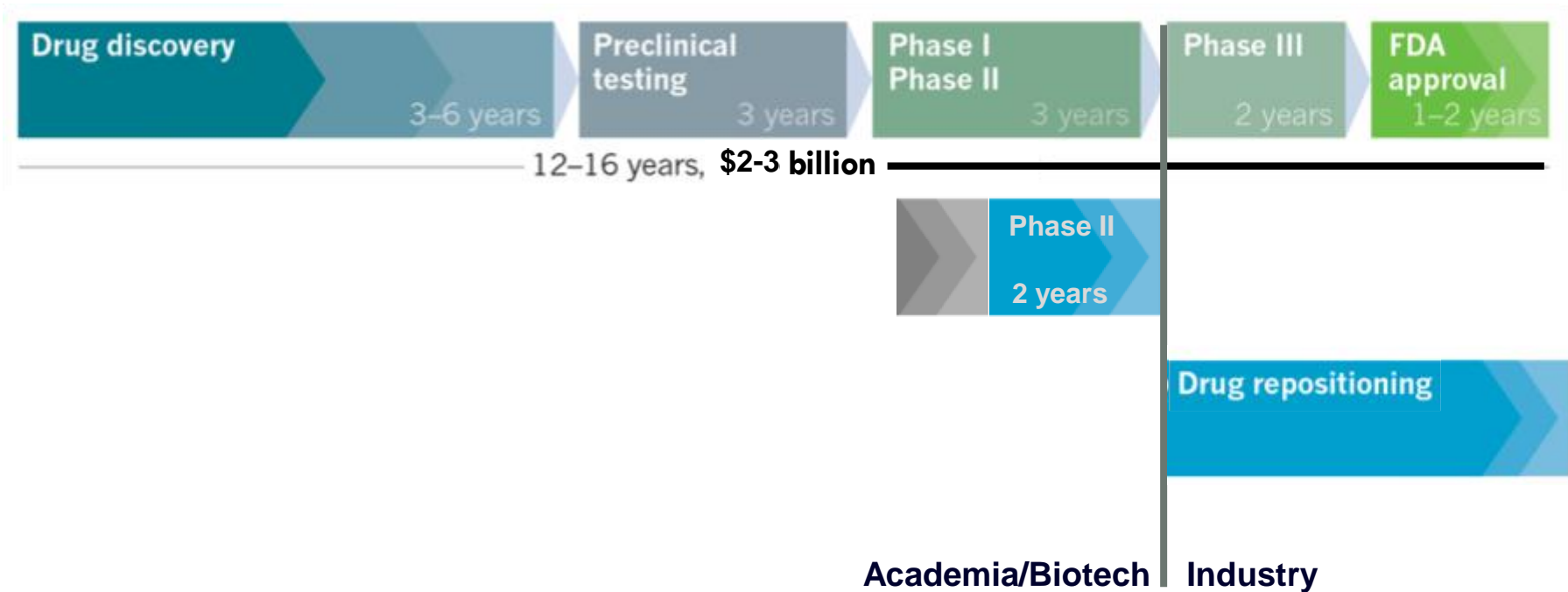


# Network Medicine and Pharmacology for Rare Diseases

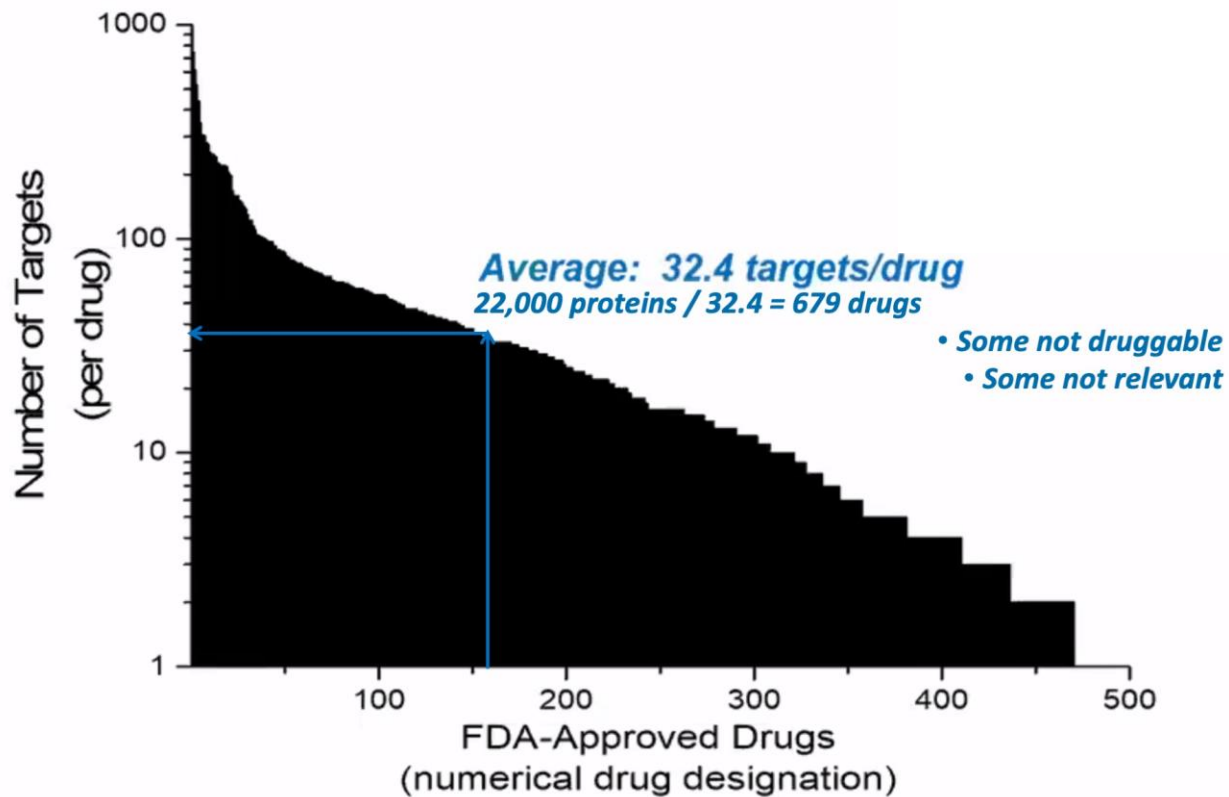
