

INCENTIVES FOR A FAVOURABLE ECO-SYSTEM



BioPontis Alliance

Biopontis Alliance Rare Disease Foundation: an Innovative Model for Early Stage Rare Disease Therapy Financing and Development

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Co-founding Board Member

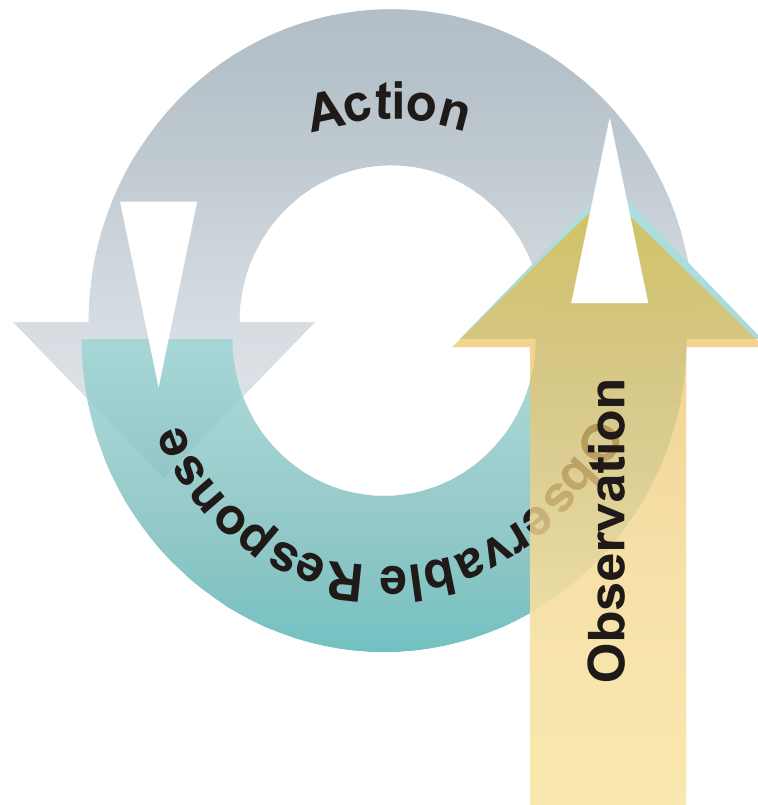
BioPontis Alliance Rare Disease Foundation

Presentation Objectives



- Describe the drive to advance patient-centric healthcare
- Discuss the challenges of developing medicine and diagnostics for rare diseases
- Introduce the BioPontis Alliance Rare Disease Foundation as a new initiative and role for a philanthropic innovation joint venture model targeting pediatric rare diseases

