Real-World Evidence and Advanced Soft Tissue Sarcoma: An Unbreakable Bond (ESMO Sarcoma and GIST Symposium 2020, Milan, February 4, 2020)

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Randomized controlled trials (RCTs) are the gold standard of evidence-based medicine, although they should not be regarded as the only way to address clinical questions. Highly controlled conditions and carefully selected patient populations mean that RCTs may not be truly representative of treatment situations and patients encountered in actual clinical practice. Moreover, for rare diseases, recruiting sufficient numbers of patients to achieve statistical power may be impossible.

Soft tissue sarcomas (STS) represent more than 170 distinct disease entities as per the most recent WHO classification; the majority of these sarcoma subtypes have an incidence of fewer than 2 cases per 1,000,000 inhabitants/year [1]. Gathering sufficient evidence about individual subtypes to address concerns of regulatory authorities who may be accustomed to evaluating far more common cancers, and creating the clinical trial data required for evidence-based clinical decision-making is a major challenge.

Observational research (e.g., retrospective and registry analyses) captures how patients are treated in clinical practice and is often used to address issues that RCTs cannot. As nearly all clinical decisions involve a degree of probabilistic reasoning and uncertainty, a realistic goal of observational research may be to achieve the level of certainty required to inform a diagnostic or treatment decision. To this end, real-world data and real-world evidence are playing an increasing role in healthcare decisions, in-

cluding in the sarcoma field. Real-world data relate to a patient's health status and/or healthcare delivery collected routinely from a variety of sources (e.g., electronic medical records, insurance claims, billing records). Real-world evidence is the distilled knowledge about the use and potential benefits/risks of a medical intervention derived from analysing real-world data.

Thanks to international collaborative efforts, the sarcoma field has a unique opportunity to gather a comprehensive repository of real-world evidence. To explore the contribution of real-world data to the development and use of available therapies in advanced STS, a symposium entitled "Real-world evidence (RWE) and advanced soft tissue sarcoma (STS): an unbreakable bond" was held in Milan, Italy, on February 4, 2020. The symposium opened with an overview of real-world data and real-world evidence, defining the terms and outlining the strengths and limitations of using such data. This was followed by case studies which illustrate the contribution of real-world data to informing research and development and directing treatment decisions in advanced STS.

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Conflict of Interest Statement

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The author contributed significantly to the conception of the article. The author has participated in drafting and/or revising the manuscript and approved its submission.

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