

# **Personalised HealthCare / Stratified Medicine An Industry Perspective**

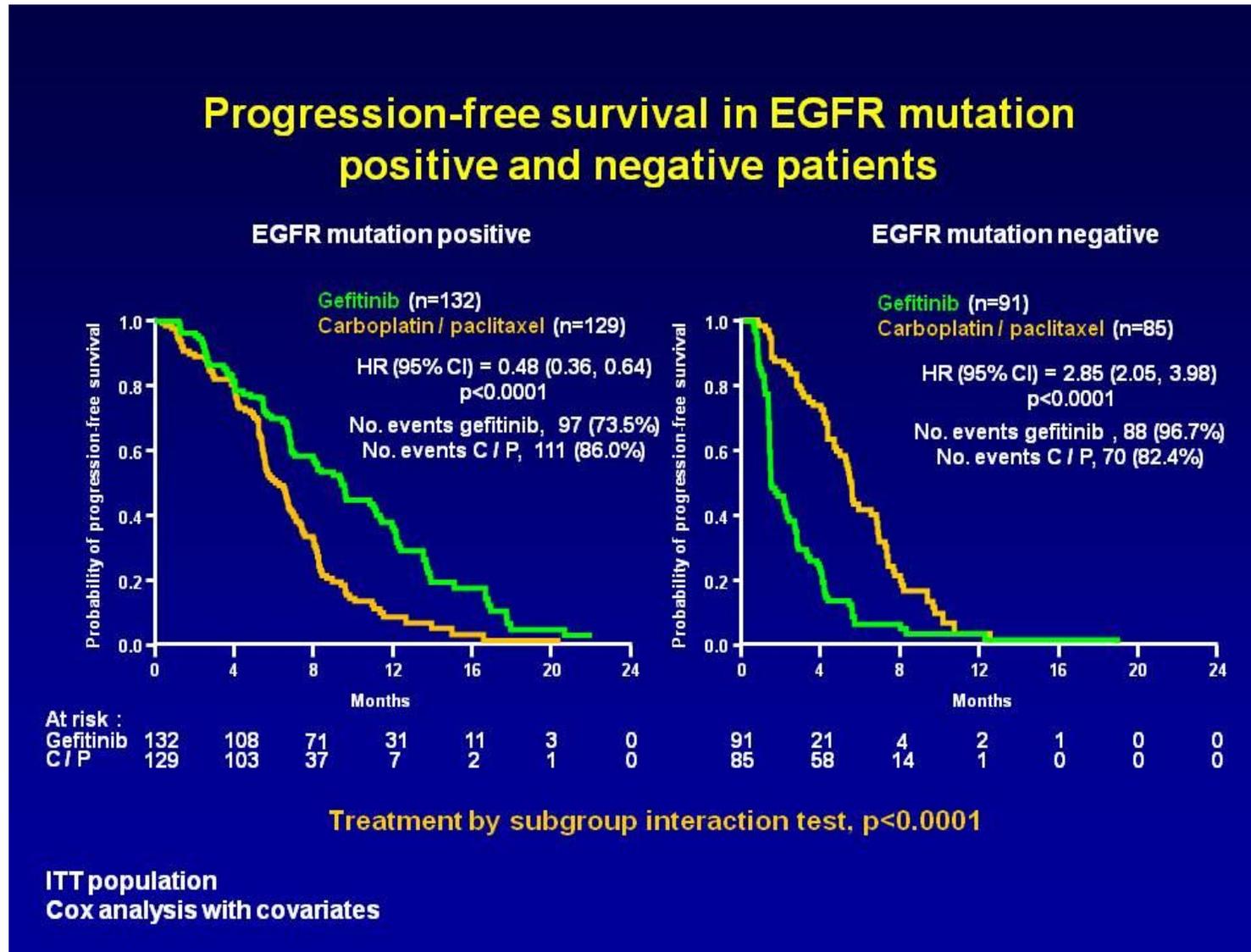
**Chris Harbron  
Senior Principal Statistician**

**2013 Network of Hubs for Trials Methodology  
Annual Meeting**

**4<sup>th</sup> February 2013**



# A Success Story : Iressa



2 Now marketed in over 64 countries



# Iressa

## What Made Iressa A Personalised Success?

- Strong scientific rationale
- Unmet medical need
- Robust test
- Efficacious & tolerable drug (in the right patient population)

**Strong Evidence  
for the Drug-  
Diagnostic  
Combination**

- **BACKED UP BY ACTIVITIES TO ENSURE:**
- Awareness amongst physicians
- Availability of diagnostic kits
- Understanding and overcoming barriers to testing

**Adoption and  
availability to  
patients**

- PHC will be an integral part of our business
  - Decreased pipeline attrition
  - Increased rates of successful launch and reimbursement
  - Increased value and market access



# Personalised HealthCare In AstraZeneca

## •Dedicated Personalised Healthcare and Biomarkers

### Organisation

- Diagnostic and Stratification strategic experts
- Science and Validation technology and laboratory expertise
- Strategy, Portfolio & Alliances
- Translational Science Unit Karolinska

## •Strong links to other internal groups

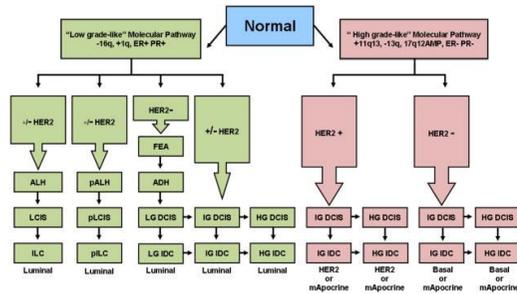
- Statistics
- Informatics
- Real World Evidence
- Translational Science
- Clinical
- Commercial

