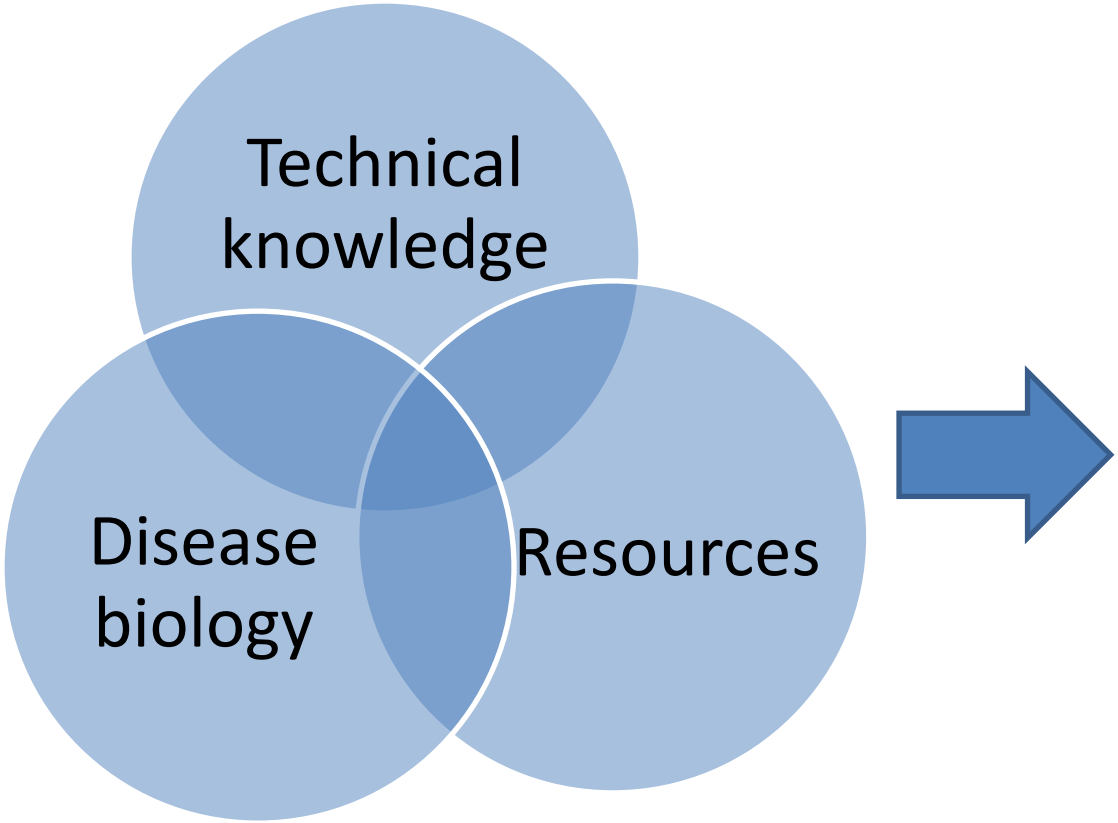
Innovative Medicines Initiative

DEVELOPING AN AETIOLOGY-BASED TAXONOMY OF HUMAN DISEASE

Duncan McHale
UCB

Need for public-private collaboration



Change > 200 years of disease classification

Objectives of the full project

- Propose new aetiological / mechanism based taxonomy in 2 disease areas
  - Immunoinflammatory disorders (RA and SLE)
  - Neurodegeneration (AD and PD)
- Initial clinical validation
- Create vision of new mechanistic approach to the classification of human disease

