Breaking silos via public-private partnerships

A paradigmatic example from AIMS-2-TRIALS, an Innovative Health Initiative

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Disclosures

- Julian Tillmann is an employee of F. Hoffmann-La Roche Ltd

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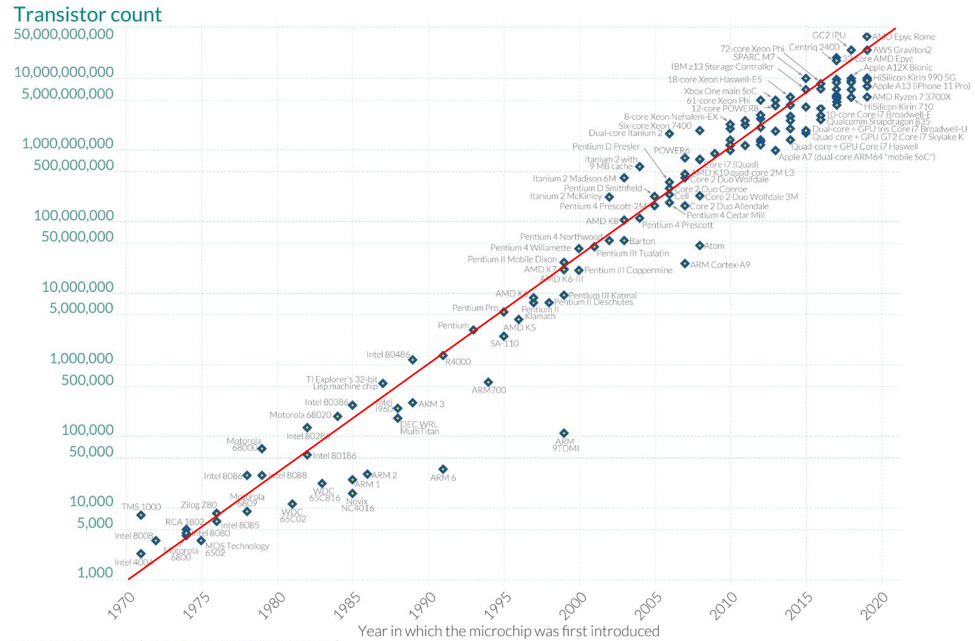
Exponential Progress: Moore's Law and Genomics

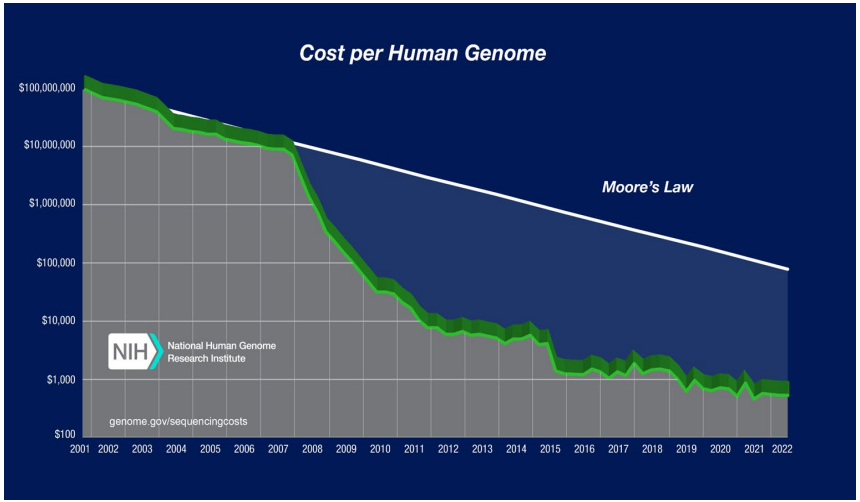
Doubling compute and halving sequencing costs every two years

Doubling of computing power every ~2 years



Data source: Wikipedia (wikipedia.org/wiki/Transistor_count)
OurWorldinData.org – Research and data to make progress against the world's largest problems.