Harald H.H.W. Schmidt, MD, PhD, PharmD

Coordinator REPO4.EU

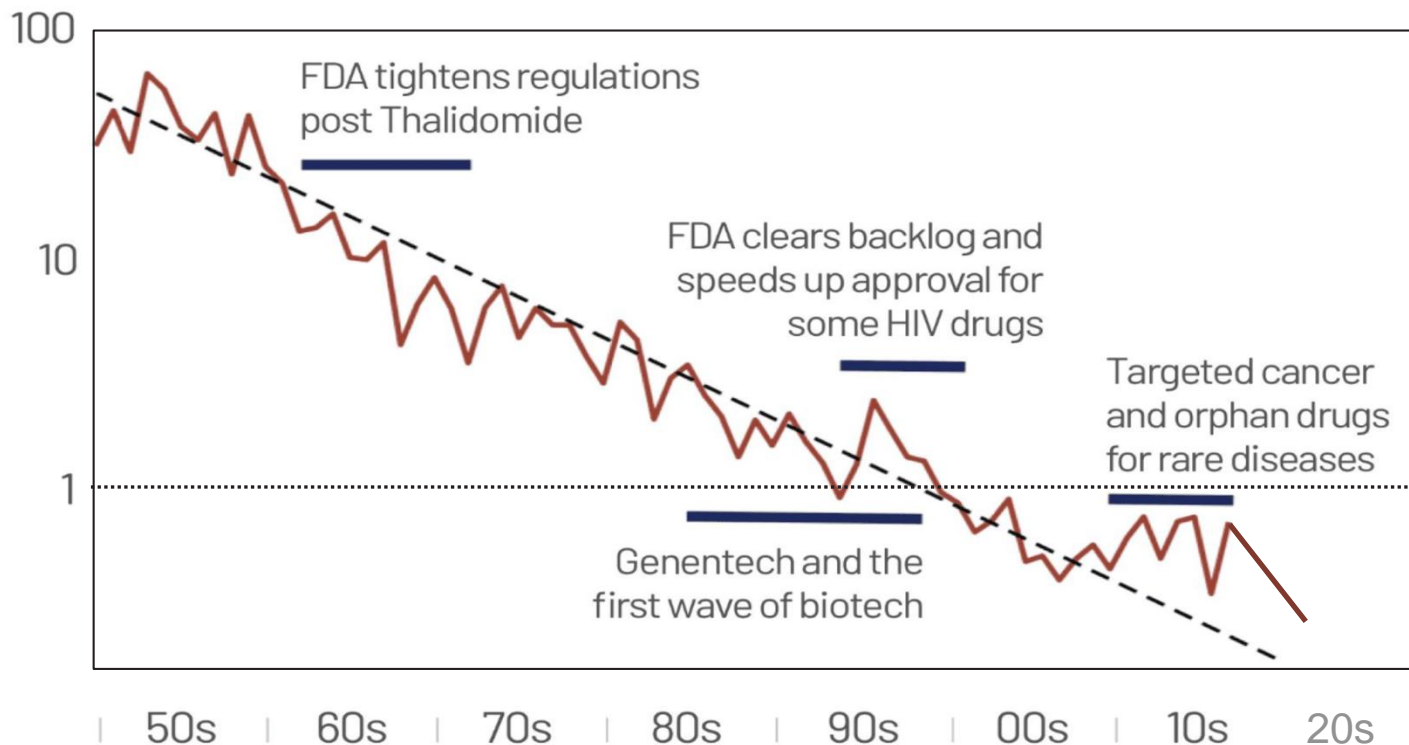
## Drug repurposing replaces drug discovery



