World PI Week Refresher on Data

What is Health Data?[1]

Health data is described as "any personal data related to the physical or mental health of a person, including the provision of health care services (doctor referrals, medical examination reports, prescriptions, laboratory tests etc), which reveals information about that person's health and medical status.[2]" Reliable health-related statistics are essential to understanding the state of health care delivery around the world and many countries lack the health information systems they need to accurately monitor health trends and inform decision-making[3].

How is it collected and used?

In general, information on a patient's health and lifestyle can be collected by individuals or healthcare professionals, during a doctor or hospital visit, and is stored as health data in patient records. Researchers, clinicians, doctors and public health experts benefit from these patient records and use them to develop or improve diagnosis approaches, clinical care, medical & scientific research and strengthen medical education. This data can also be aggregated at a larger population level, cross checked from different sources, for broader knowledge purposes and to improve overall healthcare systems.

What do we mean by "real-world" data (RWD)?

Altough there is no consensual definition, real world data is an umbrella term for different types of health care data related to the effects of health interventions, that are not collected in conventional clinical trials. Such data come from various sources and include patient data, data from clinicians, hospital data, data from payers, and social data – such as clinical and economic outcomes, patient-reported outcomes and health-related quality of life.

RWD can complement clinical trial data to support medicine assessment and authorisation, as well as regulatory decisions in smaller patient populations like PIDs. It is valuable to strengthen understanding on the effectiveness and impact of a treatment or care pathway on the long-term.

What are patient registries (or databases)?

Patient registries are repositories —for one or more purposes—of standardized information about a group of patients who share a condition or experience. They may be organised either per disease or product (treatment)[4]. Therefore, patient registries allow widespread clinical and laboratory data on selected patient populations to be gathered that can be used to foster advances in scientific research and ultimately improve patient care.[5] Patient registries are an important source of real-world data, and allow to collect such data in a coordinated way, using observational methods and following the same patients over time[6].

What is the value of health data in primary immunodeficiency (PIDs)?

Due to existing knowledge and awareness gaps and the complexity inherent to this field, the diagnosis and management of rare diseases such as Primary Immuno-deficiencies (PIDs) can be difficult.

» **Diagnosis:** Collecting & sharing data can support greater knowledge about the disease and boost testing and early diagnosis; and the development of efficient diagnostic tools.

» **Treatment & Care:** Data analyzed through registries provide important information about patients, the impact, efficacy and durability of treatment to strengthen their care, as well as help decision makers improve healthcare systems.

» **Research & Innovation:** Turning data into evidence help advance understanding of PIDs and is crucial to develop innovative therapies and new care pathways and make these accessible to all PID patients.

» **Medical Education:** Improving awareness and education for healthcare professionals and specialists through evidence-based data is important to strengthen expertise, which will allow accurate diagnosis and treatment of PIDs.

1   "What Is Health Data?" Healthy Data. Accessed February 28, 2023. https://ourhealthydata.eu/what-is-health-data
2   "Art. 4 GDPR – Definitions." General Data Protection Regulation (GDPR), March 29, 2018. https://gdpr-info.eu/art-4-gdpr/.
3   "Understanding Global Health through Data Collection." World Health Organization. World Health Organization. Accessed March 3, 2023. https://www.who.int/activities/understanding-global-health-through-data-collection.
4   "Patient Registries Are a Valuable Source of Real-World Data (RWD)." Data Saves Lives. Accessed February 28, 2023. https://datasaveslives.eu/patient-registries-are-a-valuable-source-of-real-world-data-rwd.
5   Lopes JP, Cunningham-Rundles C. The Importance of Primary Immune Deficiency Registries: The United States Immunodeficiency Network Registry. Immunol Allergy Clin North Am. 2020 Aug;40(3):385-402. doi: 10.1016/j.iac.2020.03.002. Epub 2020 Jun 7. PMID: 32654688; PMCID: PMC8183626.