Super-exponential decline in cost per genome

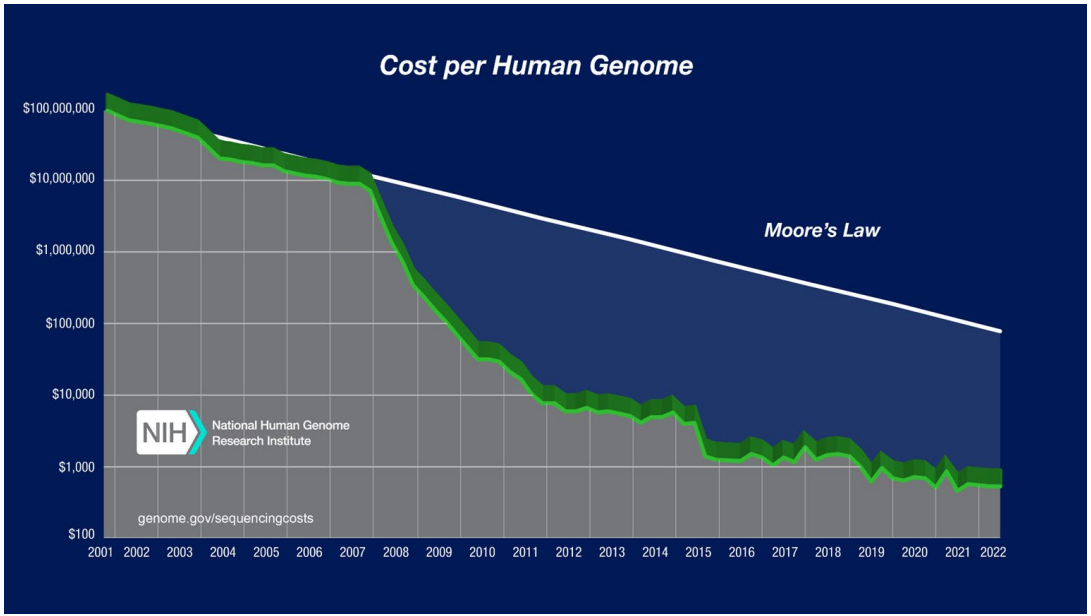


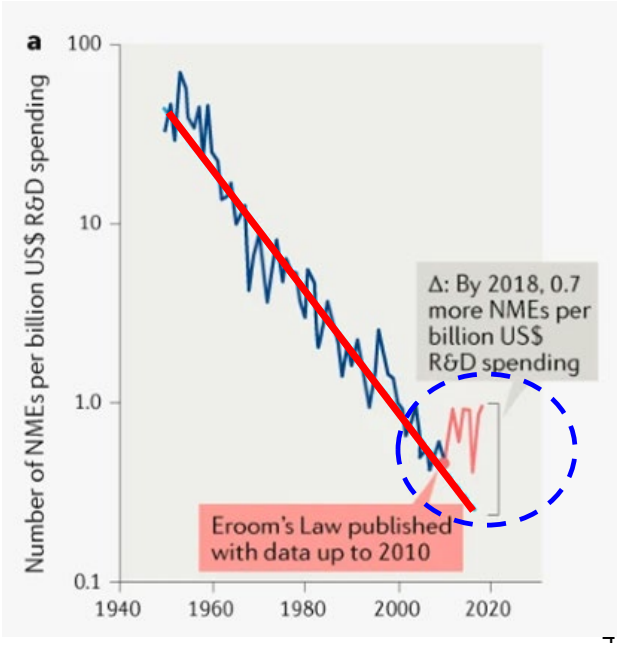
National Human Genome Research Institute (NHGRI) Cost per genome data - 2022

