EU REGULATORY AND LEGAL CONSTRAINTS TO CLINICAL TRIALS ON RARE CANCERS

Rare Cancers Conference

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1. Introduction
2. Landscape
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Rare cancers represent about 24% of all cancers.

Many unmet needs lead to a major society question.

Not much is known about the biology of rare cancers, indicating a need to learn more.

The need for international/global trials with strong translational research is very expensive.

Industries are rarely interested in addressing these questions, and there is not much funding available from other sources.

Major advances in rare cancers are made by IDCT trials, for example, 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
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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Building a bright future together requires:

WISDOM - COURAGE – VISION
FRANCOISE MEUNIER, MD, PhD, FRCP
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Thank you for your attention