Data Access Use Case Definitions

Use Case	Definition
Understanding mechanisms of disease and cancer disease biology	Real-world data can help researchers better understand how specific types of cancer appear in the wider population, and underlying causes of disease (e.g. highlight the prevalence of specific genetic biomarkers in a specific indication). This information can help pharmaceutical companies understand disease in order to shape research efforts.
Discovery and translation	Real-world data can highlight unmet need in the treatment and management of cancer and where there is the most need for new treatment or management approaches. This helps shape the research, identification and characterisation of new areas for research, discovery of new therapeutics and how this relates to clinical treatment.
Target product profile design	Real-world data and insights can help pharmaceutical companies to formulate their product in order to address unmet needs in a specific indication or population (i.e. how this treatment is administered (oral or intravenously), or the tolerable side effect profile of the treatment.)
Product development and portfolio strategy	Real-world data helps pharmaceutical companies prioritise developmental therapies within their portfolio by evaluating factors such as patient unmet need,gaps in the market, technical feasibility and scientific evidence of effectiveness.
Clinical trial design and feasibility	Real-world data and insights can help pharmaceutical companies design their clinical trials by understanding what is currently available for patients in the market, what is the best treatment comparator to demonstrate effectiveness and safety for particular patient cohorts for a new developmental treatment, and how to quickly identify sites and patients eligible for trials.
Clinical trial execution (e.g. hybrid controlled trials)	Real-world data can help pharmaceutical companies optimise how they deliver their clinical trial (i.e. whether they can structure their trials to have some in-person

	vs. remote monitoring). Hybrid control arms can also be established utilising real-world data as a potential control to compare the effectiveness and safety of the investigational therapy.
Supporting regulatory submissions	Real-world data can be used as supporting evidence by pharmaceutical companies to help them file or submit an application to a regulatory authority (e.g. Medicines Health Regulatory Authority (MHRA) in the UK) to gain approval for patients to take the new treatments. An example of this is the use of Flatiron US data to help men with breast cancer get access to life-extending treatment - palbociclib - previously only approved for women. See here.
Product launch planning	When looking to launch a treatment into the NHS, there are a series of activities that need to be undertaken to better understand the treatment landscape prior to launch in order to position the new treatment accordingly. This includes ensuring an understanding of unmet needs and how to position the new therapy accordingly.
Health Technology Assessment (HTA), Health Economics Outcomes Research (HEOR) and Market Access	When a treatment is approved for use by the NHS, it is assessed to see what relative benefit it brings against potential additional costs of the treatment vs. comparable existing treatments for patients. Cost-effectiveness studies, evaluated by an organisation called NICE (National Institute of Health and Care Excellence) in England and Wales, will make a decision based on this cost-effectiveness assessment and recommend to the NHS whether or not to provide (and fund) this treatment. Pharmaceutical companies can use Flatiron Health UK datasets to inform cost-effectiveness analysis as part of their applications to NICE.
Label / indication expansion	When drugs are approved they are typically approved for a specific indication (e.g. women with specific biomarkers or a genetic profile with later stage breast cancer).

	In the real-world, clinicians may sometimes use drugs 'off-label'. Off-label use means the use of a pharmaceutical drug for an unapproved indication (by the MHRA or NICE) or in an unapproved patient cohort. Real-world data can be used to demonstrate that off-label use of a new treatment can be done safely and effectively, which will support greater patient access to the treatment through additional approved indications (e.g. women with specific biomarkers or a genetic profile with early and later stage breast cancer).
Understanding and monitoring treatment landscape	Understanding patterns of care and treatment in the real-world, and how this maps against patient experiences and outcomes to identify unmet need or where patients may not be getting access to the right treatments (in line with guidance from NICE). This informs the strategies of pharmaceutical companies and where they develop therapies to address unmet needs.
Safety monitoring	Monitoring of adverse events or safety issues with treatments in the real-world is critical given the expanded number of patients who have access to the treatment compared to clinical trials (which is a smaller number of patients with a narrower focus) . If there are new or an increased number of adverse events following approval, the treatment will need to be reinvestigated to ensure safety and assess whether any changes to the label need to be made. Pharmaceutical companies are required by law to monitor and report such events to the MHRA.