

# Medical Innovation

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**wellcome**trust

# An acceptable diagnosis?

A world map with a color gradient from purple in the north to yellow in the south. The text is overlaid on the map.

Someone dies from HIV every 10 seconds  
1 child dies every 30 seconds from malaria  
1 child dies every 40 seconds from malnutrition

30,000 children die every day from preventable diseases

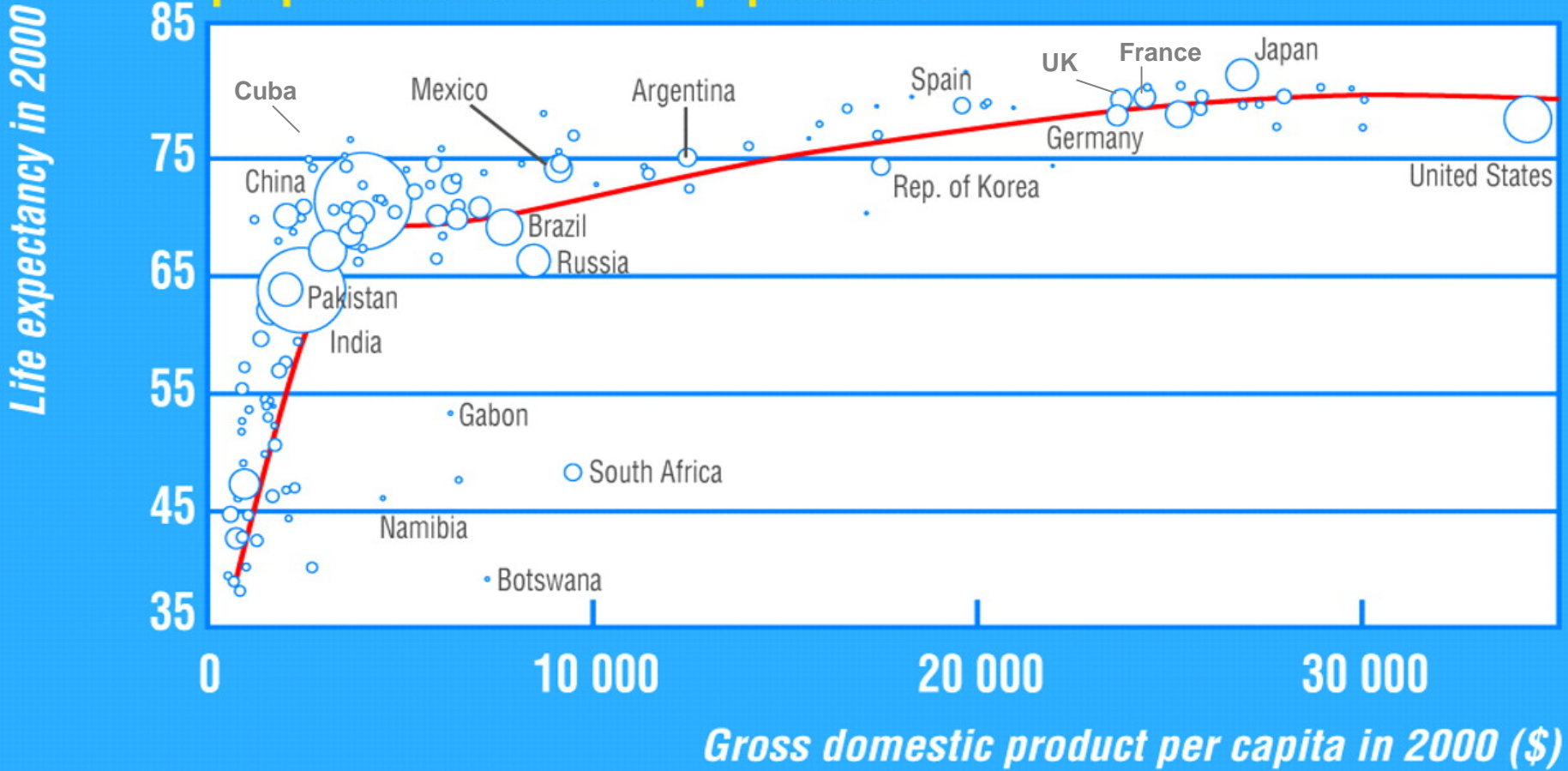
4000 people die every day from lack of access to clean water and sanitation

[UNICEF State of the Worlds Children 2005]

The diagnosis:

# Health & wealth are linked

**Life expectancy and income per capita; circle size is proportional to size of population**



Source: World Development Report 2006



# Unmet need...

- centuries-old diseases:
  - malaria
  - tuberculosis
- new and emerging threats:
  - HIV
  - avian influenza
- rise in chronic non-communicable disease:
  - coronary heart disease
  - diabetes
  - obesity
- mental health
- reproductive, maternal & sexual health
- nutrition



# The future... Science

- **Infectious diseases**

- no longer seen as orphan conditions
- vaccines

- **Genetics**

- greater understanding of disease pathways
- more targeted therapies
- measuring the environment

- **Phenotyping people**

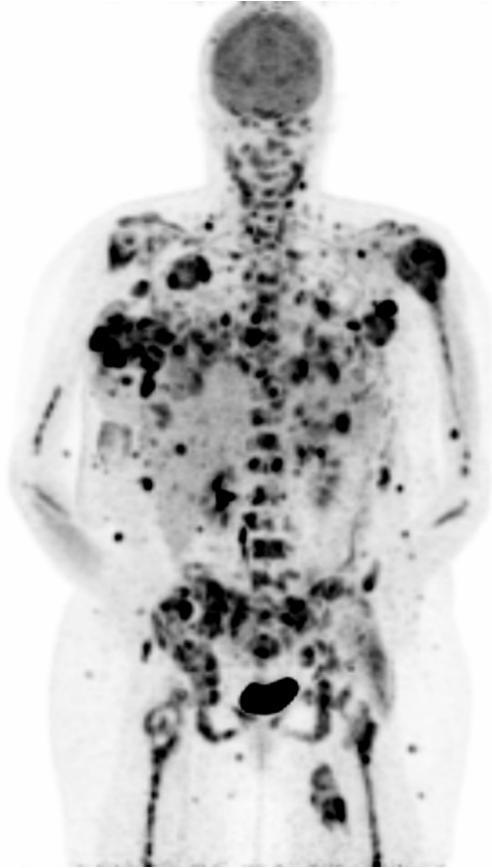
- physiology
- pathology
- medical imaging

- **Many growth opportunities**

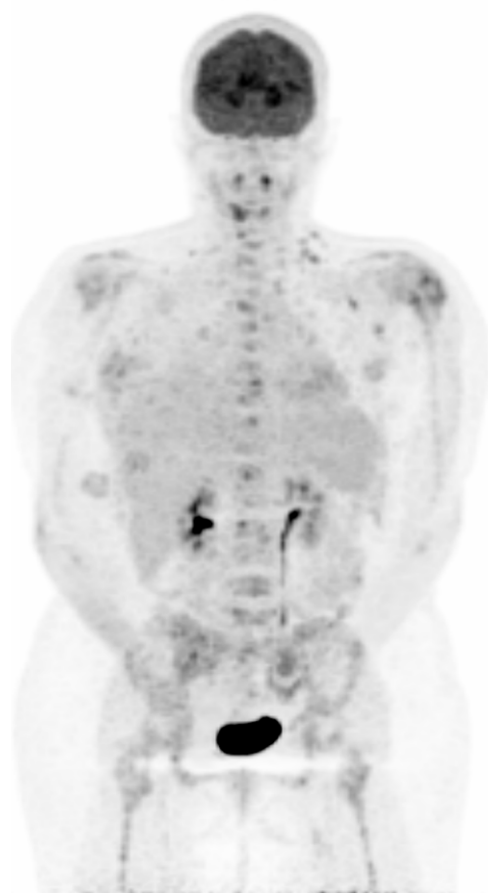
- nanotechnology
- bioengineering
- computational sciences
- digital pathology
- decision support systems

Early genome:

Treatment of metastatic malignant melanoma with selective inhibitor of BRAF V600E (Plexxikon 4032)



Before



15 days after

**February 2001: Publication of initial Working Draft Sequence**

**June 2002: Scientists identify BRAF involved In 70% of melanomas**

**January 2010: A comprehensive catalogue of somatic mutations from a human cancer genome**

# The market place:

changing global environment for health innovation

- **Number of healthcare consumers is growing** - from 1 billion to 4 billion - and expectations are rising
- **Number of innovators is increasing** - changing geographical trends in R&D activity and investment‘
- **Relationship between innovation and market share is changing** - decline of blockbuster drugs and rise of personalised medicine
- **Affordability** - Governments seeking to manage increasing health and pharmaceutical budgets
- **Innovation agenda is broader** - traditional emphasis on developing new technologies balanced by increasing focus on innovation in health services



