Regulatory and Policy Considerations for Public-Private Partnerships for Clinical Cancer Research

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CONFLICT OF INTEREST

Principal and Chief Medical Officer: Beat AML, a Limited Liability Corporation of The Leukemia and Lymphoma Society (unpaid)

Consultant (unpaid): AbbVie, Allogene, Newave, Orange Grove Bio, and Kartos

Research Funding: AstraZeneca, Newave, NIH

Scientific Advisory Board: Vincerx (Chair), Newave (Chair), Orange Grove Bio (Chair), Kurome (member), and Kartos (member)

Equity holder in publicly traded company: Vincerx Pharma

Equity holder in private company: Eilean Therapeutics, Kurome

WHAT IS BEAT AML?

- LLS convened partners (2016) with a goal of identifying more efficacious and tolerated therapies based upon lack of success despite more than 1/3 of research budget going to this

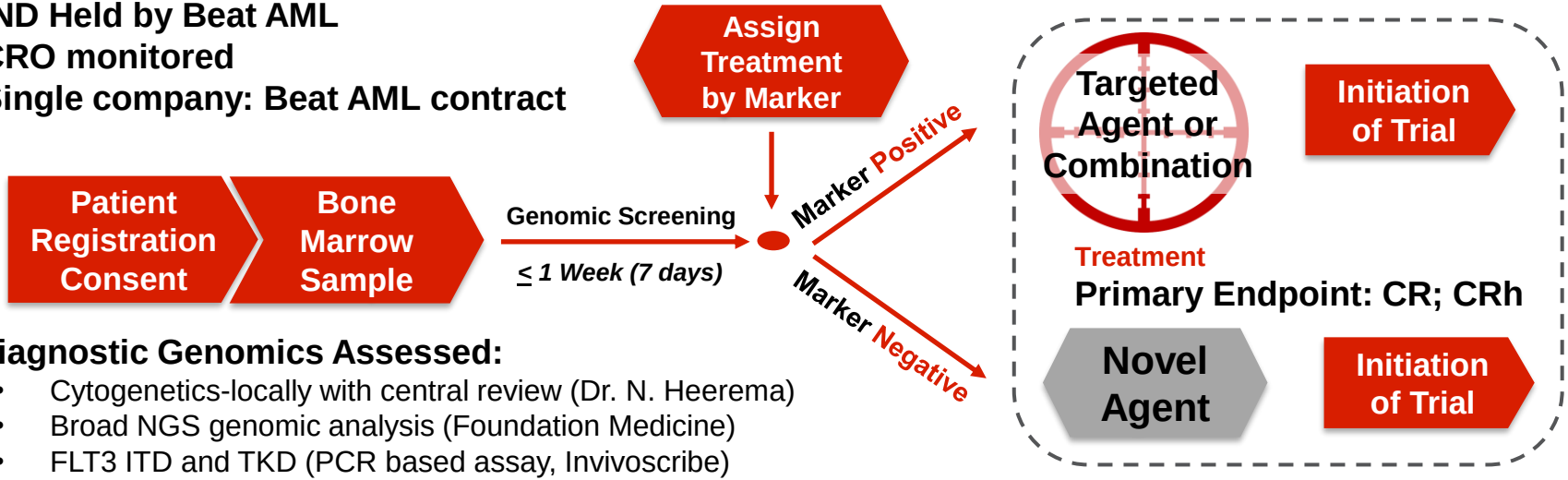
  -Academic institutions
  -Pharmaceutical Companies
  -Regulatory agencies (FDA)
  -Genomic testing
  -CRO and trial vendors

Patient focused

- Platform study that assigns therapy based upon cytogenetic and genomic features
- Central rules: Can't care about individual credit and stay nimble

