HOW CAN WE BUILD ON EXISTING REGISTRIES?

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Disclosure of Interests

- Member of the Fabry Registry Board (Europe) supported by Gezyme, a Sanofi Company. Also received grants for research and consultancy fees.

- Research grants and consultancy fees from Shire HTC.

- Research grants and consultancy fees from Amicus Therapeutics.

- Consultancy fees from Orphan Europe, a Recordati 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

Ultra-orphan diseases

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Limited experience and knowledge

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Incomplete characterization of disease

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Lack of data on long-term outcomes

Rare disease registry

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Aggregated longitudinal data

↓

Increased understanding of disease

↓

Improved quality of care and patient outcomes
Process Map—Current Situation

Regulatory authorities

Pharma support

“Board(s)” of “Experts”

Recommended Schedule of Assessments

English language

Clinics(s)

Data Fields

Focused data collection

Patients/Families

? Improved Care

Analysis

Publications

Questions

Answers

Support for Board
Data “platform”
Statistics
Medical Writing
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

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

Practical Guidelines  Communication Strategy
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

  \[ \begin{align*}
  \text{Country specific} \\
  \text{Clinically specific} \\
  \text{Age/Gender specific}
  \end{align*} \]
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 now

- 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

- Proposed
- New
- Process
- Map

Schedule of Assessment

- Independent Support
- Regulators
- Industry

- Essential
- Desirable
- Optional

Board

- Fixed Term
- Elected

Communication Strategy

- Improved Patient Outcomes

KDIGO

- Modified Delphi
- Patient Generated Data

Clinicians

- Data

Patients

Analysis

Publication