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Office of Technology Development

# Biomedical Accelerator Fund

Office of Technology Development
Harvard University

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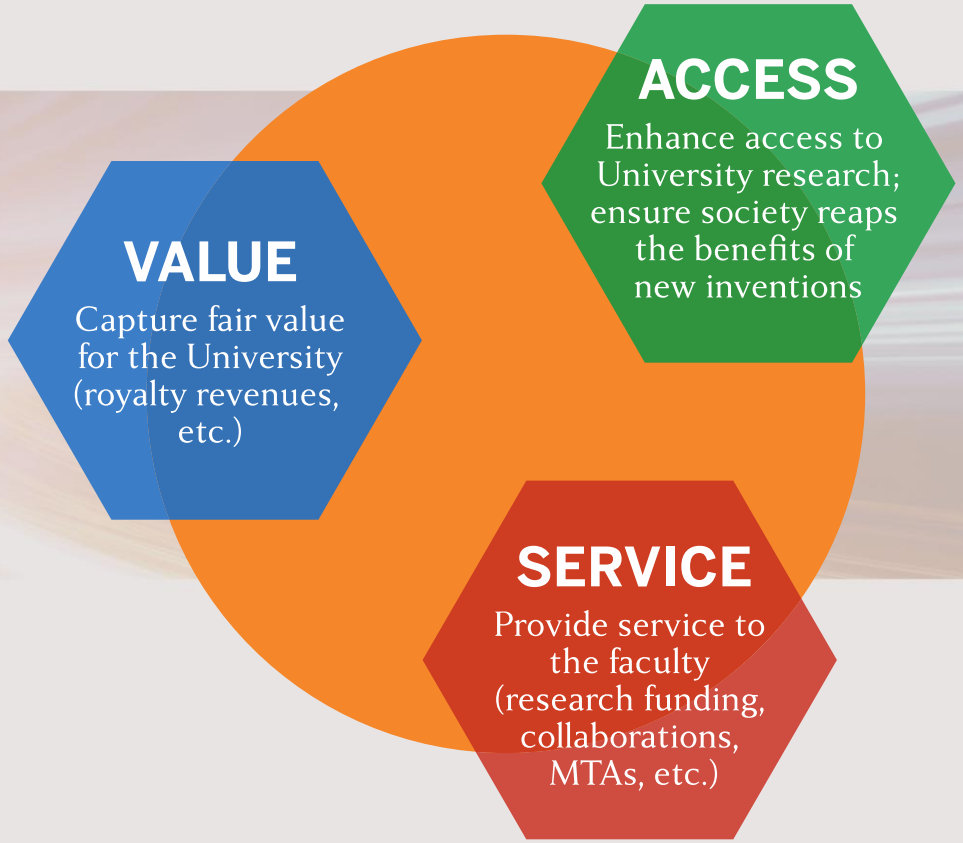
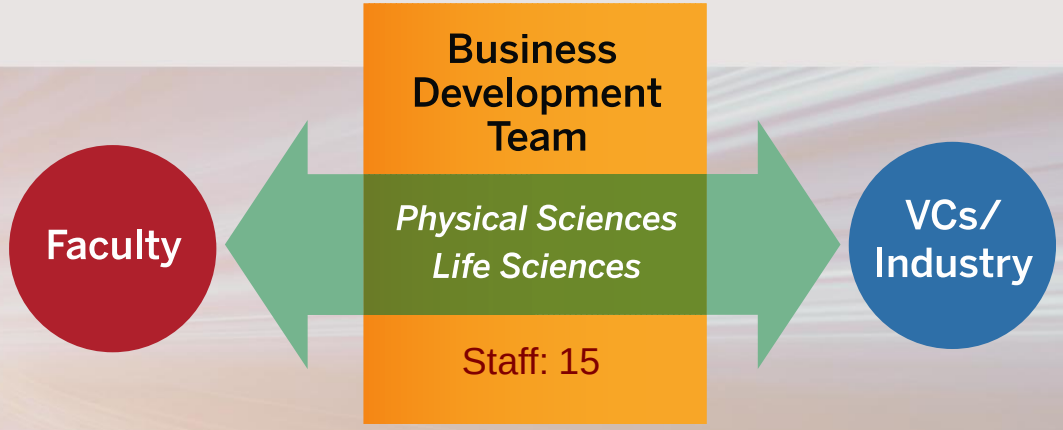
# Mission of Harvard's Office



