The Need for RWE Studies in APAC and the Impact on Product Life Cycle Management

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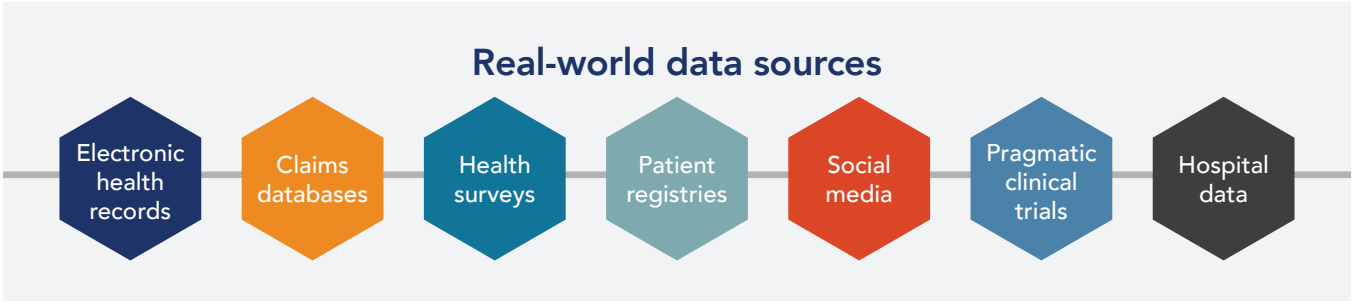
Advances in technology and globalization have enabled significant growth in the pharmaceutical industry over the last decade. Digital initiatives and focusing on patients have also conditioned researchers to think beyond traditional data collection to support product value propositions. As a result, real-world evidence (RWE) is gaining in importance and becoming an important part of managing the product life cycle, not least in Asia-Pacific (APAC).

Home to over half the world's population, APAC is a vast commercial opportunity for pharmaceutical and healthcare companies. Many APAC markets are still emerging and promising rapid market growth, while Japan's and China's market sizes make them priorities for pharma. Markets such as Singapore, Australia, Korea, Taiwan, Thailand and Indonesia are also potentially rewarding. APAC healthcare expenditure will be approximately US $2.4 trillion by 2022, driven by aging populations, increased incidence of chronic diseases, establishment of universal healthcare systems, enhanced reimbursement mechanisms and government's increased focus on the developing healthcare infrastructure.

Although APAC can be a productive region, market access is complex and diverse. Policies in major Asian markets such as Japan, China, Singapore, and South Korea frequently shift and refocus. There is an increasing requirement for evidence, not only from clinical trials but also different forms of RWE. Understanding the evidence risk is an important step for companies entering APAC. Market access and commercial success depend on the evidence produced to address local unmet needs.

Generating evidence to support value propositions to patients, providers, regulators, payers and policymakers is an important requirement for success. Randomized, controlled trials (RCTs) are still the gold standard of evidence for safety and efficacy for marketing authorization of pharmaceutical drugs. However, evidence generated in RCTs lacks general application to clinical practice in real-world settings. RWE is evidence or insights derived from analyzing real-world data derived from sources such as electronic health records, claims data, health surveys, social media and patient registries. RWE is a collection of data supporting treatment patterns, patient pathways, actual health outcomes and safety data beyond RCTs to provide a more comprehensive view of a product's real-life effectiveness and economic value. It bridges evidence from RCTs and from routine clinical practice.

RWE traditionally has been collected to support post-launch activities by demonstrating long-term safety, efficacy, and cost effectiveness in real-world patient populations. However, greater value is available if RWE collection is begun early in product development. The figure below shows the value of RWE across the life cycle of a pharmaceutical product. In the early stages, RWE insights may help



in identifying the focus area for research or supporting decision-making for clinical development. During development, RWE, if used responsibly and in a timely manner, can improve clinical trial programs, making them more cost effective and reducing time to market. Thus, eventually, RWE can affect the approval, access, reimbursement and uptake of the product. Post-launch, RWE studies can help to understand physician prescribing patterns, patient persistence and adherence, treatment switch, discontinuation, burden of disease, healthcare resource use and associated costs, optimal co-therapies, and practices followed in routine clinical care. Such insights are not available from clinical trials but are essential for continued commercial success.

