European Alliance for Personalised Medicine

Need for Harmonized Pathway for prognostic/predictive Biomarkers

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Abstract

For personalised medicine to advance, it is vital to have more well validated prognostic/predictive biomarkers, however since diagnostic information is not fully valued within our healthcare system, there is not a well defined development and approval process, resulting in lack of clear rule for marketing clearance and mechanisms of reimbursement (except for companion diagnostics which are linked to a specific drug and to which drug approval regulations are applied).
Such prognostic/predictive biomarkers are of primary interest for “personalized medicine” because they are usually aimed to address major unmet needs like improving the tailoring of standard therapeutic options or improving the selection of patients for targeted agents on the market. For instance, adjuvant therapies are commonly used in many cancer types but “average benefit” and the “one size fits all” trial design is resulted in exposing the majority of patients to either an undue overtreatment or an in-effective therapy, with only a minority really needed the treatment.
Despite the obvious benefit for many stakeholders that can derive by adopting effective “prognostic/predictive tools” for the adjuvant treatment decision making, there is an unacceptable lack of common rule and pathways for approval and reimburse. Therefore, with the present regulation, a diagnostic company after having developed a potential clinical useful diagnostic tool needs to deal with each European country facing undefined, unclear and heterogeneous pathways for evaluation, approval and reimbursement. This uncertainty discourages investments in the field of biomarker development. Moreover, the different pathways of approval and reimbursement among countries lead to an unacceptable heterogeneous access to potentially useful device. There needs to be an evolution in approach where biomarkers become an integral to all healthcare research rather than an optional element.

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Objectives

1. Define for policy makers suggested evidence requirements framework for biomarkers in oncology both in terms of research and clinical practice for both regulatory and reimbursement perspective
2. Highlight emerging trends in oncology research that could impact future regulatory and reimbursement decisions:
   - Study Design Methodology: ie Big Data analysis or Adaptive approaches
   - Technological: ie. NGS – panel of genes, imaging, etc
   - Biological insights: liquid biopsy, immunotherapy
   - Healthcare structure: centralization vs. localization, addressing smaller populations
   - Patient rights: Privacy, informed consent
3. Importance of clinical utility
4. Exemplars: KCE in Belgium – NGS, NICE Diagnostic Pathway

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Messages

1. Biomarkers are needed to advance personalised medicine
2. The current regulatory and reimbursement environment is inhibiting biomarker development
3. The field is in its infancy and getting more complex, so clear frameworks for evaluation are needed

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