Need for public-private collaboration

- Translational research (TR) is a cornerstone of the IMI mission

- TR is essential to provide new insights into disease progression, biomarker discovery, patient stratification, safety interpretation (etc) leading to reduced attrition and timelines for drug R&D.

- Yet:
  - There is no stable, open, community led KM platform or <u>service</u> to support TR
  - The standards for translational data integration and analysis are weak

- The effect of this is:
  - Increased costs and inefficiencies for each study (lack of core services)
  - Increased complexity in data interpretation (poor standards & methods)
  - Increased risk of loss of data post project (no long term repository)
  - A weak bio-medical informatics community enabling innovation in TR KM

- There is an immediate need to establish TR KM infrastructure and the delivery of services for existing and future IMI calls, as well as other PPPs.

Objectives of the full project

- Step change efficiency gains in cross-organisation TR study project execution through standardised, quality TR KM services

- Single access point to standardised TR study information

- Sustainable, interoperable, collaborative, open TR platform, based on open, agreed standards

- Development of an active TR analytics & informatics community leading to innovation in TR data interpretation.

Objectives of the full project

- Create a TR KM consortium to support TR projects:

  - **Infrastructure:** KM Research & development building on Johnson & Johnson's TranSMART system to create the required open platform

  - **Content:** Populate with existing and active TR Study Data
    - Clinical Study Data
    - Pre-Clinical Study Data (e.g. in vivo)
    - Biomarker data associated with Studies: 'omics, genetic, etc.
    - Background knowledge (e.g. molecular pathway data, literature)

  - **Standards:** Development and adoption of TR information standards

  - **Services:** Support for IMI (& other EU) TR Studies re KM data services
    - TR project KM consultation, curation support, historic data curation
    - Platform maintenance, enhancements & code control
    - Administration, exploitation support, training, awareness

  - **Research:** Research & Development of new analytics methods and tools

Pre-competitive nature

- TR KM is an enabler of EFPIA R&D, not a core deliverable.

- Lack of an open TR infrastructure, services and innovation in analytics is limiting competitive and pre-competitive TR: recognised cross-EFPIA gap.

- Proven pre-competitive opportunity: tranSMART platform built on open architecture (i2b2) and already providing pre-competitive support for TR collaboration, KM and analytics:
  - 3 IMI projects (U-BIOPRED, OncoTrack & SAFE-T) with adoption planned for 4 others
  - Multiple US Academic Medical Centres (e.g. CINJ, U. Minn. Cancer Center)
  - Sage Bionetworks/Genetic Alliance CTCAP initiative

- Need for eTRIKS consortia to coordinate with community investments:
  - ESFRI, notably EATRIS, BBMRI, ELIXIR and ECRIN
  - Other IMI KM calls re standards, especially EMIF wrt functionality.

# Expected impact on the R&D process

- Improved efficiencies for TR study set up and execution
  - No more re-inventing the wheel

- Improved TR data sharing and interpretation, through:
  - Promotion and support of data standards
  - Common access and analytics layer, lower threshold for use
  - Agreed security and publishing protocols
  - Stable repository of curated and annotated historic TR studies, enabling cross-study analytics.
  - Improved analytics & visualisation: accessible data driving innovation
  - Strengthened community of TR informatics professionals

- Will lead to enabled (quality, cost and speed) internal TR programmes as well as PPP (e.g. IMI) TR programmes.

