



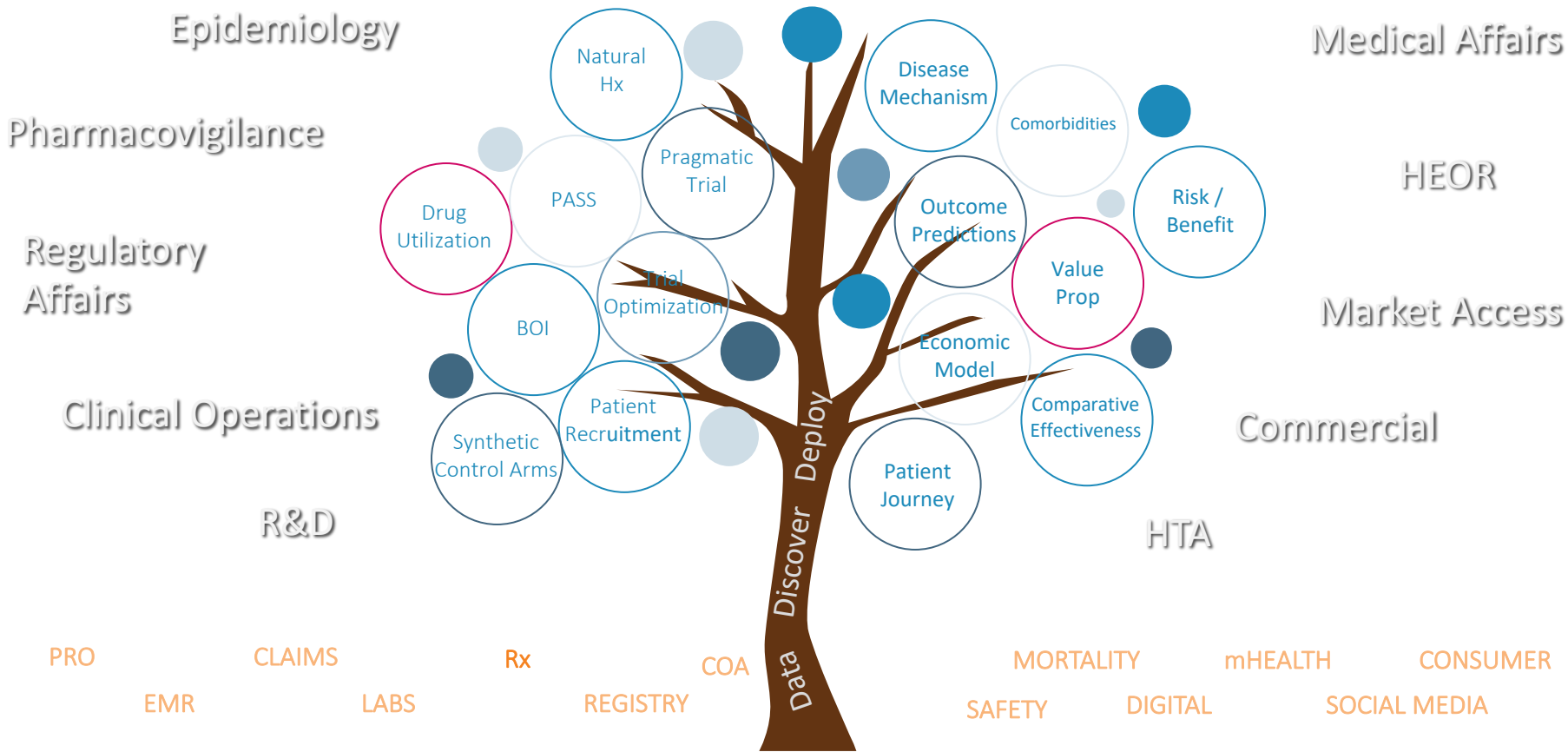
Overcoming the Pitfalls of Using Real-World Evidence in Regulatory Submissions

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Real World Evidence and Beyond



Focus areas we see the market looking to overcome

Access to broad data to inform strategy and market approach risk mitigates the heavy investment

Utilizing a broad network of provider sites is key in overcoming failure and enabling access to high quality data

The Power of Data

Bringing a product to market can cost upwards of \$2 billion, so improving clinical trial strategy and accelerating market access is critical to ROI

Cost Efficiencies

Regulatory Clarity

RWD and RWE are increasingly relevant to regulatory submissions, however the shifting regulatory landscape poses risk

Utilizing the power of data and analytics to avoid pitfalls when using RWE for regulatory submissions



Strategies

1

Start early, and think strategically

2

Develop a robust prespecified protocol and SAP

3

Optimise value from selected RWD assets

4

Ensure aligned RWE across different regions

1 Start early, think strategically, and take an integrated approach to evidence planning

Integrated evidence planning

Proactively design an evidence package based on RCT and RWE
and / or
Supplement existing RCT data with RWE on additional populations