BEAT AML Initial Study Composition and Design

IND Held by Beat AML
CRO monitored
Single company: Beat AML contract



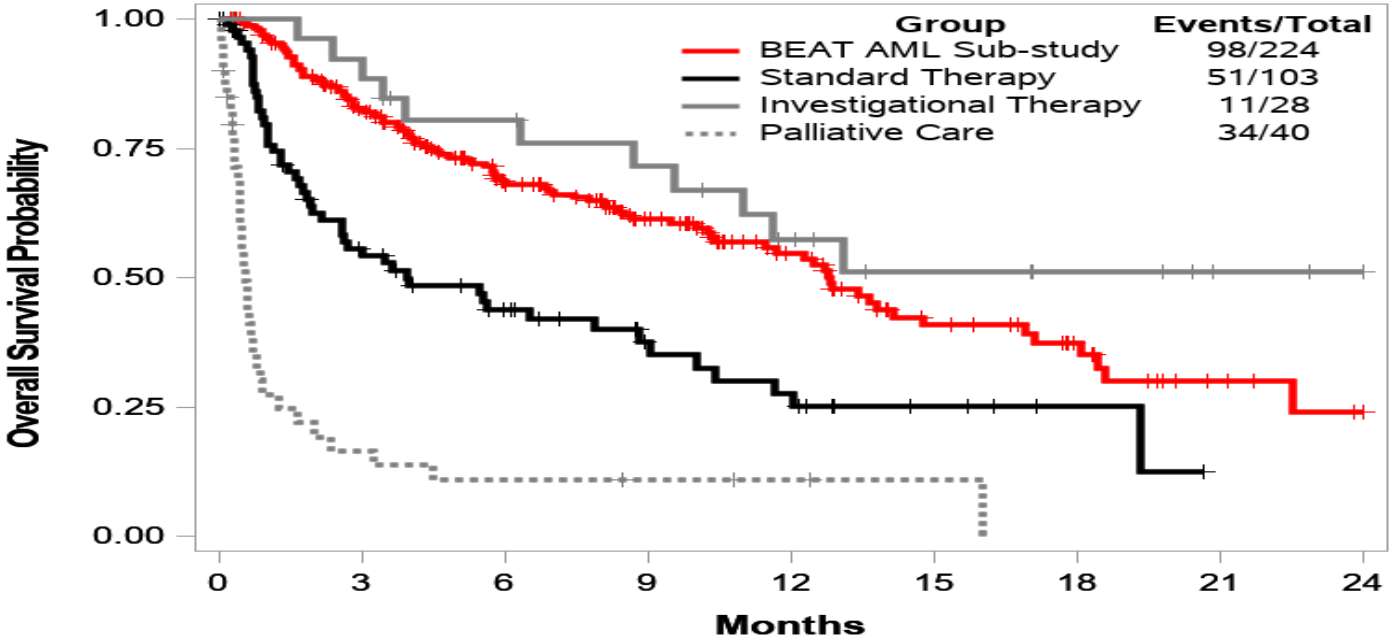
Diagnostic Genomics Assessed:

- Cytogenetics-locally with central review (Dr. N. Heerema)
- Broad NGS genomic analysis (Foundation Medicine)
- FLT3 ITD and TKD (PCR based assay, Invivoscribe)

Therapy assigned centrally
Up to 9 study arms open for specific genomic groups

BEAT AML OUTCOMES : OVERALL SURVIVAL[1]

Sub-study treatment compared to not treated on study



Current enrollment more than 1,400 pts

BEATING CANCER IS IN OUR BLOOD.

[1]Burd A, et al: Nature Medicine 26:1852-1858, 2020

LEUKEMIA & LYMPHOMA SOCIETY®

BEAT AML OUTCOMES

Contributed (along with CTEP investigators) to paving way for MyeloMATCH initiative by NCI

Ability to terminate studies early based on futility metrics

- Avoid large Phase 3 studies that likely will not meet endpoint

Provide guidance for NCCN guidelines

- Enasidenib for newly diagnosed patients with IDH2 mutations (AML treatment without hospitalization)

Data utilized to change clinical practice

- 16 ASH abstracts, 1 ASCO abstract, 1 EHA abstract
- 10+ peer reviewed publications

BEAT AML EVOLUTION

Necessitated by several things moving forward in field

- SARS-COV-2 Pandemic
- MyeloMATCH (NCI effort to disseminate our approach to community)
- Changing landscape of treatment (venetoclax + azacitidine approval)

New areas of focus (Trials)

- Beat AML study, Opti-AML, comparing 28 or 14 days venetoclax + azacitidine to maintain efficacy and improve safety/tolerability (pivot)
- Broad targeted agents + ven/aza to move to MyeloMATCH (phase 2)
- Select targeted therapy + ven/aza in select genomic groups (phase 1) to move to MyeloMATCH-Syndax Pharma
- Relapsed and refractory studies focused on ven/aza resistant disease

Application of new technologies and use of acquired samples

BEAT AML PEARLS AND OPPORTUNITIES

Pearls

- Not for profit can successfully run disease specific platform studies in rare disease
- Staying nimble at top (rapid decisions) and requirement to not care about credit enabled our success

Opportunities

- Improved venue options to refine dosing of approved regimens (i.e. Opti-AML, ven/aza) to assure they are usable in community
  - Consider empowering FDA to mandate this to companies or funding mechanism to perform
- Improve paths to encourage companies to file secondary NDA indications when data support
- All points made by Dr. Doroshow relative to contracting, etc.