Suggested architecture of the project

- Presumption that the project will initiate from a proven platform: tranSMART, enabling TR study service support from day 1.
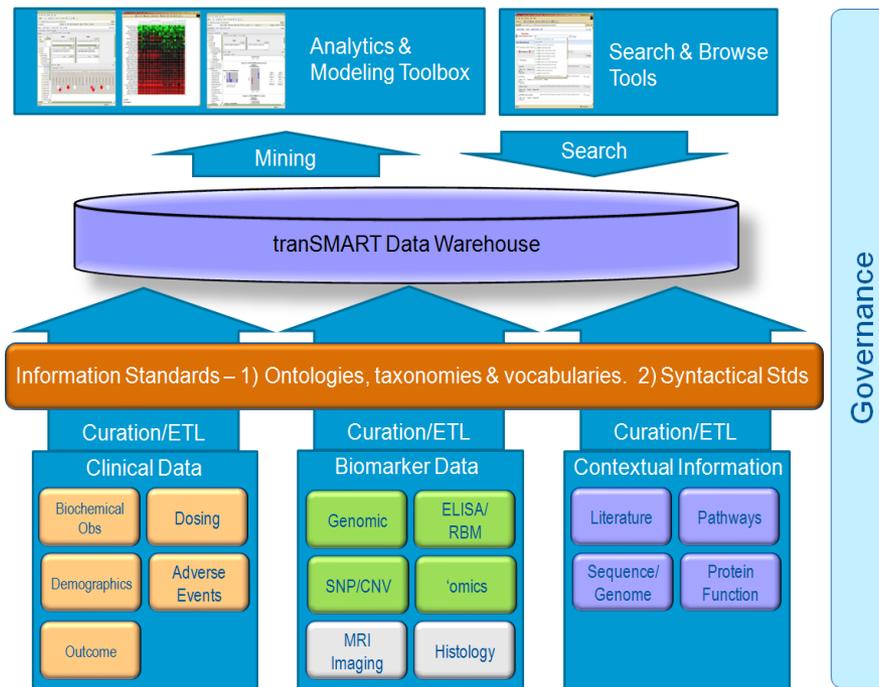
- WP1: TR Study KM Services

- WP2: KM Platform Research

- WP3: Standards Research/ Coordination

- WP4: TR Analytics Research

- WP5: Governance & Business Models

- WP6: Community Engagement & Outreach

Expected contributions of the applicants

- Co-located group of 12-15 FTEs for 5 years, typically 3-5 member organisations.

- Focus and priority on service provision and TR impact (not technology)

- Vision & know-how to develop into a NFP sustainable public service

- Key skills and experience
  - TR KM *service* delivery – proven record in supporting TR projects.
  - TR KM research - data management and analytics
  - Collaborative software engineering
  - Information management, including security models & cloud computing
  - TR information standards & standards bodies.
  - Curation, QA/QC and data handling experience: Clinical, 'omic, imaging, etc
  - Clinical KM regulatory policies to ensure compliance and promote sharing
  - Community engagement and outreach, including TR funding community.

Expected (in kind) contributions of EFPIA members

- Technology:
  - Transfer of tranSMART code and associated processes
  - Some tools, methods, standards
- Training on tranSMART system and processes:
  - installation/maintenance,
  - software engineering,
  - ETL / Curation
  - end-user trainings
- Expertise in:
  - Professional information services delivery
  - Database/Software engineering
  - Curation and ETL
  - Informatics (clinical & pre-clinical)
  - Translational / Clinical Science
  - Data governance
  - Legal, compliance and regulatory policies

What's in it for you?

- **Chance to establish a 'game changing' service set to transform Translational Research in the EU.**

- Potential to show significant impact in the TR community in less than 6mths of project initiation: quick read out.

- In depth awareness and influence of tranSMART architecture and design over the next 5 years.

- Unparalleled access to multiple TR projects, and potentially associated data sets, as part of service provision.

- Opportunity to lead the definition and implementationof standards and ontologies to represent translational data.

- Chance to work with Pharma KM/Ix professionals with years of TR information experience, *but never collectively shared till now*...

- Prospect to create a new community of information professionals..

Key deliverables of full project

- Open, hosted TR KM & analysis platform
- TR KM & analysis research & development
- TR KM services to EU TR studies :
  - Curation support for active and historic data
  - Guidelines and best practices for data curation
  - Quality control processes and services.
- Stable repository of curated and annotated translational studies
- Platform support: mirroring and data export facilities
- Training
- Standards: Independent, published and adopted
- Governance: data, security, standards
- Active community of translational informaticians & KM professionals.

tranSMART Overview

- **J&J implemented award-winning industry-proven TR platform**
  - Portable Software Based on Open Source & Academic Partnerships

- **Use Cases**
  - what is the correlation between animal models and human data
  - what is the best biomarker strategy for a given compound
  - what is the best indication for a given compound
  - how can a disease stratified based on clinical data
  - is there support for a target of interest based on clinical data

