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Crucial Collaborations: Key for Success of Pediatric Oncology Clinic Trials

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Why Collaborations are Essential for Pediatric Oncology?

- Small patient population
- Based on biologic stratification the populations are getting smaller and even “ultra-rare” especially if biomarker selected eligibility criteria
- Complimentary science: correlative biology
- Maximize efficiency in trial design/conduct
- Leveraging financial resources: pharmaceutical, federal, philanthropic

Types of Trial Collaborations

NCI-Sponsored Trials	Hybrid Trials (Investigator Initiated Trials)	Industry-Sponsored Trials
• NCI & Drug Company Contract Only (CRADA)* • Standard NCI Support Only for a cooperative group trial (eg COG, PEP-CTN, Alliance etc) • PI is appointed by cooperative group and cooperative group writes the trial • NCI CTEP holds the IND	• NCI Trial with cooperative group & Drug Company Contract • Institutional/Consortia & Drug Company contract with additional philanthropic/institutional funding • Purely Academic trial with grant/institutional/philanthropic funding • PI/consortia propose the study and write the trial • Individual/Institution, consortia or company holds the IND	• Fully Sponsored by Industry • COG & Drug Company Contract, No NCI Involvement • Institutional/Consortia & Drug Company contract • Company writes the trial • Company holds the IND

- Cooperative Research and Development Agreement, https://ctep.cancer.gov/protocolDevelopment/agents_drugs.htm

Advantages of the Types of Trial Collaborations

NCI-Sponsored Trials	Hybrid Trials (Investigator Initiated Trials)	Industry-Sponsored Trials
• NCI & Drug Company Contract Only • NCI Central IRB • Use of templated protocols/consents • Minimum requirement from Industry is drug supply so least cost to industry	• Most flexible with significant control of trial design by PI • NCI Trial with cooperative group & Drug Company Contract: multi-site single contract • NCI Central IRB if using NCI resources • Potential for funding of correlative studies	• Most feasible for international study • Most financial support for site(s) • Cooperative group & Drug Company Contract, No NCI Involvement: multi-site single contract • Potential for shorter timelines

Dis-Advantages of the Types of Trial Collaborations

NCI-Sponsored Trials	Hybrid Trials (Investigator Initiated Trials)	Industry-Sponsored Trials
• Direct communication between NCI & company • Multi-step review process • Limited funding to sites and no funding for correlative studies	• If NCI collaboration still multi-step review process • Funding may be insufficient from a single source: requires multiple agreements/contracts	• Variable ability of PI to influence trial design and conduct • Largest 'burden' on site(s) eg most data collection • Limited access due to site selection

Examples

Industry and Cooperative Group: Osteosarcoma Maintenance Therapy with OST131-164 (OST-164-01) – Primary Results

Dr. Damon Reed, Memorial Sloan Kettering Cancer Center

Industry and Academia: Targeting TGF-beta Signaling in the Tumor Microenvironment as an Effective Therapy in Osteosarcoma

Dr. Kristen VanHeyst, University Hospitals/Rainbow Babies and Children's Hospital

Investigator Initiated and Academia: RNA PRIME – Harnessing Tumor RNA for Immunotherapy Against Osteosarcoma

Dr. John Ligon, University of Florida

Discussion