Validation of New Enabling Technologies to Accelerate Cancer Research

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Cancer Moonshot Implementation Team

Overall Goals of the Cancer Moonshot

- Accelerate progress in cancer, including prevention & screening from cutting edge research to wider uptake of standard of care

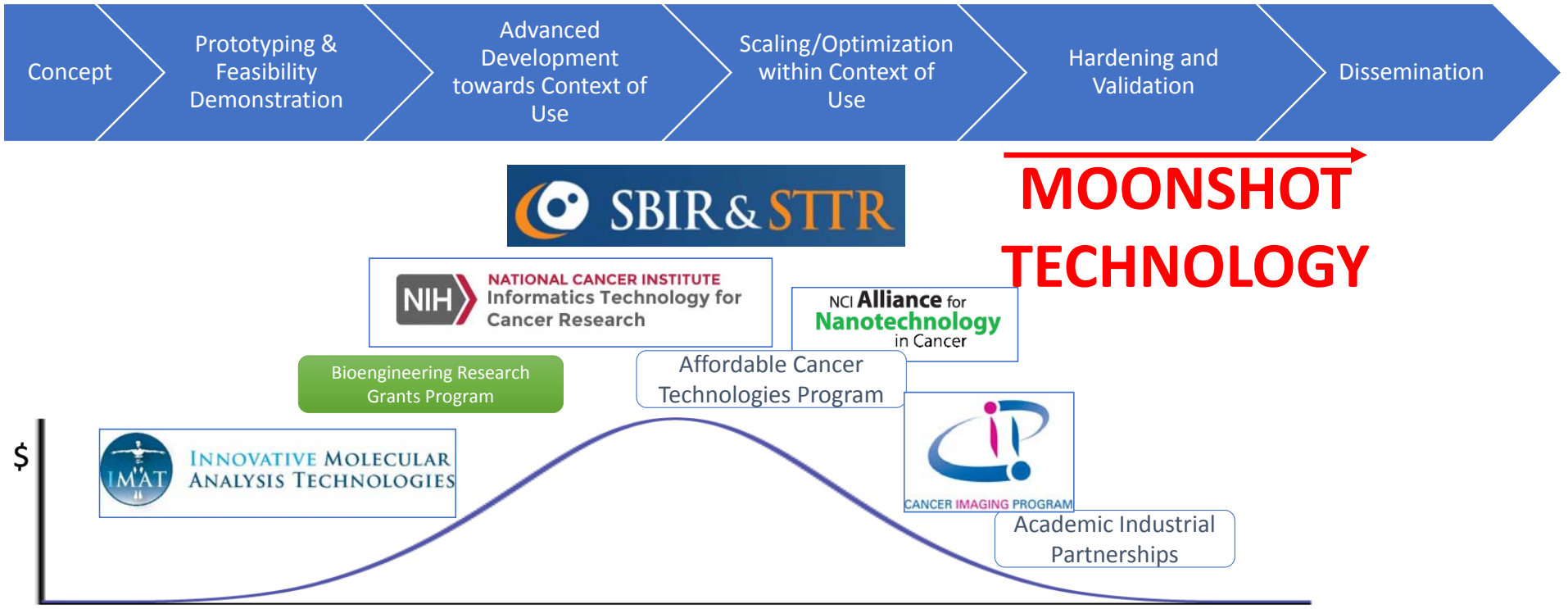
- Encourage greater cooperation and collaboration within and between academia, government, and private sector