## Drug Repurposing goes beyond registered targets



## Drugs per \$1B R&D: Eroom's Law



# Existing drugs lack precision: High numbers needed to treat (NNT)

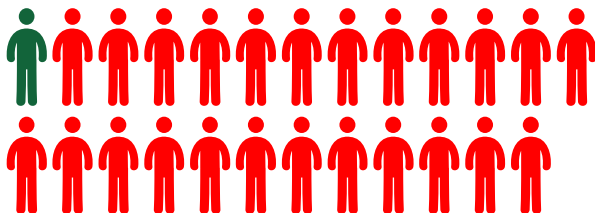
**ABILIFY (aripiprazole)**

Schizophrenia



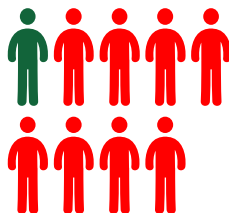
**NEXIUM (esomeprazole)**

GI



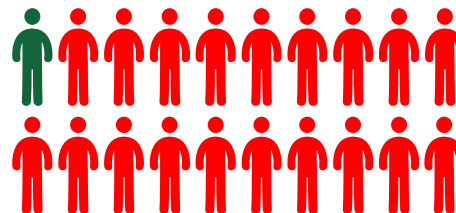
**CYMBALTA (duloxetine)**

Depression



**ADVAIR DISKUS (fluticasone propionate)**

Asthma



**ENBREL (etanercept)**

Psoriasis



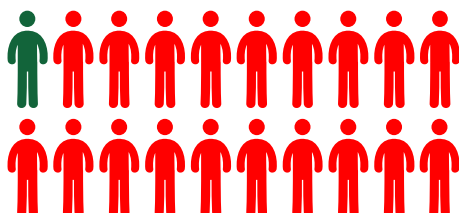
**HUMIRA (adalimumab)**

Arthritis



**CRESTOR (rosuvastatin)**

High cholesterol