- **Data**
  - Clinical data, clinical and pre-clinical gene expression, protein profiling, SNP, PD markers, omics
  - In-house – immunology, oncology, CV, psychiatry, neuroscience
  - Public and commercial
  - Curated text & Text indexing
  - Master data, ontologies, vocabularies and metadata

- **User Interfaces**
  - Search: Gene, pathway, disease, compound, trial, and combinations
  - Hypothesis testing: Cohort selection and comparison/analysis
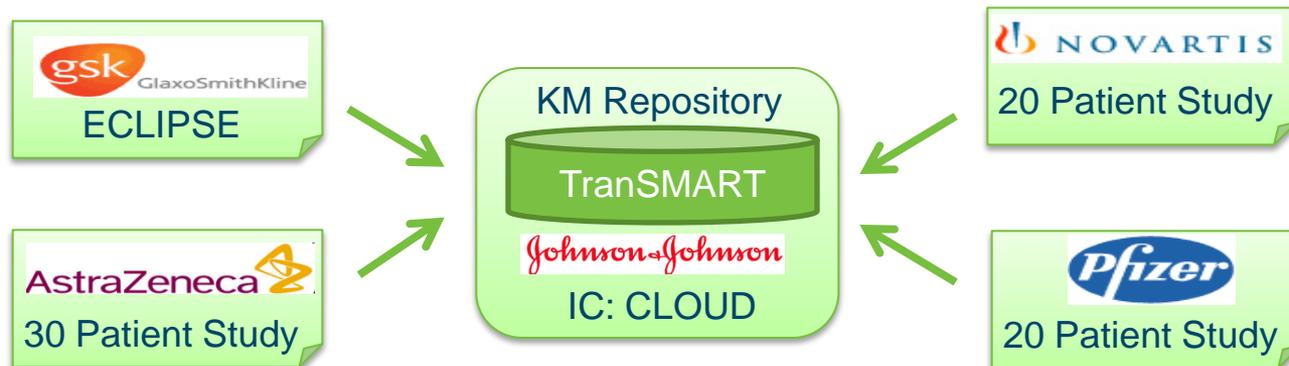 - Hypothesis generation: Gene signatures
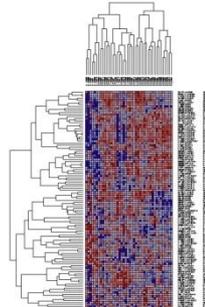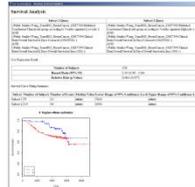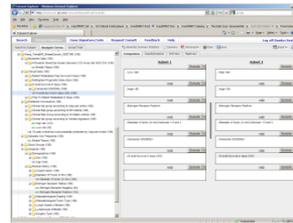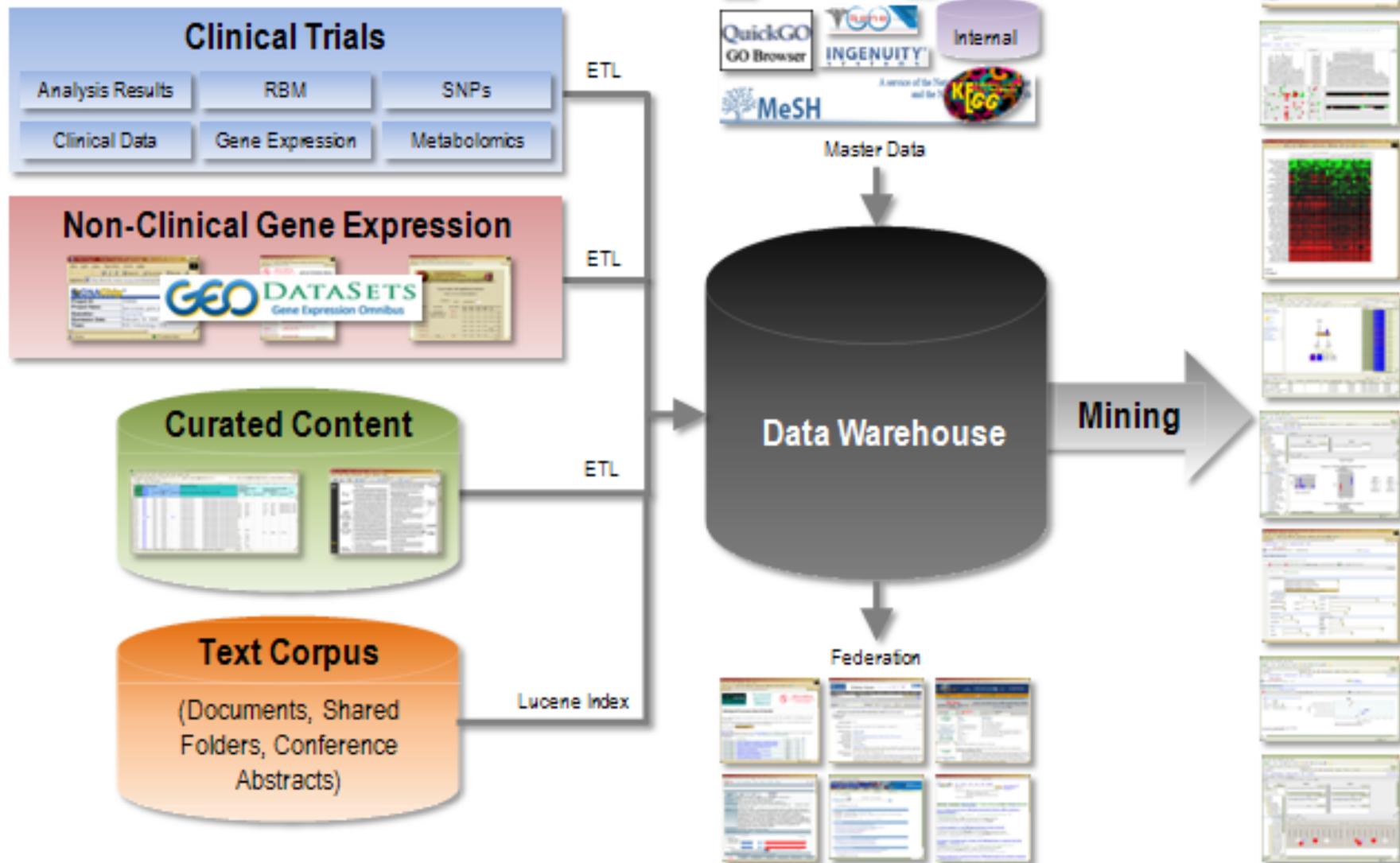
tranSMART Example: U-BIOPRED