## •Partnerships with leading diagnostics companies

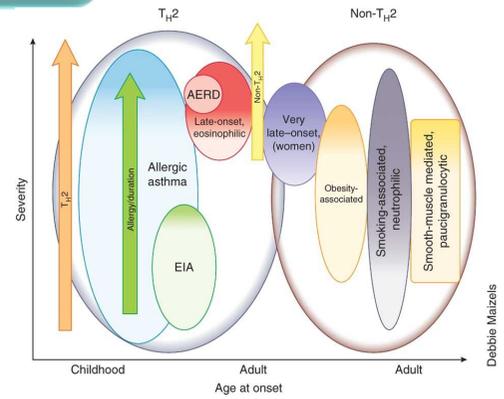
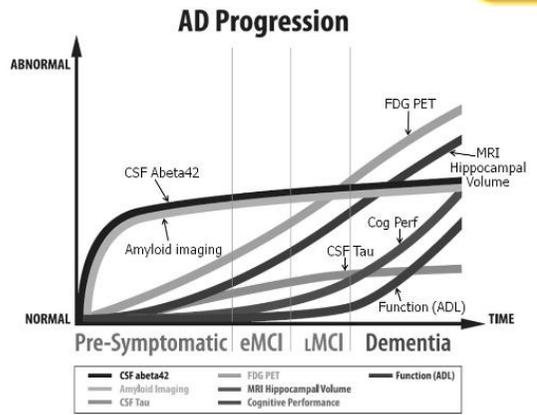
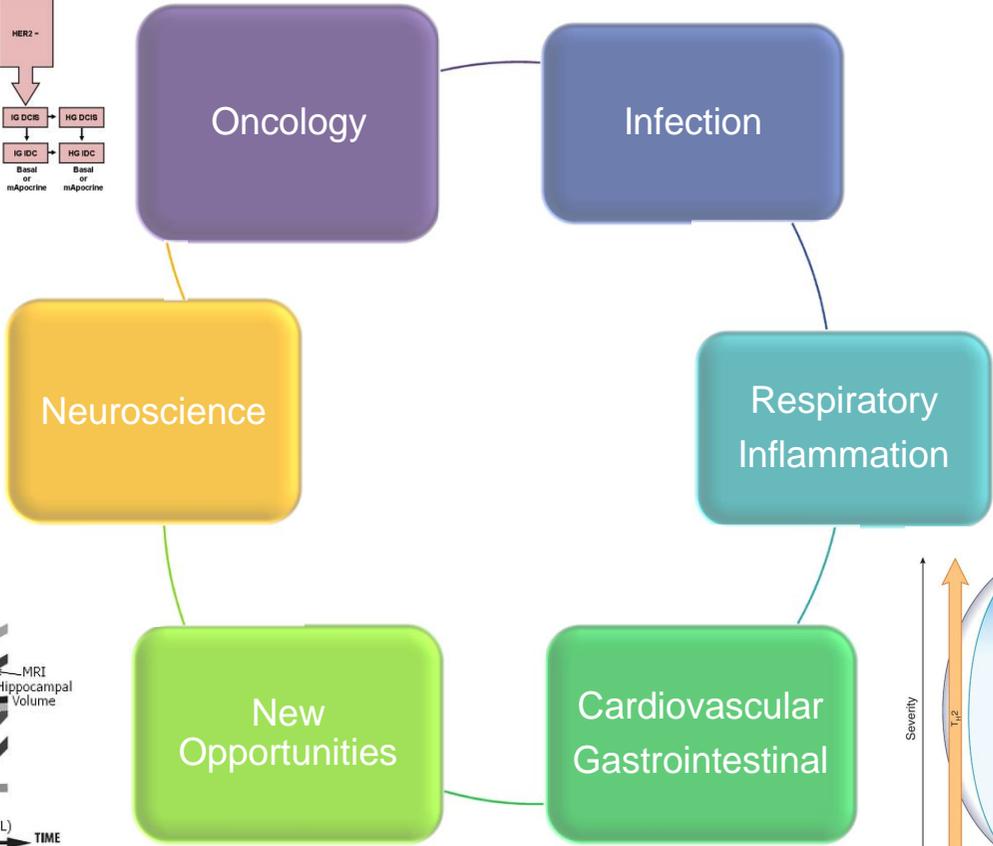


