet endpoint
  - Immunotherapy will have an increasing role in prostate cancer,
    - Provenge useful, but takes a long time to have a clinical effect
    - Other agents may have a more rapid action: Checkpoint blockade (Ipilimumab, PD-1, PDL-1), CAR-T cells?