6 Opportunities for Improving Pathways to Market: A Global Perspective on Dementia

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The UK Prime Minister's ambition to find an effective cure for dementia

Dementia Summit, 11 Dec 2013

- Brought together health and science ministers from all G8 countries
- Resulted in **Declaration and Communique** that set out vision for international collaboration and a series of high level actions
- Appoint **World Dementia Envoy**
- Formation of the **World Dementia Council**
- Attract new sources of finance
- Work in partnership WHO, EC and national Governments
- Hold series of high level events

G8 UK
UNITED KINGDOM 2013

Exposed depth and global size of the problem
Called for political leadership at the global level

G7 UK Legacy Event, 19 June 2014

Global action against dementia

New action to accelerate progress on dementia drugs, with a focus on R&D and affordable access to new medicines

- Appointment of **Raj Long** of the Bill and Melinda Gates Foundation to develop a **global initiative of HM Government** and bring together an international team of key research and regulatory experts in dementia, to gain insights on critical learnings related to the development science of dementia drugs
- **Highlight opportunities** for innovative thinking and new R&D approaches towards timely, effective and affordable new medicines
- Appropriate governance to ensure **non Conflict of Interest**

World Dementia Council Envoy

Governments, EC & International Organisations

Dementia Integrated Development Initiative

HM Government

Networks

Global Business

Patient Advocacy

Academia

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Dementia Integrated Development

HM Government

10th Nov 2014 (Geneva) 1st Global Dementia Regulators Workshop - 11 regulators from 10 agencies including the **US, EU, Canada, Japan, Switzerland, Germany, Italy, Denmark, the Netherlands, UK, leading clinical experts** and a **patient representative**

- **Dementia Research Gaps**
- **Development Challenges**
- **Regulatory Science**

Collaborative Exploratory Discussion

1. **Attrition Analysis**
2. **Industry Perspective**
3. **Patient Perspective**
4. **Neurologists/Clinical Practitioners**
5. **Cross Regional Regulators x11**

- **First time 10 agencies converged to look at Dementia with a single lens.**
- **Outcome: Identified 6 initial key areas as potential areas of impact**

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1. Attrition Analysis

- Identify R&D challenges by analysis of attrition data of dementia development failures in the last 15years.
- Initial attrition analysis IMS Lifecycle R&D DB show N = 250. NB: 76% cite no reason for termination
- Scale to include input IFPMA & 3 other CT DBs (US, EU &WHO)

2. Clinical Trial Efficiency

- Integrate lessons from oncology, RA & AMR where applicable to Dementia CTs e.g. Master Protocols (MPs)
- e.g. similar to oncology can MPs positioned into study trial networks minimising the need to run separate trials for each "sub-type" of Alzheimer Dementia.

3. Multilateral Cooperation

- Potential international platform of regulatory agencies to foster opportunities for multilateral dialogue e.g. in AD ‘Confluence’ of multiple key regulatory expertise within current regulatory framework

4. **Modelling & Extrapolation**
   - Trials in AD & dementias challenged by individual variability (symptoms & clinical measures).

   e.g. explore potential for extrapolation models that translate rare genetic forms of dementia (FAD) to the wider population, based on an empirical model of human disease.

5. **Composite Endpoints**
   - Early stage AD – challenged by lack of standardized & validated tools for the quantification of cognitive impairment. Acknowledgement that this needs to be stage and disease specific.

   e.g. RA In rheumatology, professional groups were brought together in a scientific workshop to work out and accept a common battery of endpoints.

6. **Risk/Benefit Analysis**
   - A concept paper to consider how best to balance possible benefit given the high level of uncertainty (in the context of what is not known about the disease. Ethical, Legal/indemnification concerns.

- **Item 1 – led by UK HMG**
- **Items 2 to 6 – led by regulators & supported by ID/SMEs**
- **Early stage dialogue on HTA role**

Next Steps

- **2nd regulators meeting planned – 1H 2015**
- Continue **patient, academia and industry engagement**
- **3rd regulators meeting planned - 2H 2015** – possibly with more jurisdictions. Other regions have expressed interest and ongoing dialogue for wider engagement.

Better understanding of the Research Science
Improved Development & Regulatory Science
Incentivise through Increased Success of R&D in Dementia.