SBIR Phase IIB Bridge Award

Presented to

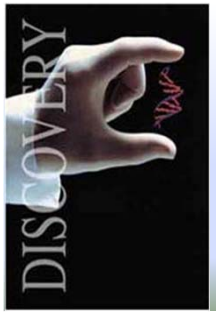
National Cancer Advisory Board

Presented by

Andrew J. Kurtz, PhD

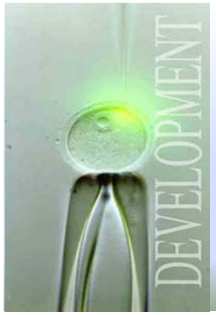
September 13, 2011

SBIR & STTR: Three-Phases



PHASE I – R41, R43

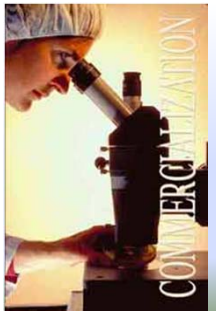
- **Feasibility Study**
- **$150K and 6-month (SBIR) ***
- ***or* 12-month (STTR) Award**



PHASE II – R42, R44

- **Full Research/R&D**
- **$1M and 2-year Award (SBIR & STTR) ***
- **Commercialization plan required**

Phase IIB Bridge Award



PHASE III

- **Commercialization Stage**
- **Use of non-SBIR/STTR Funds**

* Note: Actual funding levels may differ by topic.

SBIR Phase IIB Bridge Award

Competing Renewal Program for SBIR Phase II Awards

- Provides additional NIH funding to extend selected projects
- Involves another peer-review cycle to evaluate progress & future plans
- Accelerates commercialization by incentivizing partnerships with third-party investors & strategic partners *earlier in the development process*

How do we accomplish this goal?

- NCI gives competitive preference and funding priority to applicants that can raise substantial third-party funds (i.e., ≥ 1:1 match)

  "…Applicants are expected to leverage their previous NIH SBIR support, as well as the opportunity to compete for additional NCI funding under this [funding announcement], to negotiate and attract third-party financing needed to advance a product or technology toward commercialization…"

Mutually Beneficial Program

What does the NCI get?

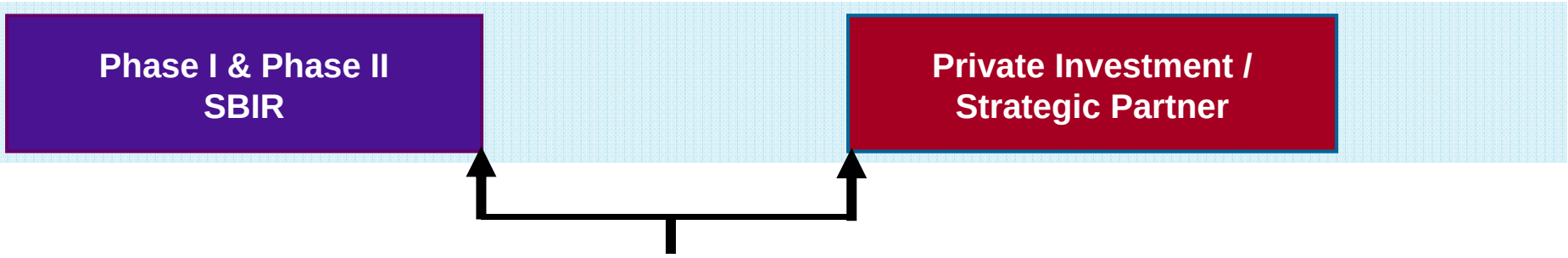
- Opportunity to leverage millions of dollars in external resources
- Valuable input from third-party investors:
  1. Rigorous commercialization due diligence prior to award
  2. Commercialization guidance during the award
  3. Additional financing beyond the Bridge Award project period

What do the third-party investors get?