# Personalising Over Disease Areas

## Complex Heterogeneous Diseases Require Stratification



**Gram Negative** **Gram Positive**

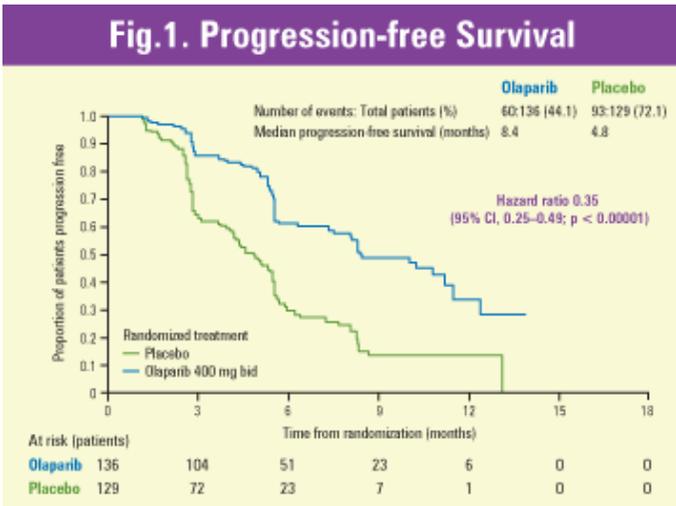


# Range Of Types of Personalisation

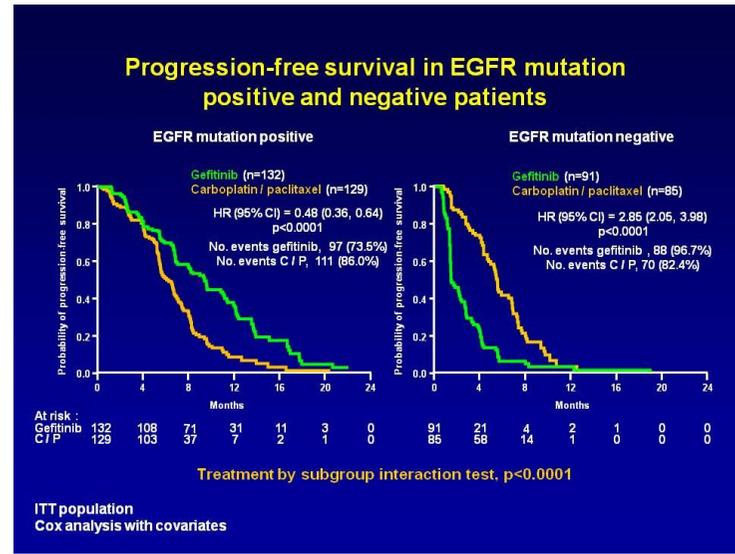
Clinical Parameters

Clinical Decision Tools

Molecular Diagnostics



Significant PFS in Phase 2 serous ovarian population with ≥2 previous platinum regimens



For Regulators

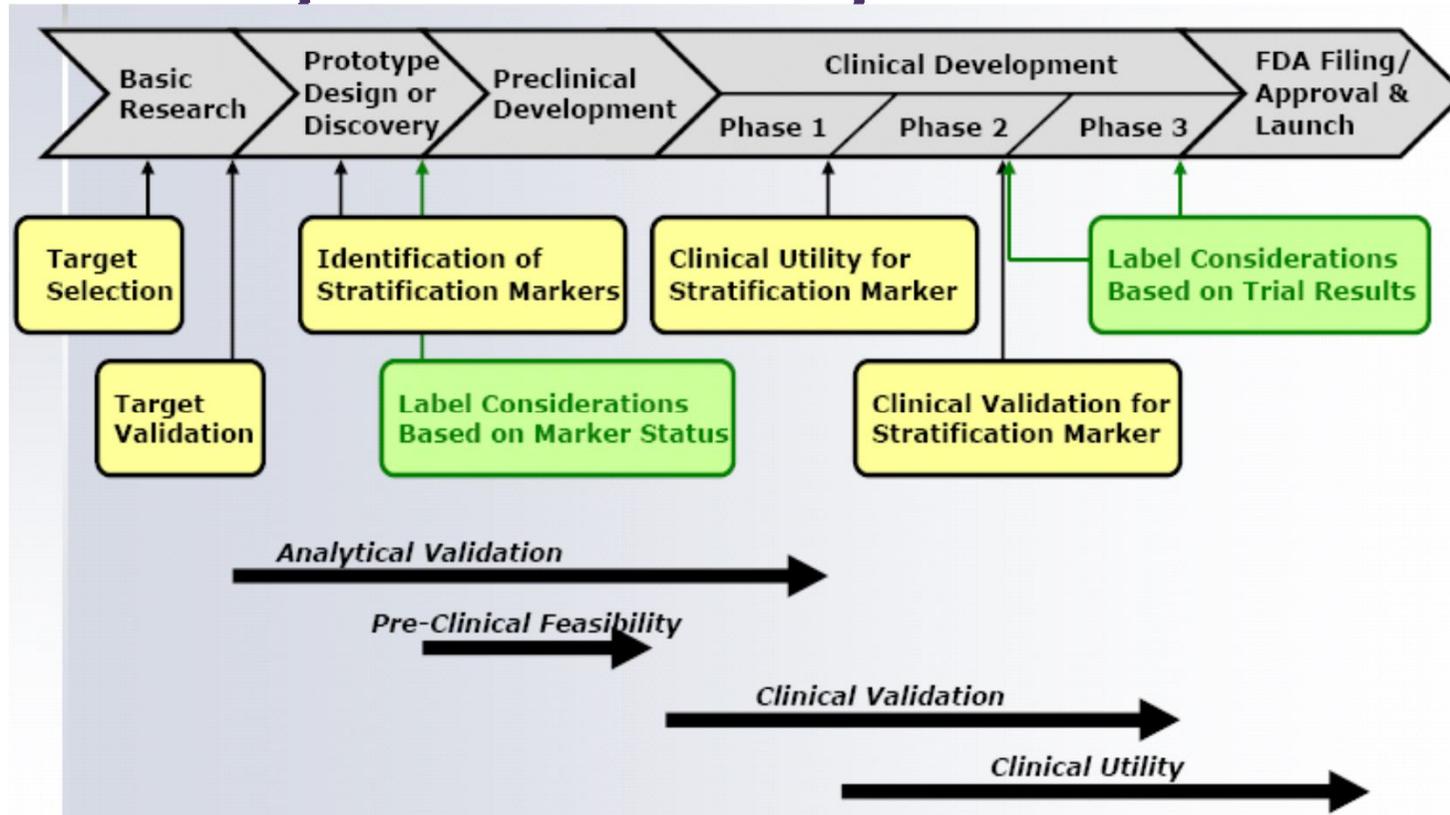
For Payers

For Patients



# Challenges

## Two Interdependent Development Processes



There are numerous benefits and challenges  
Here will focus on some with a statistical element