Old Development Paradigm: Trial and Error



**Successful When it
Leads to Innovation
and Improves Standard
of Care**

**Fails When We Settle
for “Trial and Error”
Medicine AS the
Standard of Care**

Personalized Healthcare



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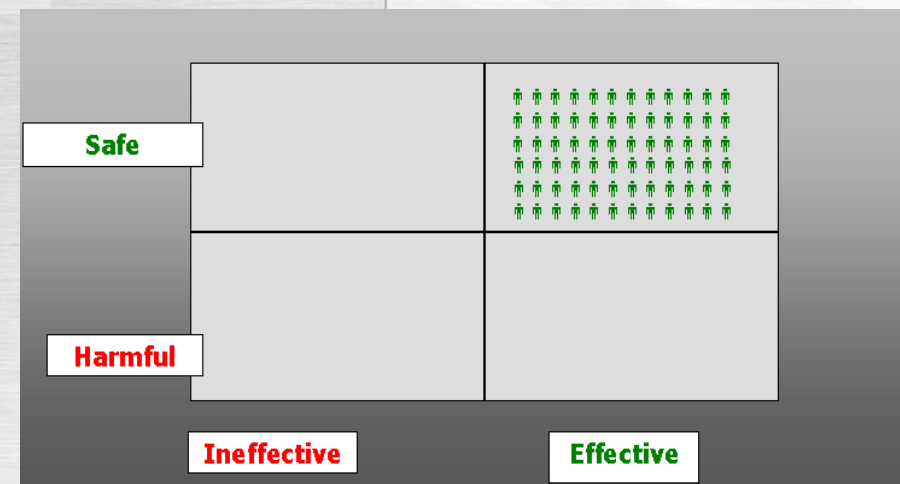
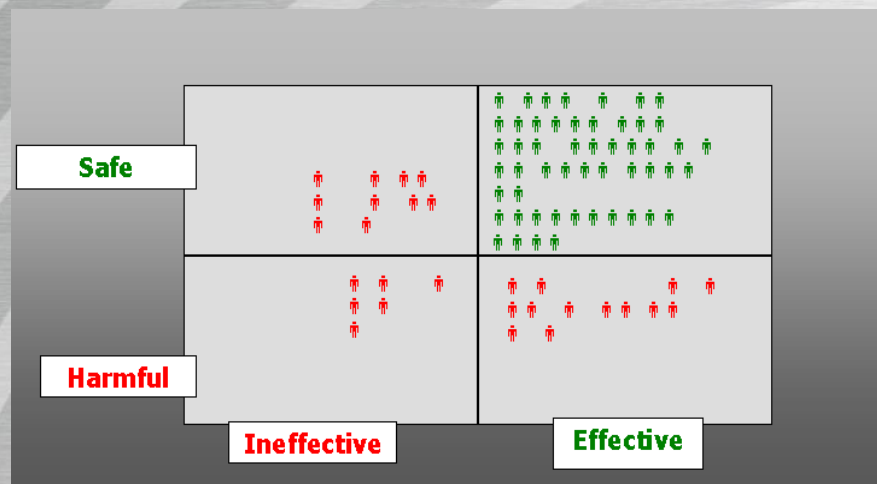


Challenges for Medicine Development



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All humans are different but treatments are standardized
Drugs are ineffective for many and harmful for some



“The vast majority of drugs – more than 90% - only work in 30 or 50% of the people”

Source: Allen Roses, Senior Vice President, Genetics Research, GlaxoSmithKline

Factors creating the 'Valley of Death' in 2014



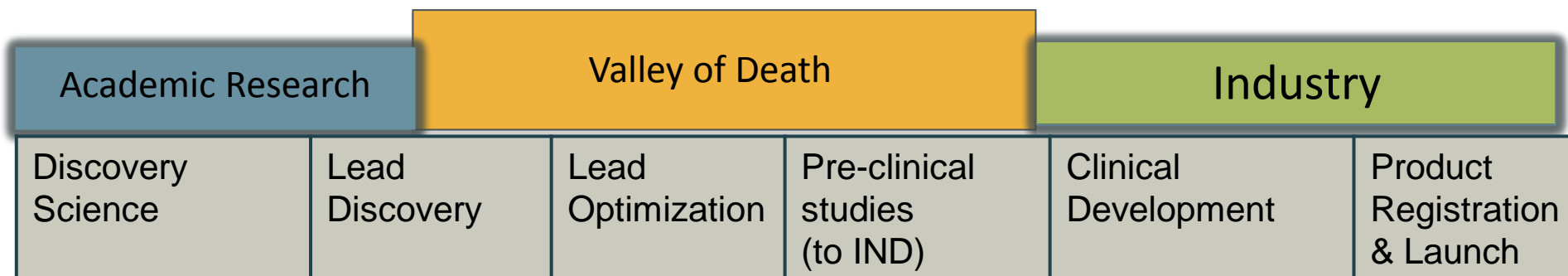
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Retreat of early stage capital due to poor ROI

Licensing hampered by misaligned incentives and interests

Complex R&D requires flexibly accessible, broad selection of technical capabilities

Product candidates % fail in human studies = poor ROI



Drug discovery process often driven by promises instead of data

Drug discovery technical know how resides in Industry

The Choice of Society: *Patient-Centric Healthcare*



- Really exists since the dawn of medicine, but using new technologies
- New technology includes combination of testing and therapy
 - → right therapy for the right patient at the right time
- Rare disease field & orphan medicines is precursor:
 - much more innovation and less me-too products
 - Clinical trials more complex +/- more costly:
 - smaller patient groups
 - Major improvement in efficacy:
 - less side effects + better compliance
- Need high quality tests with clinical utility (& accredited labs)
- Networks/centers of excellence per disease
- *Societal debate to include ethical issues*