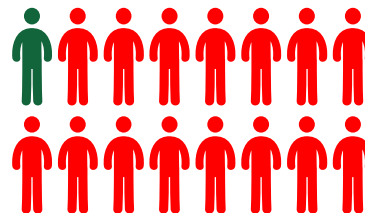
**REMICADE (infliximab)**

Crohn's disease



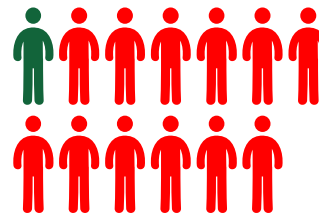
**COPAXONE (glatiramer acetate)**

Multiple sclerosis



**NEULASTA (pegfilgrastim)**

Neutropenia



# 19<sup>th</sup>/20<sup>th</sup> Century Diseases and the Structure of Medicine

## By organ

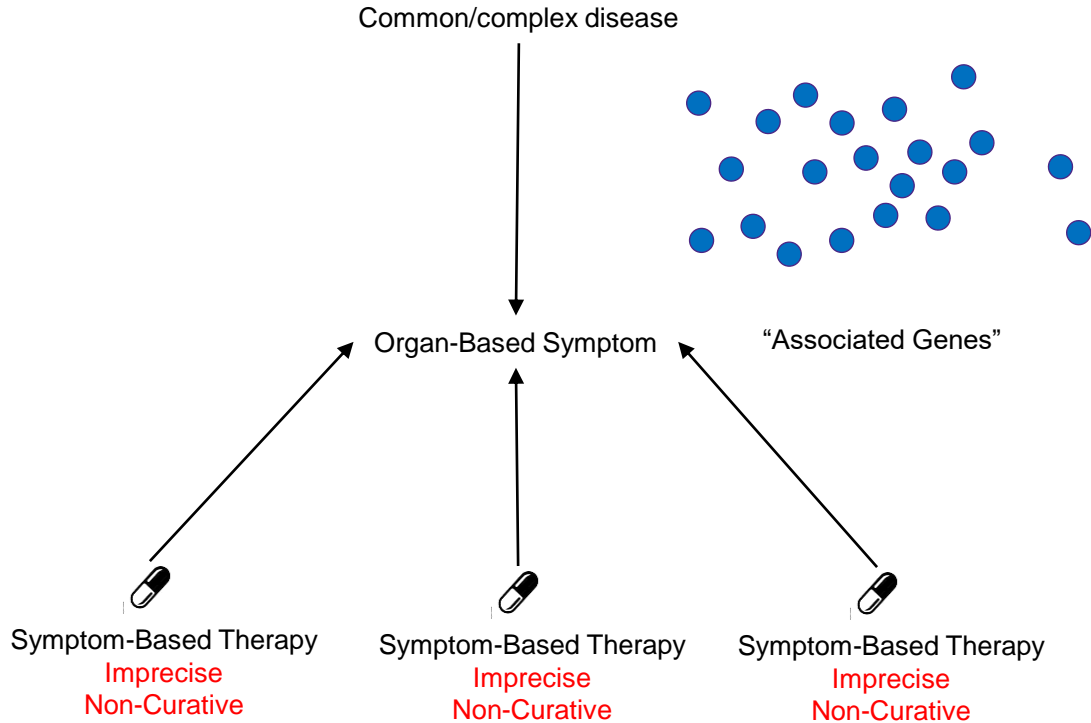


## By the name of a doctor



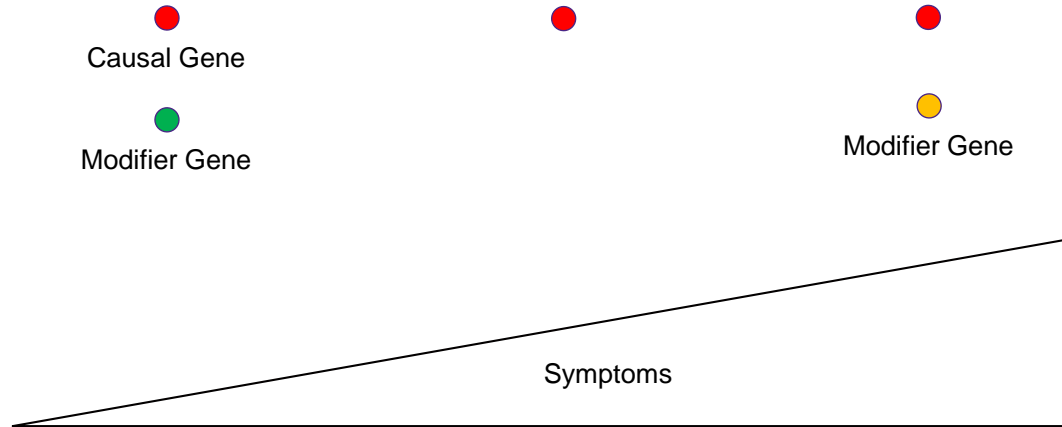
## By symptom

# Most diseases are defined by organ or symptom

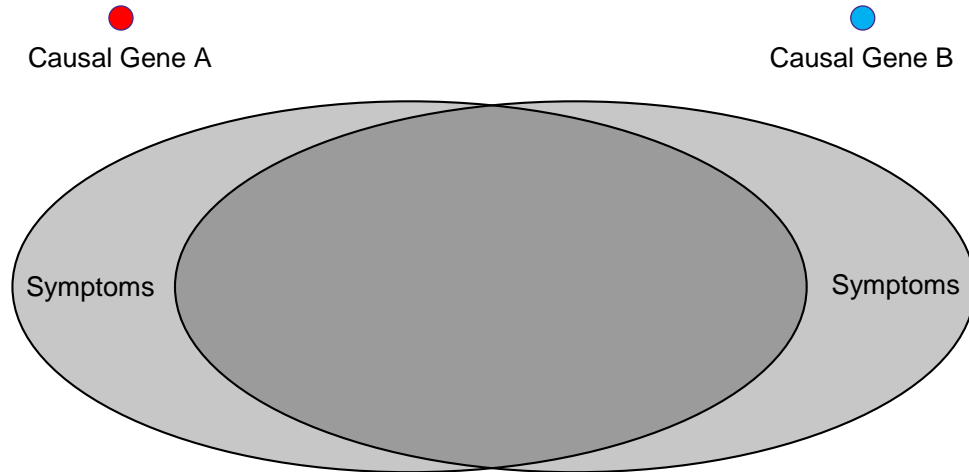




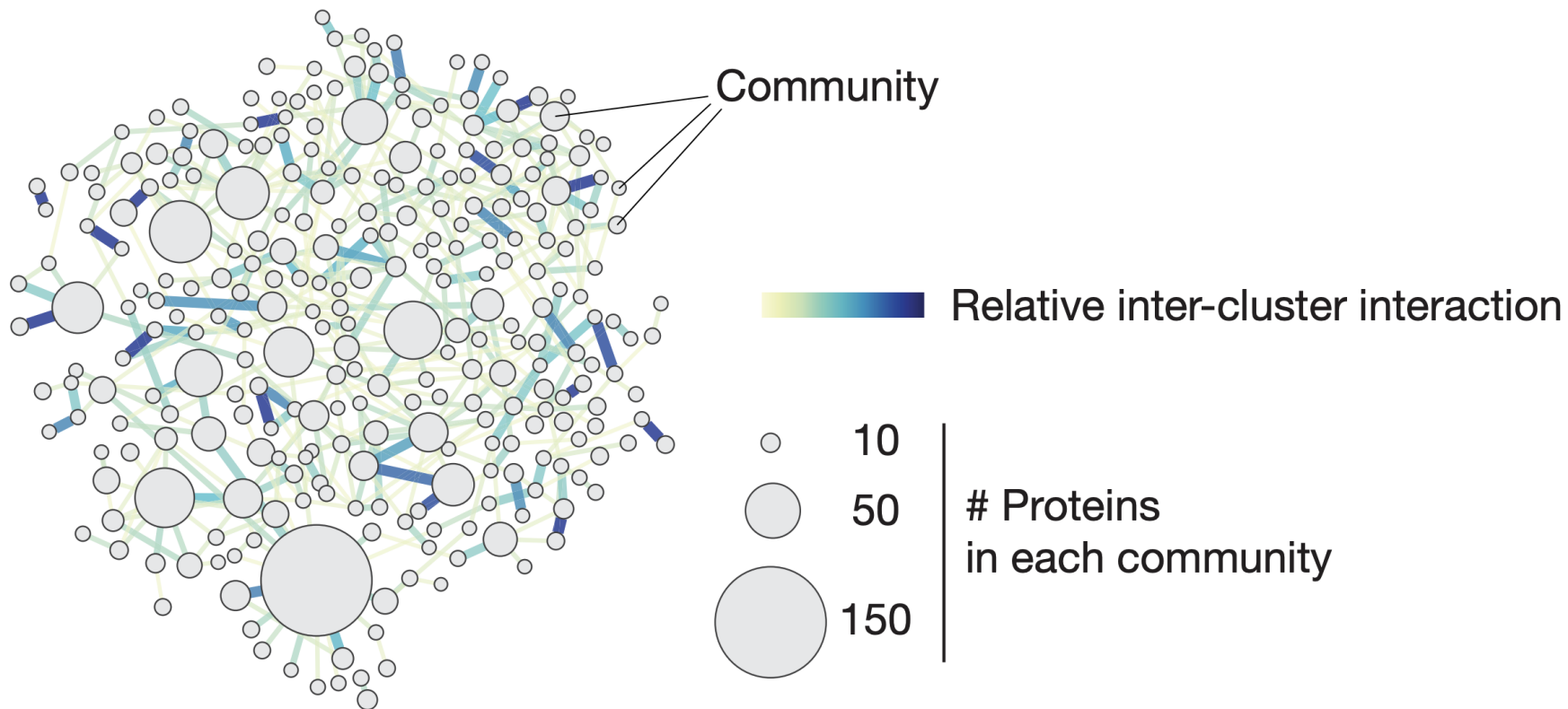
# ... how monogenic are monogenic rare diseases?