- Opportunity to partner with small businesses to develop & commercialize:
  1. Technologies that have been vetted by the NIH peer-review process, **AND**
  2. Projects for which a substantial amount of proof-of-concept data already exists

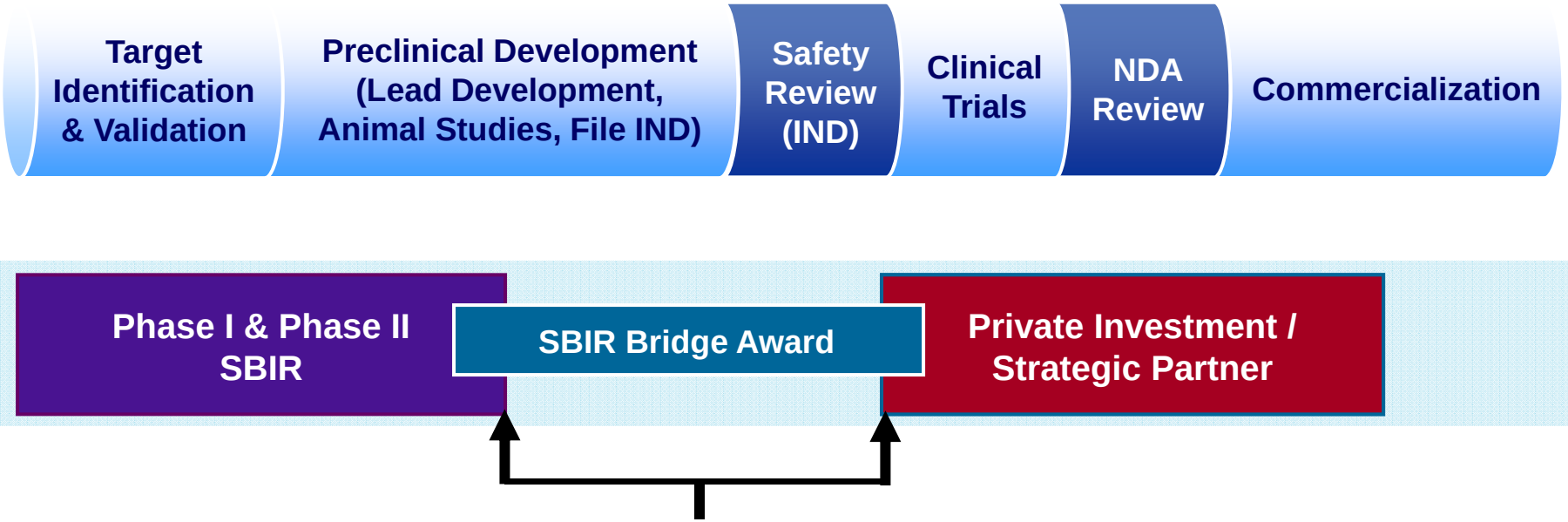
➢ **Opportunity to share in the early-stage investment risk with the NCI**

EXAMPLE: Drug Development



The "Valley of Death" is the problem

EXAMPLE: Drug Development



SBIR Bridge Award is designed to bridge the "Valley of Death"

Up to $1 M per year for 3 years ($3 M total) from the NCI

EXAMPLE: Drug Development

Competitive applicants for the SBIR Bridge Award are expected to secure additional funding from Third-Party Investors to share in the investment risk

Technical Scope

SBIR & STTR

Cancer Therapeutics (FY09)

- Small molecule anticancer agents
- Anticancer biologics, including therapeutic vaccines
- Multifunctional cancer[illegible]hnology
- Anticancer drug deliv[illegible]

Opportunity to impact >75% of the Phase II projects in NCI's SBIR portfolio

Cancer Imaging Techn[illegible] & *In Vivo* Diagnostics (FY09)

- Medical devices for i[illegible]guided interventions
- Radiation therapy de[illegible]es
- Imaging agents, inclu[illegible]als
- Devices and technol[illegible]s

In Vitro and *Ex Vivo* Cancer Diagnostics and Prognostics (New in FY10)

- Molecular diagnostics and prognostics, including *in vitro* diagnostic multivariate index assays (IVDMIA)
- Image analysis tools for diagnosis
- Spectroscopic techniques for *in vivo* and *ex vivo* tissue analysis

Program Funding Solicitation

Eligibility

- Current Phase II awards & and those that ended within the last 2 years
- Cancer-related Phase II projects initially funded by other NIH institutes

