Why companies should work more closely on developing market access materials

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What needs doing to get to, and through health technology appraisal?

- Once a drug has been developed, and a Phase III trial / regulatory trial completed, you will need (as standard):
  - An understanding of treatment pathways
  - Systematic reviews of the evidence of
    - Comparator treatment efficacy / effectiveness
    - Utility data
    - Resource use data
  - Data on the burden of illness to inform discussions / populate an economic model
  - Things to fill the gaps – utility mappings, registry analysis, etc.
  - An economic model
  - Maybe a publication of your model results (either as a poster or a manuscript)
- Only the last two items are truely bespoke to the product, and even then, not entirely in the case of a model

What does it cost?

- Broadly speaking, the suite of materials will cost a company around £200,000 to £400,000 and take a minimum of a year
- However, this assumes the materials are always used – in reality many drugs do not make it to market, with work that has already started
- The 'top' person/people or group(s) can only work with a limited number of companies (or even only one)
- …and in many areas a limited number of patients on whom data can be collected

3

How would working together get past some of these issues?

- It would be possible to have larger overall budgets for a more thorough study (and a lower cost per company) versus a basic study undertaken by a company independently
  - Cost for one company = £100,000
  - Cost for four companies = £50,000 each, £200,000 in total
  - This is also a benefit given as of the four drugs, not all are likely to make it to market
- Credibility is increased by broader review, and being more impartial versus a single company study
  - As a collective, companies are able to access leading experts who may be reluctant to connect with a single company
  - Patient groups are able to more easily and willingly engage with a collaboration
- By working together, repetition of efforts may be avoided and materials may be made available in advance of when they are required

4

And then the ethics

- To collect data from patients and then not let it be used it is difficult to justify
- Patients enter trials and risk their own health, to help patients like them
  - Data for placebo treated patients can be used beyond the licensing of a single drug, without harming the company who collected it (with appropriate controls in place)
- The same for data on drugs which will no longer be developed
  - Companies here could enhance their profile – something needed in an industry where we have (deservedly at times) had reputational issues
- And even for drugs that are developed – data on non-sensitive areas such as patient height and weight should be able to be shared
- This has been implicitly recognised by the pharma industry with initiatives like Project Data Sphere (https://www.projectdatasphere.org/)
- Transparency is also valued by health technology appraisal bodies, and the public – important in an industry relying on the public acceptability of its business model i.e. patent protection

Examples of collaboration in practice

- Diabetes
  - The CORE diabetes model has many companies involved
  - Mount Hood meetings are an example of joint working
- Rheumatoid arthritis
  - By the use of a broadly standardised model (the BRAM – Birmingham Rheumatoid Arthritis Model), input values can be used in competitor models
  - Although companies keep independently rebuilding the model framework, at least it saves having to conceptualise it each time
- Open source modelling
  - A small movement, but growing
  - Various models are now available freely, particularly in R
- Duchenne Muscular Dystrophy
  - This will be discussed more by other speakers

Where is collaboration possible? And where is it more difficult?

- Likely areas it will work well
  - Previously understudied areas (rare diseases)
  - Where multiple companies are *developing* products i.e. at an early stage
  - With smaller companies who have fewer internal people

- Areas it may not be an option
  - Where there are marketed products in direct competition
    - Companies will be competition for market share, with data an a tool to do this
  - Where companies are far apart in timings
    - Companies entering Phase II will have different needs to those finishing Phase III
  - Where there is a very finite patient pool, or a natural monopoly
    - Vaccines for a national schedule will be in a winner takes all market
  - Where there are competition concerns
    - Companies must tread carefully where there are legal ramifications – a formal collaboration should be set up to avoid any accusations of collusion / price fixing

Thankyou

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Further reading:

Hatswell AJ, Chandler F. Sharing is Caring: The Case for Company-Level Collaboration in Pharmacoeconomic Modelling. PharmacoEconomics 2017;35:755–7. doi:10.1007/s40273-017-0516-2

COMPANY PERSPECTIVE ON COLLABORATION OF DEVELOPING MARKET ACCESS MATERIALS

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9

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10

WHAT NEEDS DOING TO GET TO, AND THROUGH HEALTH TECHNOLOGY APPRAISAL?

- Once a drug has been developed, and a Phase III trial / regulatory trial completed, you will need (as standard):
  - An understanding of treatment pathways ✓
  - Systematic reviews of the evidence of
    - Comparator treatment efficacy / effectiveness ✓
    - Utility data ✓
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  - Things to fill the gaps – utility mappings, registry analysis, etc. ✓
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- Only the last two items are actually bespoke to the product, and even then, not entirely in the case of a model ✗

11

WHAT DO WE MEAN BY COLLABORATION?

- **col·lab·o·rate**[1]
- *intr.v.* **col·lab·o·rat·ed**, **col·lab·o·rat·ing**, **col·lab·o·rates**

  1. To work together, especially in a joint intellectual effort

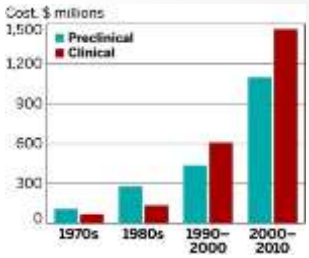
  2. To cooperate treasonably, as with an enemy occupation force in one's country

1. Oxford English Dictionary 2018

12

COST AND RISK OF DEVELOPMENT

- Development costs for NCEs (new chemical entities)
- Competition within drug development (e.g. immunoncology)