Study Sizes

Predictive Accuracy

Data Quality

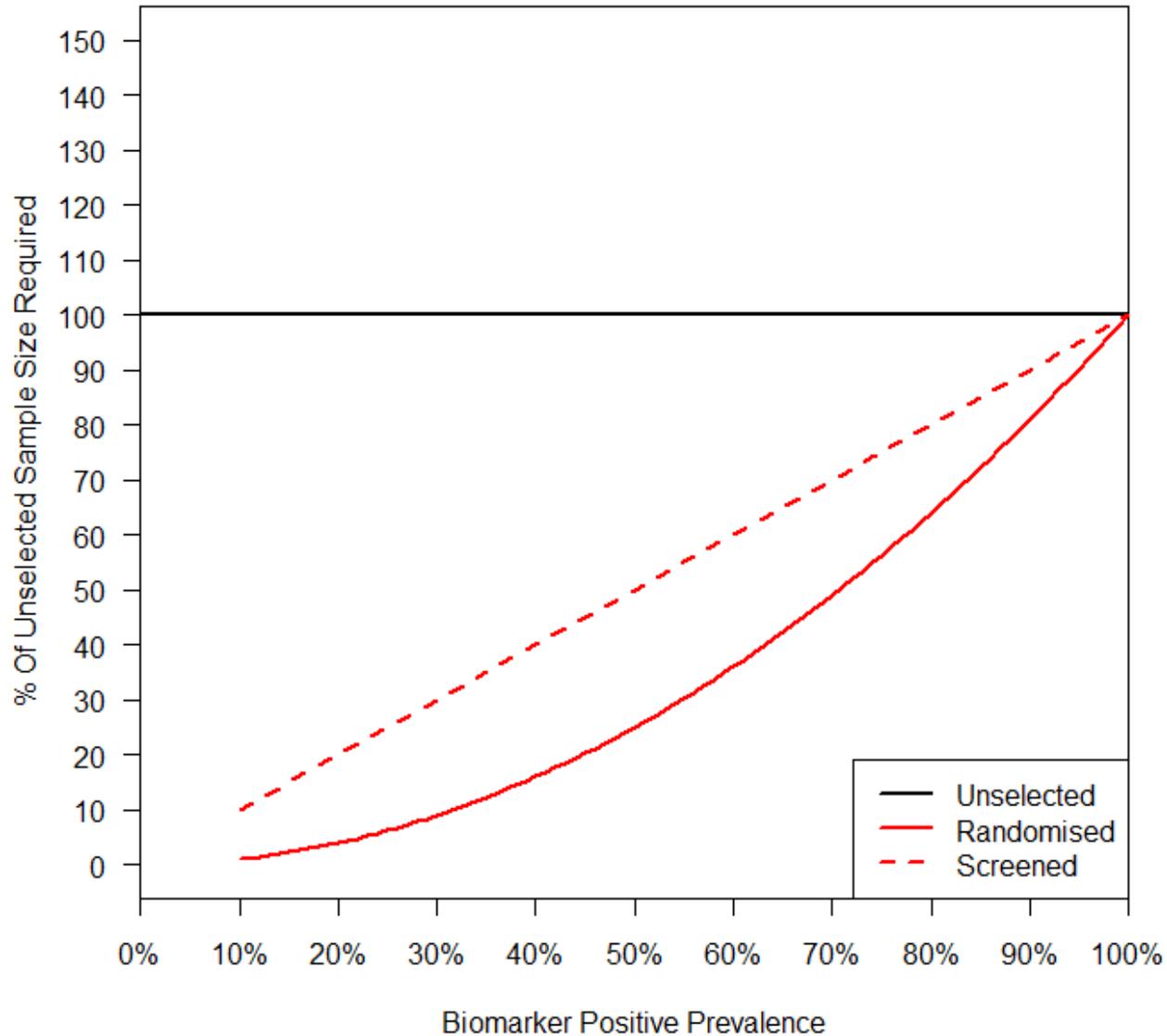
Variability



# Benefits

## More Efficient Studies

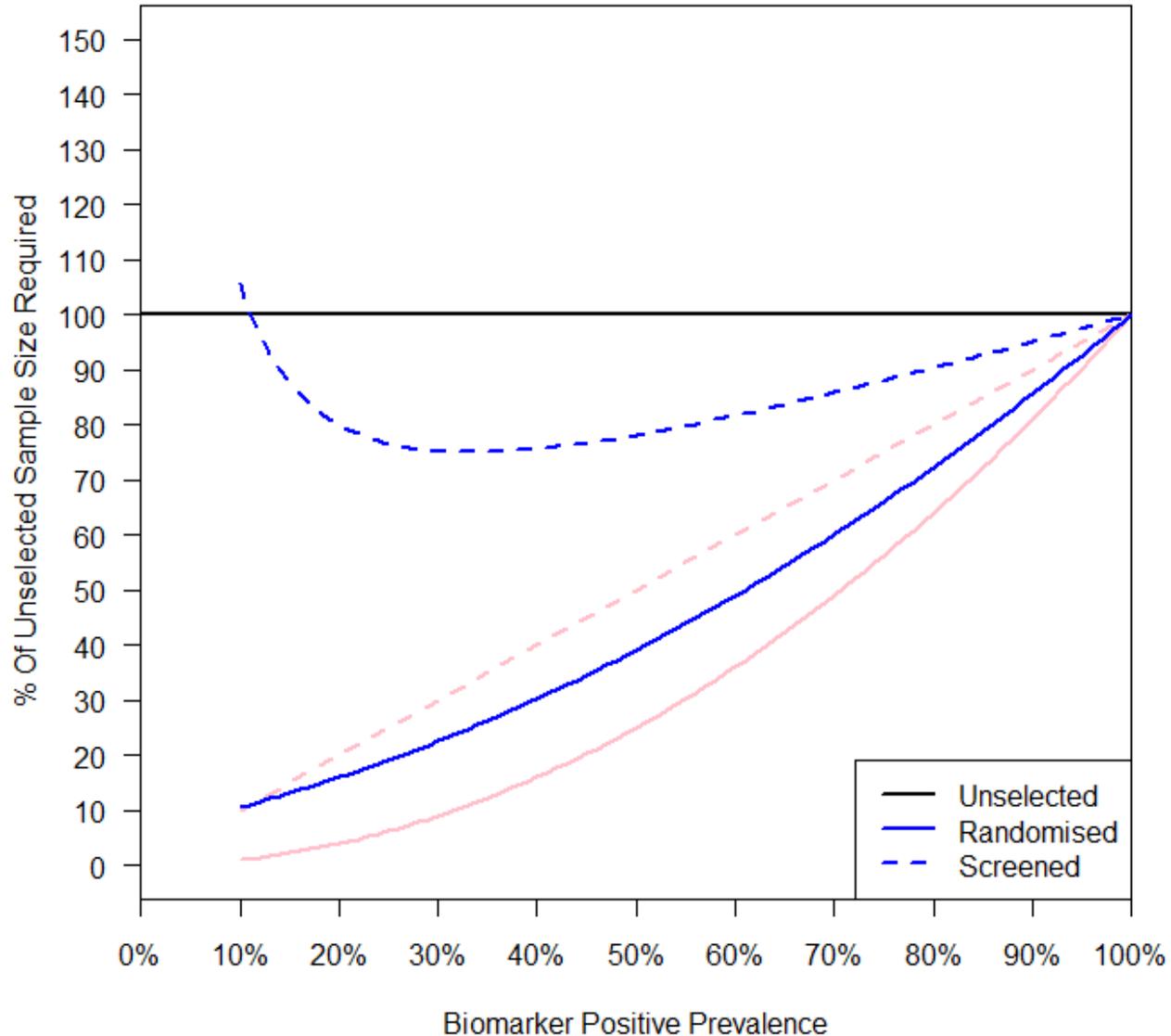
No Treatment Benefit In Biomarker Negative Population