# Organizational Structure of Harvard OTD




**Technology Transactions Team**
Staff: 7

**Finance and Administration Teams**
Staff: 9

**Intellectual Property Team**
Staff: 7

**Alliance Management Team**
Staff: 3

Total Staff: 41

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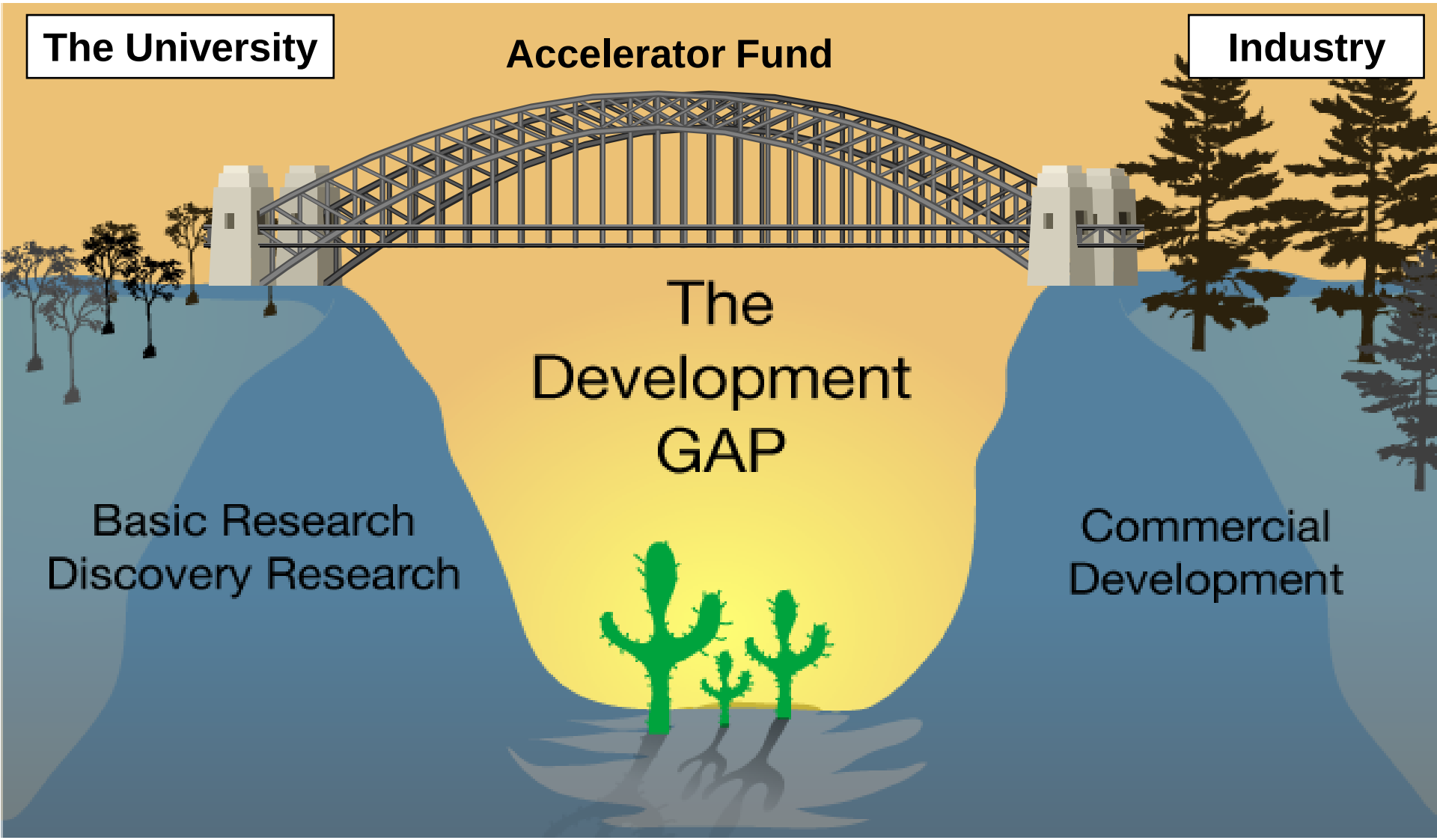
# Bridging the Development Gap



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# Engines of Innovation

**Problem insufficient innovation in biomedicine by pharma and biotech**

- Biotech and pharma are failing to generate truly novel approaches to treating disease
  - New targets for challenging diseases
  - Fundamentally new therapeutic modalities and strategies
  - Integration of new technologies (e.g. diagnostics and personalized medicine) with therapeutics
- This problem has been exacerbated by decreases in venture capital funding for activities at the "development gap" stage

**Solution university-based research → basic science, also translational**

- The culture of innovation at universities positions them perfectly as engines of fundamental innovation in biomedicine
  - Drug discovery
  - New approaches to translational medicine
- However, universities need increased funding, in particular "gap funds," to fully exploit this opportunity

***"The seeds for the future exploitation of scientific advances for drug discovery already have been planted and the existing culture in academic settings is perfect for this type of work, although the funding will have to be expanded significantly."*** **From A New Model for Distributed Partnering in the 21st Century, Kauffman Foundation, January 2010**

