

# Repurposing an old drug in a new ultra-rare indication: pilot of sirolimus in EHE

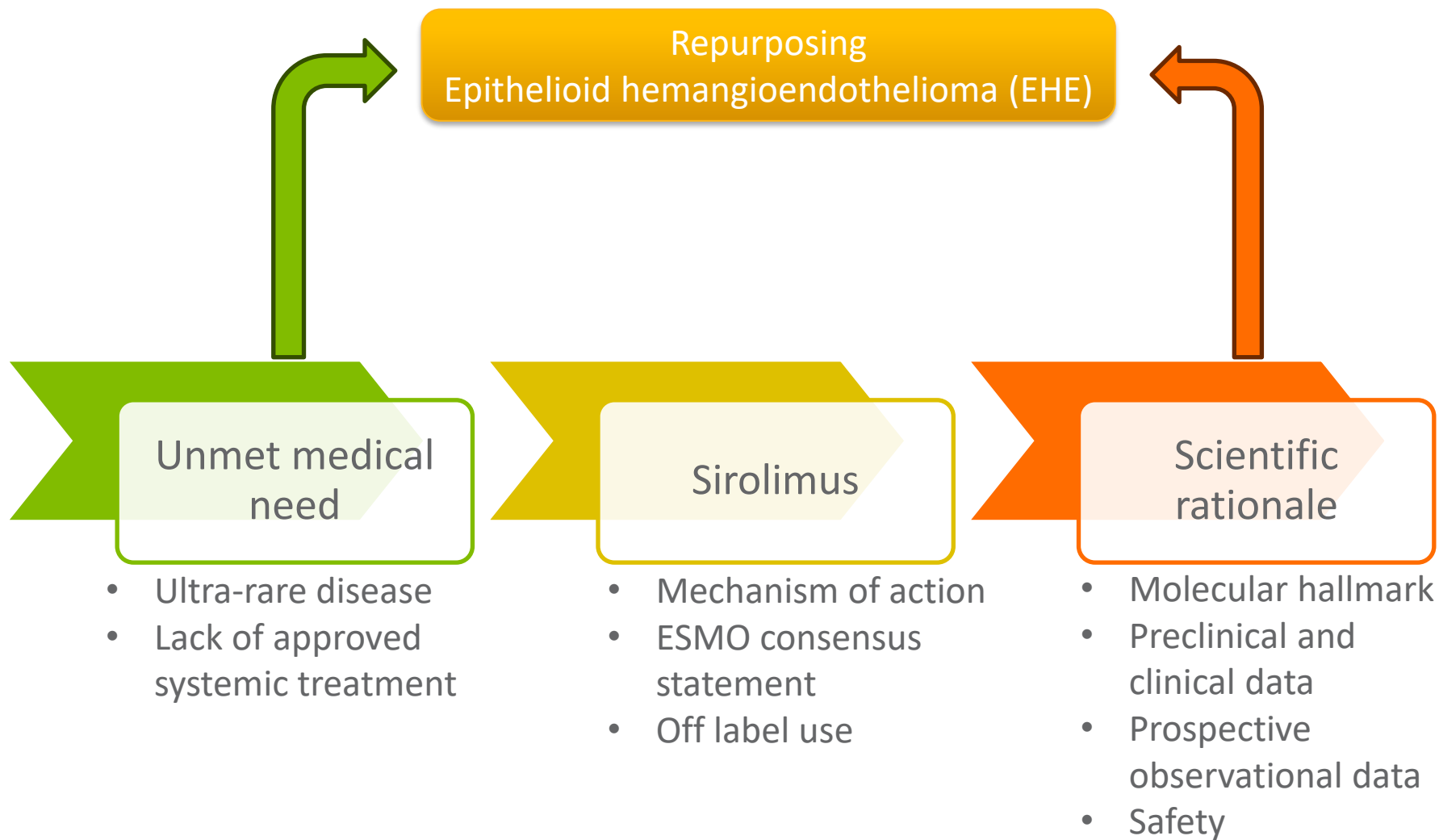
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No conflicts of interest

Pan Pantziarka

No conflicts of interest



# Repurposing

- **Molecular hallmark**

- The molecular hallmark of EHE, oncogenic fusions YAP/TAZ, are involved in activation of mTOR complex 1