- Enhance data sharing

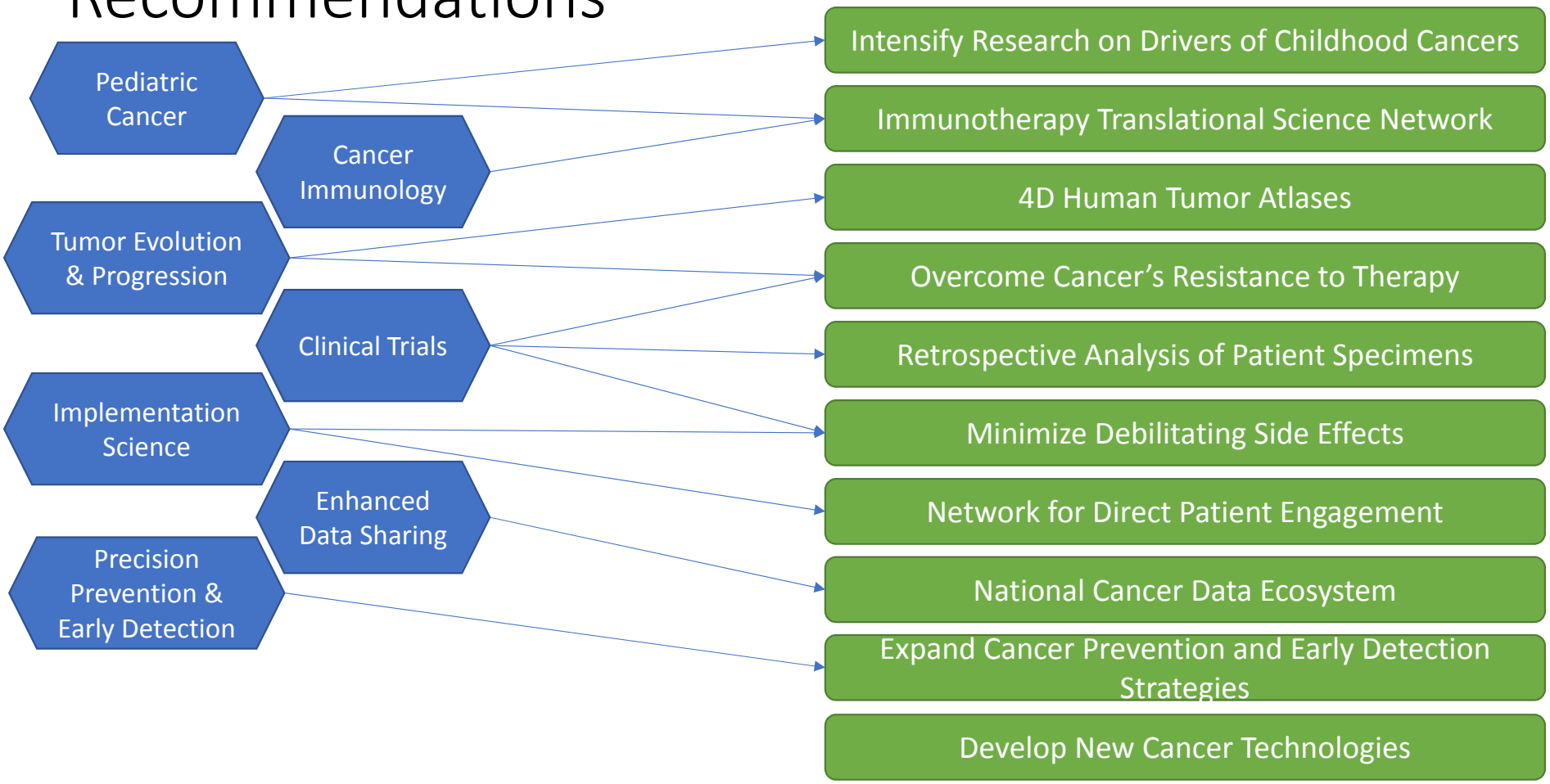
Motivation for the Concept

- Address the needs for technology development highlighted by Blue Ribbon Panel
- Technology development pipeline is lacking later-stage validation
- Accelerate the convergence of emerging complementary technologies