Pharma products have received approvals/line extensions using RWE as controls. The Blincyto study (blinatumomab) is an example of RWE optimizing the registration of a product. Blinatumomab was initially approved by the FDA under accelerated approval with a control arm made of historical data from 694 comparable patients extracted from over 2,000 patient records in the EU and the U.S. A further study in an RCT was required by the FDA to verify the clinical benefit. In another example, Natalizumab was registered for multiple sclerosis in Japan on the premise that an RWE study was conducted to assess its safety, efficacy and tolerability in clinical practice. Between 1998 and 2019 there were 17 cases (including avelumab and nusinersen from Japan) in which RWE was used for new drug applications and 10 for line extensions. RWE was applied in new drug applications/line extensions across therapeutic areas (oncology, immunology/hepatology, neurology, gastroenterology, orphan diseases). For line extensions, RWE was used to support a new indication (blinatumomab, paliperidone palmitate and a medical device), label completion (tolerability and outcomes of fosamprenavir in pregnant women; dosage and administration of nusinersen) and expansion to subpopulations (alglucosidase alfa for children under 8 years old; eculizumab for children; etravirine for children and pregnant women; and palbociclib for adult men). These are a few examples; however, the broader applicability of RWE in accelerating drug development, registration and access to the patient is yet to be fully exploited.

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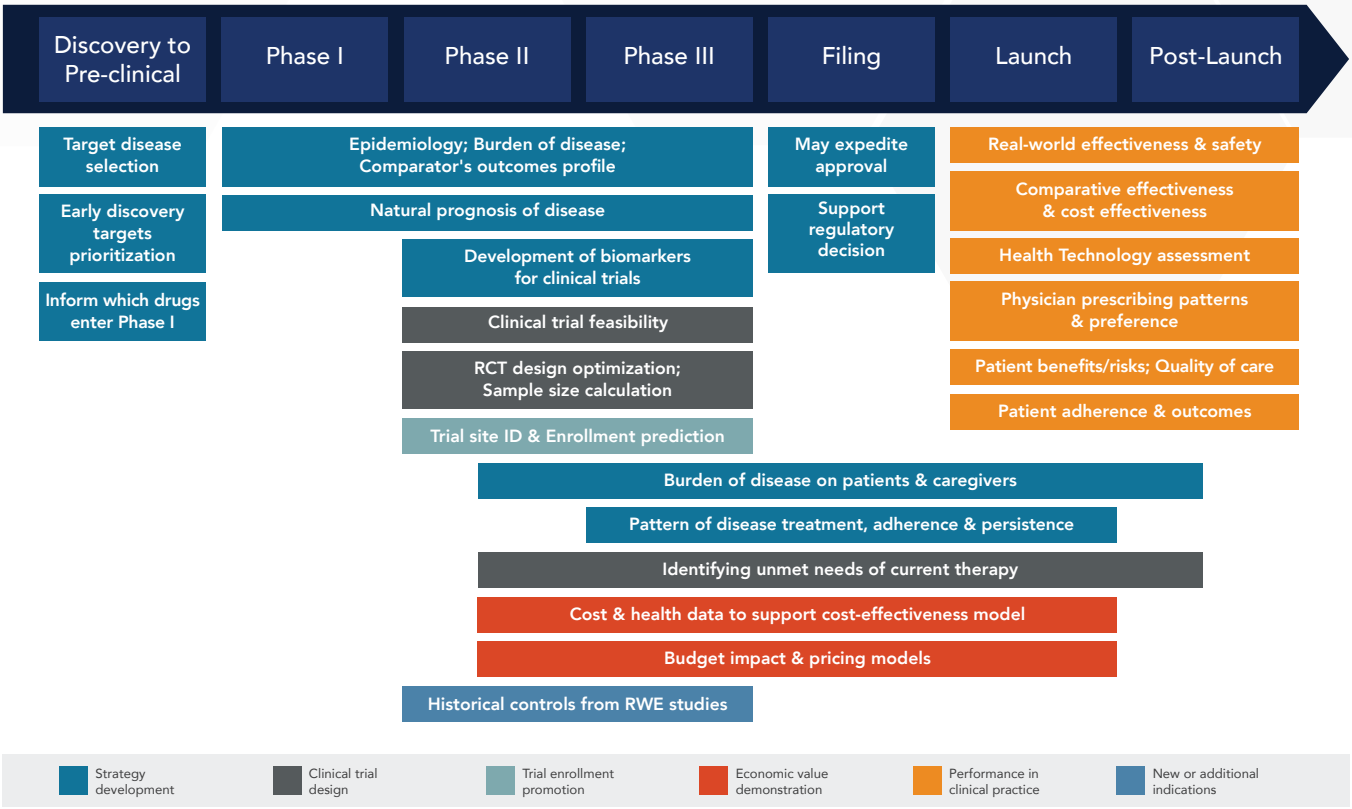
The utility of real-world evidence across product life cycle management



region is appealing for substantial investment from global pharmaceutical and life sciences companies; however, a mix of developed and developing markets presents unique opportunities and challenges for business in this region. With the right strategy in place, pharmaceutical companies can leverage the power of RWE to identify drug targets, optimize clinical trials, accelerate time to market and generate compelling evidence for payer and policymakers.

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