EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Histology-independent indications

and resulting challenges in the context of orphan designations

Orphan office perspective

3rd Industry Stakeholder Platform on R&D support, 18 May 2018

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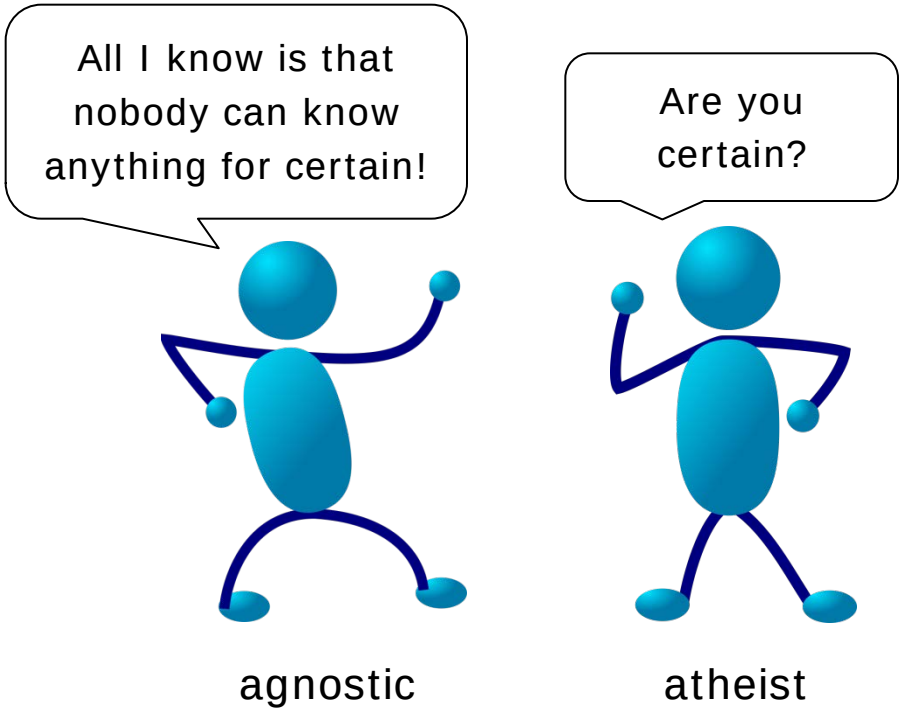
An agency of the European Union

Tissue agnostic / histology independent



The EU orphan condition

- Regulatory context dependent and may not be the same in a non-orphan regulatory setting.
- For this purpose COMP has not defined new conditions to serve the regulation or the development of a specific product but relies on established terminology and classifications.
- Does not need to translate into the therapeutic indication directly, but will broadly cover the indication.

Outside the EMA

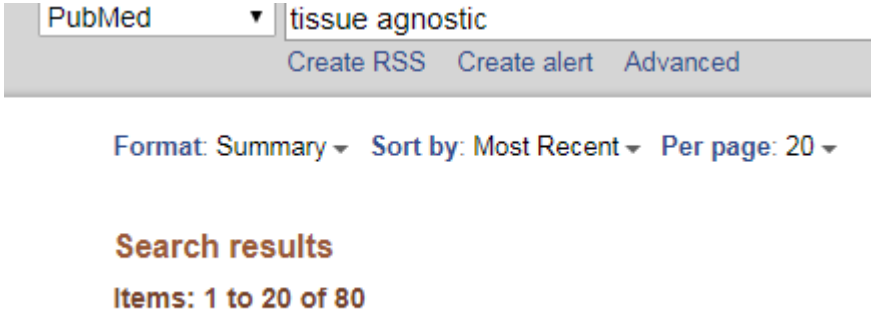
"If a biomarker will, in essence, define the disease indication, then it should be developed through the collaboration of multiple stakeholders including commercial sponsors, device manufacturers, academia, and patients."

S. Lemry et al. NEJM Oct 2017 (FDA)

Limited publications on the topic.

Search:

- "tissue agnostic" 9 hits
- "histology agnostic" 4 hits



Challenges

- How to define a histology independent condition to reflect a “disease”?
- Alternatively, designate all underlying cancers + biomarker subsets.
- If this was overcome, how justify significant benefit?
  - many products would likely be approved for the patients
  - but not the same product for across histologies
- How to establish the natural history of the histology independent condition?