Pre-competitive nature

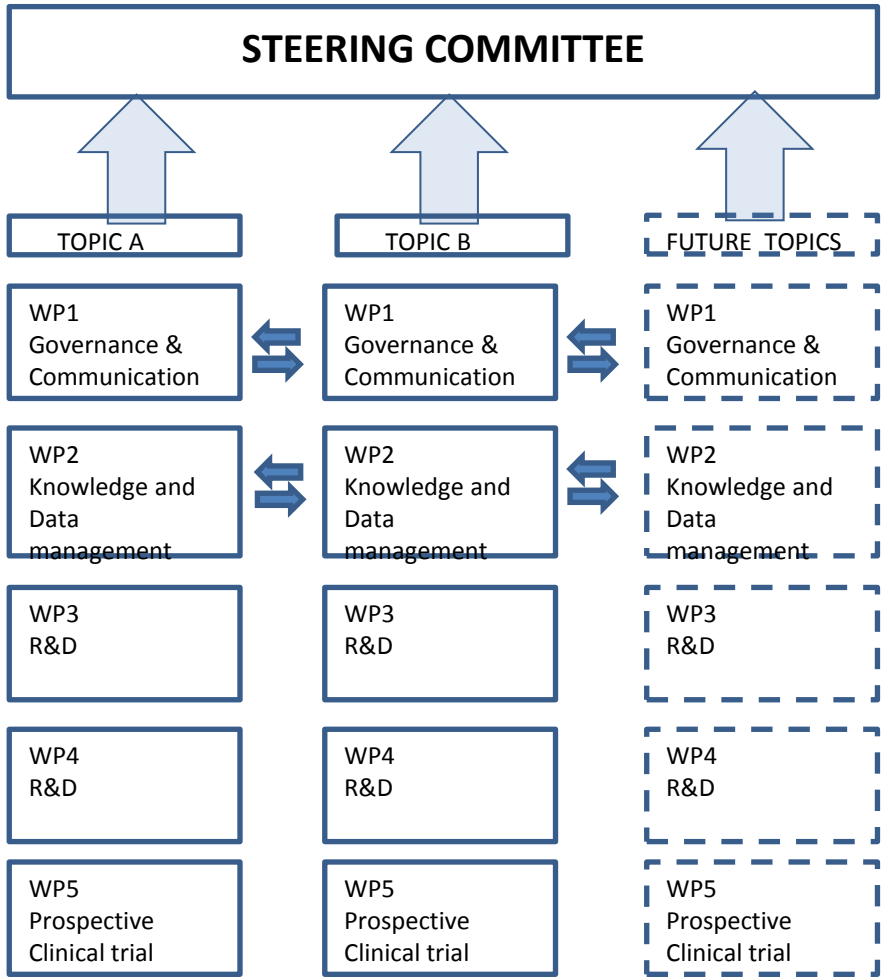
- Mechanistic classification of disease **is pre-competitive** and will help
  - Patients,
  - Physicians
  - Regulators
  - Industry
- Agreed new classification will aid in driving getting the right drug to the right patient

Expected impact on the R&D process

- Increased homogeneity of disease will:
  - Decrease trial sizes
  - Improve benefit risk profiles of drugs
  - Increase speed to patients
- Improved understanding of the disease
  - Ensures patients can access the right treatments for them regardless of phenotypic presentation
  - Increased confidence in target
- Reduce drug discovery costs by impacting attrition

Suggested architecture of the project

- Build evidence base from:
  - Literature
  - Current data
  - Shared data
- Develop potential new classification
- Prospective validation



Expected contributions of the applicants

- Disease area expertise
- Multidisciplinary approach
- Access to pilot data and samples
- Access to patients to test new classification system
- Thinking beyond current confines of “today’s phenotypic classification”

Expected (in kind) contributions of EFPIA members

- Baseline clinical data from clinical trials
- Some molecular data and samples
  - Genetics
  - White cell mRNA
- Pharmacology expertise from current therapies
- In-vivo assay expertise
- Informatics and biostats expertise

What's in it for you?

- A mechanistic taxonomy
  - Optimise benefit risk of therapies (remove patietns with no chance of responding)
  - Identifies patients likely to benefit from new therapies despite different phenotypic features
  - Reduce overall cost of clinical development by reducing trial sizes and increasing success
  - Improves academic research by providing more homogenous diseases to study

Key deliverables of the full project

- Proposed taxonomy for:
  - RA
  - SLE

  } Topic A

  - AD
  - PD

  } Topic B
- Initial validation
- Formal validation plan
- New diseases nominated

Taxonomy call

- 2 topics initially to form 2 separate projects
- The successful applicant consortia will work together where approaches are similar to create harmonisation
- Expect successful applicants to collaborate with other initiatives which will add value to this one e.g. eTRIKS
- If successful then additional calls focussing on other disease areas will follow

Questions?

- Contact the **IMI Executive Office**

E-mail: infodesk@imi.europa.eu

Website: www.imi.europa.eu