How can we build on existing registries?

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- Consultancy fees from Orphan Europe, a Recordarti Company.
- Consultancy fees from several other companies not conflicting with Fabry disease

How can we build on existing registries?

By not re-inventing the wheel

By looking at other Registries and organisations supporting Registries

By cooperation

How can we build on existing registries?

By not throwing out the baby with the bathwater

What is good about the current Registries?

What can be improved?

What do we really want from Registries

Registries—

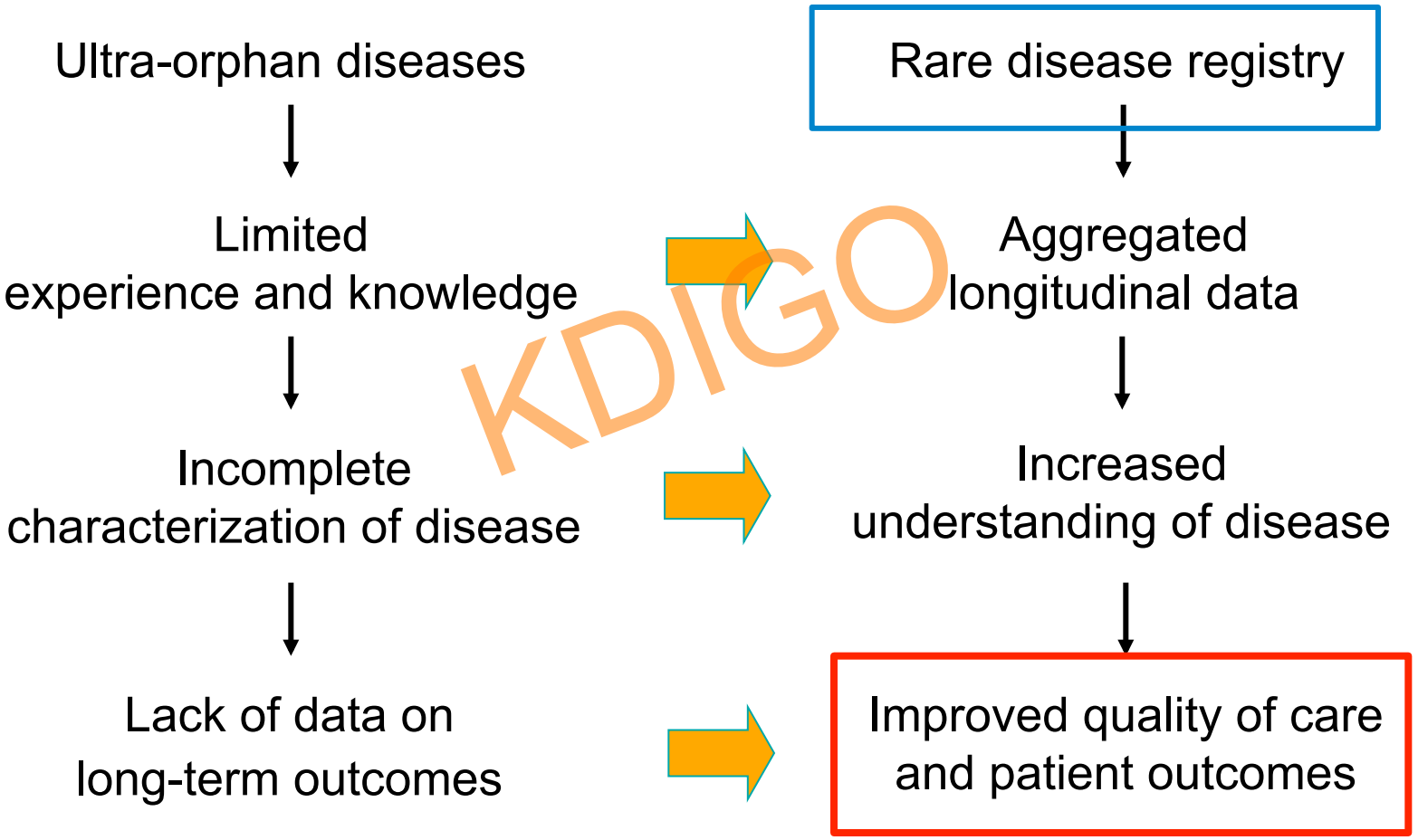
Are not CLINICAL TRIALS

"Whatever is done they can never be perfect"