NCI Support for Technology Development



Blue Ribbon Panel Recommendations



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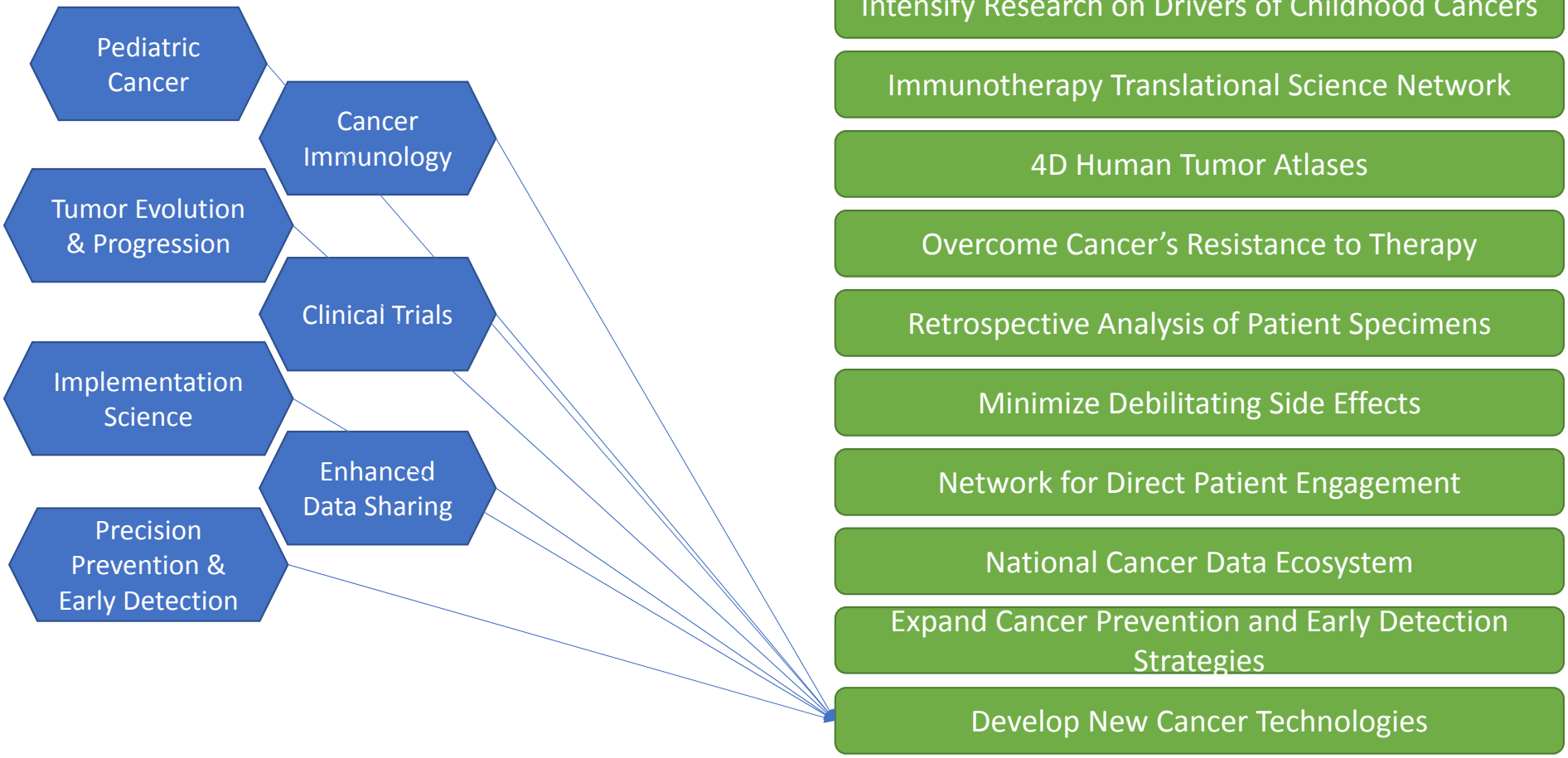

