



21 June 2016
EMA/328790/2016
Human Medicines Research & Development Support Division
Human Medicines Evaluation Division

EMA-ESMO workshop single-arm trials in cancer drug evaluation

30th June 2016, 9:30-16:30, 3A

Chairs: Francesco Pignatti (EMA); Paolo Casali (ESMO)

| Agenda | Time |
|--|---|
| Registration | 08:30 - 09:30 |
| Welcome & introduction <ul style="list-style-type: none">Problem statement - F. Pignatti (EMA) | 9:30 - 9:45 |
| Scenarios and case studies: from early molecular concept to regulatory/HTA decisions <ul style="list-style-type: none">Crizotinib in patients with ROS1+ Non-Small Cell Lung Cancer: Rationale and results – M. Rothenberg (EuropaBio/Pfizer)Use of single-arm cohorts/trials to demonstrate clinical benefit for breakthrough therapies – E. Rubin (EFPIA/MSD)Uncontrolled evidence in rare cancers - P. Casali (ESMO, RCE)Discussion | 09:45 - 10:05 10:05 - 10:25 10:25 - 10:45 10:45 - 11:15 |
| <i>Coffee</i> | 11:15 - 11:45 |
| General issues and methodological considerations I <ul style="list-style-type: none">Perspectives on non-randomized trials in oncology drug development - G. Blumenthal (FDA)Can we improve the reliability of SAT in Oncology? - P. Bruzzi (INT)Project Data Sphere®: Progress & Promise of this Digital Library-Laboratory - M. Murphy (Data Sphere LLC)Single-arms within platform trials - R. Kaplan (MRC)Discussion | 11:45 - 12:00 12:00 - 12:15 12:15 - 12:30 12:30 - 12:45 12:45-13:15 |
| <i>Lunch</i> | 13:15 -14:15 |
| General issues and methodological considerations II <ul style="list-style-type: none">Clinical trial design- a patient perspective - B. Ryll (ESMO PAWG)Clinical research in small patient populations: the investigator point of view - J. Rodon (VHIO, ESMO)Single-arm trials: a good step towards faster access/reimbursement of drugs? - M. Neyt, KCE, EUnetHTA)Discussion | 14:15 - 14:30 14:30 - 14:45 14:45 - 15:00 15:00 - 15:15 |
| Open discussion <ul style="list-style-type: none">Need for further regulatory guidanceWrap up and outlook | 15:15 - 16:30 |

