Business Concepts for Life Scientists: Strategy

Deb Dauber, PhD, MPH
Healthcare Consultant
UCSF Alum

Sandy Waugh-Ruggles, PhD
Founder and Innovator
UCSF Alum

Content Contributors
Michael Penn, MD, PhD
Anatol Kreitzer, PhD
Kinkead Reiling, PhD
Brad Grueter, PhD
Calli Merkel, MBA, PhD
Business Concepts for Basic Scientists

Strategy

Finance

Development
Strategy course objectives

By the end of class you will be able to:

1. Define strategy and how scientific enterprises use strategy
2. Identify the value proposition of a scientific enterprise
3. Determine key stakeholders for a scientific enterprise
4. Discuss how organizational context impacts budget and resource allocation
Defining strategy

- **Strategy is how a scientific enterprise plans to conduct activities in order to achieve a set of overarching goals**

- The purpose - to act as a road map that guides decisions and establishes boundaries on the scope of work

<table>
<thead>
<tr>
<th>In biotechnology…</th>
<th>How the company will achieve clinical vision and corporate growth targets</th>
</tr>
</thead>
<tbody>
<tr>
<td>In academia…</td>
<td>How the lab will decide which research questions and grants to pursue</td>
</tr>
</tbody>
</table>
Value Proposition

The enterprise’s technology and market context

Organizational Context

Capabilities, structure and funding of the enterprise

Strategy
Value Proposition

The enterprise’s technology and market context

Organizational Context

Capabilities, structure and funding of the enterprise

Strategy
The Value Proposition addresses the value of the technology to key stakeholders

Value Proposition

- WHY is the work important?
- WHAT is unique about the technology or work product?
- WHO has a stake in the outcomes of the work and what do they value?
**WHY** is the work important?

- **Market Need**
  - Meet an unmet need
  - Build a better mousetrap
Akashi therapeutics: a company focused on a specific market need

Company Overview

Akashi Therapeutics is a clinical stage biopharmaceutical company. Our mission is to develop treatments for Duchenne muscular dystrophy and other rare pediatric diseases. Akashi was founded by leading patient organizations and biotechnology industry veterans and is managed by a seasoned team of drug development experts. Akashi is developing a cocktail of medications with the goal of transforming Duchenne from a 100% fatal, aggressive muscle-wasting disease to a chronic, manageable condition.

WHY?
WHAT is unique about the technology?

Technology Attributes

- Efficacy
- Potential for portfolio growth
- Competitive technology
- Barriers to entry for competition
Catalyst Biosciences: Founded based on a unique scientific platform

**Natural Protease** → **Catalyst Proteases**

**Natural Target** → **Disease Targets**

**WHAT?**
The path to a robust strategy

Need-based

WHAT?

Technology-based

WHY?
The path to a robust strategy

Need-based

WHAT? → WHY?

Technology-based

WHAT? → WHY? → WHAT?
Two different paths: both incorporate the WHAT and the WHY

1. Expertise in Duchenne Muscular Dystrophy (DMD)
2. Identify and acquire rights to promising compounds
3. Develop drugs for DMD

1. Expertise in protease biochemistry and selection
2. Identify a disease-specific target of interest
3. Create & test novel proteases as treatments for that disease
Understanding key stakeholders is fundamental to creating value

Need-based

WHAT?

WHY?

Technology-based

WHAT?

WHY?

WHO?
Academic Stakeholders

FUNDERS
- Granting agencies
- Corporations

RESEARCH PEERS

EMPLOYEES
- Students
- Post-docs
- Lab managers

COMMUNICATIONS
- Publishers
- Conference organizers

EMPLOYERS
- Department chairs
- University administration

EXTERNAL

INTERNAL
Pharma Industry Stakeholders

CUSTOMERS
• Providers (Physician or hospital)
• Patients
• Payers (Insurer, CMS)

INVESTORS

REGULATORS

VENDORS

INFLUENCERS
• Caregivers
• Patient advocacy groups

MANUFACTURERS

EXTERNAL

INTERNAL

BOARD OF DIRECTORS

EMPLOYEES
Value proposition must take into account differing stakeholder values

Patients
- Drug safety & efficacy
- Drug convenience
- Drug cost

Investors
- Revenues
- Current portfolio
- Long-term sustainable growth

Employees
- Mission and portfolio
- Culture
- Long-term sustainability
Re-cap: Important elements to a value proposition

Value Proposition

• WHY is the work important?
• WHAT is unique about the technology or work product?
• WHO has a stake in the outcomes of the work and what do they value?
Advanced topics

Much work is done in support of the development of the value proposition, including:

- Competitive analysis
- Market research
- Stakeholder or customer mapping
- SWOT analysis

A scientific enterprise’s value proposition drives articulation of:

- Mission and vision
- Product or brand positioning
- Messaging
Parallels with Academia: Q&A with Anatol

- Would you consider your lab to be primarily need-based or technology-based?

- Tell us about how you think about the Why’s and the What’s of your lab’s research program.

- Who are the stakeholders that you think about the most? Can you give an example of how you might address stakeholders differently?
Value Proposition

The enterprise’s technology and market context

Organizational Context

Capabilities, structure and funding of the enterprise

Strategy
The Organizational Context defines the situational confines of the enterprise

**Organizational Context**

- Where in the development cycle is the enterprise?
- What is the structure of the enterprise?
- What resources are available to achieve the objectives?
The developmental stage of the enterprise shapes the strategy

Product development follows a cycle from concept to the clinic to market, and the enterprise short and long term strategy will vary based on the number of products underway, and their place in the product development cycle.
The organizational structure shapes the direction of growth.