The R&D Efficiency Crisis: Moore's vs. Eroom's Law

Technological growth is not translating into R&D productivity

Scannell, et al Nat Rev Drug Disc, 2012
Ringel, et al Nat Rev Drug Disc, 2020





Why Public-Private Partnerships are Essential

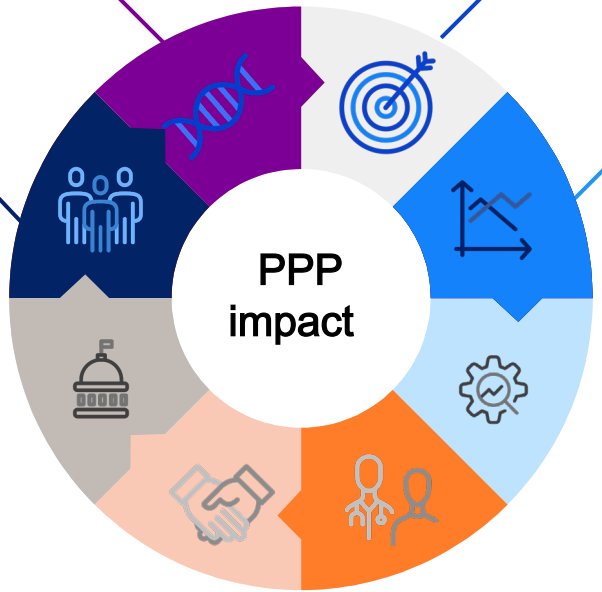
Breaking R&D silos to address common innovation challenges in precision neuroscience

Biomarker development

- Broad array of candidate biomarkers and novel technologies developed/tested

Cohort studies

- Large-scale deeply phenotyped longitudinal cohorts to unlock new insights



Accelerate innovation of breakthrough science & understanding of foundational targets

Sustained focus at scale

- Needed to enable discovery or translation;
- Needed to create sustainable data assets and complex analytical pipelines

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"A rising tide lifts all boats"

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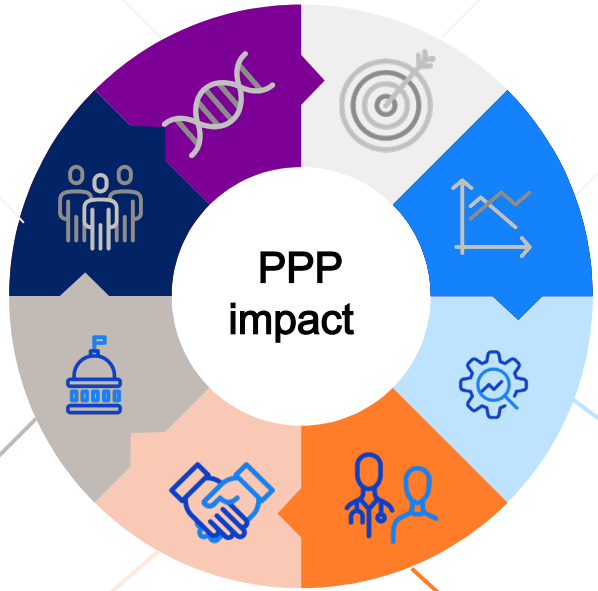


Regulatory acceptance
- Qualification of new technologies; engage in scientific exchanges

Standardized frameworks and models

Community co -creation

Multidisciplinary expertise & centers of excellence

AIMS-2-TRIALS

Apply a precision health approach to autism

First joint EU-North American trial

2 PhII trials

EU-wide clinical trials network with trial-ready cohorts

Clinical Trial Network with recontact

Trial capactiy & trial-ready cohorts

Co-creation through lived experience

Autistic advocacy shapes the research agenda

>82 active research engagements with a panel of 30+ autism representatives

Sustained policy impact

Supported European Brain Council Value of Treatment project
ACCESS-EU study, 10 points for change

Data & analytics infrastructure

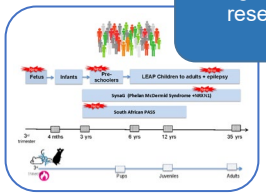
Data lake of deeply characterized cohorts