RWD planning in parallel to study design planning

BC Platforms' Global Data Partner Network provides the ability to source appropriate data to meet the exact requirements of each study, from multiple sources

Develop a robust prespecified protocol and statistical analysis plan, and proactively share

- Engage with regulatory bodies early and often when designing each study
- Take a proactive and stepwise approach to ensure buy-in to overall study objectives, protocol, and SAP
- Ensuring cross-sponsor stakeholder visibility throughout is key



3 Optimise value from selected RWD assets

A

Clinically rich EHR data with enriched variables like genomics, lab values, and imaging has significant value in regulatory submission success and achieving comparability with active trial cohorts

→ Especially valuable within rare and ultra rare disease populations

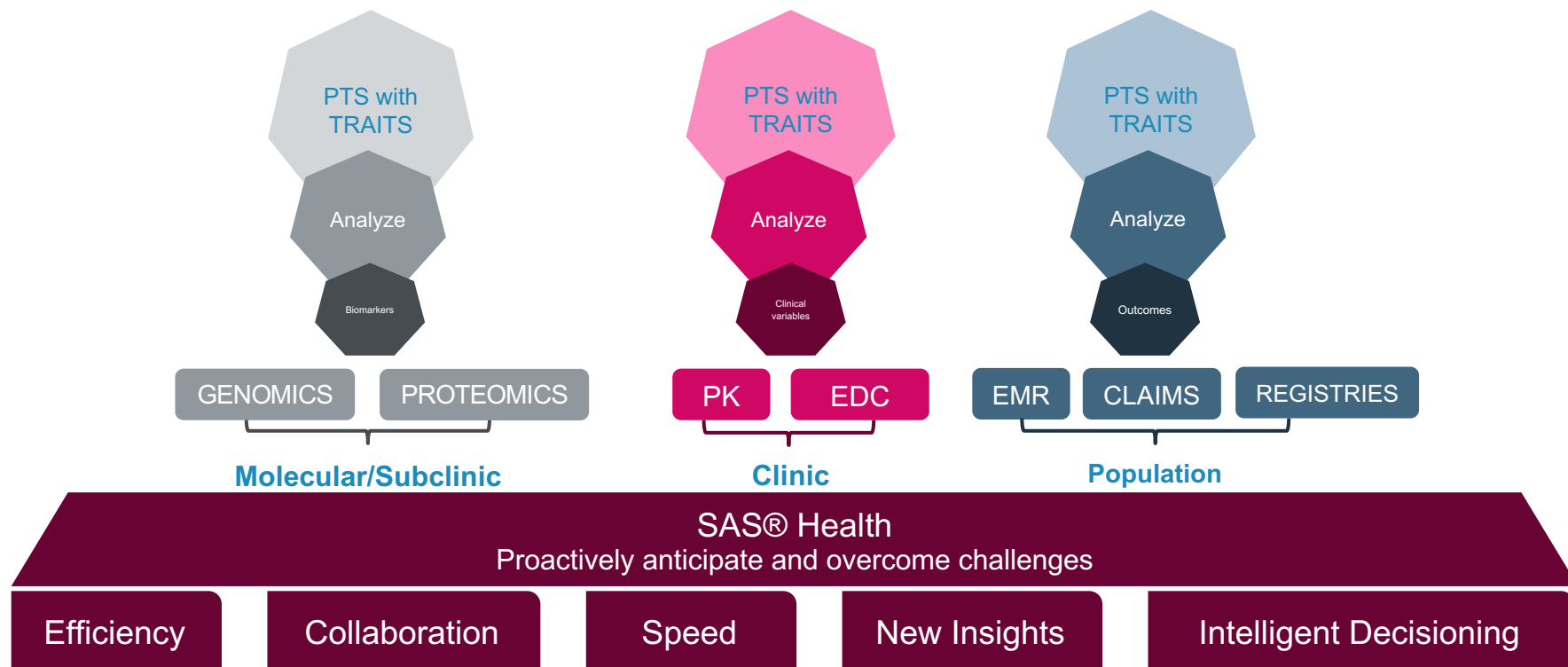
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Compensate for data missingness by outsourcing your cohort, to allow for statistical correction on analysis. This can help compensate for variations in Standard of Care, EHR structures, regional coding, ...