- Exploratory COPD study (ECLIPSE) sponsored by GSK
  - 3000 Patients, 6 Time points over 3 years
  - 1800 Clinical Attributes (inc Lung Function, Exacerbations and Sputum cell counts & protein markers)
  - Blood Samples for all patients for all time points
    - 200 Patients with Micro-arrays of baseline blood
  - 60 Patients with Micro-array of baseline sputum
- Successful Pilot
  - Training, tech transfer, tranSMART instance construction and loading of 300 patient cohort from ECLIPSE completed in 3 weeks.

tranSMART Consortium

KM Repository

tranSMART

SAGE Bionetworks

KM Repository

tranSMART

CINJ

KM Repository

tranSMART

U. Minn Cancer Center

North America Consortium (4/29)

Millennium Pharma,
VA Boston,
Harvard/Partners,
SAGE Bionetworks,
U. Michigan Cancer
Children's Hospital
Boston,
U. Minn.,
Astra Zeneca,
Sam Waxman
Int. Medical Corps.,
West Wireless
Healthcare,
Kimmel Cancer Center,
Merck,
Xyntek,
Cancer Institute of NJ,
Recombinant Data,
Sloan Kettering,
fNIH,
Critical Path,
FDA, NIH, J&J…

Open Source Code Base

Global Data Sets

tranSMART Consortium

IMI eTRIKS

AstraZeneca
GSK
Pfizer
J&J
Lundbeck
Merck Serono
Roche
Sanofi-Aventis
Bayer
Eli Lilly

KM Repository

UBIO PRED
tranSMART

IC: CLOUD

KM Repository

OncoTrack
tranSMART

TBD

KM Repository

Safe-T
tranSMART

TBD

For More Information...

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Big Scary Diagram