# Benefits

## More Efficient Studies

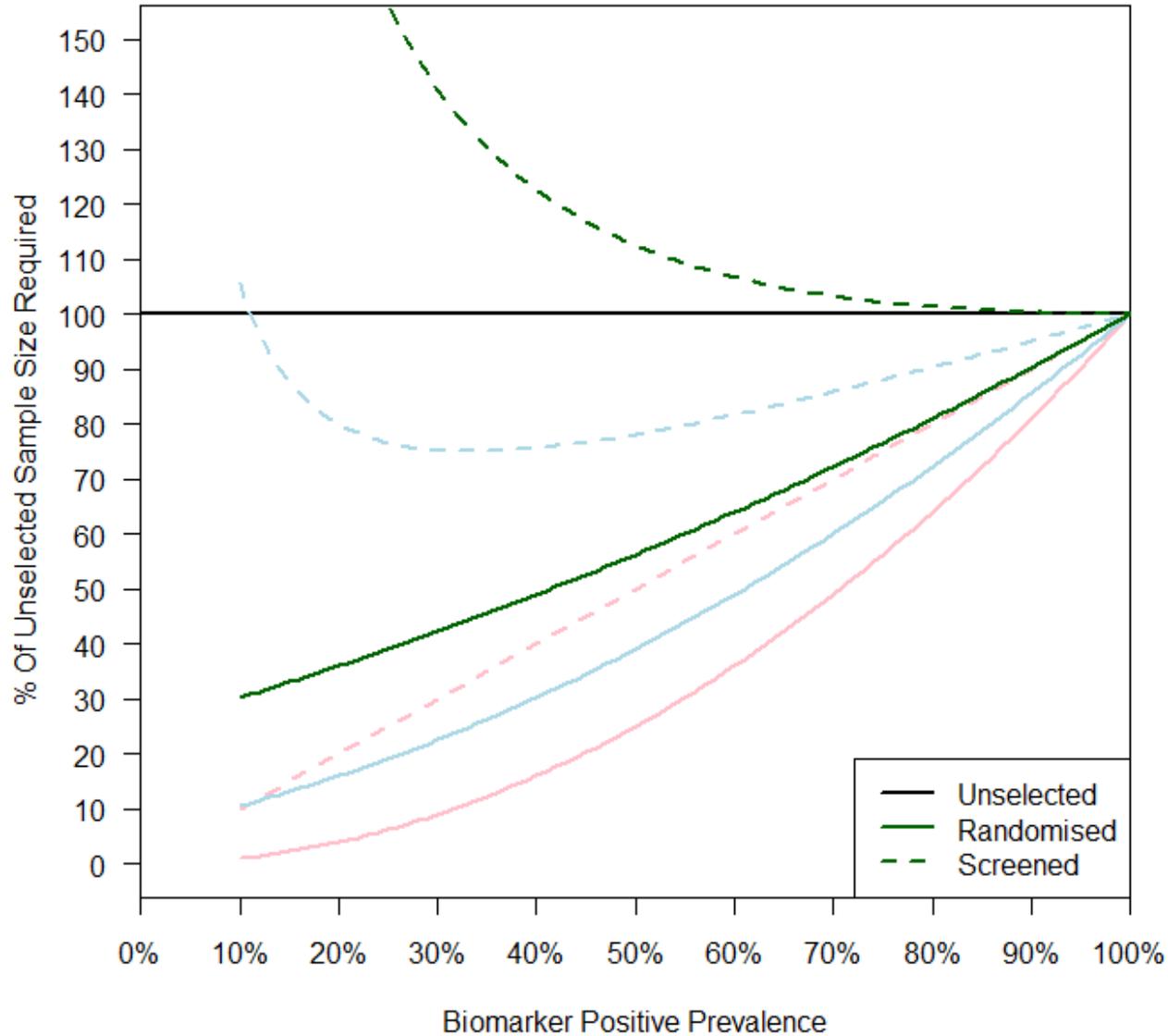
Biomarker Negative Population Gets 25% Treatment Benefit Of Biomarker Positives