Need to Better Incorporate *Patient Values and Patient Inputs*



- Patient values may differ from physician perception
- Challenge “cost-effectiveness” and “cost-utility” approach by “real-life” impact data and long-term outcomes
- Train patients and public to better engage in health economic and evidence-based deliberations
- Develop a values-based decision making framework & train members and public for value-based deliberations
- Bring the patients and clinicians who represent the “real-life” perspective into the decision committees
- Make processes publicly accountable & transparent

Innovation Gaps Occur in Many Industries



- High failure rate of translating ideas into products
 - because science is complex and largely poorly understood
- Industries that rely on technological or scientific innovation for their products must source it continuously
 - Big companies tend to be poor innovators
- Good sources of creative discovery and invention include academic institutions
- Academic inventors often lack the essentials for product development beyond initial prototype:
 - Capital
 - Know-how to design/engineer to product definition
 - Effective patenting strategy and/or tactics

Health is the Greatest Wealth....



Needs are continuously increasing

- Ageing population
- Difficult to treat, degenerative and chronic diseases
- Rare diseases

Higher expectations

- Quality of life

More is possible !

- Exploding knowledge base
- Innovation
- Growing technological toolbox (e.g. through biotechnology, genetic diseases become treatable)

but... costs are escalating and budgets more limited

The Need for PPP's: *Public-Private Partnerships*



- Drug discovery and development processes change to meet the needs of patients (instead of focusing on creating “markets”)
- None of the players (industry, academia, regulators...) have all necessary knowledge
→ multidisciplinary collaboration to minimize size and cost of clinical trials
- Efficient model of “open innovation”: academia, clinicians and large and small companies to be true partners in a win/win collaborative effort

Introducing a *non-profit* Innovation Joint Venture



We are founding a collaborative *non-profit* 'cures continuum' for rare diseases.

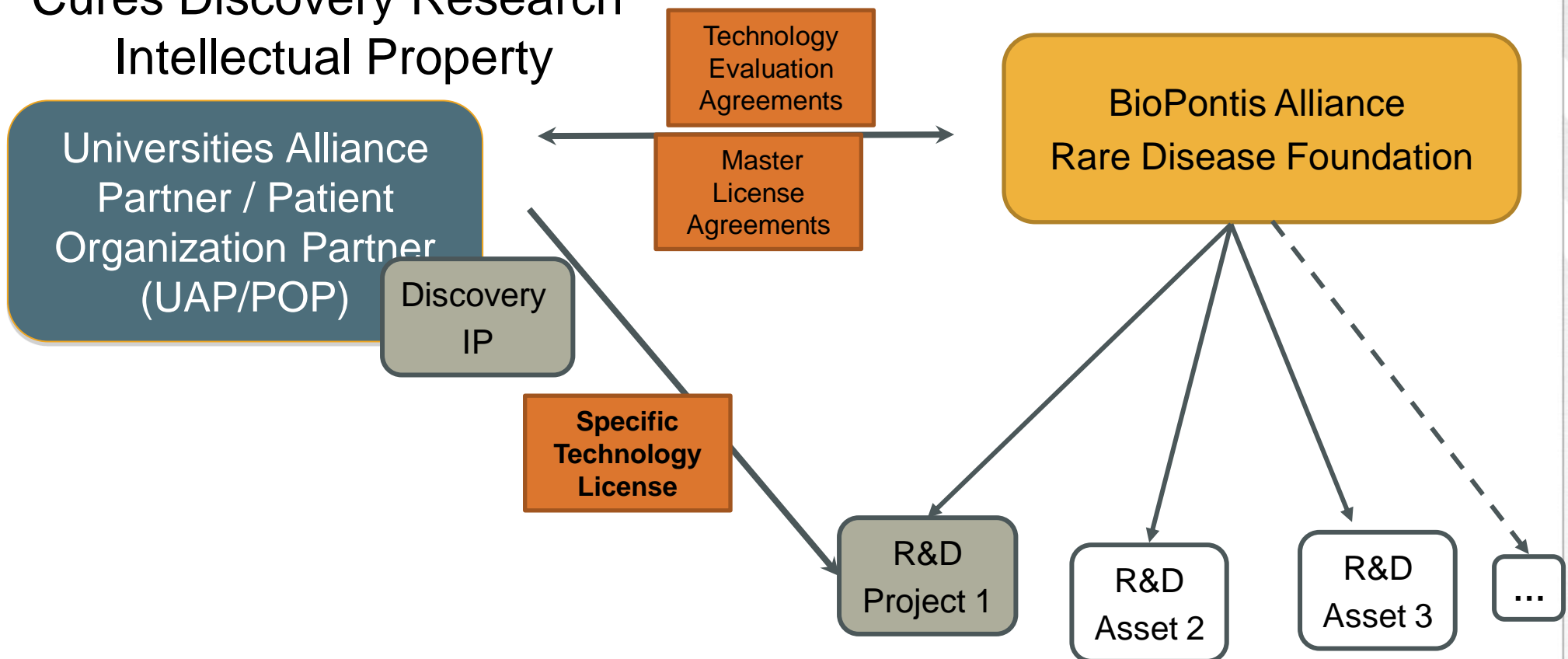
The mission of the **BioPontis Alliance Rare Disease Foundation** is to:

- ❖ Establish a scientific bridge across the innovation gap that will serve as an enabling hub to independent disease foundations, *providing a nonprofit partner able to develop drug candidates.*
- ❖ Establish a financial bridge between cures 'push' of philanthropic resources and the commercial 'pull' of private sector.

The Academic Partnerships of the BioPontis Foundation



Cures Discovery Research Intellectual Property

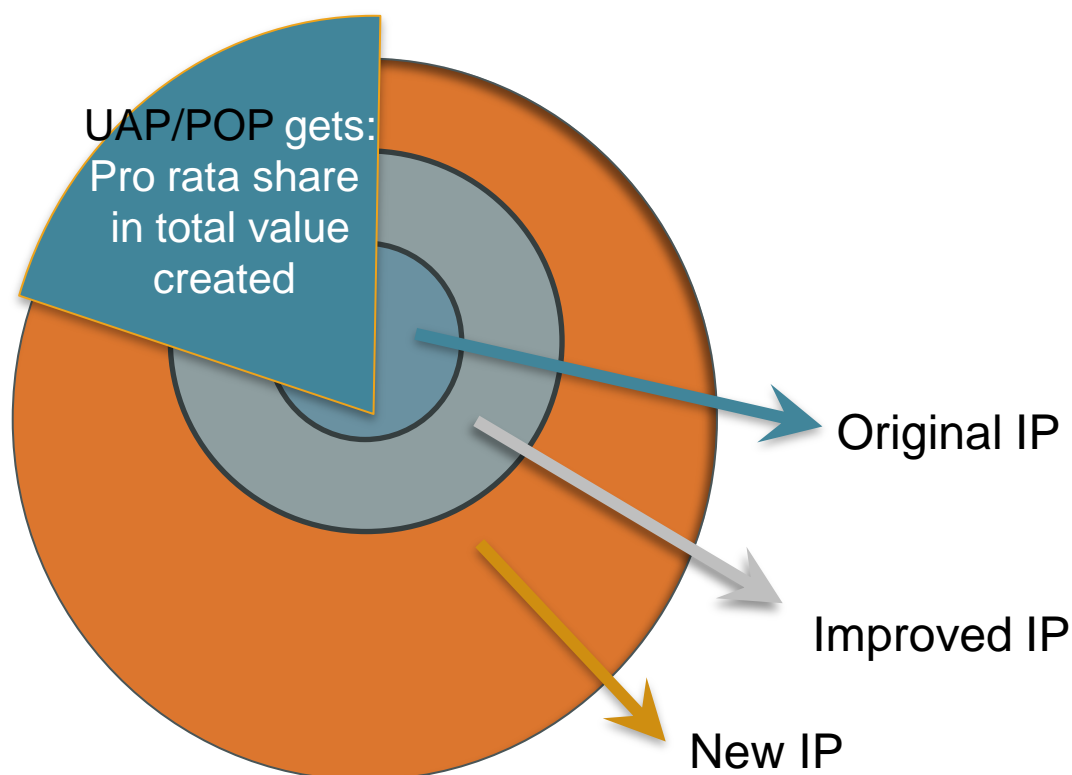


Shared IP *and* Economics Model



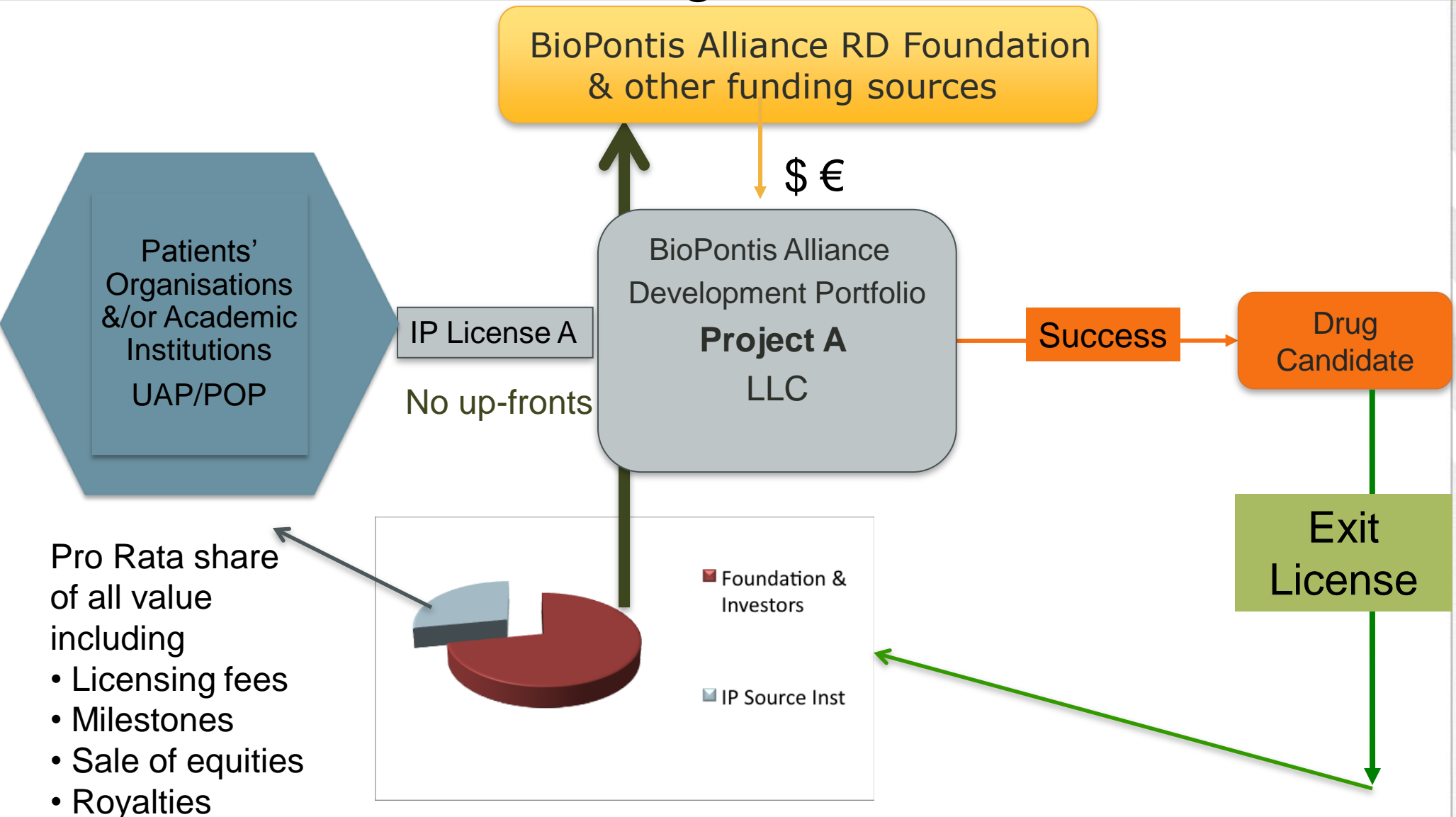
Transformational Economic & IP Sharing

- Replaces upfront IP cost with shared exit IP value
- Value generated from the entire patent portfolio is shared between the IP source and BARDF

