AI/ML in Clinical Development

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Conversation:

- Q: "What are the differences between data scientists and statisticians?"
- A: "A group of statisticians working in Bay area called themselves data scientists, that was how it started …"

AI/ML Challenges in Clinical Development

- Quick overview of AI in healthcare
  - Revolution in healthcare delivery
  - Decision support
  - Medical imaging
  - Diagnostics
  - Drug discovery and development

Clinical Trials vs. AI

- What are the differences?
- Clinical trials: (controlled environment)
  - Highly regulated, confirmatory, prospectively specified
  - Highly targeted, highly structured, validated data from limited, highly selective patient population
  - Causal inferences, reliability, reproducibility, generalisability (e.g. by randomisation)
  - Time, cost constrains
- AI: (real world)
  - Data driven
  - high dimensions, high volume, unstructured, multiple-source data
  - Broad relationships, e.g. clusters, association, prediction etc. within the data

AI in Clinical Trials – challenges and opportunities

- **Challenges – time and cost**
- Where are the opportunities?
  - Core evidence ?
  - Decision support ?
  - Intelligence ?
- Don't forget evidence generation - optimisation
  - Improve systems and workflow
  - Improve trial efficiency, optimisation
  - **Improve clinical trial design**
  - Improve understanding of the evidence (insight)

Population – case study

- Phase 3 study
- Identify patients who are super sensitive to the drug
  - AI: predictive model using past trial data, combine pk, pd, science and biology
  - Examine predictors and subgroups by toxicity profile
  - Optimise eligibility criteria

Comparator – case study

- First treatment in rare disease
- Post approval commitment study
- Key endpoint: overall survival
  - Drug registry (prospective) – data on active treatment
  - Develop AI predictive model using reliable, representative disease databases
  - Small part-historical, part-prospective control
  - Generate digital control (simulated control)
- Compare treatment effect

Operations – case study

- Improve overall trial efficiency
  - Intelligent feasibilities using historical operational data and RWD, e.g.
    - EHR, medical claims, labs, prescriptions, GP visits, life styles, etc. etc.
    - Prediction on site performance, individual compliance, prediction on drop out etc.
  - Centralised remote screening/monitoring using wearable device and cloud/AI analytic platform
    - Baseline physiological data – training/learning to generate personalised predictive model
    - Post baseline – automatically detect abnormalities based on individualised model, improve model
    - Send safety alarm to investigators, automatically trigger data collection at time of abnormalities

Analytics - examples

- Risk based monitoring – error detection, fraud detection etc.
- Data exploration – subgroup identification
- Supplementary data – linking trial patients to other medical or public records as an alternative to follow up, e.g. survival (special case)
- AI model tumour/lesion evolvement – provide better understanding of tumour growth, better prediction on longer term treatment effect
- Automated reporting using natural language processing
- etc

AI in Clinical Development

- Great potential, at early stage, key to success
  - AI and conventional RCT are complimentary to each other
  - Step-wise approach, setting achievable targets and deliver
  - Focus on decision support, intelligence and optimisation, and make a difference
  - **Understand limitations** – deep understanding of source data and data source is very important
    - AI find insight/patterns that exist within the data
    - If source data does not represent the population that it is to be applied to (bias), the "insight" would only be a biased view
    - Current data used to develop AI were not generated by AI themselves, in the future when AI generated data mixed together with other data -> this may lead to potential re-enforcement of "bias" – this could be a potential AI that can do self-exclusion

Questions?