From Science to Medicines: Bridging Translational and Regulatory Gaps in Paediatric Development

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Summary

- Disclaimer
- General comment, which pediatric drugs are we missing ?
- Gaps in the development of paediatric drugs
- clinical development ,
- marketing & reimbursement
- Conclusion

Disclaimer

- The content and the opinion expressed during this presentation are my personal point of view.

Which pediatric drugs are we missing ?

- Infective diseases are well covered with Vaccines & Antibiotics

  (possible gaps are in the use off-label and in the formulation for very young children)

  (the target is the infective agent, so it is the same for adult, children and animals)

- Non-infective disease treat the host and by definition the host is very heterogenous.
  - Furthermore, the human development can be easily divided in different stages from birth to adulthood (at least 4 stages can be separated for children)
  - Smaller target populations either by age or by genetic marker may suffer from the "rare" status, implying little or no market incentives and less well studied pathogenesis. (Any personalised medicine is "rare" by default (i.e., for a small population or n=1))

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Which are the gaps in the development of paediatric drugs ?

- All steps of the pediatric drug development do suffer :
  - Basic research in the pathogenesis of children diseases
  - nonclinical development for lack of adequate models
  - clinical trials for small population
  - marketing & reimbursement difficulties linked to a smaller market

Basic research

- Human development is still largely a black box due multiple reasons :
  - no animal models is really sufficiently similar to model for human long development from birth to adulthood
  - genetic diseases often starts in utero during the fetus development
  - we have precious little knowledge of the environment effect on children development
  - the human population is highly heterogenous (which is valid also for the adults)

nonclinical development

- The current models of drug development are based on the combination of small molecules and animal models, which have been highly effective in the last 80 yrs. However, increasing cost and length of drug development hints to an inherent complexity for non-infective diseases to be treated.
  - Moving from animals to in vitro models will take a lot of effort in validation (still they must tackle human heterogeneity and full organism interactions).

Clinical development

- Effort are undergoing to develop statistical models applicable to small population (or n=1 cases).
  - (More will be discussed in the upcoming panel discussion today.)
  - Such methods are needed whenever the study population is restricted by age, genetic or other factors so it is not strictly “pediatric”
- A more general change come with the introduction of the Pediatric Investigation Plan for all new drugs

Marketing & reimbursement

- The cost of innovative drugs is rising exponentially with the reduction of the number of patients treated. Few patients means higher cost.
- Providing incentives for small populations (orphan drug incentive) has not been proven to be very effective

Conclusion

- The acceptance of innovative clinical models is making possible to perform clinical trials in smaller pediatric population.
- Progressive advance in in vitro models will give more insights in the human pathogenesis either adult or pediatric
- The inadequate for-profit model of drug development need a revisitation with alternate pathway to marketing.