# Driving innovation to compete

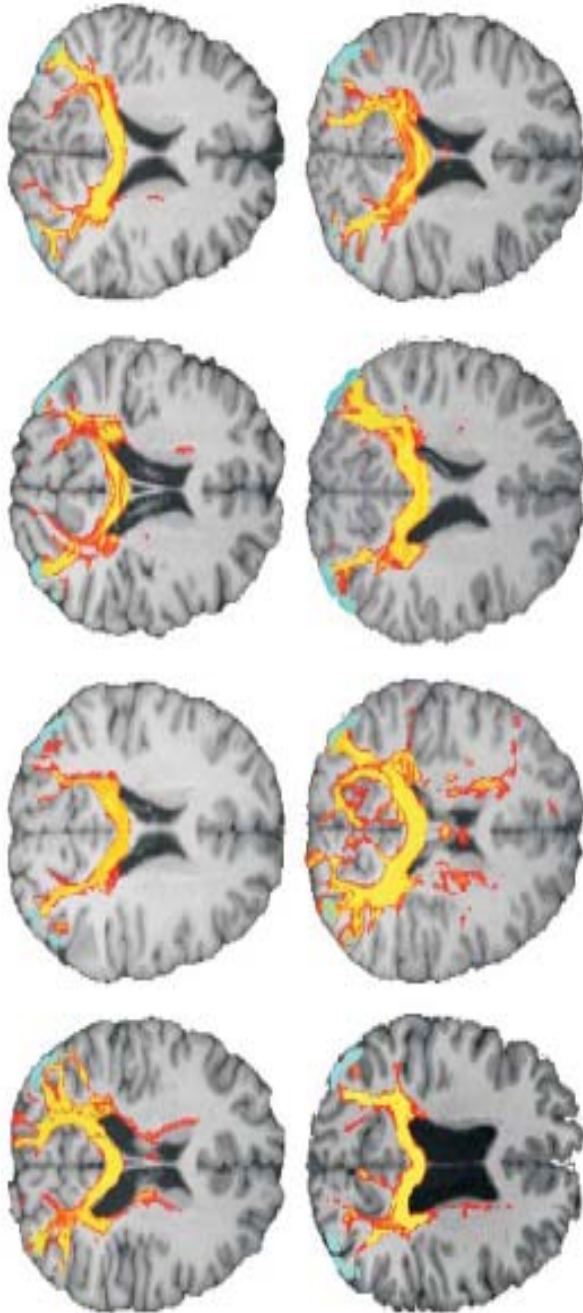
- **Structural:** establishing innovation centres and clusters
- **Financial:** increasing the availability of funding and financing
- **Skills-based:** developing the current and future workforce
- **Regulatory:** creating tax incentives and removing regulatory barriers
- **Cultural:** developing a culture that celebrates, rewards and enables innovation
- **Market-driven:** building demand for innovative products and processes



# The future... Pharma

- **increased volume**
  - unmet clinical need
  - price differentiation to supply the globe
  - improved distribution and access
  - BRIC recognition of I.P.
- **improved productivity**
  - open innovation
  - personalised therapies rather than blockbuster drugs
  - increased role of knowledge hubs
  - increased inter-disciplinary working
  - new models for public-private partnerships

