EU REGULATORY AND LEGAL CONSTRAINTS TO CLINICAL TRIALS ON RARE CANCERS

Rare Cancers Conference

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CONTENT

1. Introduction
2. Landscape
3. Current barriers
4. What is already being addressed
5. What is not yet addressed - proposals

LANDSCAPE: GENERAL

- Rare cancers represent about 24% of all
- Many unmet needs -> major society question
- Not much is known about biology of rare cancers -> need to learn
- Need for international / global trials with strong translational research -> very expensive trials
- Industries are rarely interested to address these questions & not much funding is available from other sources
- Major advances in rare cancers are made by IDCT trials: e.i. GIST sarcoma or glioblastoma

LANDSCAPE: 2 TYPES OF TRIALS

- **Investigators Driven Clinical Trials (IDCT): major missions**
  - to understand the biology of rare cancers
  - find state-of-the art innovative treatments
- **Drug development**
  - industry is rarely interested (despite orphan status) to run trials in rare cancers -> not a worthwhile investment
  - it can simply be not in line with the business plan
  - need for a reliable & transparent partnership with the industry
  - need to preserve criteria of academic independency

CURRENT REVISION OF THE DIRECTIVE EXPECTATIONS

- **Single electronic submission portal in English for CA & EC (all inclusive, no additional "national" submissions)**
- **Coordinated Assessment Procedure**
- **Single communication of decisions of countries (listing all countries where trial can start & mentioning opted-out MSs)**
- **Risk based approach and requirements fit to the risk**
- **Revision / clarifications of key definitions (e.i. IMP, Sponsor)**

HOW COULD THIS HELP RARE CANCERS?

- EASY, QUICK and FINACIALY sustainable activation
- Many would qualify for a medium to low risk trial -> more trials would be feasible again
- With comparator, concomitant and background medication clearly being non-IMPs, more trials would be done within the existing budgets: science will progress faster
- Industry may be more interested in registering new indications (worthwhile investments)

WHAT IS NOT (yet?) ADDRESSED (1)

- Funding international IDCT :
  - A European Fund should be created for addressing unmet needs in rare cancers (funding clinical trials)
  - Solution should be found for these patients to have access to promising drugs, including when industry is not interested to explore them for rare cancers
  - Europe should think about long term sustainability of its expertise and capacity to run large international independent academic clinical trials:
    currently existing national support & solutions are not sufficient, unsustainable & does not take into account specific international needs

WHAT IS NOT (yet?) ADDRESSED (2)

- Authorization of platforms instead of individual trial (complementary trials, consequent trials, multi-cancer type screening trials etc…)
  - Simultaneous start
  - Maximization of the use of data and biological material
  - Maximization of resources: scale economy

WHAT IS NOT (yet?) ADDRESSED (3)

- Support and stimulation of translational research:
  - Residual material is frequently wasted
  - Storage is limited in time (e.i. 15 years)
  - Patient's consent for future research is discouraged
- Stimulation of global trials
  - Divergent requirements (EU versus US versus Australia etc…)
  - Need for a local representation of sponsor
  - Drug distribution & supply barriers
  - Etc…

WHAT IS NOT (yet?) ADDRESSED (4)

- Extension of label:
  - Nobody else, but the industry can currently extend the label… what if industry is not interested? and what about generic drugs?
  - Feasible trials may not fit regulator's requirements for authorizing a new indication
- Drug development:
  - What if no available drug fits the purpose?
  - How can Europe have rare cancer oriented drug development agenda?

CONCLUSION & TAKE HOME MESSAGE

- Over 22 % of cancers are rare cancers
- Numbers are rapidly increasing: fragmentation of sub-types / better characterization of tumor biology
- Personalized medicine is a reality
- Challenges of research in rare cancers & orphan indications should be urgently addressed
- Law should not be a barrier but a frame

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Thank you for your attention

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