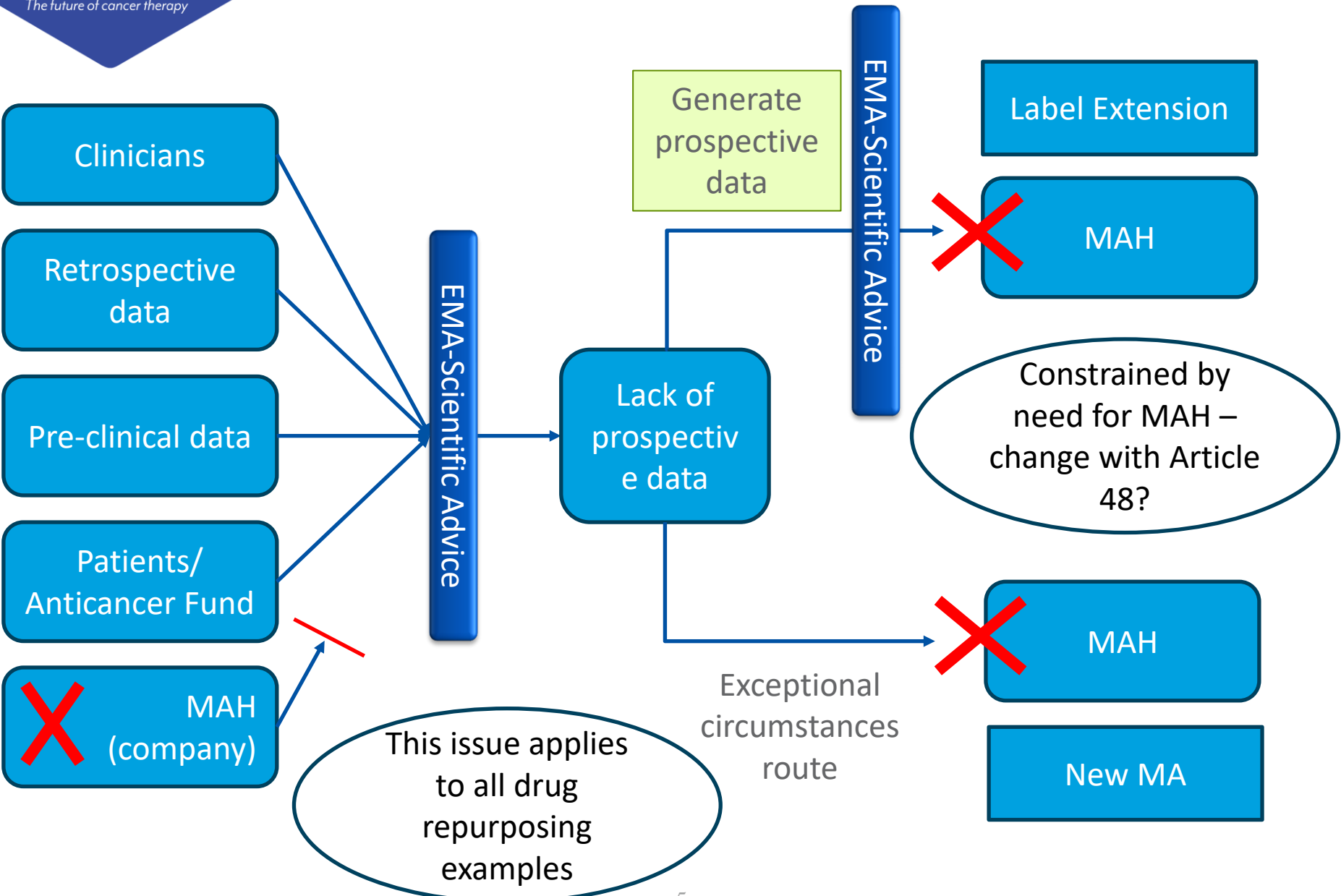
- **Pre-clinical data**

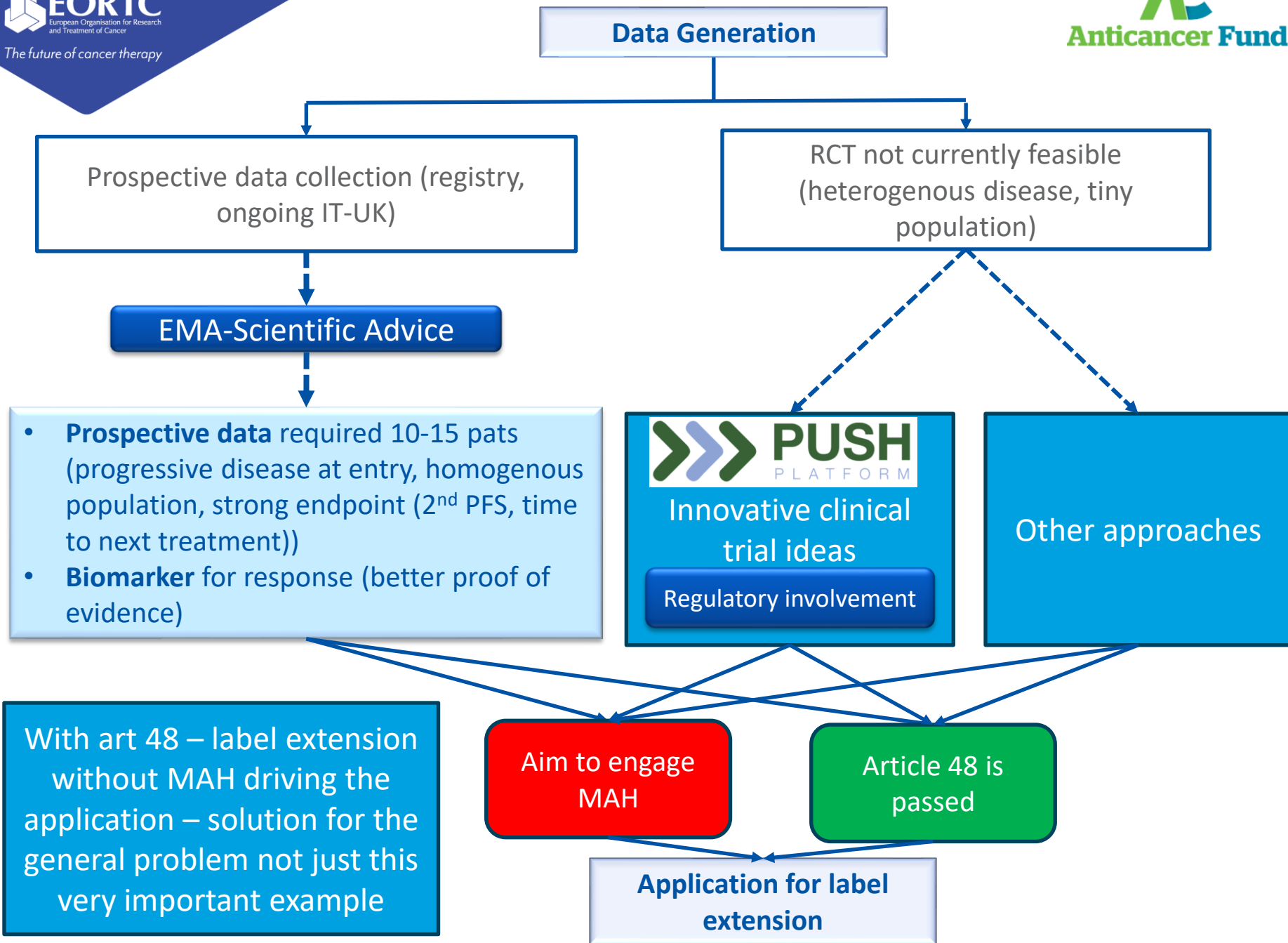
- Preclinical data regarding the comparative activity of doxorubicin and sirolimus in an EHE, WWTR1-CAMTA1-translocated patient derived model → sirolimus induced an 80% tumor volume inhibition, negligible for doxorubicin.

- **Clinical data**

- **Prospective** data: no prospective data on the activity of sirolimus/mTOR inhibitors are available at the moment. A prospective registry study (Italy and UK) is on-going.
- **Retrospective** Italian study with 38 pats (including 13 with serosal effusion) with PD at baseline. No Gr4-5 toxicity observed. (DOI: 10.1002/cncr.33247).

|                | EHE<br>N=38 | EHE pats with serosal effusion<br>N=13 |
|----------------|-------------|--|
| Evaluable pats | 37          | 12                                     |
| Median PFS     | 13 mo       | 4.8 mo                                 |
| Median OS      | 18.8 mo     | 10.6 mo                                |





## Questions:

- Can we formalise requirements for prospective data collection for ultra-rare cancers?
- When do we think article 48 will be effective?



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