CLINICAL TRIALS FOR REGULATORY AND REIMBURSEMENT NEEDS: DOES ONE SHOE FIT ALL?

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ISSUES PANELS - SESSION II, Monday, 5 September 2016: 3.45-4.45pm

Clinical trials: Fit for purpose?

What is the purpose?

- Timely, sustainable and equitable access to medicines that change the lives of people, and their families, living with disease.
- Evidence to support discussions with multiple stakeholders: regulators, payors, clinicians, patients
- Safe, effective and cost-effective therapies for the right patients at the right time and at the right price
- Rational investment to sustain the ability to invest in future R&D – to support expanded access for existing therapies and for therapies of the future

All things to all people?

Is it possible to meet the needs of all stakeholders?

- Answer the question, with greatest certainty, of the safety, effectiveness and value for money of a medicine:
  - In accordance with latest clinical practice
  - Applicable to local patient care
  - Applicable to local treatment practice
  - Relative to local Standard of Care
  - With a measure of benefit that is meaningful
  - Whilst doing the least harm, and
  - To bring benefit to patients and payors as early as possible

Answering the question

By molecule, by class, by therapeutic area, by company?

Reference: Biopharmaceutical Research & Development, The Process Behind New Medicines. PhRMA, 2015

All things to all people?

Is it possible to meet the needs of all stakeholders?

- International jurisdictions for regulatory requirements
  - Regional harmonisation exists
    - Legal obligations of application by members
- International requirements for funding
  - National ⇄ Regional ⇄ Fund level

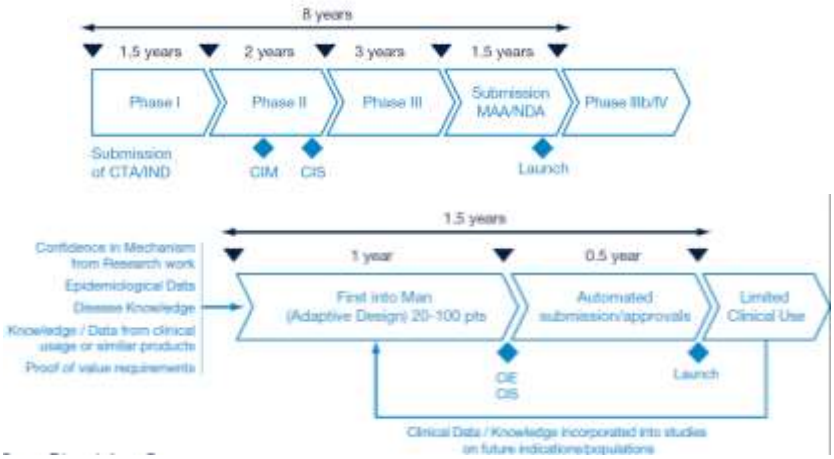
includes social, ethical, and legal aspects of health technology use

Payer archetypes

Ref: InVivo, The Business and Medical Report, Pharma Survival in a Transforming Global Payer Environment, September 2015

Evolution of evidence generation

How do we continue to evolve the debate?



PricewatershouseCoopers, Pharma2020, Virtual R&D. June 2008

Evolution of evidence generation

How do we continue to evolve the debate?

- Regulatory reform
  - International recognition
  - Remove proof of efficacy requirement
- Development of value frameworks and Harmonisation of HTA
  - Scientific societies (ASCO, ICER, Sloan Kettering Cancer Institute, National Comprehensive Cancer Network)
  - EUnetHTA Core Model
  - Green Park Group
- Maximise value of registries
  - Commonly used for Rare Diseases
- Utility of Real World Evidence
  - Vaccines utilise RWE with notification of disease fulfilling the requirement of evidence of effectiveness
- Greater utilisation of post marketing experience

Panel recommendation

- Registry based trials
- Evidence generation - Fit for purpose
  - Outcomes based trials +/- RWE
- Practice based evidence
- Managed entry schemes and coverage with evidence development
  - For registration and funding

Working toward a common purpose

Can we shape evidence generation to meet the needs of all?