# Benefits

## More Efficient Studies

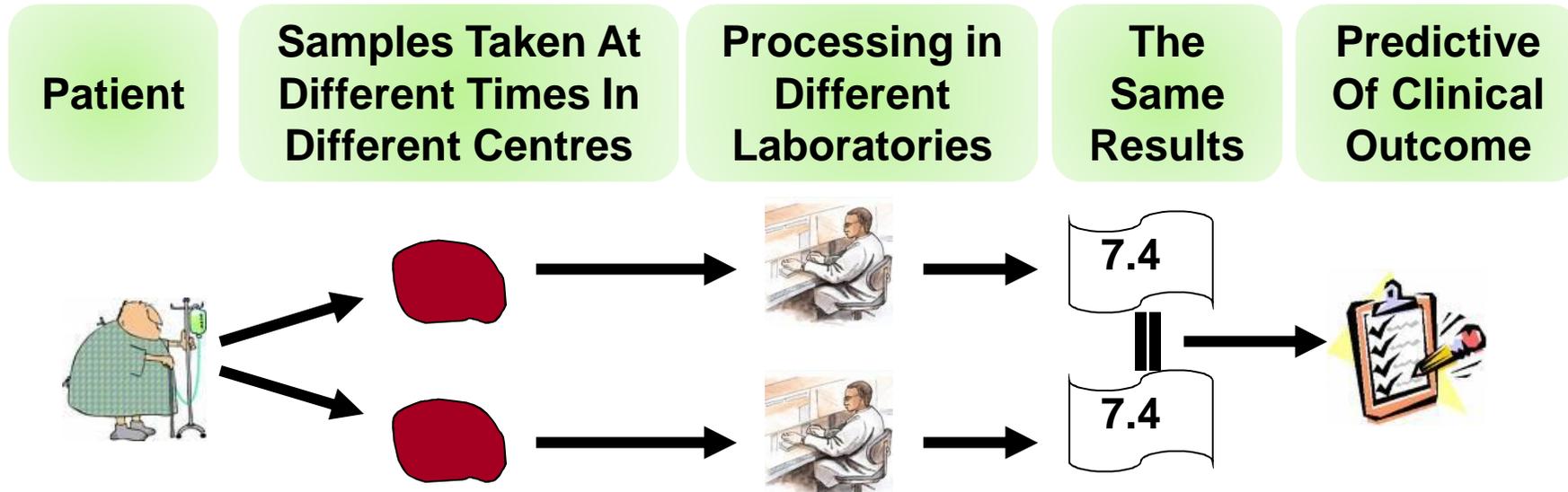
Biomarker Negative Population Gets 50% Treatment Benefit Of Biomarker Positives



# The Perfect Diagnostic Assay

To be demonstrated over a number of patients, covering the range of results and disease subgroups within the population

An ideal study would cover many laboratories to allow extrapolation to a more general clinical setting



If the study fails to demonstrate concordance, then additional studies may break down and identify the critical sources of variability