- **Special Review to Evaluate Technical and Commercial Merits**
  - Reviewers are academics, clinicians, industry professionals, venture capitalists
  - Emphasizes important commercialization considerations such as intellectual property (e.g., patents) and strategy for gaining FDA approval
  - Requires complete disclosure of applicant's SBIR commercialization history

➢ **Third-Party Fundraising plan**

- **Preferred Types of Funds:** Cash, liquid assets, convertible debt
- **Sources of Funds:** Another company, venture capital firm, individual "angel" investor, foundation, university, state or local government, or any combination

"Valley of Death"

SBIR & STTR

How are we sure that they need more money from the NCI?

Applicants must provide a concise "Statement of Need" that includes answers to the following questions:

- What is the perceived "Valley of Death" for the product/technology?
- Why is additional government funding critically needed to accelerate the development of the product or technology toward commercialization?
- What activities are being proposed that would not otherwise be possible through independent third-party investments OR would be significantly delayed without additional NIH support?
- To what extent would a possible award advance the product or technology far enough to attract sufficient, independent third-party financing and/or strategic partnerships to carry out full commercialization?

Success Rates

71 unique applications received to date (17 were resubmitted)

10 projects funded to date

Overall success rate: **14%**

		Applications Received					
RFA #	**FY**	**Date**	**Therapeutics**	**Imaging**	**Diagnostics/ Prognostics**	**Total**	**Funded**
CA08-021	2009	Sep 2008	11	12	0	24	→ 2
		Feb 2009	9	10	0	19	→ 4
CA10-009	2010	Mar 2010	8	10	8	26	→ 4
CA11-002	2011	Apr 2011	5	7	7	19	→ 2*

