CPSA Foundation Clinical Trial Consortium

Why CPSA?

- Power Host
  - Can deliver where individual organisations cannot
- Trusted
- Neutral
- Inclusive
- Engaged
- Community
- Patient focussed
- Forward thinking
- Committed to delivering
- Responsive

Vision

- Change the standard of care, e.g.
  - Under-represented groups
    - Rare diseases
    - Vulnerable groups
    - Unmet need
    - Orphan drugs
  - Outcome can be used on a larger scope to change the standard of care, i.e. COVID testing
- Demonstration that patient centric sampling / home sampling and analysis are at least as good as current venous phlebotomy and analysis workflows
- Overcome barriers to acceptance

Deliverables - Goals

- Data quality
  - Quality sample and analysis regardless of site
  - Comparable with current standards and between patient centric technologies
  - CPSA Stamp
- Inclusive of all measures required
  - E.g. not just PK; immunosuppressants to include creatinine, etc
- Decision making / diagnosis
- Patient acceptance
- Healthcare provider acceptance
- Society acceptance
- Reimbursable
- Outputs need to be able to be used by member organisations

- May be logical to start in Europe

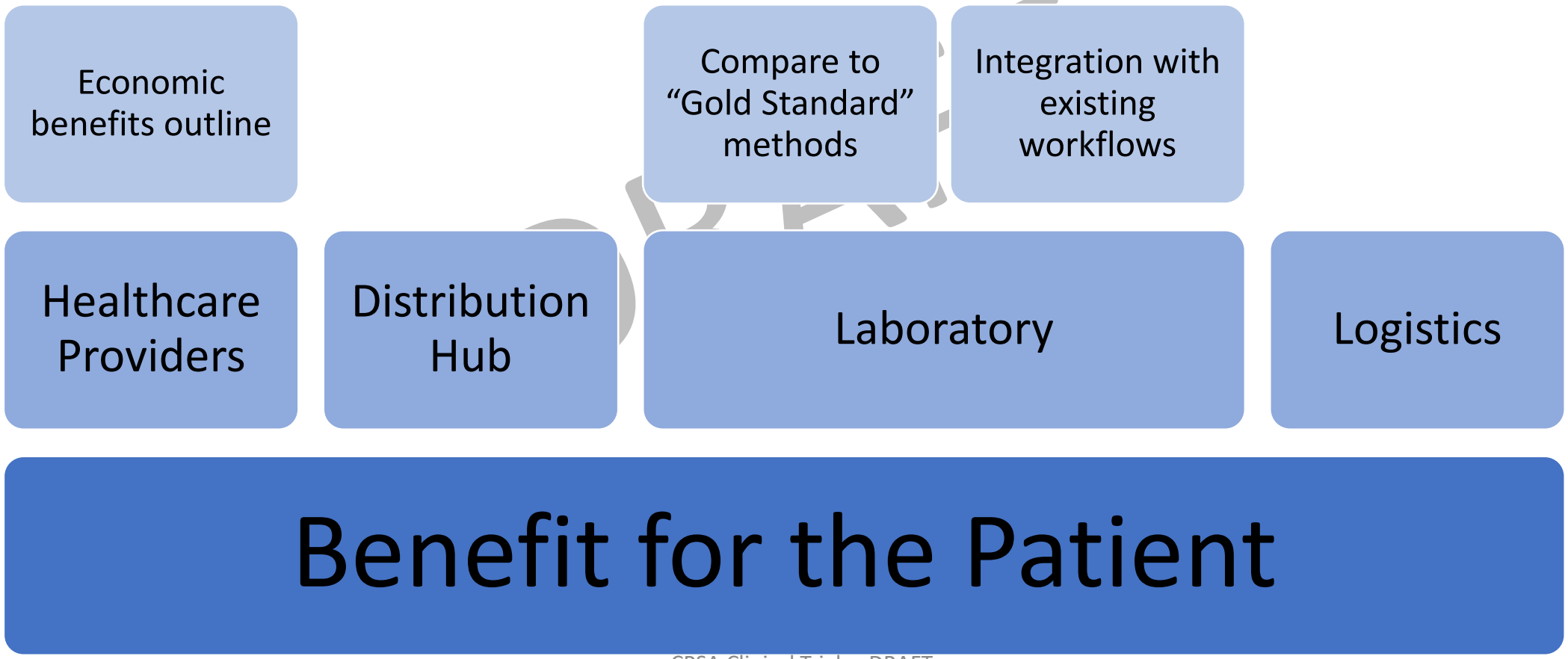
Deliverables - Means

1-3 Clinical Trials per Year

- Identify what is required, NOT how it is delivered
  - E.g. date stamp, identity of patient, precise volume, etc
- Understand the needs of different markets (Pharma vs healthcare) and countries / regions for home sampling
  - Define cost benefit balance
- Check (legal) that each deliverable does not do more harm than good for development & routine implementation of the technology
- Turn into day to day reality
  - Published standards
  - Work with central regulators, etc