- Fully integrated therapeutics company
- Contract Research Organization
- Virtual Drug Development
- Clinical Development Partnership
The resources available to the enterprise affect the strategy

Funding and organizational capabilities constrain the actions of the enterprise and influence the strategy.

- Budget constrains near term actions and investment in growth.

Organizational capability is the capacity of the enterprise to reach the goals and objectives.

- Organizational capabilities effect the speed and type of work that can be accomplished by the enterprise.
Recap: The Organizational Context defines the situational confines of the enterprise

Organizational Context

• Where in the development cycle is the enterprise?
• What is the structure of the enterprise?
• What resources are available to achieve the objectives?
**Value Proposition**
The enterprise’s technology and market context

**Organizational Context**
Capabilities, structure and funding of the enterprise
The strategy reflects both the long term and short term organizational goals.

**Long-term Strategy (5+ years):**
- Aspiration

**Mid-term Strategy (3-5 years):**
- Preparatory, Directional

**Short-term Strategy (1-2 years):**
- Tactical, Focused, Incremental

Annual goals and objectives
Catalyst in Year 1

VALUE PROPOSITION

Treat significant diseases with novel proteases against targets not amenable to antibody therapeutics.

1. Expertise in protease biochemistry and selection
2. Identify a disease-specific target of interest
3. Create & test novel proteases as treatments for that disease

ORGANIZATIONAL CONTEXT

1. Developmental Stage: Pre-clinical discovery / Basic research
2. Organizational structure: Fully integrated biotherapeutics company
3. Funding: $10.2 MM Series A financing
4. Organizational capability: 10 employees focused on protease biology, cell-based assays and establishing animal studies
Catalyst Biosciences Year 1 Strategy

Short-term Strategy (1-2 years): Through internal and partnered discovery research, advance compounds out of pre-clinical discovery.

Goals and Objectives:
- Show proof of concept in cell-based and animal models for one disease target
- Build out the pre-clinical development infrastructure and engineering process
- One pre-clinical partnership

Mid-term Strategy (3-5 years): Build a portfolio of products and partnerships at the clinical stage to prove the technology in patients.

Long-term Strategy (5+ years): Build to become a publicly-traded, fully integrated therapeutics company with direct, measurable patient impact.
Strategy has a circular nature

Large companies often follow an annual strategic planning cycle using strategic decisions to drive the goals and objectives for the coming year.
New leadership can drive a change in enterprise strategy

- 2007 – Founded, a “WHAT” company based on induced pluripotent stem cell (iPSC) technology
- 2011 – Patent protecting the utility of human iPSC technology; senior staff fired and shifts focus.
- 2012 – Back to drug discovery basics, pivot to a “WHY” company focused on developing novel therapies for neurodegenerative disease, specifically discovering monoclonal antibodies.
- 2013 – iPierian spins out True North Therapeutics- founded to develop orphan drug indications.
- 2014 – BMS buys Ipirian for $175 MM down and up to $550 MM in milestone payments. Nancy Stagliano continues at the helm at True North.
Advanced Topics: Developing Strategy Requires

Analysis of the following areas:

• Competitive landscape
• Market opportunity assessment
• Market segmentation

Use of the following skills sets:

• Budgeting
• Forecasting
• Enterprise growth planning
• Metrics
• Portfolio plan
• Commercial planning
• Program management
Parallels with Academia

- What is the development cycle of projects in your lab?
- How many projects do you have in each part of the cycle?
- What are organizational capabilities of your lab?

- Tell us about your near and long term strategies for your lab?
INTERLUDE: How do you assess company strategy from a website?
Case study

Break-out groups for discussion

- Choose 1: Pharma, Medical Device, or Academics
- Choose a notetaker and a presenter.
Strategy course objectives

You should now be able to:

1. Define strategy and how scientific enterprises use strategy
2. Identify the strategic focus of a scientific enterprise
3. Determine key stakeholders for a scientific enterprise
4. Discuss how strategy impacts budget and resource allocation
Wrap Up

Resources to learn business strategy

- UCSF courses
- Coursera or iTunes University
- Harvard Business Review (HBR)
- FierceBiotech daily newsletter (or sister publications)
- Earnings calls from public companies of interest.

Reminder of next seminar
INTERLUDE: How do you assess company strategy from a website?
Leadership and company-level descriptors
Development pipeline

19 targets supported by GENETIC VALIDATION

The industry's largest toolkit with 11 MODALITIES*

A mix of INNOVATIVE MOLECULES, POTENTIAL NEW INDICATIONS, AND BIOSIMILARS

A robust and differentiated pipeline, leveraging state-of-the-art science to create medicines for serious illness. Amgen is focused on high-quality candidates that demonstrate large, clinically-relevant effects. Human genetic validation is used whenever possible to enhance the likelihood of success.

Download Pipeline Chart

<table>
<thead>
<tr>
<th>THERAPEUTIC AREA</th>
<th>MODALITY</th>
</tr>
</thead>
<tbody>
<tr>
<td>All</td>
<td>All</td>
</tr>
</tbody>
</table>

Enter Keywords

PHASE ONE
- AMG 172
- AMG 208
- AMG 211

PHASE TWO
- AMG 139
- AMG 157
- AMG 181

PHASE THREE
- AMG 334
- Aranesp®
- BLINCYTO®
- Epoetin alfa
- Mylotarg®
- IMLYGIC™
- Kewebi®
Currently marketed products

Amgen is one of the world’s leading biotechnology companies. A values-based company, deeply rooted in science and innovation, Amgen transforms new ideas and discoveries into medicines for patients.
Investor Relations: SEC filings, corporate presentations, and earnings calls