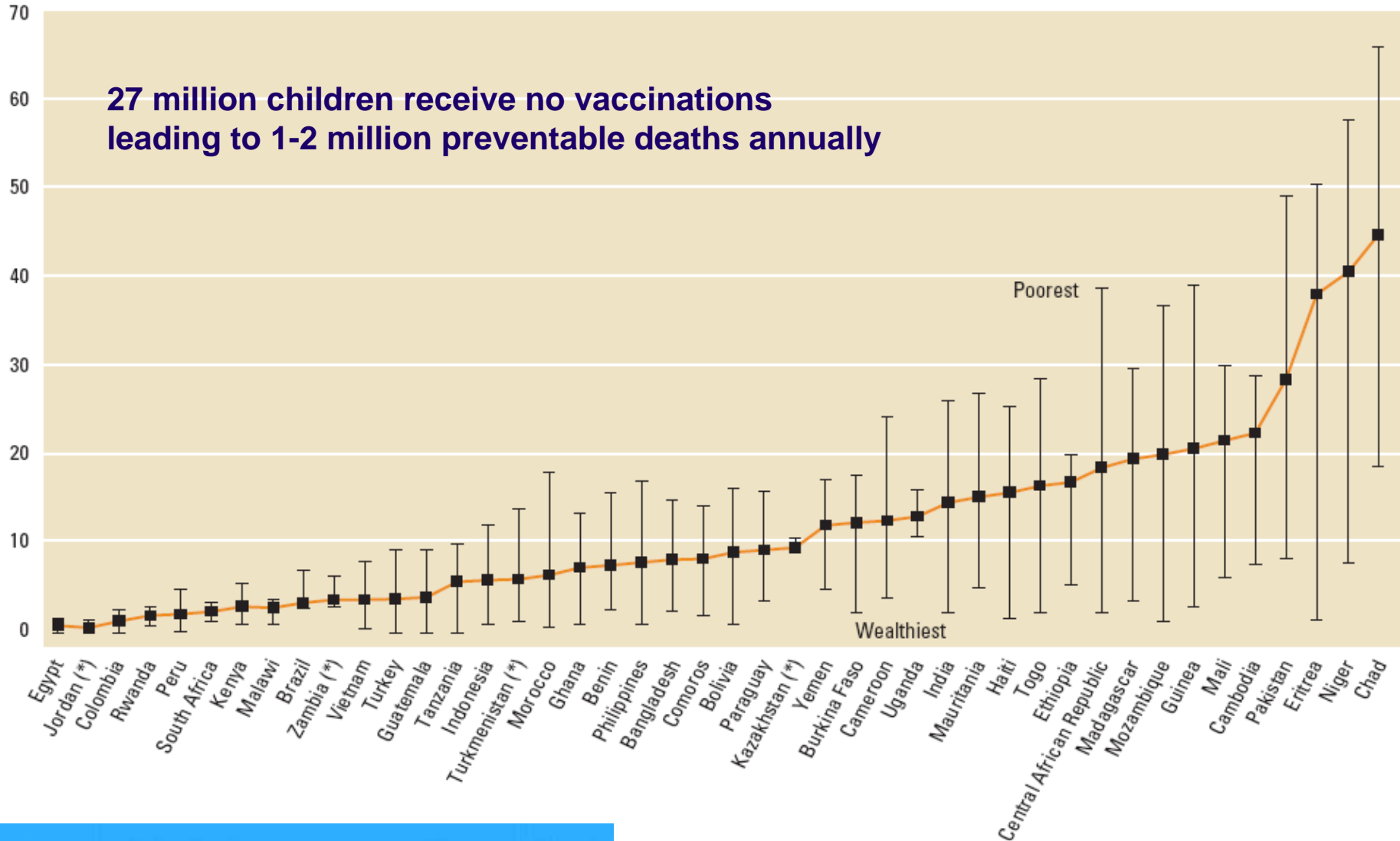
# The future... Healthcare



- biomarkers - huge opportunities for new therapeutics and diagnostics
- focus on wellness
  - early health vs late disease
  - individual accountability for health
  - rewarding prevention
- harnessing ICT:
  - linking large datasets
  - personalization
- efficiency:
  - take health to the patient
  - 'L plating' of therapies
  - registering therapies in a different way – determining benefit before therapeutic is priced
  - the end of the mega clinical trial – look for therapeutics that have big effect (e.g. anti-TNF)

# Equity: Access and global distribution

Percentage not covered



Source: World Development Report 2006

Affordability: The cost of healthcare

# For example obesity in the UK

## Calculating future costs of elevated BMI (£ billion/year)<sup>1</sup>

|  | 2007 | 2015 | 2025  | 2050  |
|--|------|------|-------|-------|
| Total NHS costs of diabetes  | 2.0  | 2.2  | 2.6   | 3.5   |
| Total NHS costs of coronary heart disease  | 3.9  | 4.7  | 5.5   | 6.1   |
| Total NHS costs of stroke  | 4.7  | 5.2  | 5.6   | 5.5   |
| Total NHS costs of other related diseases  | 6.8  | 7.4  | 7.8   | 7.8   |
| Total cost (all related diseases)  | 17.4 | 19.5 | 21.5  | 22.9  |
| NHS cost increase above current, due to elevated BMI (overweight and obesity)                            | –    | 2.1  | 4.1   | 5.5   |
| NHS costs attributable to elevated BMI (overweight and obesity)  | 4.2  | 6.3  | 8.3   | 9.7   |
| NHS costs attributable to obesity alone (see Table 4 in Modelling Future Trends) <sup>1</sup>            | 2.3  | 3.9  | 5.3   | 7.1   |
| Wider total costs of overweight and obesity, taken at 7x direct costs (figures include rounding effects) | 15.8 | 27   | 37.2  | 49.9  |
| Projected percentage of NHS cost @ £70 billion   | 6.0% | 9.1% | 11.9% | 13.9% |

- Obesity-attributable NHS costs projected to rise to £9.7bn in 2050
- By 2050 the wider cost to society could be £49.9bn



# Our future

UK must remain world class:

- Science
- Leadership
- Partnering



# Philanthropy

Catalysing innovation...