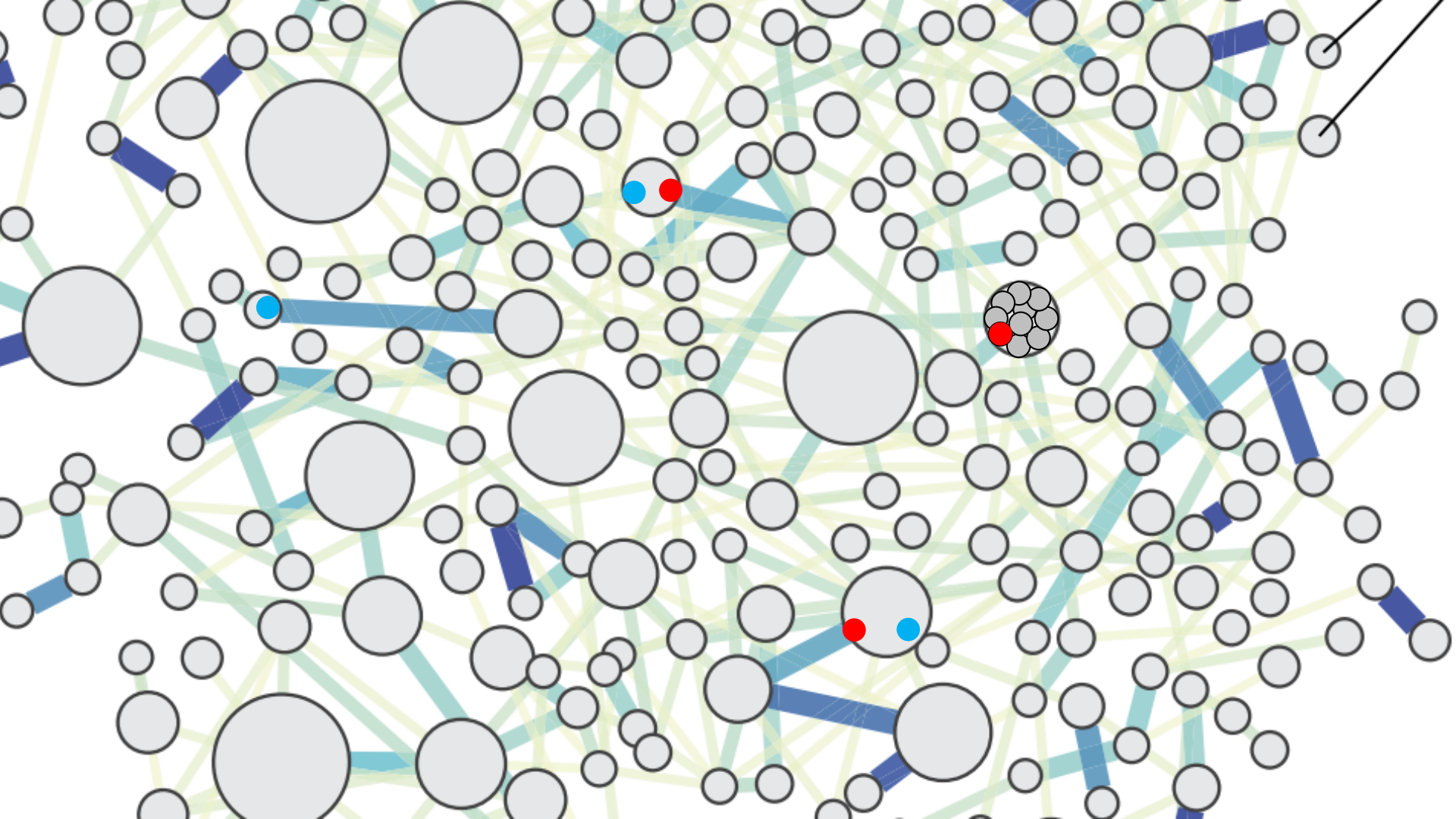


... how different are different monogenic rare diseases

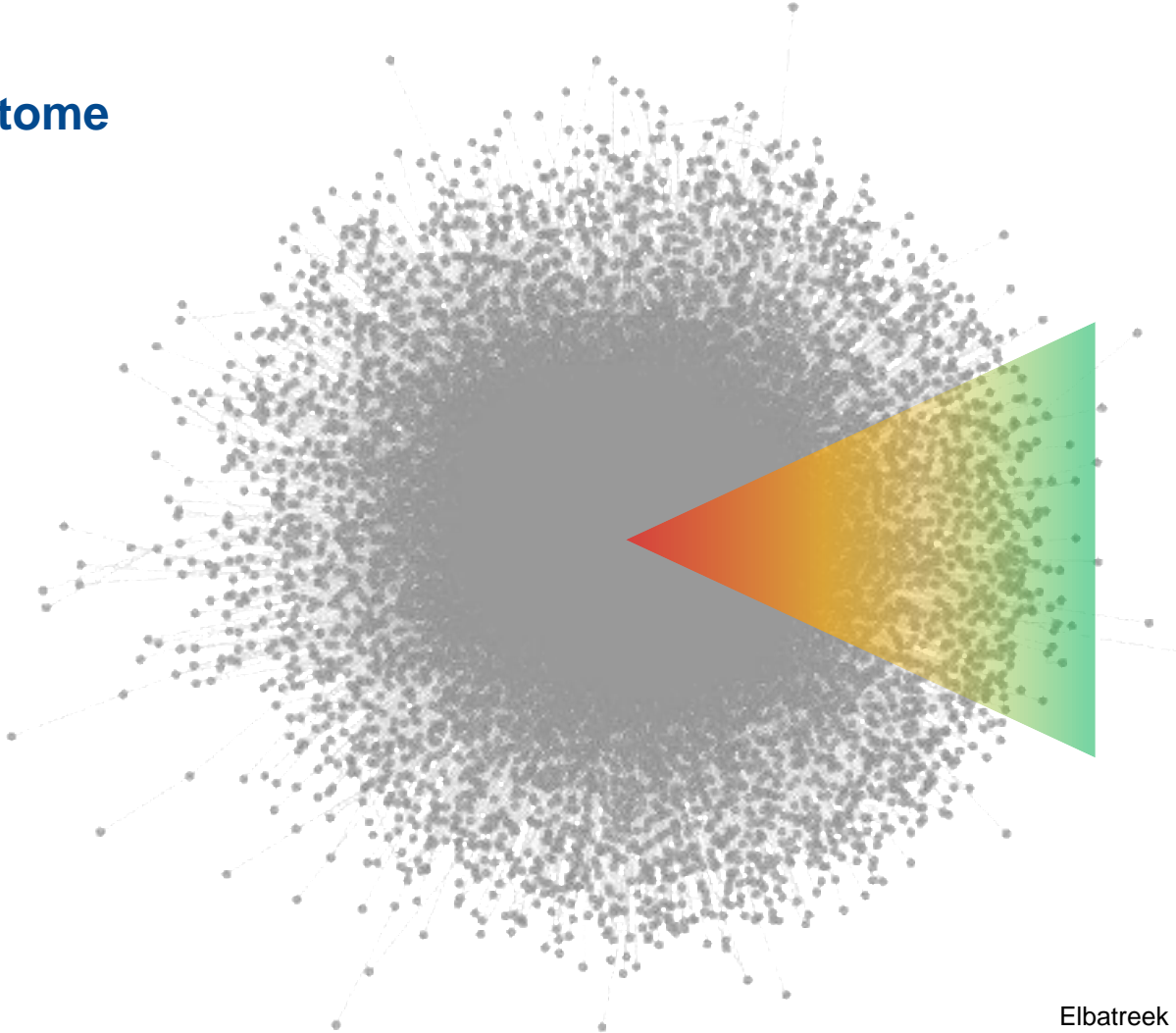


# Single cell proteomics revolutionise our understanding of disease