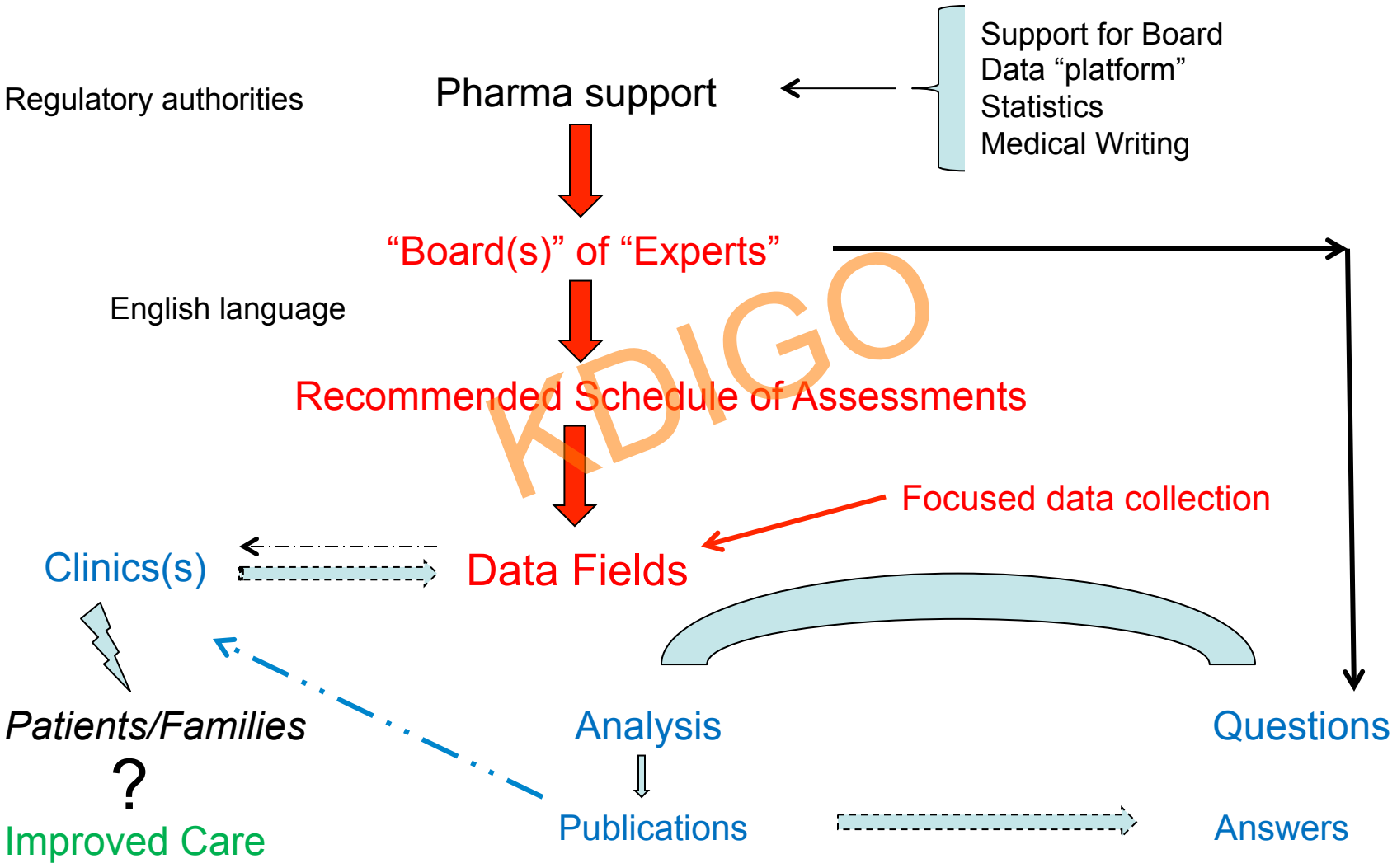
"They are an important Public Health and Clinical Tools