## • Projects related to charitable mission

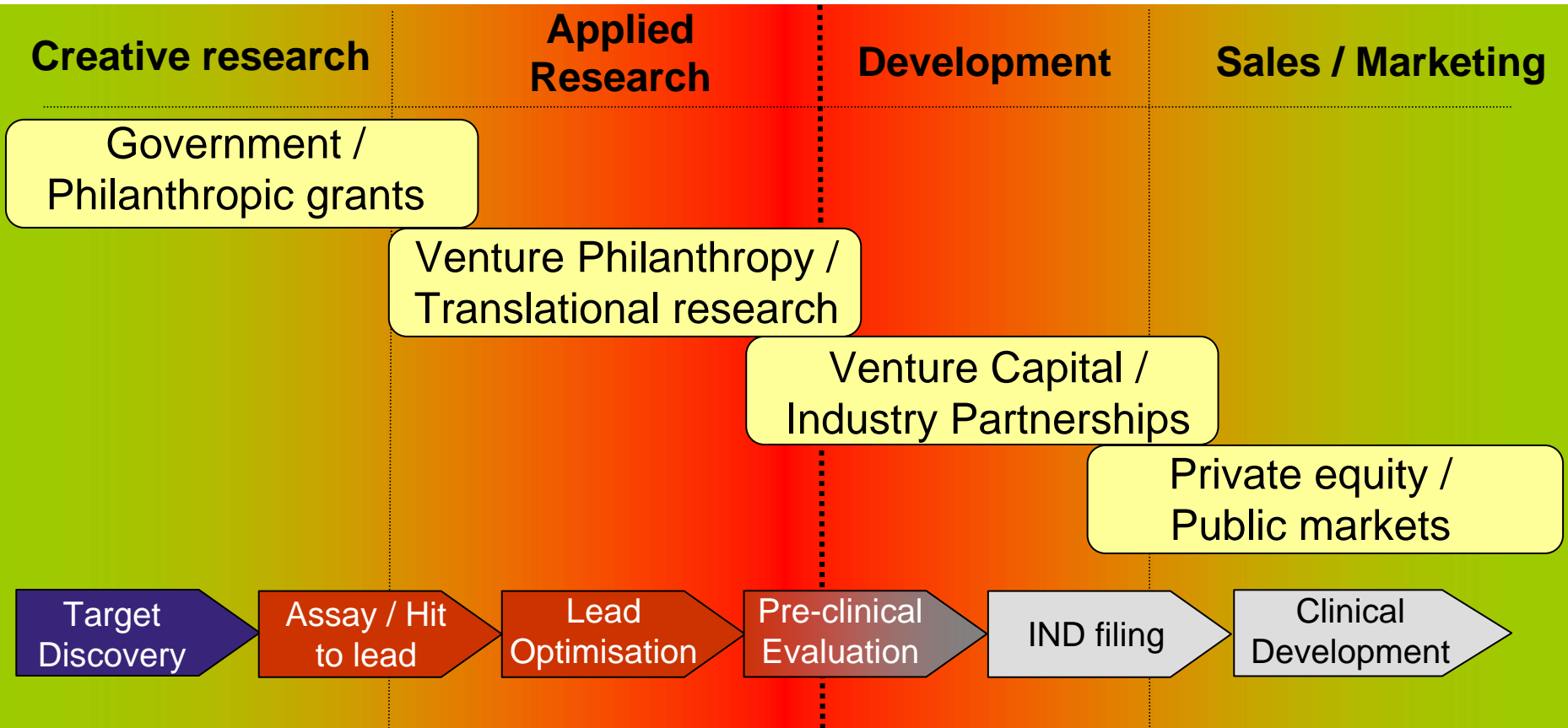
- usually disease-focused
- sometimes problem-oriented
- always fulfil an unmet need
- offer solutions that are 'fit for purpose'