** Pending Award*

Ten Bridge Awards: FY09/FY10

SBIR & STTR

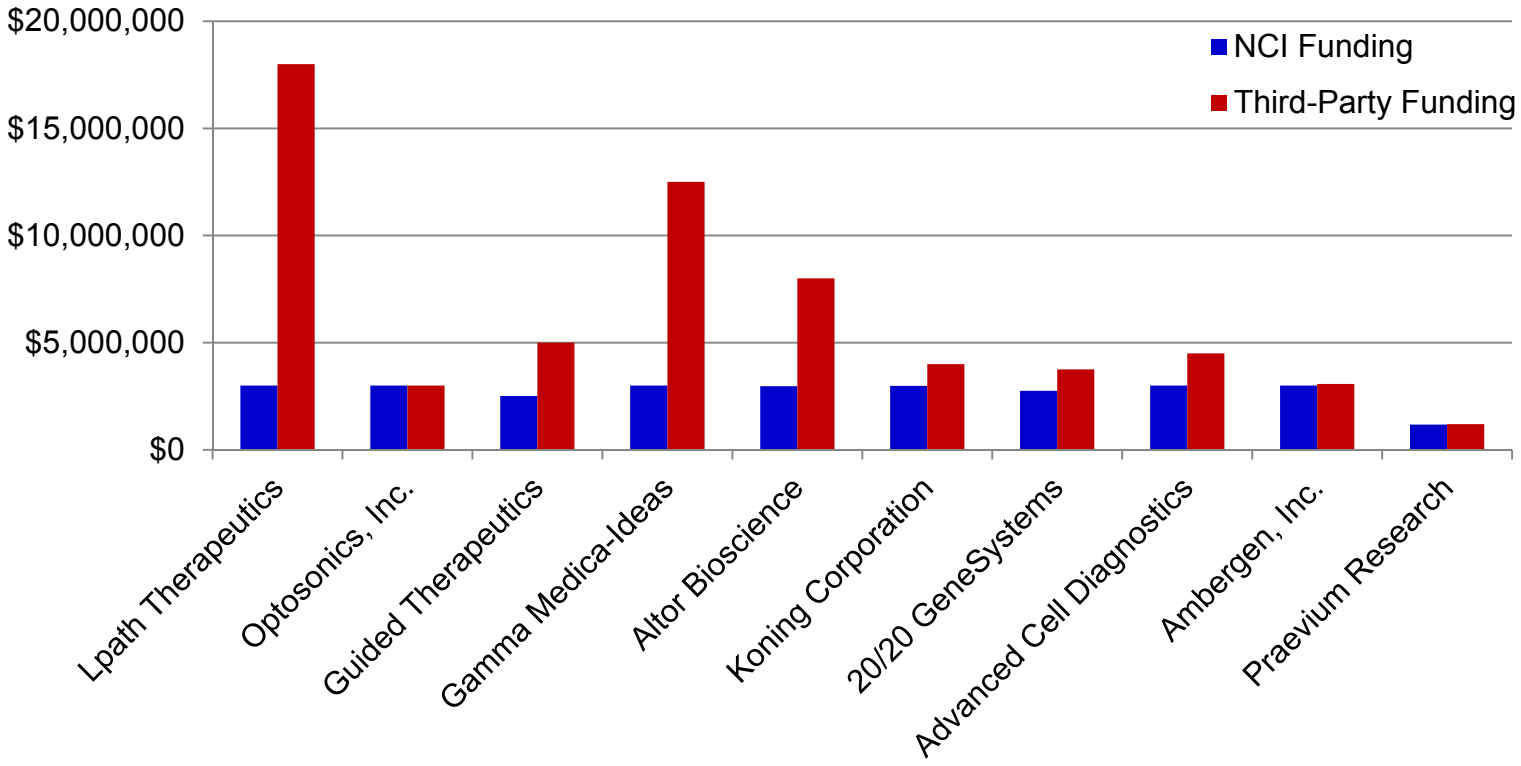
FY	Company	Technology/Product	Award Size
2009	**Lpath Therapeutics**	Humanized monoclonal antibody for treatment of prostate cancer	$3,000,000
2009	**Optosonics**	Photoacoustic CT for preclinical molecular imaging	$2,997,247
2009	**Guided Therapeutics**	Fluorescence/reflectance spectroscopy for detection of cervical cancer	$2,517,125
2009	**Koning Corporation**	High-performance breast CT as diagnostic adjunct to mammography	$2,986,453
2009	**Gamma Medica-Ideas**	Molecular imaging to detect metabolic activity of breast lesions	$3,000,000
2009	**Altor BioScience**	Tumor-targeted immunotherapy for treatment of p53-positive cancers	$2,969,291
2010	**20/20 GeneSystems**	mTOR companion diagnostic assay	$2,750,000
2010	**Advanced Cell Diagnostics**	*In situ* RNA detection assay for analyzing circulating tumor cells	$2,996,450
2010	**Ambergen**	Expression-based prognostic assay for recurrence of colorectal cancer	$2,998,830
2010	**Praevium Research**	High-performance imaging engine for optical coherence tomography	$1,180,420

↓

2 therapeutics
5 imaging technologies
3 diagnostics

NCI Total	**$27,395,816**
Third-Party Investments	**$62,950,000**
Leverage	**> 2 to 1**