E.U. discussion document 2015

Fabry Registry Construct



Process Map—Current Situation



Gap Analysis—Assessments

- Who decides?
  - Limited input
- Too many
  - No prioritisation
- Language
  - Understanding
- Availability
- Patients???

Gap Analysis—Data

- All voluntary
- Entry decided by clinicians
  - "Carrots"
- Missing data
  - Big problem
- Selection bias
  - e.g. Males v Females v Age
- Issues of consent
  - Increasingly important

KDIGO

Verification difficult

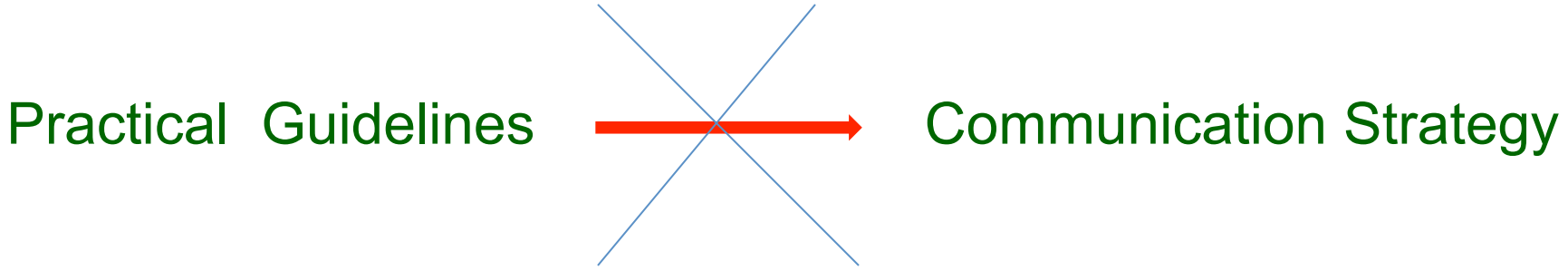
Variation in standards

Gap Analysis—Support

- Perceived Bias
- May limit participation
- May restrict patients populations
  - By treatment
  - By geographical area

Is there evidence that current registries have improved patient care?

NOT ? REALLY



Improvements—1 Assessments

- Greater involvement of stakeholders in PLANNING
  - Modified Delphi [or similar]
- Include the voice of the PATIENT
  - P.R.O.M.S.
  - Patient generated Q.o.L measures
- Prioritisation
  - Essential
  - Desirable
  - Optional

Country specific

Clinically specific

Age/Gender specific

?

Improvements—2 Data

- Better Ownership
  - Resolve the Data protection issues
- Understanding
  - Language
  - Importance
- Empowerment
  - Clinician
  - Patient

Clear Understandable Instructions/Guidelines

Patient driven
-- “patient view”

Communication

Verification
--quality control

Improvements—3 support

Pharma have done a good job till know

- Need to remove potential bias
- Need to increase access
  - Geographical
  - Therapeutic
  - Phenotypical
- Link with others—progress through cooperation
  - EDTA etc.
  - Europe—
    - European platform for rare diseases [EPIRARE]
    - EC Expert Group on Rare Diseases [EUCERD]
    - European Reference Network
  - USA
    - NIH/NCATS GRDR®

- National/International Rare Disease Policies Should be Supportive of Registries

Proposed New Process Map