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Chemotherapy regimens in rare solid tumors

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Preface

Rare tumors (RT) are an increasing problem in oncology. A tumor is defined as "rare" if its incidence is ≤ 4 cases/100,000 people/year. Taken globally, such carcinomas are approximately 15–20% of all tumors, and represent a highly relevant health care problem.

The main characteristic of RT is the low frequency of the event, that determines difficult and sometimes late diagnosis and a great uncertainty in diagnosis and treatment. The expertise (i.e., knowledge about a specific RT and the number of cases managed in one year by a single clinician or group of clinicians) is the most important element for an adequate treatment of a patient with RT, together with a multidisciplinary approach. In fact, the occasional nature of the event, the scarcity of evidence-based proofs that can help diagnosis and therapy, and the lack of high-quality available medical literature expose the oncologist to the risk of diagnostic, prognostic, and therapeutic errors, especially if the information exchanged between the professionals involved is poor. As a consequence, the patient with RT often feels alone in facing the disease, because he/she does not find in other clinicians, especially if they are not part of a specialized center, the expertise and skills to manage the disease.

The most common results are, on the one hand, a forced migration of patients far from their residence in order to find doctors or centers of expertise, and on the other hand, a reliance on "miraculous treatments" of alternative medicine.

Moreover, RT can cause difficult problems in health care organization. Two very different organizational models can be found:

- a concentration of cases in a few National Centers of Excellence, as happens in Scandinavian countries; and
- a diffuse sharing of expertise in all hospitals, each authorized to treat patients with RT, as happens in the German model.

In the first model there is a risk of long waiting lists; in the second there is an increase of health care costs related to the proliferation of centers and the possible disparity of results between institutions with high expertise and general hospitals.

A better balanced model is the spread of the clinical knowledge in order to guarantee a good level of performance in early diagnosis and in first-level exams in general hospitals; while concentrating patients in referral insti-

tutes during the most complex periods, such as surgical intervention, therapies with innovative technology, or experimental medical treatments.
Since RT are neoplasms with different anatomic site of distribution, not a single expert is needed. For instance, pleural mesothelioma should be referred to a thoracic surgeon while a uterine sarcoma is a gynecological problem. So, the multidisciplinary team for every RT requests a complex organization to be realized in a wide geographical area.
Leading centers should be identified, in order to obtain from them support and learning, to diffuse the knowledge of these diseases, and to highlight the fundamental steps in diagnosis and therapy: this is the best way to assure the most adequate treatment to patients with RT.
This book cannot and does not try to offer solutions to all the problems related to RT; either it is not a simple handbook, making such a complex topic trivial. The aim is to offer a little help in spreading the knowledge of low-incidence tumors in oncology, in order to alert the nonexpert clinician to take them into consideration, and to show the most accredited and updated medical treatment for some of the rare oncologic diseases. This booklet is not conclusive, but we hope to give help in prescribing treatments for the main RT, underlining the need of a multidisciplinary approach in these tumors, as well as cooperation with the centers of expertise.
For the sake of brevity and competence, only solid RT are included; for the lymphoproliferative or hematologic low-incidence diseases we refer to hematologists. Each chapter provides brief information about the epidemiology of and clinical aspects for every RT; and, following each chemotherapy schedule, we report a short description of the hematologic toxicities related to the treatment. In this edition only the treatments approved by regulatory Agencies are reported, in order to avoid the off-label use, which is frequent and sometimes overused in RT. In few cases, we have indicated schedules and therapies not yet approved, but which are supported by strong literature evidence. These cases are specified.
We thank in advance all the readers who may assist us to improve the text giving us their advice and suggestions. We hope to be useful for everyday clinical practice.

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