## Biological Variation

- Temporal
- Site
- Spatial

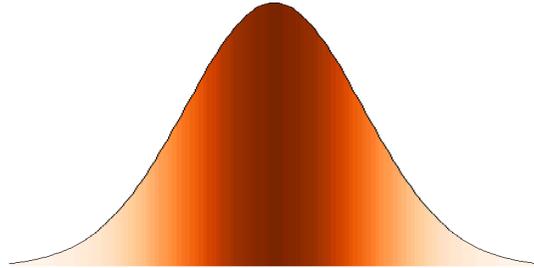
## Laboratory Variation

- Procedures
- Equipment
- Technicians



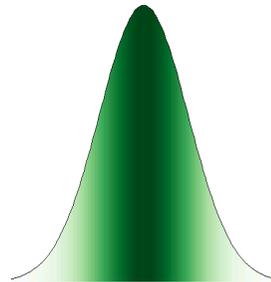
# Variability In Results

**Overall  
Variability**



=

**Biological  
Variability**

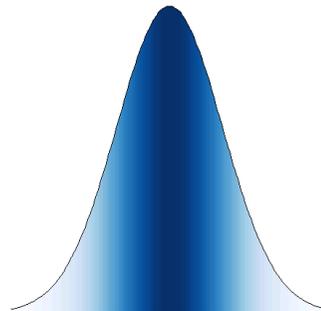


- Day-to-day
- Time of day
- Tumour Heterogeneity

**Limits of  
Application of  
this Biology**

+

**Technical  
Variability**



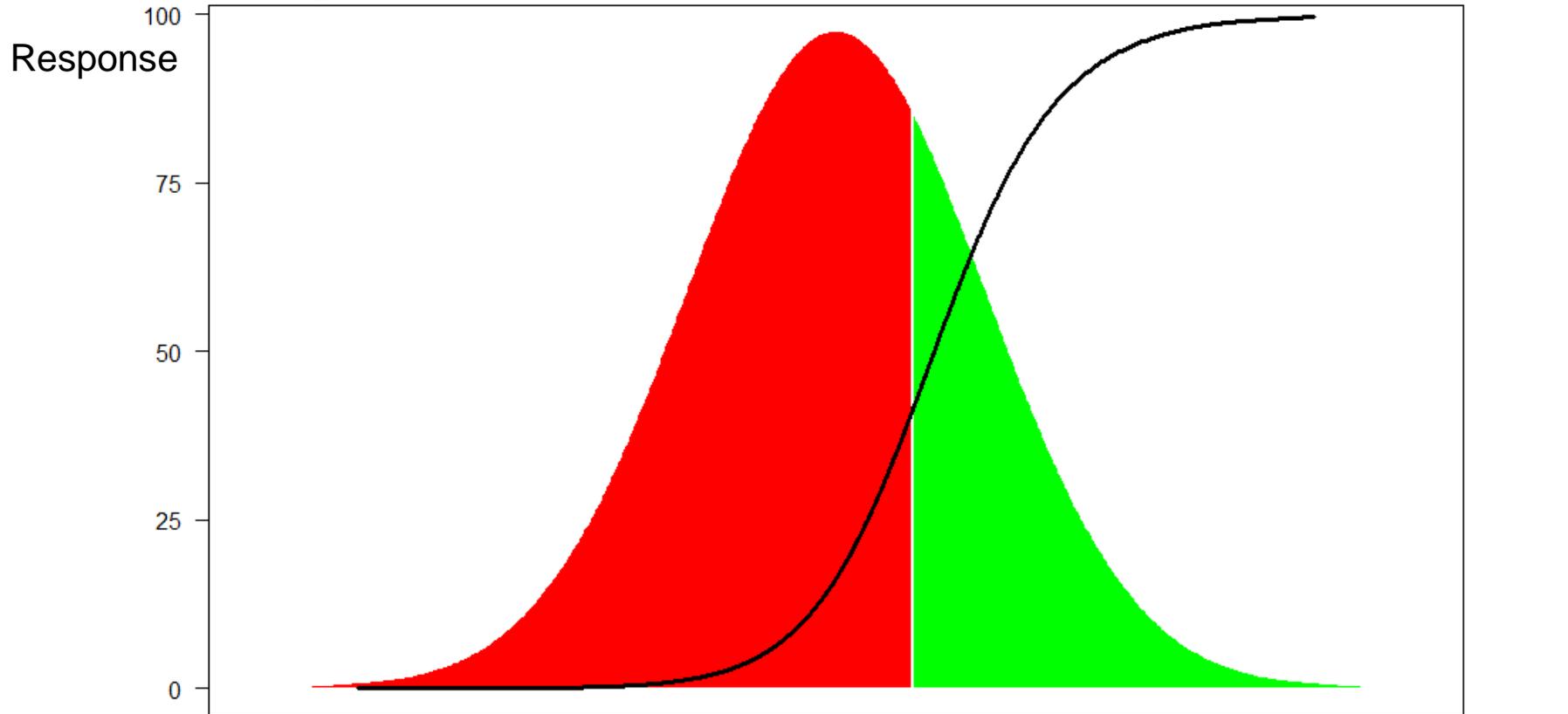
- Sample Collection
- Storage
- Processing
- Sample Quality
- Reading

