Investigator-led studies
An Industry perspective

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Imperatives for the pharma industry

- Develop medicines that meet unmet medical need
- De-risk development
- Make good decisions earlier
- Reduce time and cost of development

Leads to a desire to work increasingly in partnership with academia

The common goal

- Most of the major advances in medicine have been made possible by new drugs.
- Incredible breakthroughs in biology have yet to realise their potential in improving human health.
- Neither academics nor industry can realise that potential alone.
- Developing safe, effective and better medicines is one of the greatest challenges in science.

The environment is changing

Less focus on blockbusters
Emerging interest in
orphan diseases

Tap into academic expertise
Exploit academic innovation

Tap into drug development expertise
Exploit technologies and manufacturing capabilities
Translate basic science or innovative hypotheses

A great fit, so where have we gone wrong?

Collaborations negotiated at wrong level (SVP to Dean)

Endless contract deliberations

Unrealistic views on IP

Academic Institutions unwilling to bear any financial risk

Different approach to publications

Industry mergers and reorganisations lead to changes in strategy – leaving projects high and dry

High turnover of Industry staff - Leads to constantly changing point of contact

Where do Investigator-led studies fit in?

- Innovative and motivated individuals
- In-depth understanding of the target, disease and patient
- Hypothesis-driven
- Focus on un-met medical need and differentiation
- Explore the full potential of a novel drug
- An extension of a project team

Investigator-led Studies: Common Industry Myths

- Outside core project strategy
  - Nice to have, speculative, low priority, “Not invented here” syndrome
- “High risk”
  - May generate a new and unwelcome adverse event profile if a different patient population is used
- Lower quality
  - Level of GCP-training?
  - Level of patient safety monitoring etc?
- Additional administrative burden
  - Need to incorporate data into CIB updates, IND and NDA annual reports etc
- **SLOW!**

What do we consider when reviewing proposals for investigator-led studies?

Is it ethical?

What are the scientific and medical merits?

Does the study align with the overall global drug development strategy?

Will the results address specific data gaps and/or address a specific issue?

Could this be seen as a back-hand way of rewarding HCPs or influencing prescribing practices?

Might this be perceived as promoting off-label use?

Are the investigators qualified and competent?

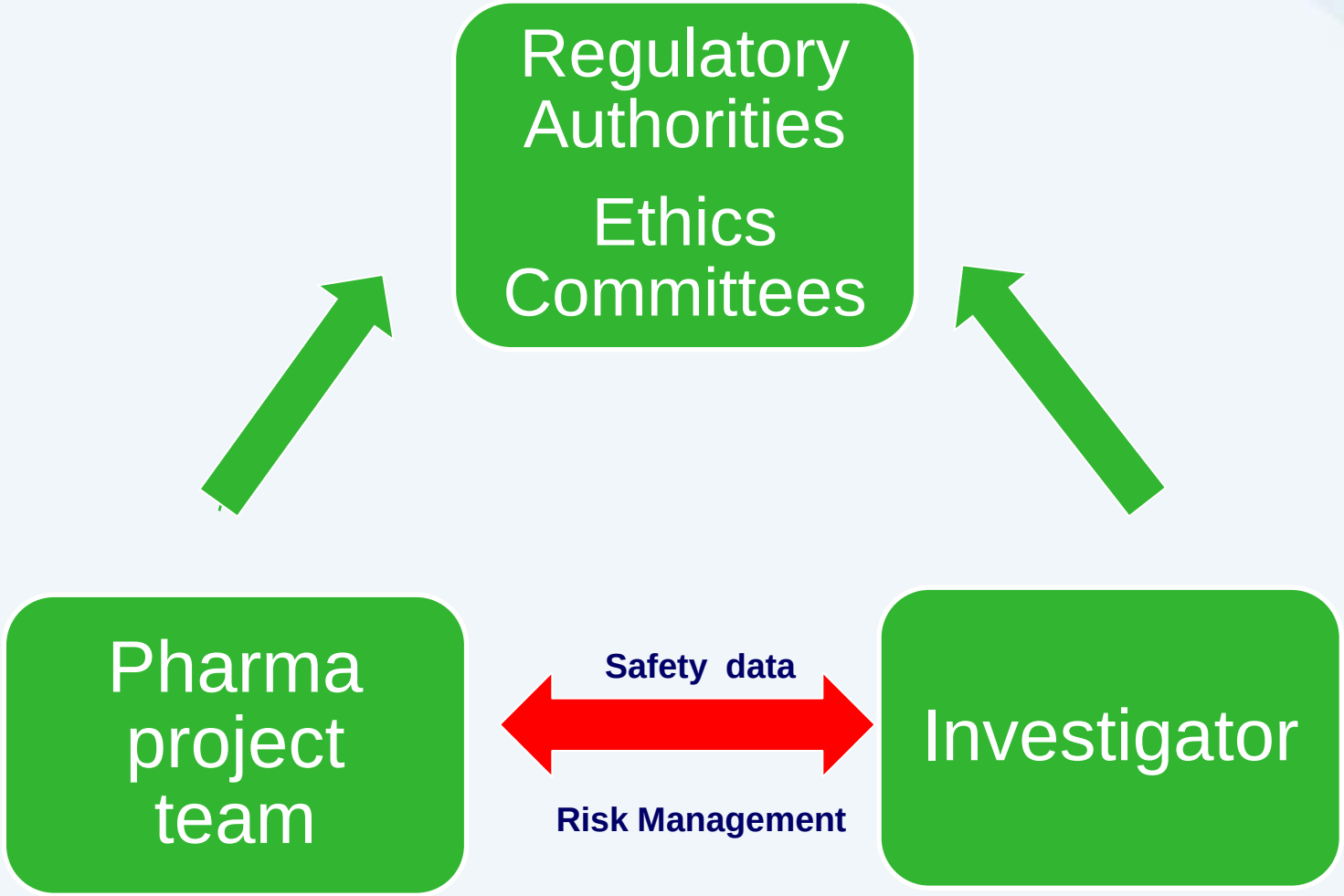
Is the budget based on current fair market value?

Are blinded comparators or unique formulations of active drug or placebo required?

Are robust safety and clinical data disclosure procedures in place?

If testing in a new indication: Where next?

The Golden Triangle of Communication



How can we make it work?

Academic and Industry PIs approach each other as equal partners

Complete transparency about who will do what, when and how

Meet frequently to review data, exchange ideas and update plans

Agree publication policy in advance

Collaborate on a clinical plan, not just one-off studies

Have a clear procedure for safety issues and updates

Make contingency plans for
- compound attrition etc.

Keep reminding ourselves of the common goal!

Our mission

To improve the quality of **human life**
by enabling people to

do more
feel better
live longer