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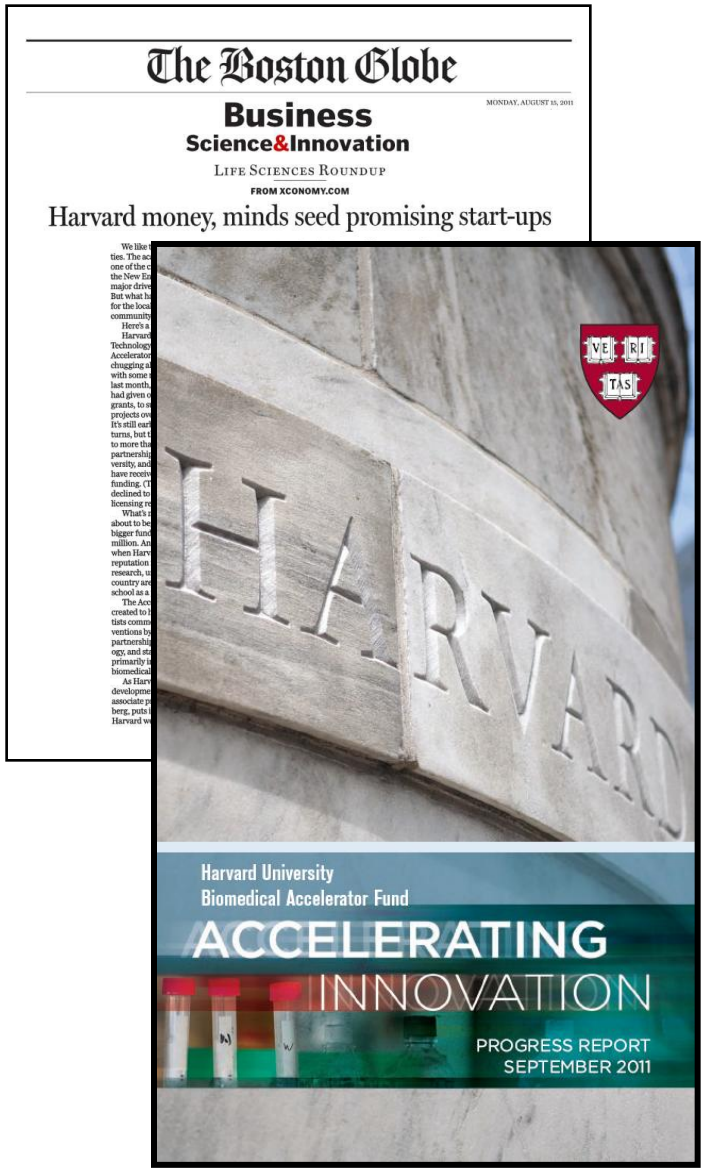
# Accelerator Fund Overview




- Philanthropic fund launched in 2007
- $10 million initial fund
- Focused on Harvard's public service mission while also increasing the value of Harvard's IP assets
- Dedicated to life sciences and biomedical technologies (primarily therapeutics)
- Key success factors include:
  - Top-tier advisory board of industry leaders
  - Dedicated Chief Scientific Officer
  - Technologies with clear roadmap for R&D and commercialization
  - Completely integrated with Harvard's business development activities
- Structured as an evergreen fund
- Promoting an ethos of entrepreneurship at Harvard, translational medicine mindset

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# Project Selection

- The technology must address an important societal need
- Strong indications of commercial interest at the outset
- Realistic project plan for which a few hundred thousand dollars can make a significant impact (near a “value inflection point”)
- Patentable technology with freedom-to-operate
- Engaged, committed principal investigator

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# R&D Plans – Types of Studies

Accelerator projects tackle key questions that currently stand in the way of commitment from outside partners

- Demonstrate the chemical tractability of a novel drug target
  → fund high-throughput screens of compound libraries
- Proof-of-concept testing for a target and/or a ligand to that target
  → fund testing in *in vivo* disease models
- Determine suitability of a particular chemical scaffold for significant medchem efforts
  → fund analog synthesis / SAR studies
- Differentiate or benchmark technology vs. existing modalities

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# Driving Partnerships

- Over 200 technologies have been evaluated in 6 funding cycles
- 33 projects have been funded from around Harvard University
- Of 25 completed projects, nearly half have been partnered
  - Four start-up companies
  - Many exclusive licenses with existing pharmaceutical or biotech companies
  - Co-development partnerships that include an option to Harvard background IP generated during Accelerator projects
  - Accelerator projects have generated more than $14 million of new industry-sponsored research funding for Harvard
  - There is the potential for significant milestone payments and future royalties as products are developed and reach the marketplace

