Short intro into biomarker and big data

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EFSPI regulatory statistics workshop, Basel, October 6, 2017

Traditional predictive biomarker analyses

- Attempt to find a subgroup of the population defined by a biomarker where a therapy only works or works much better than in other patients
- The main advantages are to avoid treating patients with a therapy which would not work or on the other hand increase the potential of a drug by concentrating on the subgroup in which it really works
- Biomarker analyses traditionally based on clincial trials and specific biomarker information

New world approaching...

- In future we will have more information available:
  - Larger datasets (based on many more patients) inside and oustide clinical trials
  - Availability of much more information for each patient:
    - Complete sequencing information
    - Complete imaging information
    - Digital biomarker
    - More complete biomarker profiling

"After careful consideration of all 437 charts, graphs, and metrics, I've decided to throw up my hands, hit the liquor store, and get snockered. Who's with me?!"

- How relevant is this information and what do we do with it?

Predictive biomarker approach limited success

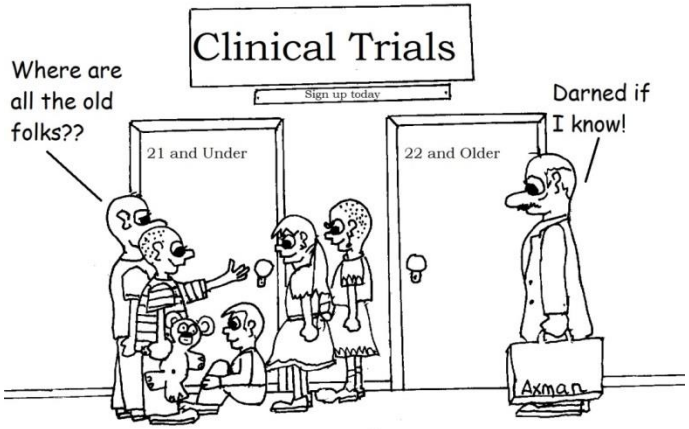
- Sometimes, response to therapy can better be characterized by a predictive biomarker
- Sometimes despite we feel that there is a predictive bioamrker guided therapy we fail to show it
- Often, response to therapy is independent of biomarkers although there is heterogeneity in treatment response by patients
- Again:
  How relevant is this new information contained in large and highdimensional datasets and what do we do with it?

Future of predictive biomarkers

- We need to accept that field of predictive biomarkers will evolve of the next years with the increased availability of
  - Large data
  - High dimensional data
- We may be able with additional data to idendentify more complex subgroups of responders driven by more than one predictive biomarker
  - Example: We may find subgroups determined by a group of predictive biomarkers
- But people will also try out event prediction modeling as soon as large and sufficiently rich (i.e. high dimensional) data are available
  - This at least may further increse precision of predicting response even when high accuracy levels may not be reached

Some additional questions rising...

- How good can such event prediction models be?
- Would it be acceptable in the future to characterize a predictive subgroup by « as soon as predictive probablily for the patient in model A is > 0.5»?
- Do we need to learn and accept different concepts of deriving evidence moving away from p-values?
- Do we need to accept black box approaches?



Thank you! &