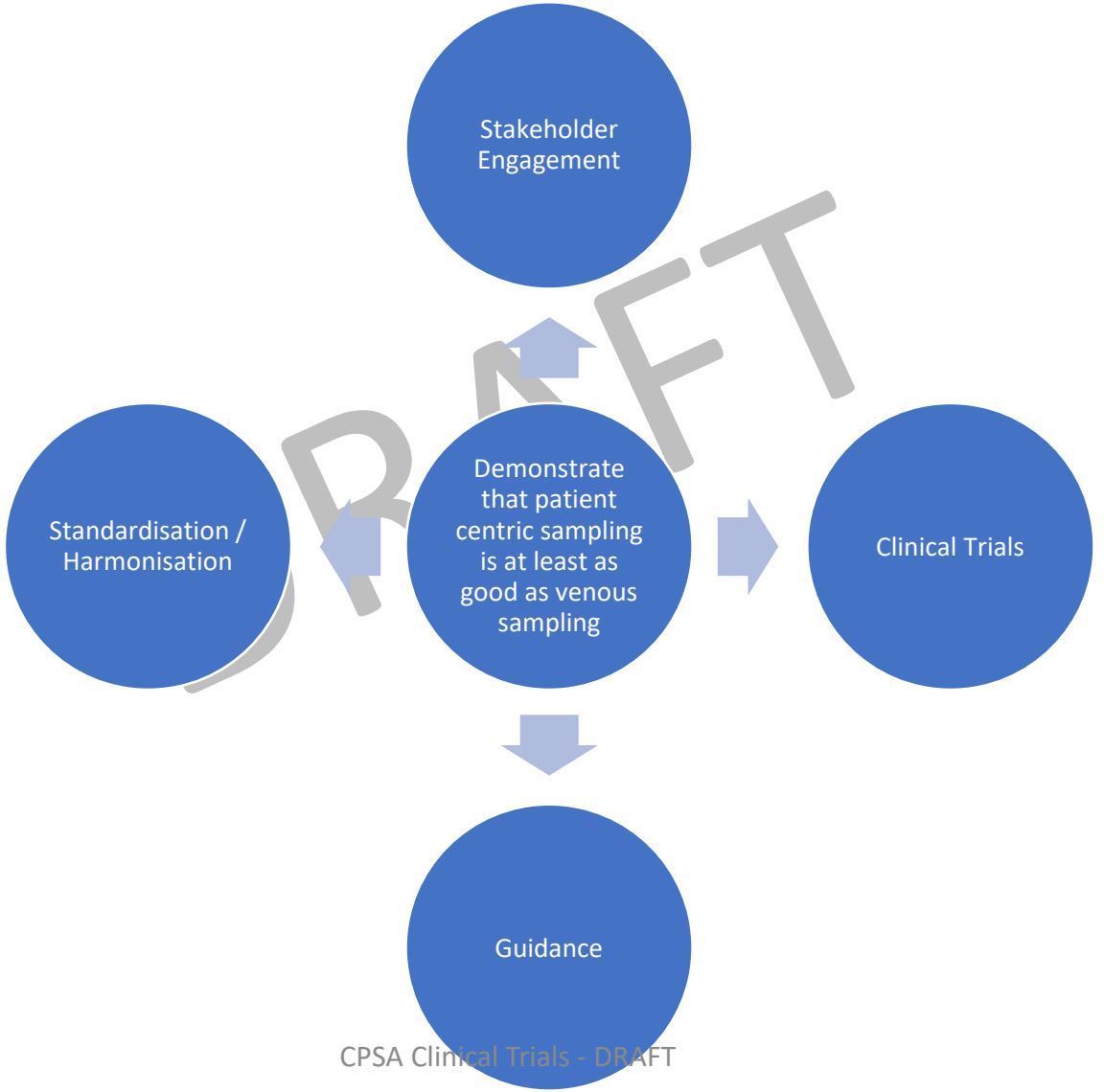
- Create standards – minimum required, rather than extensive & prescriptive
  - Technology components - devices
    - Patient identifier, GDPR, time stamp, etc
  - Logistics
    - How to package, send, receive and handle the samples
      - E.g. in the post, etc
    - Sample stability – assay validation vs sample monitoring
    - Connecting sample to donor – barcode, etc
    - What happens if sample goes into wrong hands?
      - Inclusion of personally identifiable information – e.g. EU / USA differences, Pharma / central labs differences
  - What level of bad samples / failed tests is acceptable?
    - How - Training materials
  - Patient safety – successful replacement of nurse / doctor
    - Proving what the sample is – linked to logistics
    - How - Bar codes, Apps, DNA
  - Laboratory procedures
    - Suitable minimum validation procedures – e.g.
      - Contamination during extraction
      - Dilution procedures – not just for MS
      - Selection of appropriate internal standard

