

Symposium 13

SY – 13 – 1 Rare cancers; off label use

Tatsunori Shimoi^{1,2}, Kan Yonemori^{1,2}, Akira Kawai^{2,3}

¹National Cancer Center Hospital, Department of Breast and Medical Oncology,

²National Cancer Center, Rare Cancer Center, ³Head of Musculoskeletal
Oncology and Rehabilitation Medicine, National Cancer Center Hospital

Rare cancers according to RARECARE definition account for as many as 15% of new cancer cases in Japan. Because of the rarity, rare cancers are difficult to study. The concept of off-label use is so widespread in rare cancers. A European consensus position paper published in 2014 said that regulations should cover appropriately and timely approval and have some relaxation in rules on the compassionate and off-label use of drugs in rare cancers. There are various drugs which have difficulties to handle in rare cancers treatment. It might be solved with centralization. Moreover, concerned with new therapeutic development, the enrollment in a study of an adequate number of eligible patients is not feasible in rare cancers. Some appropriate statistical designs for rare diseases are proposed. For example, control of statistical probability, Bayesian-design trials, as well as adaptive trials design. I would like to discuss future management of rare cancers in Japan.