European consensus on methodological recommendations for clinical studies in rare cancers

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One out of every five new cancer patients is diagnosed with a rare cancer, yet the clinical evidence needed to effectively treat these rare cancer patients is scarce. Indeed, conventional cancer clinical trial methodologies require large numbers of patients who are difficult to accrue in the situation of rare cancers. Consequently, building clinical evidence for the treatment of rare cancers is more difficult than it is for frequent cancers.

Dr. Jan Bogaerts, EORTC Methodology Vice Director, points out, "For rare cancers, we need alternative ways to conceive study designs and to analyze data. Also, the possibility to combine results would be

particularly advantageous. Certainly, in the situation of rare cancers, collaborative networking is indispensable, and, of course, all of this needs to be funded properly."

In order to develop effective clinical trials for patients with rare cancers, international cooperation of experts with complementary skills and knowledge are required to find new ways to treat these diseases. In this light, the recommendations put forth by Rare Cancers Europe were the result of a consensus reached through multi-disciplinary and multi-stakeholder discussions. Rare Cancers Europe is dedicated to putting rare cancers firmly on the European policy agenda, and the EORTC is among the Cooperating Organizations.

These recommendations cover clinical decision-making in rare cancers, study designs in rare cancers, surrogate end-points in rare cancers, and critical organizational aspects of clinical research in rare cancers. In effect, they represent a road map showing the way to design trials to test new treatments in patients with rare cancers.

Rare Cancers Europe's recommendations on the methodology for clinical studies in rare cancers are available in *Annals of Oncology*.

More information: www.dx.doi.org/10.1093/annonc/mdu459

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