Elements

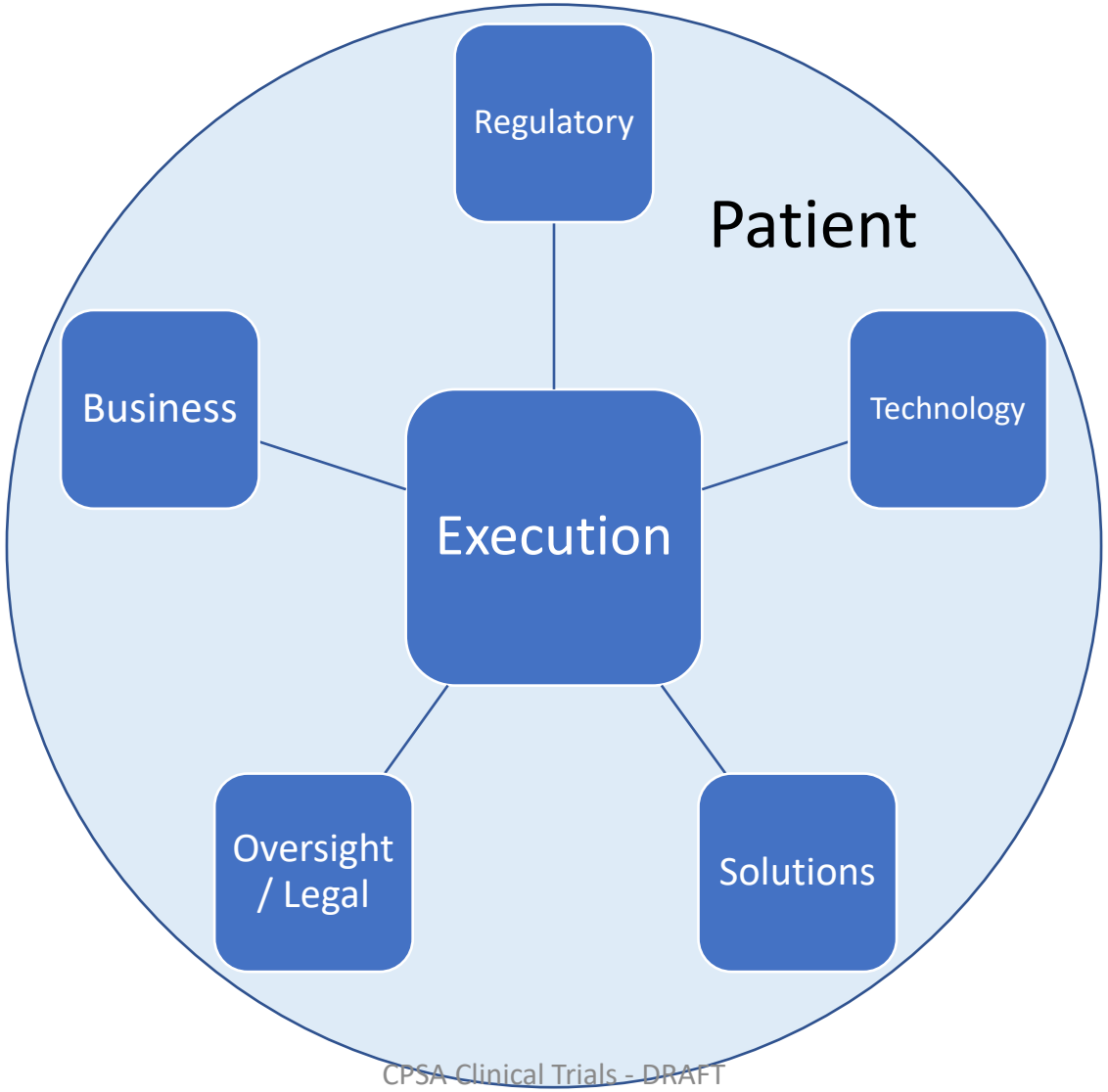

Economic benefits outline
Compare to "Gold Standard" methods
Integration with existing workflows
Healthcare Providers
Distribution Hub
Laboratory
Logistics
Benefit for the Patient

Outcomes

Must work with current workflows in addition to revolutionary workflows



Organisational Structure



Markets

- Patient Groups
- Healthcare providers
- Wellness
- Pharma
- Current instrument providers

NOT

- Current central labs
  - No impetus to change the status quo

Focus Areas

- Rare disease
  - ALD Connect
  - Duchenne UK
- Vulnerable groups
  - E.g. Transplant patients
    - Immunosuppressants and all other factors measure, e.g creatinine
- Unmet needs
- Orphan drugs
- Need lists of measurables that are a must have and a nice to have
  - Feedback from Regulators, Healthcare Systems, Pharma, etc
  - Leads into being able to create a standard, e.g. ISO

Membership

- Annual fee - $2-4K
  - Gives opportunity to participate in the Community
  - Pharma, technology companies, automation companies, etc
- Clinical trials vs Gold Standard (1-3 per year)
  - Equal share of trial costs paid by each company signing-up to each trial
  - Demonstrate that technology meets required standard
    - NOT comparison of technologies
- Quality Standard – CPSA Stamp
  - Demonstration that device and / or assay meets acceptable standard
  - $1K per assay and/or technology per year
  - Similar fee for analytical laboratories demonstrating that they meet the standard?