Integration of Real-World Data and Evidence into Assessments to Support Decision-Making in the Pharmaceutical Sector

English summary

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SUMMARY

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Background

In the pharmaceutical industry, the approaches traditionally preferred for demonstrating the efficacy and safety of medications rest on randomized control trials (RCT), and such knowledge forms an important basis for assessments in regulation and public coverage of drugs. This situation is evolving: the desire to support the decision-making process with data that are representative of the implementation setting, as well as the growing availability of vast databanks, have led to real-world evidence (RWE) being considered as a complementary and useful source of knowledge. The nature of the underlying data as well as the methods required for their collection, analysis and appraisal, however, raise several issues regarding the integration of RWE in evaluations aimed at supporting decision-making. The decision was made to produce a state of knowledge on this topic, focused on three main operational objectives:

- establish a common organizational understanding of concepts related to RWE and real-world data (RWD);

- equip the production teams at INESSS with methods to appraise RWE/RWD in their assessments;

- support the implementation of institutional requirements for the information needed when submitting a drug assessment request that includes RWE, as well as the initial acceptability of such evidence for integration into evaluation projects.

Methods

The objective of the present state of knowledge report is to clarify the following: 1) definitions and characteristics of RWD and RWE; 2) potential uses for RWE during the various phases of the life cycle of drugs; and 3) considerations related to using RWE in assessments to support decision-making in the pharmaceutical sector. Therefore, a narrative review of the scientific and grey literature was carried out, more specifically with respect to drug assessments although several other areas relevant for INESSS's activities were also included. The state of knowledge report is solely for informational purposes and does not constitute a position paper nor recommendations.

Results

There is no Canadian or international consensus-based definition for RWE nor for RWD. Various definitions and characterizations have been identified, which are not fully consistent and coherent with each other. However, two dimensions in particular appear to be useful and applicable when characterizing RWE: the setting or context that generated the data and refers to the source of the data (e.g., clinical or community milieus), on the one hand, and the methodological approaches used to carry out the study (such as its design and level of pragmatism), on the other. The main benefits of RWE with respect to drug assessment are the type of information collected and the external validity of the studies, whereas the challenges concern the quality of the data, internal validity, and ethical and governance issues.
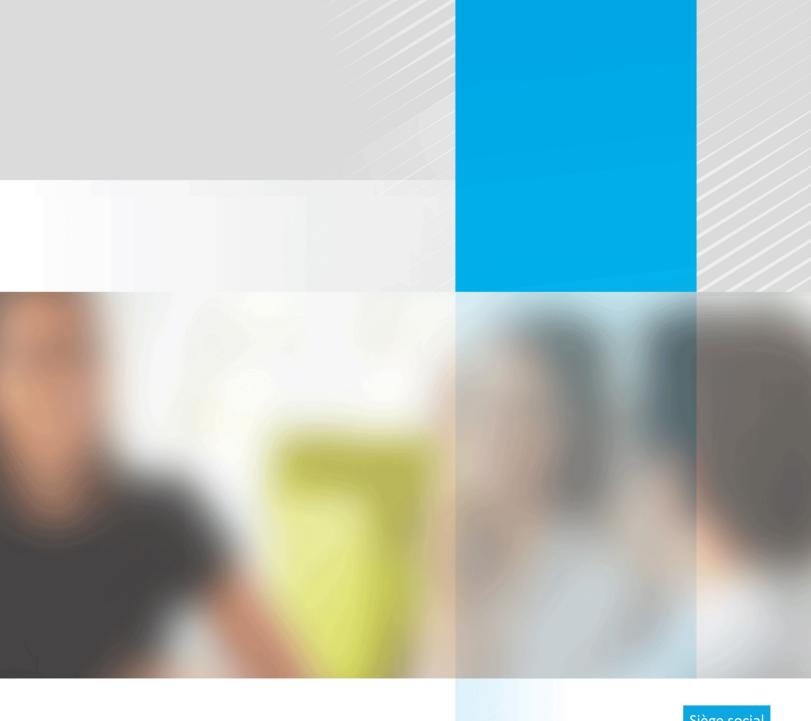
Use of RWD and RWE in the pharmaceutical sector is noted to occur at various times in the life cycle of products. A review of practices, however, shows that regulatory and assessment agencies remain relatively cautious about using RWE as a primary source to support decision-making. Its acceptability would be linked to use in specific situations, such as when it is not possible to conduct an RCT, in assessments for rare diseases or advanced therapies, in cases of unmet health care needs, and when a major treatment effect is expected.

INESSS identified a large number of methodological tools to appraise study quality, with these tools generally being classified according to how treatment is assigned (randomized or not). Appraisal of the quality of both the sources and the data themselves is not consistently considered, and only two tools were identified that directly address these elements: the "Assessment of Real-World Observational Studies critical appraisal tool" and a "Questionnaire to assess the relevance and credibility of observational studies to inform health care decision-making". Recommendations also exist to promote transparency in reporting results from real-world studies, particularly for observational studies that use data routinely collected in pharmacoepidemiology (RECORD-PE: Reporting of Studies Conducted Using Observational Routinely Collected Health Data Statement for Pharmacoepidemiology).

Lastly, the suitability of RWE to support regulatory or drug listing decisions can be viewed as an appraisal of the potential or capacity for the evidence to respond to a specific decisional question. Despite their differences regarding some points, the various conceptual frameworks identified recognize that this suitability for use results from the interaction between aspects related to the data themselves, the data collection and analysis methods, and the decision-making context.

Conclusion

Based on a review and analysis of the literature, there is broad national and international discussion of RWE, as well as abundant documentation. Many applications of RWE are suggested during the life cycle of medications, but the standards and rules for the integration of RWE into assessments to support decision-making are still being developed. Authors suggest in particular that the various organizations involved work together to harmonize the following: 1) recommendations on quality standards for real-world data and studies; 2) requirements with respect to the acceptability of RWE for the various types of questions regarding decisions; and 3) practices for integrating such evidence in decision-making. Lastly, the topics covered by this state of knowledge report, as well as other related subjects, represent a rapidly shifting area, and the work carried out by various regulatory and assessment agencies will be monitored to enhance learning. Despite the challenges related to integrating RWE into assessments, the model for demonstrating evidence is evolving, and the various stakeholders involved are working to adapt to this changing environment.

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