13

WHEN DOES IT MAKE SENSE TO COLLABORATE

- Likely areas it may work well
  - Previously understudied areas (e.g. rare diseases), but opportunities may be limited
  - Well established diseases (e.g. diabetes, hypertension, depression), but not many companies are investing here
  - General epidemiology studies
  - Maybe literature reviews
  - Registries, but with access limitations
  - Different stages of disease
  - Finite patient populations – competition for patients limits opportunity to gather evidence
  - Broader definitions of value – QoL
- Areas it may more challenging
  - Where there is reasonable competition
    - Early stage – how evidence on place in therapy might evolve
    - Where evidence is a key differentiator – investment in key data
    - Timing of development is somewhat irrelevant
- "All animals are equal, but some animals are more equal than others" – George Orwell, Animal Farm

14

COLLABORATION IN ACCESS

- So why are we suggesting collaboration?
  - Decrease cost?
  - Decrease duplication?
  - Improve decision making?
  - Improved patient access?
- Industry concerns
  - Fairness and reasonableness of data interpretation
  - Timing
  - Risk/uncertainty
- Overcoming evidence limitation or addressing the decision making process/framework?

15

PATIENT PERSPECTIVE

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SOME CHALLENGES FOR DUCHENNE

- Rare disease – 1:3500 boys and 1:50 million girls
- Around 1000 different variations in gene mutations (dystrophin)
- Raising awareness with academics, understand the complexity of the condition in order to ask the right questions and get the right answers when developing the protocols
- Advocacy – Time it takes, what do we say, how do we collect the data needed, how do we present the data
- Understanding payer needs early on – **only thing worse than not having a therapy is having a therapy stuck on a shelve not being able to access it**

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WHY WORKING WITH THE NFP SECTOR IS SO IMPORTANT

- NFP are Engaged – Want and welcome the opportunity to collaborate
- Rare Disease – Limited experience both in industry and government, small patient populations, often hard to recruit to trials especially ones targeting specific mutations
- Understanding the landscape – Knowledge of Country specific / Care / Community / Reimbursement processes / clinicians (Who? Where? Why?)
- Data – Work with the NFP to gather data and gain insight and information into available date sources / international collaborations
- Registries – Is there one? National and international, what data do they collect,
- Advocacy – Regarding the HCA / reimbursement processes
- Meaningful – Develop meaningful understanding bench to bedside

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NFP ARE INFORMED – LIVED EXPERIENCE

- Essential knowledge base of the community.
- Knowledge of the standards of clinical care / clinical care pathways.
- Knowledge of what is 'meaningful' when measuring endpoints.
  - For example walking for 2 more years
  - Improved hand function
  - Respiratory function stabilised but not cured
  - Fewer side effects to a more traditional therapy – like steroids
  - Reducing carer burden – less transfers, being able to feed themselves ……..
- The patient perspective highlights elements of the condition that may not be appreciated or understood by Pharma, academics and payers.

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RESEARCH AND DEVELOPMENT

- Helping with development of the protocols –what the community are willing to accept because what seems unreasonable to an ethics board / payer may be seen as acceptable to the community for example length of time for placebo group, number of muscle biopsies, travel
- Helping with the ethics – having patient advisors can been looked on favorably and can save time in the long run
- Registries – access to the community, their data and natural history
- Master protocols being considered by disease groups – trials
- Sharing data – Pt data, clinical trial, biopsies (broad consent / informed)
- Recruitment and support for trials
- Community engagement and facilitation of information – social media

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NEVER TOO EARLY, FREQUENTLY TOO LATE

- You need us – we need each other (Training, help us to help you)
- Don't leave it too late- Can't get through HTA and reimbursement without us
- Registries – recruitment, natural history data and post market surveillance tools
- Knowledge of treatment pathways
- Knowledge and engagement with the clinical experts / community
- Clinical Trial Networks – NFP working smarter to improve clinical trial capacity
- Hercules and Duchenne – an exemplar of international multi stakeholder collaboration between academia, industry, and the community to develop tools and evidence to support the HTA and reimbursement decisions for new treatments for Duchenne, higher credibility and higher quality. Engaged with NICE, TGA, PBAC and world leaders in the respective fields. Much cheaper. Professional.

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DELAYS IN REIMBURSEMENT

- Not just painful for companies, excruciating for the community.
- It can take more than 2 years to get decisions on reimbursements, In a progressive condition like Duchenne, there is no time to waste
- International examples – Exondys 51 in the USA and Translarna in Europe. Patient / community advocacy was instrumental in the conditional approval and successful reimbursement decisions
- Working together, reducing repetition of efforts and the necessary materials may be made available in advance of when they are required (HERCULES)
- Faster reimbursement, Faster access, Improved quality of life
- Lives saved and lives transformed

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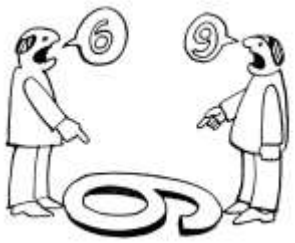
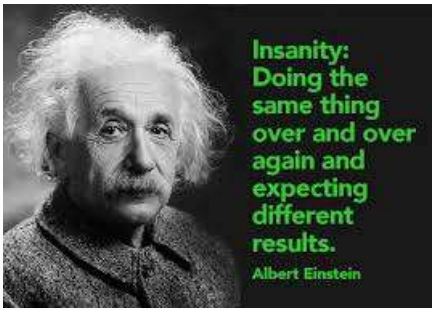
THE IMPORTANCE OF COLLABORATION

- Same problem just different ways of looking at it
- Same goals – good drugs to patients quickly
- Collaboration is key





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THANK YOU

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