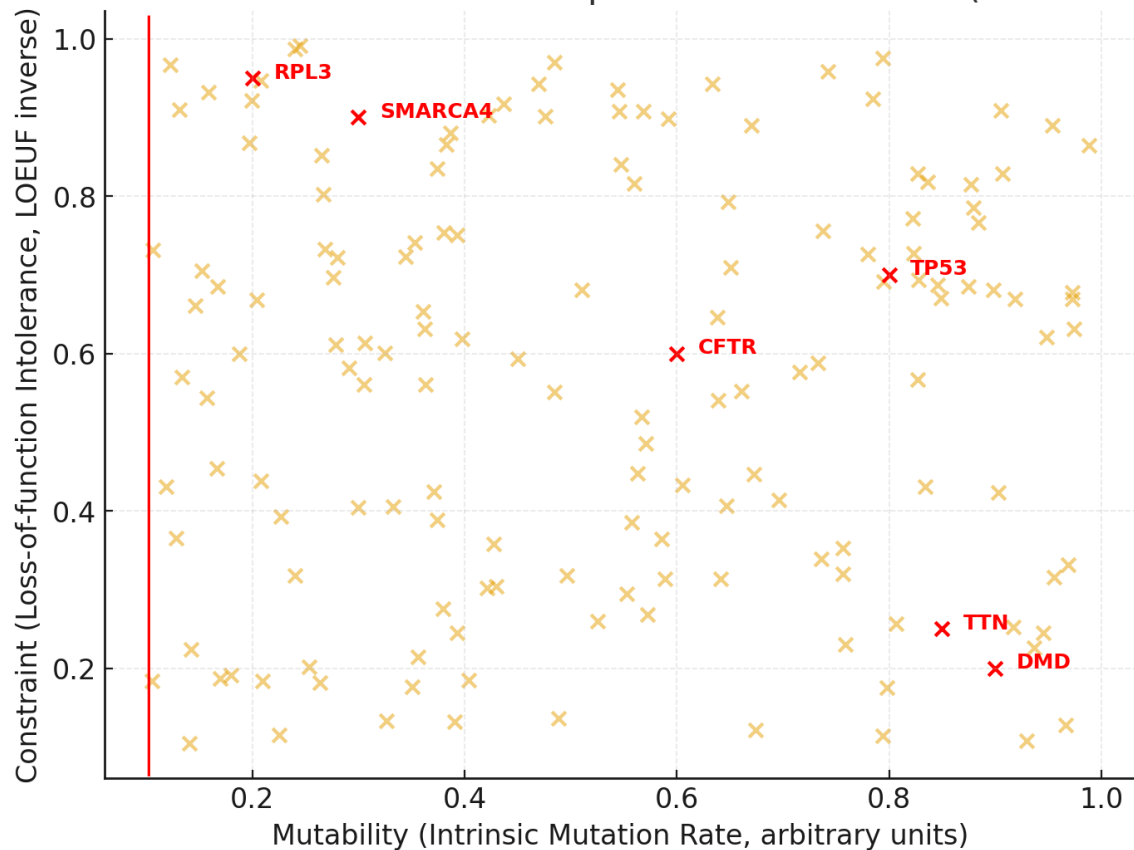




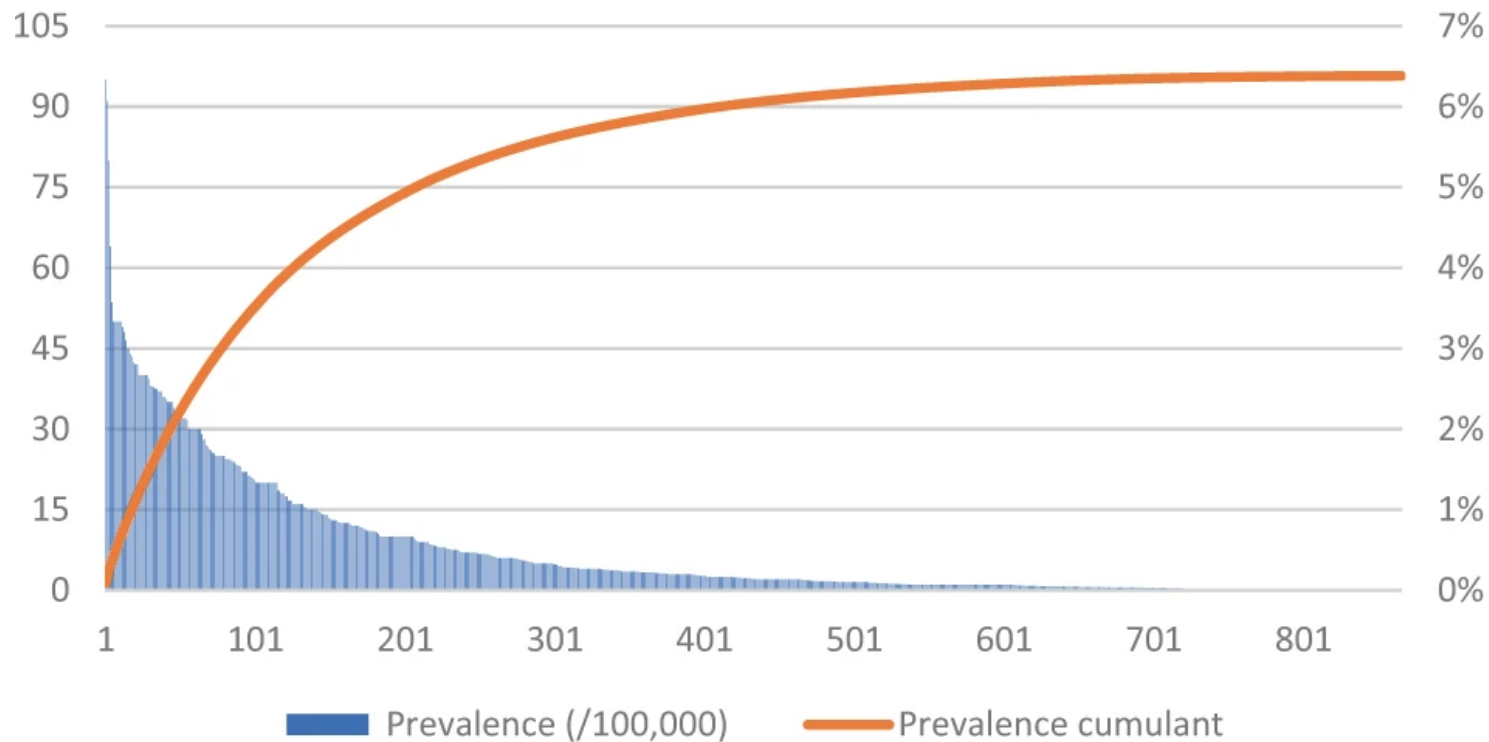
# The interactome of PPIs



## Mutability of genes vs. disease outcome

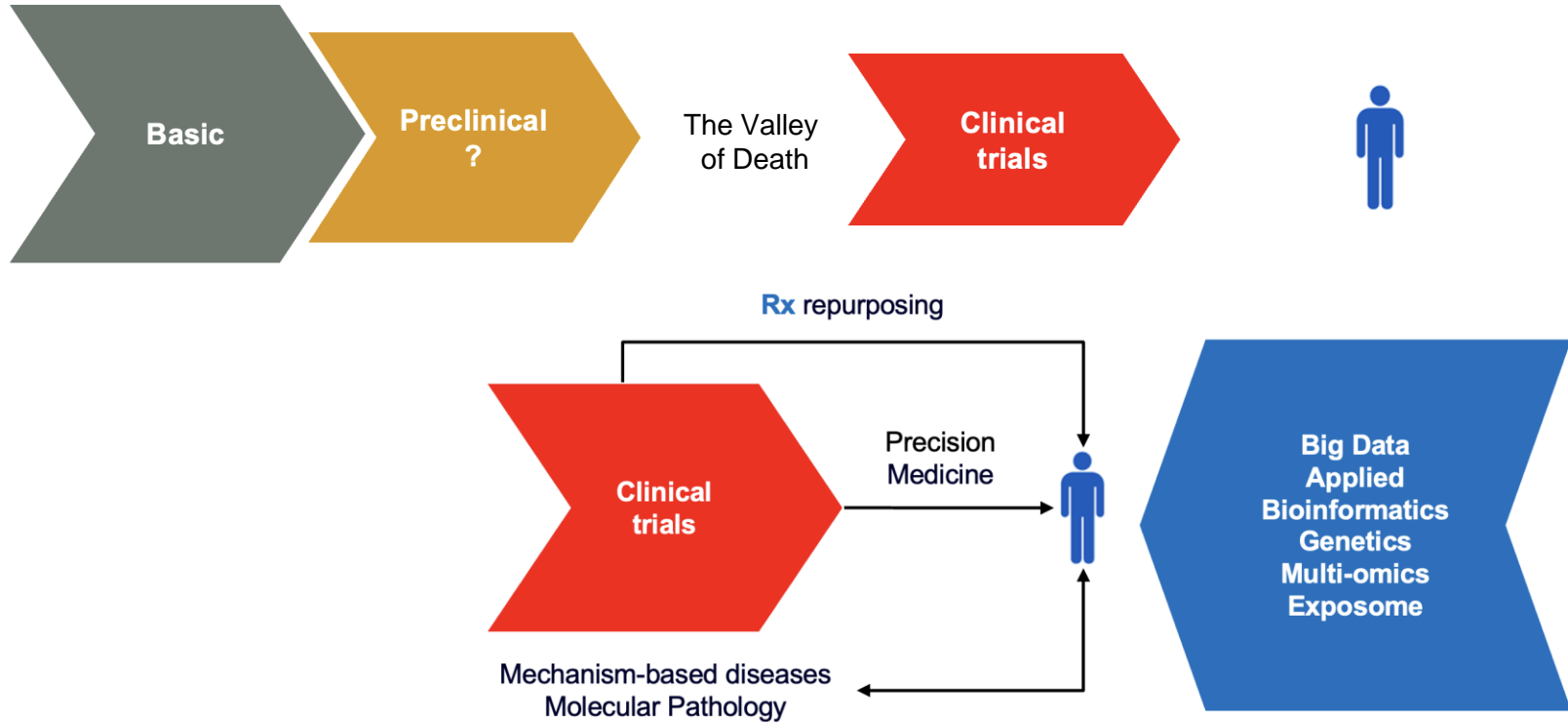