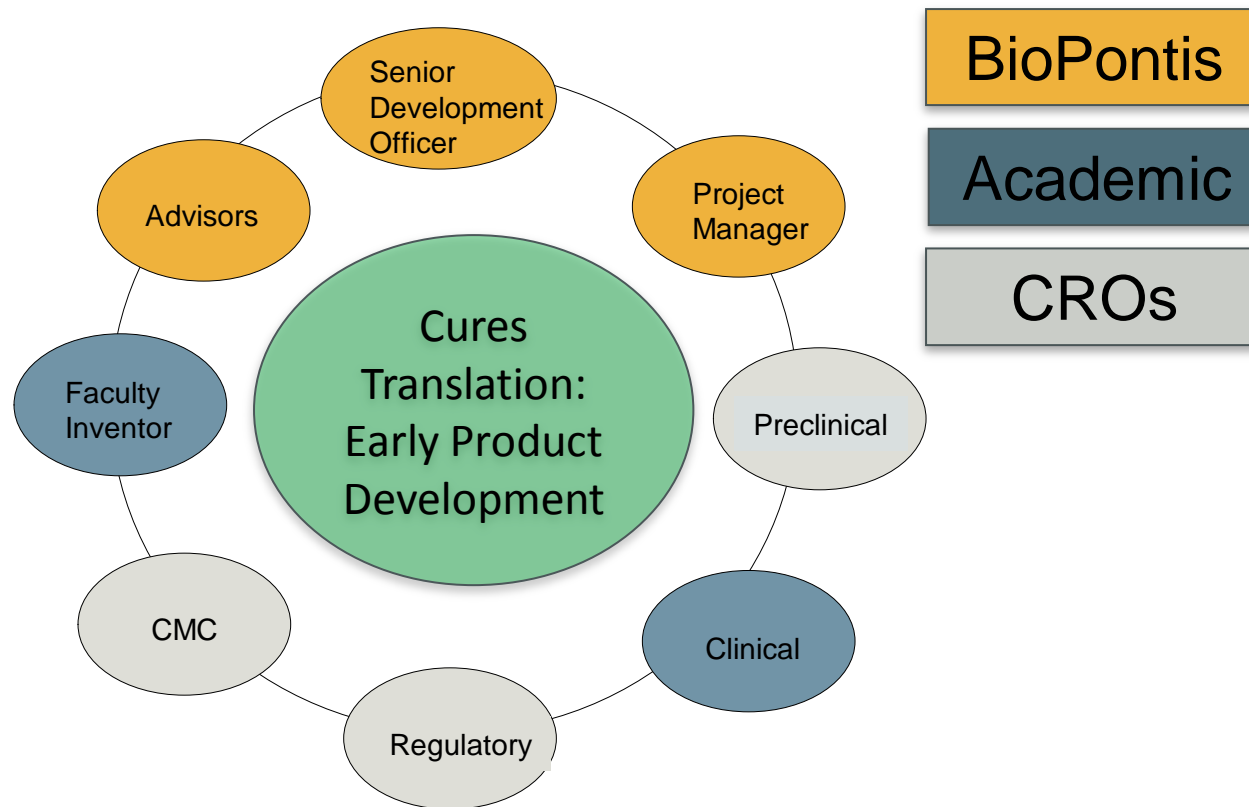


- Removes concerns about IP work around
- Incentivizes all to maximize contributions toward success
- Promotes transparency and full collaboration

BioPontis Alliance RD Foundation Economic Sharing Model



How Applied Development will Happen → the Project Team



BioPontis creates a multidisciplinary team for each program bringing specific expertise around the inventing scientist that enables rigorous checking and advancing.

Call to Participate and Act. BioPontis Alliance Rare Disease Foundation



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Also YOU can support the launch of the non-profit
BioPontis Alliance Rare Disease Foundation:

- Donate (a US 501(c)3 tax exempt foundation)
- Rare disease projects (especially in children)
- Board and Scientific Advisory Committee
- Can this idea also work across borders in the EU?

Contact Us for More Information



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Dankon al vi

Nagyon köszönöm

Дуже дякую

Ευχαριστώ



BioPontis Alliance

ありがとうございます

Cảm ơn cô

Mange tak

Dank je wel

THANK

Merci beaucoup

Благодарам

Tānan teid

YOU

شكر

Dziekuje

Paldies

Grazie

Multumesc

धन्यवाद

Obrigado

Tesekkür ederim

Toda raba

Paljon kiitoksia

Dakujem vám

Attyu

Hvala lijepa

Danke schön

谢谢

Tusen takk

Gracias

Dêkuji

Благодаря

Tack så mycket

Спасибо

Faleminderit