The Convergence of Technology in Life Sciences and the impact on Drug Discovery and Development

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Interdependent evolution of technology and science

Faster pace of progress and greater interdependency

- Novel therapeutic modalities / new targets
- Diagnostic and Molecular Profiling technology
- Digital technologies and data processing
  - Data
  - Technology
  - Analytics
  - Connectivity
- Behavioural Science
- Personalised Healthcare
- Real World Data Science

IMI Innovative Medicines Initiative
Digital Technology in the Context of Data Collection

Digital technology in the context of data collection

Electronic health records
Primary care data, hospital records

Claims data

Patient and caregiver surveys

Registries
Existing disease
Registries / new product registries

Prescription databases
Drug utilisation

The Future
Social media data

Patient derived data (via smart phone or web based technologies)

With thanks to M. Berntgen, EMA
Personalised Healthcare 2.0 at Roche

Access meaningful data at scale
- Diagnostic / molecular data
- Clinical trial data
- Real-world data

Create insights
- Advanced analytics of integrated data

Generate value
- Drive more effective and efficient R&D
- Improve access & personalised patient care
Development of innovative medicines often requires innovation of the entire ecosystem

• At the outset of a development program, particularly in diseases with significant unmet need, much needs to be reviewed:
  – Methods of diagnosis
  – Development «Tool Box»
    • Conceptualization of disease
    • Current SOC and variations therein
    • Biomarkers and other stratification tools
    • Primary and secondary endpoints and methods of assessment
  – Methods of conducting clinical trials
  – Disease management tools
• Innovative technologies are applied to create more patient-centric, efficient and effective development programs
Autism Spectrum Disorders

Core and co-morbid symptoms

Incidence
• 1 in 68 births

Gender differences
• 4x more frequent in males versus females

Strong Genetic link

Treatment
• No treatment for core symptoms
• Risperdal and Ability for irritability

High medical need with no therapy available
Autism Spectrum Disorders
Europe & the World @ 2011

• No major strategy defined within Europe
• No major or concerted efforts in drug discovery
• No validated clinical endpoints
• No regulatory strategy
• Late diagnosis and poor awareness (adults)
• Poor knowledge of patient needs across life-course
• Wide range in treatment strategy with no evidence of efficacy
Novel Endpoints in Duchenne’s Muscular Dystrophy

• Existing primary regulatory endpoints > 100 years old (6 MWT, 4SC)
• Existing endpoints have major weaknesses:
  – Suboptimal sensitivity to functional change
  – Subject to observer and other error, manipulation etc.
• Qualification of 95th percentile stride velocity using wearable device as outcome measurement
• Patient function can be monitored at home - high ecological validity
• Draft guidance as a 2° endpoint for regulatory decision making