Secure, centralised database to deliver multidimensional analysis-ready datasets

Global legacy

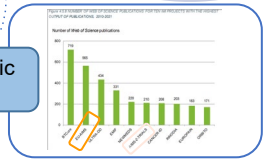
Integrated in EU data space

Longitudinal biomarker research platform



EMA letter of support for biomarker

High scientific impact



2x Top 10 IHI projects

Loth et al, Lancet Psych, 2014
Oakley et al, Nat Rev Drug Disc, 2022

Scaling infrastructure: Trial-ready cohorts &capacity

Example: European Autism Genomics Registry & Clinical trial network

EAGER
European Autism GEnomics Registry

Bloomfield et al, BMJ Open, 2024

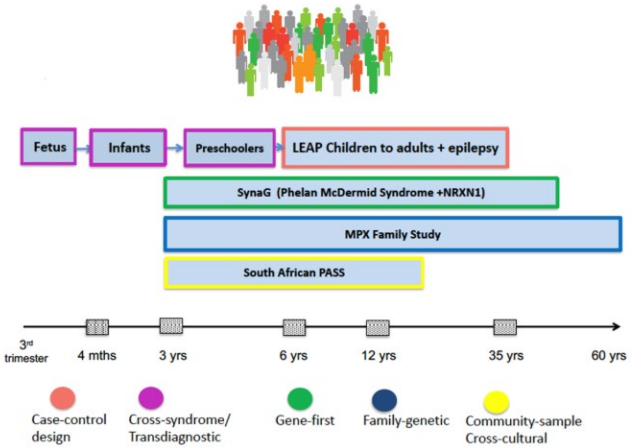
Ashwood et al, Eur Child Adol Psych, 2015

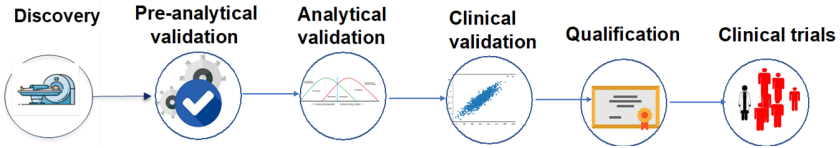
- >1,200 individuals with either autism or a rare genetic disorder: Whole -genome sequencing & consent for recontact for future research studies or clinical trials
- Design for federated data sharing

- 120 sites across 37 countries , with access to >28,000 newly diagnosed autistic individuals each year
- Supported recruitment of industry -led and PI sponsored trials

Clinical cohorts and pipeline for biomarker validation

Shared platform for significant advances from biomarker discovery to regulatory qualificati





Loth et al, Mol Aut, 2017

- **N>2500 participants in Europe and >1,400 children in South Africa** comprehensively characterized in clinical, cognitive and behavioral profiles, brain structure and function, genetics/genomics, environmental factors
- **First EMA qualification advice** for novel biomarker in autism
- **15 high candidate markers** at various stages of validation - Small Medium Enterprises approached consortium to implement pipeline
- **Scientific & data legacy** : Sustainable data sharing via the ELIXIR-LU platform

Public-Private Partnerships: An essential lever for precision neuroscience

Break up the ‘siloed’ R&D model to address common challenges

Key impacts		Remaining challenges
De-risking innovation through shared platforms & cohorts: Reduce barriers of entry &share risks and costs across stakeholders	›	**Federated data infrastructure:** Technological, legal and data standardization hurdles
Standardization & regulatory interactions: Develop standardized models, biomarkers &drive regulatory acceptance	›	**Global alignment:** Expand alignment with global initiatives and across regulatory & health authorities
Re-contactable cohorts : Capacity-building to make further research and clinical trials tractable	›	**Sustainability & impact:** How to transform assets into durable infrastructure

Doing now what patients need next