EUROPEAN MEDICINES AGENCY
SCIENCE MEDICINES HEALTH

Better Science, Better Health: New Healthcare Models: a drug regulatory view

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"The safest drug that no one can afford or that arrives too late is of no benefit to a patient"

US-based patient representative

Addressing competing objectives

Allow timely access for patients to address urgent medical need	⟷	Allow only well-studied drugs on the market
Enable precision medicine, 'difficult' indications	⟷	Rely on robust study methodology and end points

'Difficult' indications?

Smaller treatment-eligible populations ('orphanisation')

1989: one disease: 'Cystic Fibrosis'

- all patients randomised in same study

2015: multiple CF subgroups defined by mutations

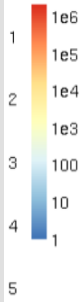
- homozygous F508del-CFTR mutation → **RCT**, **parallel group***
- F508del-CFTR heterozygous with residual function mutation on the second allele → **RCT**, **cross-over***
- Other, less frequent mutations → **n-of-1 or uncontrolled**?

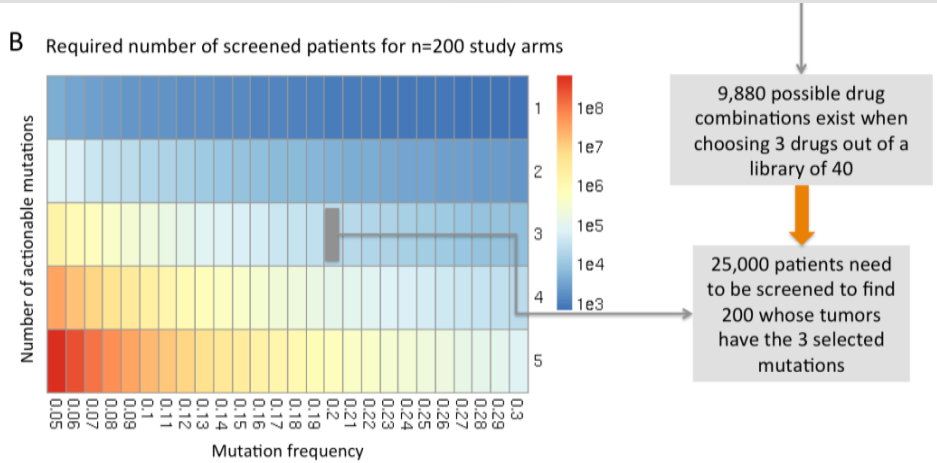
*Source: EMA website: Paediatric Investigation Plan VX-661/Ivacaftor

Is there a future for single drug interventions?

combinatorial complexity of personalised Rx combinations:

9,880 possible drug combinations exist when choosing 3 drugs out of a library of 40



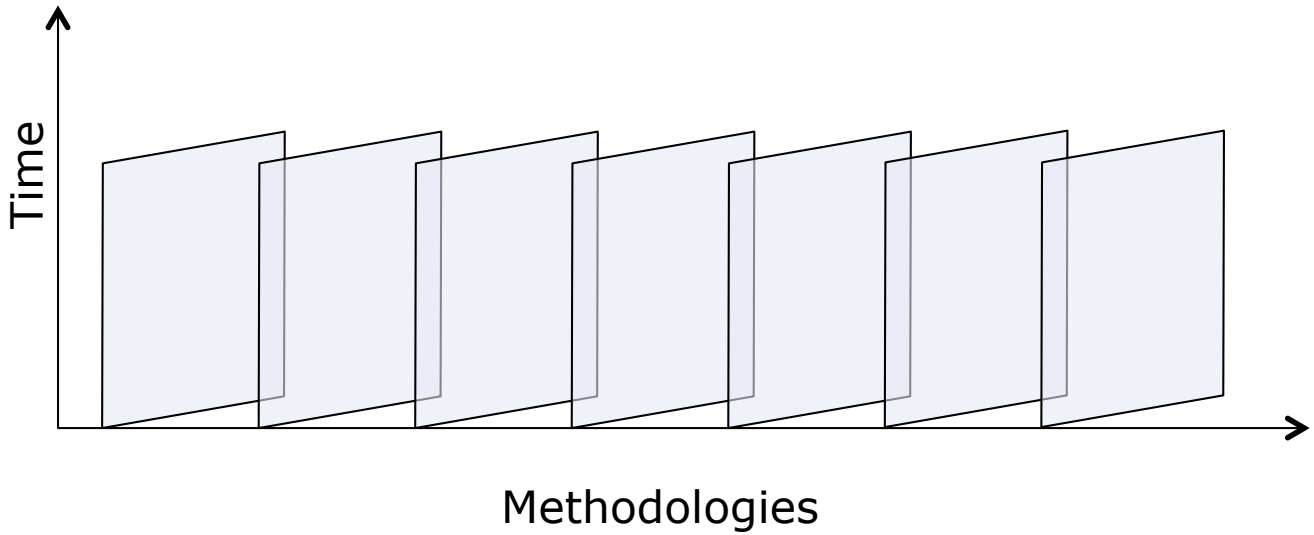


25,000 patients need to be screened to find 200 whose tumors have the 3 selected mutations

Klauschen F et al. The combinatorial complexity of cancer precision medicine. Oncoscience 2014, 1(7): 504

What's needed to address the competing objectives?

Two-dimensional 'growth' of the drug development / evidence generation processes



1) Growth along the time axis

A life-span approach to learning and evidence generation

- Blurring of the 'research-practice dichotomy'
- Continued learning during the on-market phase, capturing exposure and treatment experience
- Sharing of data or information (!) across healthcare environments; make use of the 'Darwinian space'
- Rapid cycle evaluation

2) Growth along the methodologies axis

From RCT to complete toolbox of evidence generation: horses for courses

- Need to integrate learnings from RCTs, observational studies, dedicated registries, e-health records, adverse event reports, …
- Common data standards, ideally common data model, common outcome criteria
- Methodologies to enable synthesis of different data sources

Thank you

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