## Rare vs. common: regulatory and an epidemiological gradient



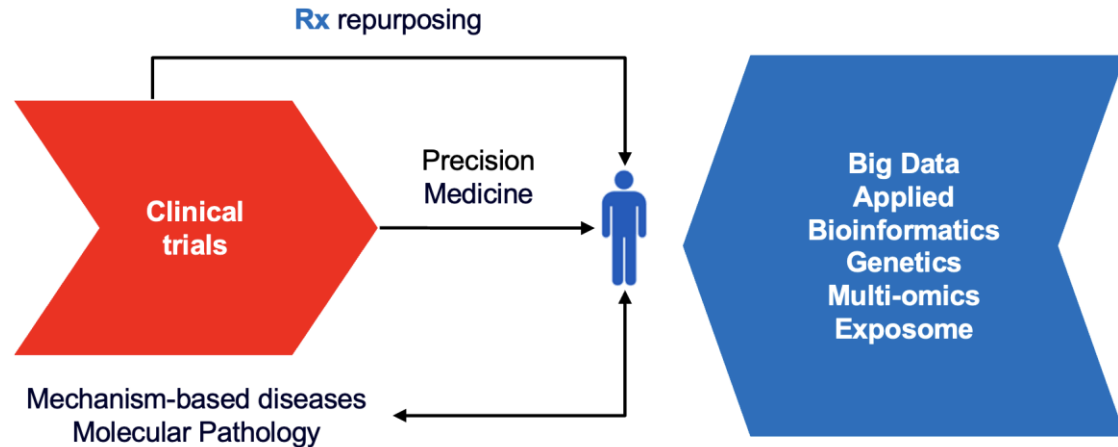
# REPO4EU's paradigm shift

From "Translational Medicine" to "Network and Systems Medicine"



# REPO4EU's paradigm shift

From “Translational Medicine” to “Network and Systems Medicine”



# The Euro-Global Platform for Mechanism-based Drug Repurposing

From imprecise drug therapy  
to precision medicine



Funded by  
the European Union