## Examples of successful exits for Accelerator projects

- Start-up formation
- Biotech license
- Co-development deal with pharma

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# Start-up Company

Background prior to Accelerator funding

- Technology for fluorinating complex molecules under mild conditions
  → potential applications in pharmaceuticals and PET diagnostic imaging

Accelerator project

- Further development of the core technology (make the process catalytic, more suitable for industrial applications)
- Synthesize commercially compelling examples and test them *in vivo*
- Significant investment in patent applications related to methods and compositions of fluorinated molecules

Outcome

- Start-up formed based on “platform company” potential

SciFluor Life Sciences
an allied minds company

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# Biotech License

Background prior to Accelerator funding

- Possible drug target for enhancing the degradation of toxic proteins

  → potential pharmaceutical applications in many diseases, including Alzheimers, Parkinsons and other neurodegenerative diseases

Accelerator project

- Optimization of preliminary "hit" compounds: improve their potency, selectivity and drug-like properties, generate back-up series
- Validate compounds in cell-based models of disease, in vivo testing

Outcome

- License to well-funded Boston biotech company with relevant scientific perspective and clinical expertise
- Very favorable deal for the Harvard inventors

PROTEOSTASIS THERAPEUTICS
Restoring Protein Homeostasis to Ameliorate Disease

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# Pharma Co-development

Background prior to Accelerator funding

- Inhibitors of cellular autophagy: screening hits from phenotypic assay
  → potential pharmaceutical applications in cancer, also as antivirals

Accelerator project

- Optimization of "hit" compounds: improve potency, selectivity and drug-like properties (especially solubility and metabolic stability)
- Identify the direct molecular target (Cell publication)

Outcome

- Further compound optimization and de-risking needed before licensing
- Entered into three-way partnership with Roche (China) and BioBay
- $millions committed for optimization and more biology work at Harvard
- Harvard owns IP, option granted

bioBAY

Roche

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# Looking Ahead – A New Fund

Building on the successes of the original Accelerator, but with expanded scope and strategy

- More significant investments in Harvard's therapeutics programs with greatest commercial potential (~$1-2 million over multiple years)
- Areas of biomedical technology that were not fully addressed by the original Accelerator Fund (e.g. diagnostics)
- Deepening of the relationship with Harvard Business School
- Goal of achieving evergreen status by 2020
- Ensures that Harvard remains at the forefront of technology transfer and carry out its academic and research missions

# Looking Ahead – A New Fund

BACK-UP

# Developing Harvard's IP Portfolio

Develop


- ~250 active principal investigators
- 2,100+ worldwide patents pending
- 1,525+ issued patents

# Value creation using CROs

## Why do we use CROs?

Goal: advance our technologies to later stages of development

- increase the number of "partnership-ready" technologies
- increase the value of our technologies

Access drug development capabilities not found at the university

- Medicinal chemistry, DMPK, industry-standard efficacy models, etc.

Leverage our modest project budgets via risk-sharing partnerships with CROs

# Creative models for working with CROs

"Traditional" arrangements

- FTE-based, fee-for-service, etc.
- Majority of Harvard's projects are still done this way, although at "discounted" academic rates
- Wide range of costs

Risk-sharing options

- Deferred payment (FTE-based)
- Milestone-based
- Co-development

# Example 1: deferred payment of FTE costs

- CRO activity: synthesis of analogs
- 3 FTEs for 6 months
- Accelerator pays 50% of FTE rate
- CRO partner "invests" 50% of FTE rate
- Upon licensing, Harvard shares revenues with CRO partner up to 2X their investment

# Example 2: milestone-based

- CRO activity: synthesis of analogs, *in vitro* ADME, *in vivo* (PK, efficacy)
- $200K budget at CRO, also money for bioassays at university
  - $50K paid over first 6 months (FTE-based)
  - Remaining $150K paid upon achievement of technical milestones
    - $50K *in vitro* milestone – generation of a novel (IP), optimized compound with specific potency and selectivity requirement
    - $50K first *in vivo* milestone – achievement of a compound with a particular PK profile (exposure, route of admin)
    - $50K second *in vivo* milestone – successfully run efficacy model, with PD component, "positive result" not required
- Additional revenue sharing up to $100K upon licensing

# Example 3: co-development

- CRO activity: full set of activities up to IND candidate
- Significant commitment of resources (double digit FTEs for multiple years)
- Harvard PI continues basic research on mechanism etc.
- Really functioning as a biotech partner rather than CRO
  - Many companies that were historically CROs now starting to do things like this…
- Downstream sharing includes royalties etc.

# Universities and CROs

Are there different considerations for universities (vs. companies) working with CROs?

- Generally smaller projects, focus on near-term objectives?
- In the absence of technical consultants, higher premium on CROs that provide services such project management and design?
- Preference for integrated CROs?
- CROs that are sensitive to difference of academic culture