**Potential to  
Tighten Up  
Processes**



# Data Quality Is Key

## Variability Could Erode The Benefits

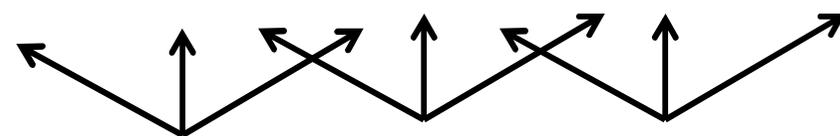


Minimal Misclassification



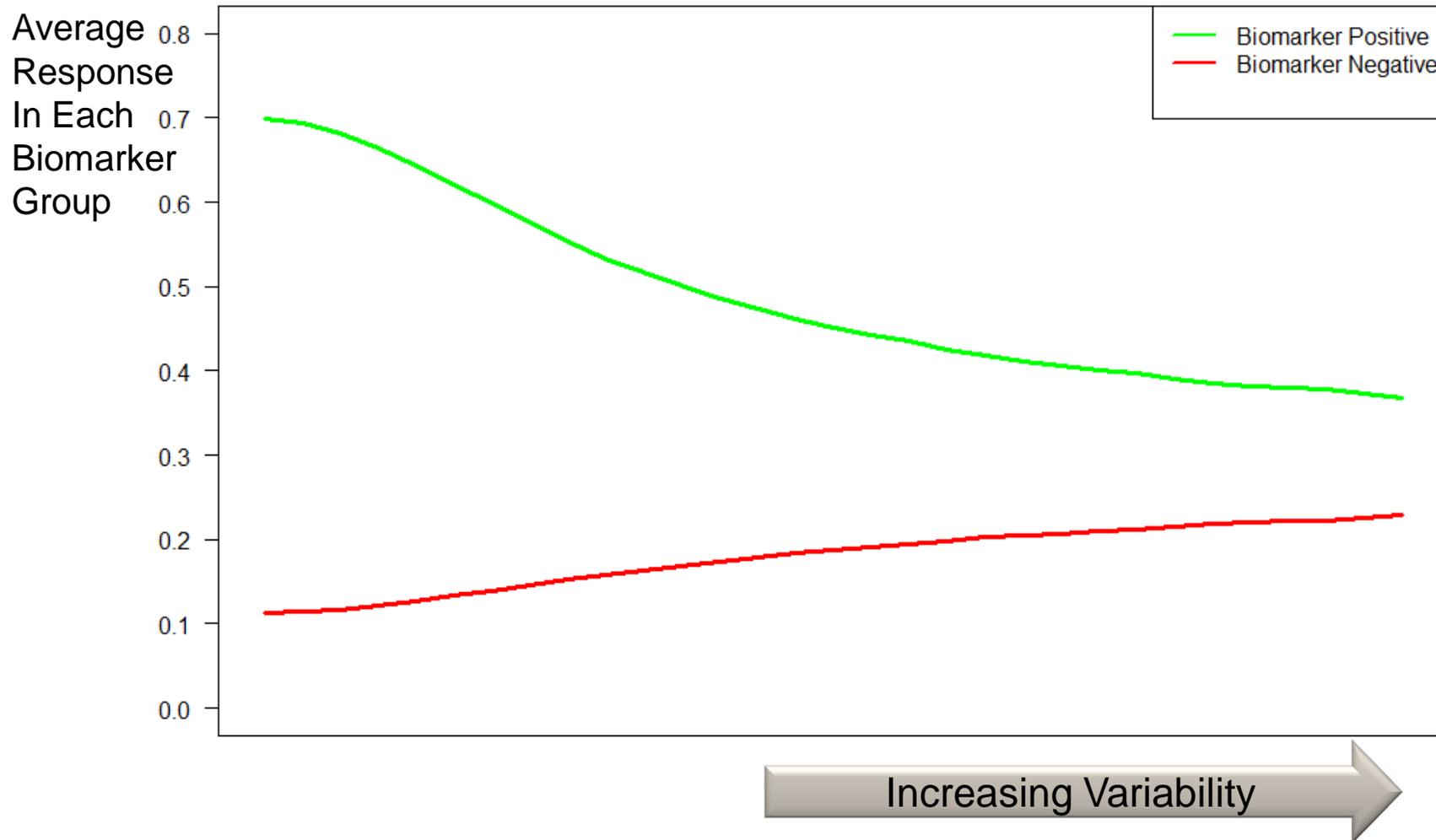
Biomarker

Increased Misclassification



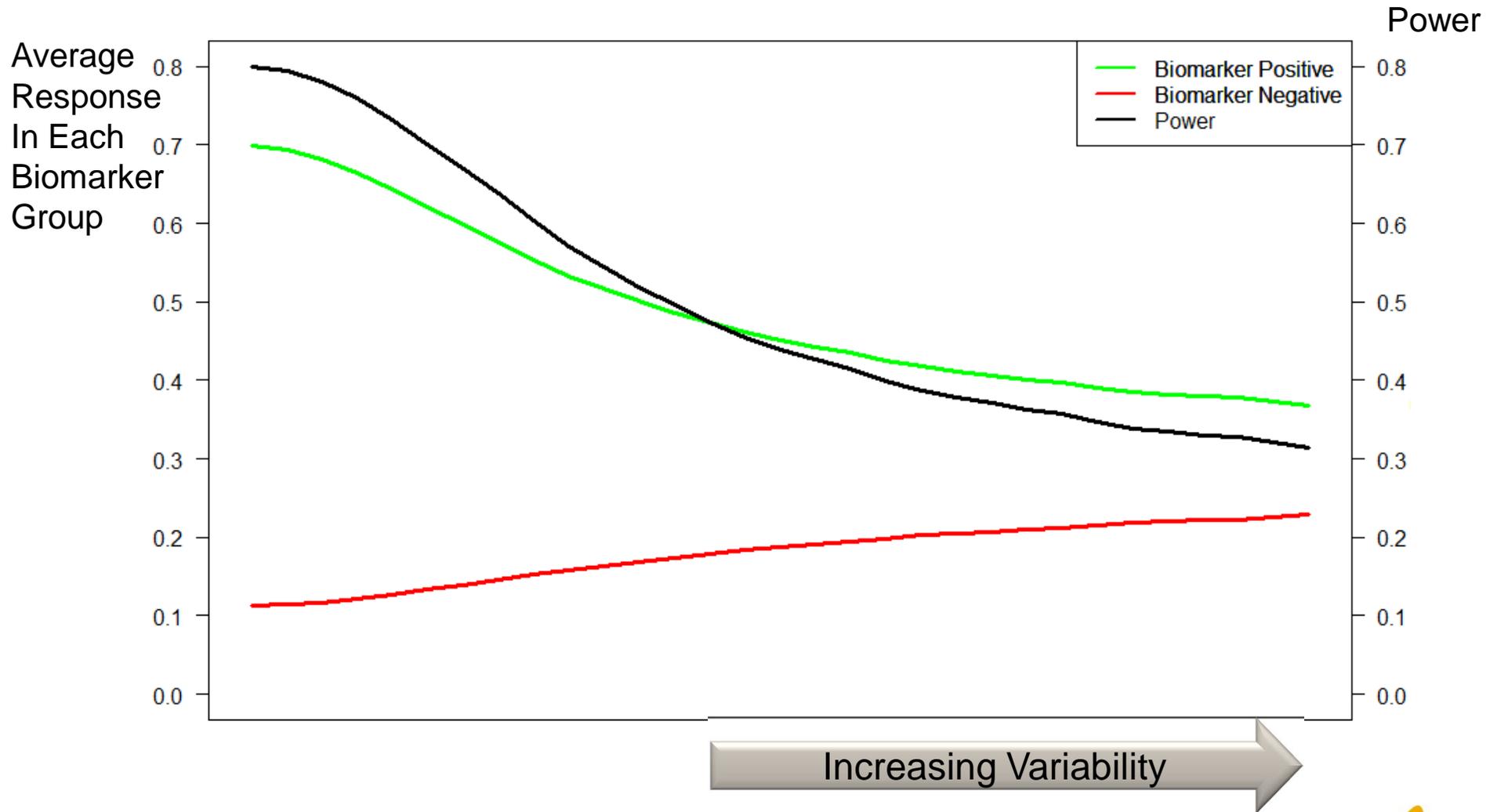
# Data Quality Is Key

## Variability Could Erode The Benefits



# Data Quality Is Key

## Variability Could Erode The Benefits



# An Ideal Predictive Biomarker?

## Predictive

- Drug is efficacious in biomarker positive population
- Biomarker positive population doesn't exclude responders
- Provides a competitive advantage for our drug

## Confidence

- Robust
- Minimal biological or technical variability
- Reproducible across sites and regions
- Strong scientific rationale

## Accessible and Available

- Widely available
- Part of standard care pathway
- Uses standard technology
- Minimally invasive to patient
- Reimbursed
- Accepted by regulators and medical community

## Beneficial to patients

- By improving medical outcomes and quality of care
- By giving patients confidence that they are receiving the best treatment for them
- By helping prescribers to find the right therapy first time



# What is Personalised Medicine to AstraZeneca?



**Who could argue with this as a worthwhile objective?**



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