

Validating Your Drug Discovery Opportunity

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Technical success is a great and hopeful milestone!

Now you must determine:

- 1. Whether it's worth bringing your potential therapeutic to market**
- 2. Exactly how that's to be done**

What is validation?

- Deciding what business you're really in
- Assessing market potential
- Analyzing options and selecting the best business model
- Assessing resource requirements to determine what's really possible and what initiatives to kill (Will you enjoy competitive superiority?)
- Deciding on outreach requirements and alliance relationships
- Assessing regulatory pathways
- Devising customer access tactics

Why validate?

- Correctly sets expectations of company execs, investors, colleagues and alliance partners
- Stimulates exploration of alternative business models and organizational structures
- Avoids commercializing the wrong therapeutic
- Avoids developing a product for too small a market
- Curtails a discovery program destined to fail

When to validate?

Therapeutic indication

Disease model

Target identification

Target validation

Compound synthesis

Screen development

Compound library screening

Lead compound optimization

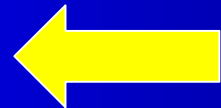
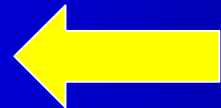
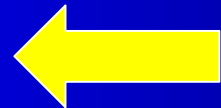
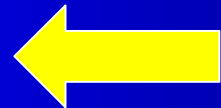
Preclinical studies

Phase I

Phase II

Phase III

Post-market surveillance



Validate early and often!

Agenda

- What's the product?
- Focus
- Serving an existing market
- Serving a new market
- Who's the customer?
- Customer solutions
- Customer attitudes
- Pricing
- Business models
- Market access
- Partnering & licensing
- Distribution
- What next?

What's the product?

- Must define the product precisely in order to:
 - Correctly assess its market opportunity
 - Develop the right product
- Who's the user, and what are the benefits of use?
Curative? Symptomatic relief? Palliative?
- Who's the payer, and what "value" (reimbursement) will be set for the product?
- How is it like and unlike competitive products — exactly?
- Does it significantly save healthcare \$\$ overall?

Focus

- The blockbuster model is (nearly) dead
- Everyone knows that focus is key, but how?
- Consider, for example, focusing by:
 - Disease category (classical M.O., e.g. cardiovascular)
 - Value chain niche (full service, drug discovery, production and marketing, DD tools, etc.)
 - Specialist niche (e.g. Novo in diabetes)
 - Delivery niche (orals, injectables, transdermals, inhalants, etc.)
 - Drug/device combinations (stents, coated catheters, drug pumps, encapsulated biologics, etc.)
 - Molecule class (small, biologic, natural, etc.)

Serving an existing market

- Market expansion unlikely; generally must take share
- More accurate estimates are possible
- Easier to describe product to prospective customers and users
- Usually easier to establish purchasing criteria and customer profiles
- “Feet on the street” often wins

Serving a new market

- Must create the market
- Little or no immediate competition
- Must face up to the adoption process
- Greater scrutiny; must overcome inherent conservatism of clinicians and third-party payers
- Likely need for greater marketing investment, but perhaps less sales investment

Who's the customer?

- The essential question: Who must be satisfied in order to achieve success?
 1. Clearly, the user must be satisfied
 2. The purchase influencer (prescribing clinician) must be satisfied
 3. Clinical thought leaders must be satisfied, too
 4. Finally, a payer usually must be satisfied
- Is your discovery program allotting adequate consideration to all 4 customers?

Customer solutions

- Don't think drugs, think customer solutions
- Consider, for example:
 - Drug/diagnostic combinations
 - Disease management programs
 - Drug cocktails
 - Drug/device combinations
 - Information products & services
 - DTC marketing channels

Customer attitudes

- Prospective customers lie!
 - When not asked the right questions by experienced interviewers who know the territory
 - When not expertly led to disclose their real attitudes
 - Because they're human and wish to please
- Must work through respondent attitudes and biases to answer the essential question:
 - What will it take for this prospective customer to choose, champion and routinely use your new therapeutic?

Pricing

- Think you can just set the price to satisfy your internal ROCE requirements? Think again!
- The market will set your price:
 - Competitive products
 - Alternative therapies
 - Payers
 - And soon, legislation?
- Your recourse: Control capital investment and minimize development time
- For a breakthrough drug, assess the potential reduction of other costs (hospitalizations, LOS, surgery, etc.) to rationalize price leadership

Business models

- Eliminate organizational “silos” that impede effectiveness
- Devise discrete business units that are customer-focused
- Push “down” responsibilities for:
 - Strategic assessment
 - Decision-making
 - Productivity
 - P&L accountability

Market access

- You will almost certainly overestimate your ability to access customers
- Most biotech firms should not consider directly accessing clinical customers ... unless access to a small cadre of specialist physicians is sufficient and feasible (e.g. multiple sclerosis)
- In any case, regional marketing partners and distributors are likely to be needed

Partnering & licensing

- Manage risks and returns; stick to what you know best; control invested capital
 1. Joint development
 - Complex and challenging to manage
 2. In-Licensing
 - From biotech: Common practice
 - From Big Pharma: Difficult to convert apparent “losers”
 - Re-purposing previously marketed or failed drugs
 3. Out-Licensing
 - Generally lackluster returns
 - Typically challenging to staff the activity

Distribution

- Objective: Engage a powerful ally to offset lower revenue per sale by achieving greater volume
- Customers often prefer dealing with a few powerful firms — more efficient and comfortable
- You must craft mutually beneficial relationships with channel partners
- Don't let the distribution process de-emphasize or derail your marketing program; it's always needed

What next?

If all looks favorable:

- Solidify your plans
- Document your findings and rationale
- Sell your validated ideas to company execs, investors, colleagues and alliance partners

If you find defects:

- Adjust your expectations and plans
- Consider redefining your product
- If necessary, alter your business model
- Re-validate

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