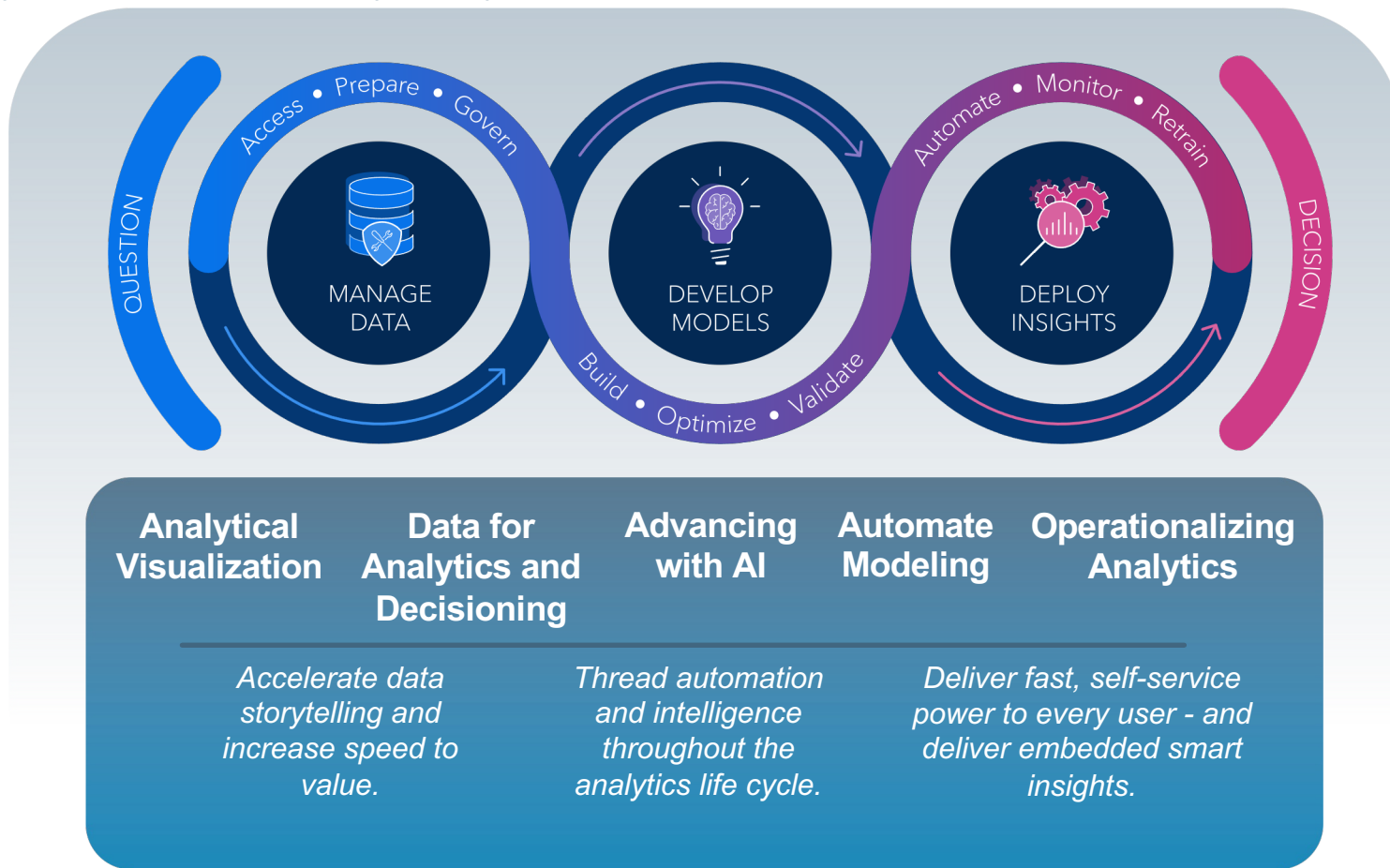
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With challenges in data heterogeneity — analytical approaches can be taken to validate data quality and enhance dataset utility. Complete audits and audit trails are critical components of a robust evidence package.

Health & Human Data Fabric – Stitching it together



This process turns complex questions into understandable, actionable insights



4 Ensure aligned RWE across different regions

Considering variations in SoC and endpoint collection in your design is critical to success, regional expertise is required

1

Variability Considerations

Physician practice, SoC, and end-point recording can vary across geographies

This can impact on overall study viability and/or result in unclear outcome measures if not considered in Protocol and SAP

2

Provider Questionnaire

Provider questionnaires can be an effective and cost-efficient initial step to confirming SOC, patient journey and available end points across potential RWD sources

3

Diversity and Inclusion

Regulatory guidance is critical when incorporating underrepresented global populations for drug development

A comprehensive network of both data collections and Key Opinion Leaders is important

Bottom line



- With Planning and the right tools it is possible to overcome challenges using RWE in regulatory submissions.
- Assembling data within a GDPR+ technology platform allows a vast network of provider sites to participate; combined with the right SAP, previously untapped global RWD assets can be unlocked to accelerate insight generation for R&D and commercialization



Clinical strategy and evidence package generation for submission

Regional knowledge to inform approach

Accelerated molecule to market

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