

Multi-stakeholder Development Partnerships for Underserved Indications

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Collaborate
to Innovate



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Overview

- Introduction: the risks of product development
- Novel types of partnerships
- Benefits to partners
- Partnership examples
- Case study
- Conclusions



Introduction

- Product development is difficult, expensive, risky
- Uncertain ROI
- Small markets: very uncertain ROI
 - Small populations (some orphans)
 - Large resource-poor populations (e.g., TB)
- Business proposition not attractive



To lower risk...

- Pursue me-too drugs
- Repurpose established drugs
- Reformulate
- Develop adjacent expanded indication
- In-license post-POC
- Build broad patent estate



To raise benefit...

- Pursue billion-dollar markets
- Develop innovative, superior products
- Serve unmet medical needs (no competition)



Alt.risk:benefit

- Redefine benefit
 - Double-bottom line
 - PR to benefit wider company
 - Public health value
- Re-distribute risk
 - Partners share in risk of venture
 - Combined knowledge bases further reduce risk
- Smart partnerships achieve both



Novel partnership types

- Government-funded research consortia
 - Immune Tolerance Network (ITN), Atopic Dermatitis and Vaccinia Network (ADVNI), AIDS Clinical Trials Group (ACTG), etc.
- Direct government procurements
 - SBIR grants, Broad Agency Announcements (e.g., DoD)
 - For company, support is non-dilutive