Priority Areas for this RFA

- *Priority Area A: Enhanced experimental and analytical capabilities addressing complexities of cancer development.*

- *Priority Area B: New capabilities for advancing precise clinical diagnosis of cancer patients*

- *Priority Area C: Novel predictive ex vivo and/or in silico modeling approaches to accelerate cancer research and development of personalized therapies.*

- *Priority Area D: New technologies/approaches to improve biospecimen and data quality.*

Priority A: ***Enhanced experimental and analytical capabilities addressing complexities of cancer development***

- New techniques to identify and analyze the complex network of genes, proteins, inflammatory/immune signatures, and biochemical pathways regulating different cell types within tumors and their immediate microenvironment.

- Examples:
  - High-resolution tumor characterization including single cell, imaging, and multi-plexed molecular methods.
  - Systematic perturbation (e.g., genome editing) along with advanced computational and predictive modeling approaches to reveal new opportunities for studies of cancer development and progression.

Priority Area B: ***New capabilities for advancing precise clinical diagnosis of cancer patients***

- Next-Generation diagnostics enabling more sophisticated biomarker signature identification (e.g. through integration of molecular assays, images and clinical records)

- Examples:
  - enhanced tracking of multiple cellular and/or molecular features;
  - monitoring interactions across length scales and population scales;
  - analysis encompassing higher levels of complexity; and/or
  - diagnosis across different populations, including capabilities focused on addressing disparities in access to care.

Priority Area C: ***Novel predictive ex vivo and/or in silico modeling approaches to accelerate cancer research and development of personalized therapies***

- New predictive modeling approaches, including organoids and “tissue slice” platforms, with particular interest in those that account for immune invasion/infiltration.

- Examples:
  - the use of both patient-derived cultures and advanced computational methods that allow for evaluation of personalized therapeutic approaches within clinically relevant timeframes
  - new microscale "normal" tissues such as liver, heart and intestine for predictive toxicology.

Priority Area D: ***New technologies/approaches to improve biospecimen and data quality.***

- New methodologies and/or standards to assure sufficient integrity of data and/or quality of tested biospecimens to allow comparison across clinical and/or large-scale basic research studies (e.g. comparing and/or using data across federated databases).

- Examples:
  - development of standardized molecular panels for profiling patients
  - standardized data collection protocols, or data quality control standards or structures that enhance comparison and integration of data across different studies

Unique Aspects of this RFA

- Focus on “technical validation”
  - Applicants must define project-specific ***performance measures*** that substantiate technical validation for their technology or method, subject to peer review for confirmation
- Extensive “non-responsive” criteria to screen out poorly-aligned applications before IRG review
- Unique review criteria for Significance and Approach
- Requires discussion of “Path to Implementation Readiness”

Justification for RFA Mechanism

- Responsive to Congressional Mandate (21st Century Cures Act) allowing clear indication for use of dedicated funds

- Assure community of NCI commitment to supporting projects, especially given accelerated timeline

- Allow for review flexibility, including ability to engage responsiveness review and assembly of a special emphasis panel by NCI with unique review instructions.

Success Metrics

- Successful Development
  - The number of products and methods successfully developed, as determined by the number of projects that meet their proposed performance measures.
  - The number of publications demonstrating progress towards proposed aims.
  - The number of patents and licenses applied for and secured by the supported institutions for the successfully developed new technologies.
- Evidence of adoption
  - The number of products and methods that are adopted by research groups in clinical settings or for other public health applications not directly associated with the project team that developed the products and methods.
  - Incorporation of technology platforms into ongoing research initiatives (especially NCI-supported efforts) and relevant dissemination initiatives (e.g. I-Corps).

Summary of Request

- Award details:
  - Activity: R33
  - Length: Up to 3 years
  - Budget: ≤$325,000/year direct cost cap

- Program Budget
  - Estimate 10 awards (no designated set aside per priority area)
  - Total cost: Estimate $5,000,000 per year for 3 years.