Real-world evidence

Registries and real-world evidence

We offer a comprehensive suite of customizable gold-standard registry solutions that efficiently deliver data-driven, actionable insights.

Study design and data collection strategies are tailored to your research question to ensure an optimal approach. Our bespoke and independent registry models enable truly fit-for-purpose design, delivering reliable and robust data that meet stakeholder requirements.

We offer exclusive access to proprietary regulatory-grade data via our independent disease registries and an integrated team of real-world evidence (RWE) and registry experts and operational expertise, providing unmatched registry solutions to accelerate drug development and meet regulatory requirements.

We offer two core models for registry design and delivery

1 Independent registry

A disease-based, multi-drug registry solution that enables subscribers to **extract regulatory-grade data from prospective, longitudinal proprietary registries** to efficiently address a multitude of real-world evidence needs.

Key characteristics

Independent model: capture broad safety, effectiveness and quality of life (QoL) data from multiple drugs/patients in indication. Cost and data sharing with the PPD™ clinical research business of Thermo Fisher Scientific and additional industry subscribers.

License data: renewable licenses granted to data (aggregated reports, cross-sectional data and analysis)

Regulatory-grade data: complete and high quality, using validated clinician and patient-reported measures

Open-ended: ongoing site and patient involvement for continuous and expanding longitudinal data collection

Unique value

- Provides critical **contextualization of your drug's positioning** relative to standard of care (e.g., utilization, effectiveness and safety)
- **Active assessments** of clinical endpoints and safety events in combination with patient-reported outcomes (PROs) – resulting in unmatched completeness
- **Flexible infrastructure**: ability to add to data collection or nest additional studies over time as evidence needs evolve
- Dedicated scientific advisors, non-profit organizations, and site networks champion **long-term ongoing registry growth**
- Over time, this model **lowers cost per patient**

Use cases

- Use pivotal Phase 3 trial endpoints collected in independent registries to bridge randomized controlled trial (RCT) and real-world evidence
- Post-approval safety studies (PASS) in support of FDA and/or EMA requirements
- Comparative effectiveness and comparative safety
- Demonstrate natural history, unmet need, and disease burden
- Real-world evidence to support Health Economics and Outcomes Research (HEOR) and medical affairs activities

2 Bespoke registry

A customized model using innovative technologies and patient-centric approaches to reduce burden, improve retention rates, enhance data quality and streamline longitudinal data collection.

Key characteristics

Bespoke model: data collection based on real-world exposure to a specific product

Own the data: full control of scope, protocol, and engagement with physicians and ownership of all data

High-quality data: meets stringent regulatory standards

Fixed duration: one-time registry to fulfill specific study needs

Unique value

- Customizable, **fit-for-purpose registry** design and data collection/analysis
- **Flexible** program planning
- Design patient-centric protocols to reduce burden and **optimize retention**

Use cases

- Assess product effectiveness
- Monitor long-term safety
- Describe real-world care patterns
- Regulatory authority-required long-term follow-up

Case study – Bespoke registry

Optimized patient engagement and retention for long-term follow-up studies

CHALLENGE

- Long-term follow-up studies monitoring Duchenne Muscular Dystrophy (DMD) treatment utilization to better understand various standard of care approaches to identify optimal patient treatment
- Study data and insights will be leveraged to improve future product development and publication

Challenges:

- Minimize site and patient burden to successfully recruit and retain for the study, given that studies are 5-10+ years in duration
- Sites and investigators were pre-identified by sponsor
- Standard of care practices vary from country to country

SOLUTION

- Proactive recruitment strategy to minimize patient burden and simplify data collection
- Protocol enabling data collection at regular office visits minimizes impact on patient and reduces investigative site burden
- Employed site survey to collect country-specific standard of care practices to eliminate unnecessary assessments and data collection
- Close collaboration and consistent communications with sites and sponsor to improve engagement and retention
- Early engagement with MSL team and sponsor
- Early and ongoing site education
- Leveraged initiation visits and training calls to build relationships between us, the site and sponsor
- Provided a study branded blanket to patients at time of enrollment as a token of appreciation
- Proactive escalation and mitigation to address recruitment challenges early
- Monthly site management calls and 3 IMVs per year to strengthen our site and investigator relationships

RESULTS

- Successful data collection approaches and close collaboration with sponsor to leverage existing site relationships improved study recruitment
- All sites activated. Currently at 96% enrollment target within projected timeline
- Extremely low termination rate at 10%

Abbreviations: MSL= medical science liaison, IMV= interim monitoring visit

Case study – Independent registry

PPD™ CorEvitas™ Rheumatoid Arthritis Registry data leveraged for post-approval comparative safety study

CHALLENGE

- Evaluate safety profile of client's recently approved rheumatoid arthritis (RA) drug
- Client needed to know how their drug performed in comparison with other advanced RA therapies
- Client needed reliable post-approval long-term safety data on their therapy in real-world practice

SOLUTION

- Extensive in-house expertise allowed us to compare incidence rates of 5 predefined targeted adverse events (AEs) over a 5-year period between patients initiating client therapy (JAK inhibitor) and those initiating biological disease-modifying herapies for the treatment of RA within the CorEvitas Rheumatoid Arthritis Registry
- Detailed comparative analysis performed leveraging a longitudinal observational registry
- Real-world patients are generally more diverse and have a longer term follow-up than patients in clinical trials. This analysis also provides context for the use of client's drug through comparison with other advanced therapies used in the treatment of RA

RESULTS

- Existing deep, high-quality longitudinal CorEvitas Clinical Registry data allowed the client to avoid the time and cost of developing their own post-approval registry
- Extensive, regulatory-grade comparative effectiveness data drawn from existing registry data (60,000+ patients)
- Results provided the longest-term real-world safety data for a JAK inhibitor (at that time)
- Provided long-term safety data from the use of client drug in real-world practice, without the exclusions found in RCTs or long-term extension studies
- Over time, this registry model lowers cost per patient

Abbreviations: JAK= janus kinase; RCT= randomized clinical trial; RWD= real-world data

Learn more at **ppd/rwe.com**