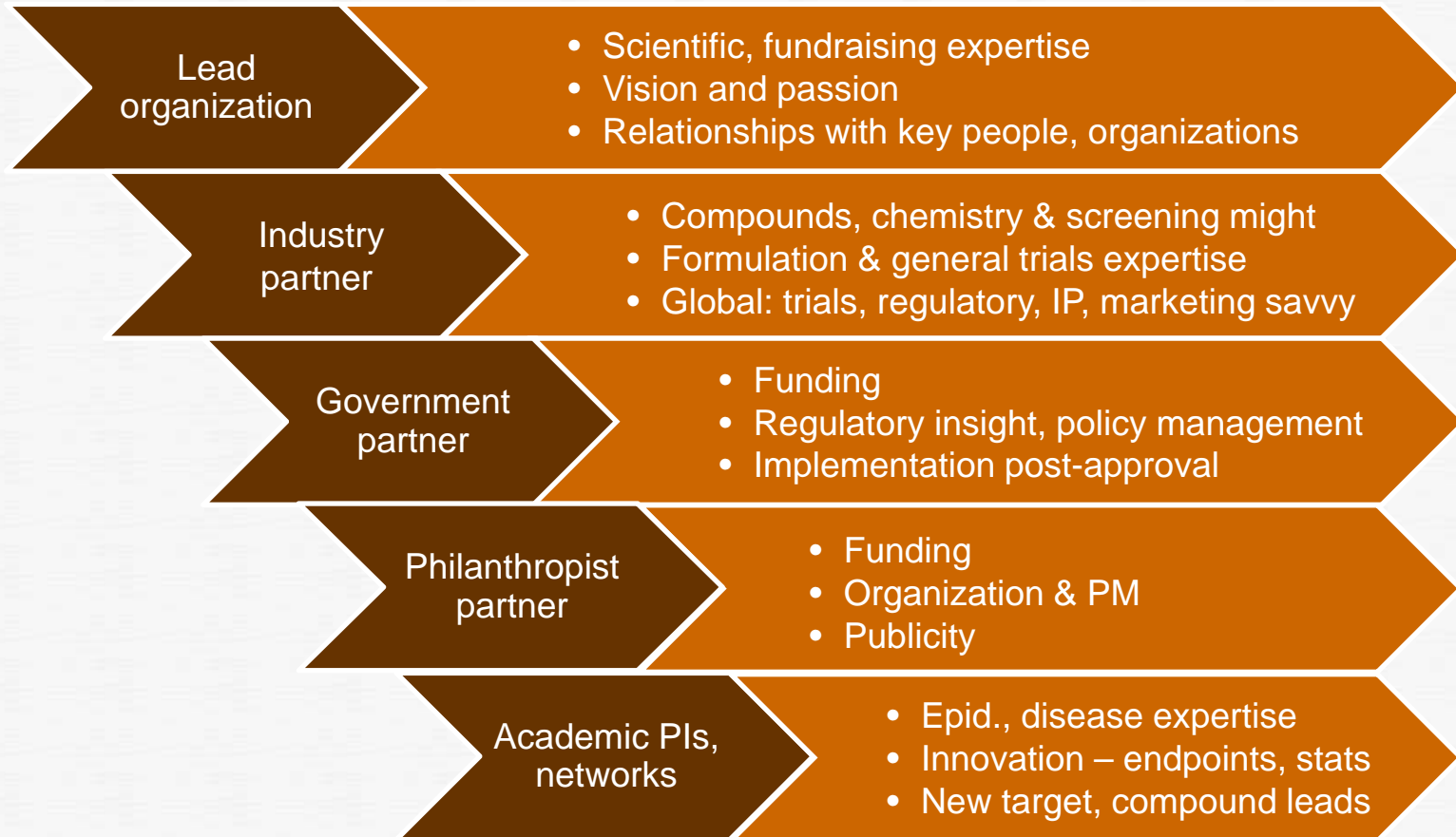


Novel partnership types - 2

- Non-profit biotech companies
 - Oneworld Health: diarrheal diseases, malaria, Leishmaniasis, others
- Minimally profitable companies
 - ReProtect: devp'd-world profits subsidize devp'ing-world sales
- Public/private product-development partnerships (**PDP**)
 - TB Alliance, IPM, MMV, DNDi, The Lilly TB Drug Discovery Initiative, etc.



PDP contributors



Key benefits to PDP partners



PDP: Pros

- Each stakeholder makes valuable contributions
- Guard is lowered; data flows more freely
- Goodwill, PR can mobilize significant industry resources



PDP: Cons

- Drug development is no less expensive
 - Held to highest safety, efficacy standards
- Harsh endemic environments, combination Rx can be challenges
- Market “success” hard to control
 - negotiation/politics, not quality/advertising
- Low/nonprofit by design – no \$\$ to support ops
 - Generics and volume sales
 - Need constant \$\$ infusion – donor fatigue?
- Multistakeholder ... not always nimble
 - Project, partner, and people management are essential



Case Study: TB Alliance

- Founded in 2000
 - Rockefeller, Gates Foundations
 - NIAID
 - Partners now include Tibotec, Novartis, Bayer, WHO, FDA, other regulators, Johns Hopkins, numerous other academic & industry groups
- Mission: redress decades' of R&D neglect
 - Accelerate, motivate development of new TB drugs and regimens



Case Study: TB Alliance Challenges

- Funding
- Thin pipeline
- Regulatory precedent not recent
- Lack of trials network
- Uncertain trial site infrastructure



Case Study: TB Alliance Strategies

- Challenge: Funding
 - Rockefeller foundation funding and in-kind assistance (offices, etc.)
 - NIAID support via project management grant to RTI
 - Gates Foundation for initial development operations



Case Study: TB Alliance Strategies - 2

- Challenge: Pipeline
 - Energetic outreach to pharma
 - Risk sharing via co-development: TMC207 (Tibotec)
 - Opportunities to expand indications: moxifloxacin (Bayer)
 - Otherwise unused drugs: PA-824 (Novartis)
 - Creative licensing – pharma retains developed-world market; developing-world market guaranteed low prices



Case Study: TB Alliance Strategies - 3

- Challenge: Regulatory precedent not recent
 - Co-sponsor development issues fora in US, India, Ethiopia, etc.
 - Cooperate closely with key FDA leaders, KOL in field



Case Study: TB Alliance Strategies - 4

- Challenge: Lack of trials network, site infrastructure
 - Conduct extensive global R&D capacity assessment
 - 39 countries; 84 sites; 72 associated labs
 - Goal: identify critical investment needs to focus investment
 - Results available in open database for community innovation



Case Study: TB Alliance Achievements

- Development innovation
 - Now 20+ drugs in its pipeline, 3 late-stage.
 - Raised bar, attracted for-profit development investment, competition
- Regulatory innovation
 - Co-founded w/ FDA, Gates the Critical Path to TB Drug Regimens (CPTR) initiative
 - Develop multiple NCEs in parallel as single operative development unit



Case Study: TB Alliance Achievements - 2

- Rich network of partners
 - Multiple philanthropic, pharma, academia links
 - New sites being brought into networks
 - Meta: partnership with DNDi to share products across indications



Conclusions

➤ Underserved markets are unclaimed business opportunities



➤ Multi-stakeholder groups can lower risk, increase wherewithal and expertise of development team

➤ Multi-stakeholder efforts require special program management efforts

➤ Participating as partner brings many ancillary benefits to organization

➤ For-profit efforts may be best path to sustainability