Ten Bridge Awards: FY09 – FY10



Venture Capital: ~1/3

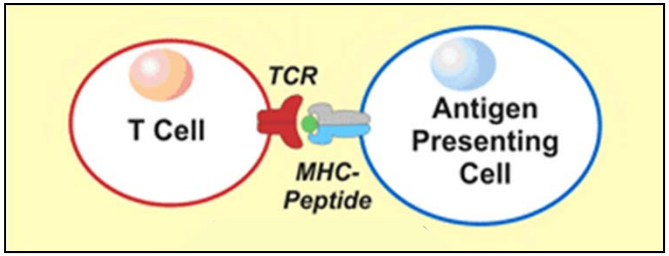
Strategic Partners: ~1/3

Individuals & Other: ~1/3

2009 Bridge Awardee

SBIR & STTR

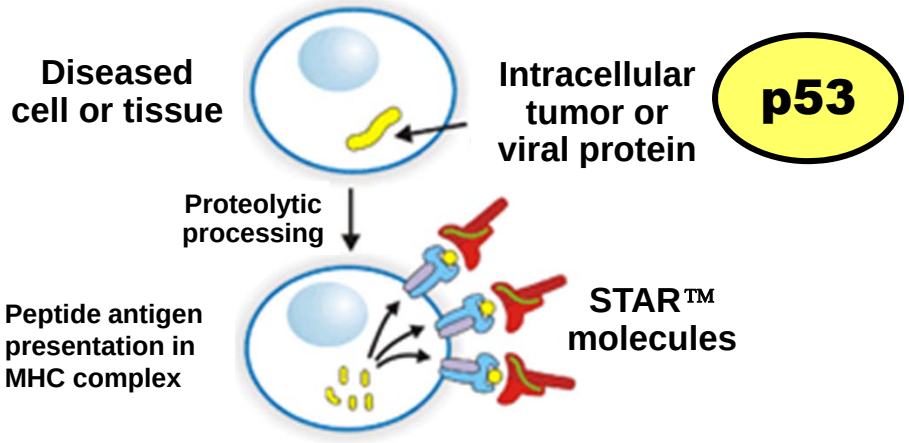
Altor BioScience
Miramar, FL



Major histocompatibility complex (MHC)

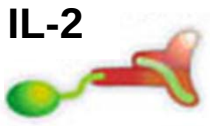
T-cell receptor (TCR)

STAR ™ ≡ Soluble T-cell Antigen Receptor

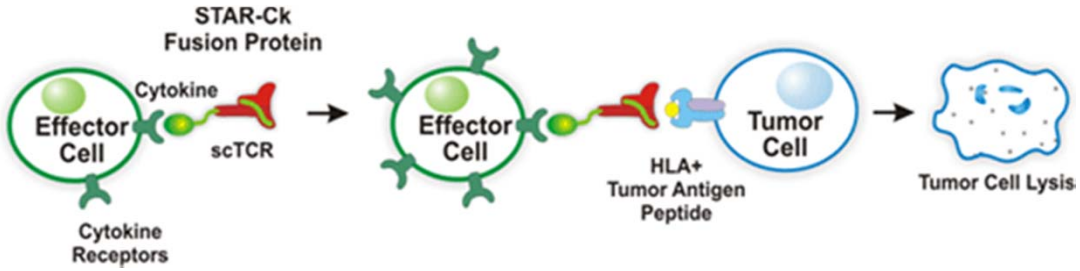


STAR™ molecules target disease-specific antigens

Drug ≡ IL-2



(ALT-801)



Altor Bioscience (cont'd)

SBIR Phase I & Phase II

- ALT-801 inhibits growth or causes regression of human-derived cancer cells when grown in animal models
- ALT-801 shows significantly better antitumor activity than IL-2 alone

- ALT-801 was advanced as a clinical candidate and evaluated in a Phase I clinical study **(ClinicalTrials.gov: NCT01029873)**
  - Treated 26 patients with progressive metastatic p53-positive malignancies
  - Primary endpoints: Safety, maximum tolerated dose (MTD), pharmacokinetics
  - Secondary endpoints: Immunogenicity and antitumor response

➢ ***ALT-801 exhibited favorable safety and PK profiles at the MTD level***

Altor Bioscience (cont'd)

SBIR & STTR

$3.0 million Phase II Bridge Award

- Further assessment of the anti-tumor activities of ALT-801 for advanced/metastatic melanoma, renal cell carcinoma, head and neck adenocarcinoma, and prostate cancer
- New therapeutic regimen has been developed for a clinical study in patients with metastatic melanoma

  (ClinicalTrials.gov: NCT01029873)

  - Eight clinical sites in the U.S. have been initiated and are screening patients for enrollment in this study
  - Results of the dose escalation phase will be used to establish ALT-801 plus cisplatin treatment regimens in Phase II clinical studies for other indications

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SANDERLING VENTURES

Third-Party Investment: $8,000,000

- In July 2008, Altor signed a term sheet to raise a total of $8.0M in a financing round led by Sanderling Ventures
- Bridge fundraising is complete, and additional funds have been raised beyond the original commitment

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Milestone-Based Awards

SBIR & STTR

Ability to raise matching funds is a component of the Phase II Bridge Award