## • Projects at the appropriate stage

- engage at various stages
- fund-raising charities are more patient-oriented
- many charities look for leverage of their funds

# Funding in the gap

Traditional model



- Productivity
- Open innovation
- Big pharma



Partnering

# Partnerships

## Working with PPPs



Medicines for Malaria Venture

### MMV Portfolio, Q4 2009

| Research                         |                                | Translational                   |                          |                      | Development            |  |                               |
|----------------------------------|--------------------------------|---------------------------------|--------------------------|----------------------|------------------------|--|-------------------------------|
| Lead Gen                         | Lead Opt                       | Preclinical                     | Phase I                  | Phase IIa            | Phase IIb/III          | Registration                           | Phase IV                      |
| Novartis miniportfolio           | KAI407 series Novartis         | MK 4815 (Merck)                 | GSK 932121 GSK           | Iv artesunate Guilin | Arterolane/POP Ranbaxy | Eurartesim™ sigma-tau                  | Coartem®-D Novartis           |
| GSK miniportfolio                | Pyridone GSK                   | KAE 609 Novartis                | Tafenoquine GSK          | Artemisone UHKST     | AZCQ Pfizer            | Pyramax® Shin Poong/University of Iowa | Coarsucam® sanofi aventis/DND |
| Broad/Genzyme miniportfolio      | DHODH UTSW/UW/Monash           | P218 DHFR (BIOTEC/Monash/LSHTM) | OZ 439 (Monash/UNMC/STI) |                      |                        |  |                               |
| Pfizer Whole cell screen         | Aminoindole Broad/Genzyme      |                                 |                          |                      |                        |  |                               |
| sanofi aventis Orthologue screen | Ozonide backup Monash/UNMC/STI |                                 |                          |                      |                        |  |                               |
| Kinases Monash                   | Quinoline Methanols WRAIR      |                                 |                          |                      |                        |  |                               |
| Natural Products 5 Projects      | DHODH Broad/Genzyme            |                                 |                          |                      |                        |  |                               |
| Whole Cell Hits St Jude/Rutgers  | KAC776 series Novartis         |                                 |                          |                      |                        |  |                               |
| Other Projects 13 Projects       | KA558 series Novartis          |                                 |                          |                      |                        |  |                               |



## Curing Malaria Together



# Strategic Plan

## 2010-20

Extraordinary Opportunities

Wellcome Trust

## Five major challenges...

- Maximising the health benefits of genetics and genomics
- Understanding the brain
- Combating infectious diseases
- Investigating development, ageing & chronic disease
- Connecting environment, nutrition and health

# Stimulating priority research areas

- Genetics and genomics
  - pioneering large-scale genomics research – WT Case Control Consortium, International Cancer Genome Consortium, 1,000 Genomes
  - major cohort studies (UK Biobank, ALSPAC, etc)
- Neuroscience and mental health
  - breakthrough studies to understand & map brain function (e.g. WT Centre for Neuroimaging);
  - development of more effective treatments – e.g. cognitive behavioural therapies
- Infectious diseases
  - major funder of malaria research (support five of top ten malaria researchers in world to 2005)
  - research to address threat of pandemic influenza
- Chronic diseases & public health
  - basic & clinical research on major chronic diseases (e.g. obesity, diabetes)
  - building capacity in public health research





# Wellcome Trust

Catalysing innovation...

## Major initiatives in area of technology transfer:

- **Seeding Drug Discovery:**  
for early-stage, small-molecule drug discovery (£91 m)
- **Electronic patient records & databases in research**  
WT, ESRC, EPSRC and MRC (£10 m)
- **Medical engineering**  
WT and EPSRC partnership (£45 m)
- **Health Innovation Challenge Fund**  
WT and DH initiative (£100 m)
- **Interdisciplinary Training Programmes for Clinicians in Translational Medicine & Therapeutics**  
integrated training programmes for clinicians (£22 m)
- **R&D for Affordable Healthcare in India:**  
safe and effective healthcare products for India (£30 m)

# Catalysing innovation

Addressing unmet need



## Translation awards

197 projects supported

Range: £25k - £11.2M

Average award, £1.3M

£257 m committed since 2003

£423 million leveraged

3 M & A by Pharma

6 product launches

10 projects into the clinic



ACHAOPEN



Diagnostics for the Real World Ltd



# Pre-market investment

Catalysing innovation...

## Drugs



Discovery portfolio for malaria



Discovery portfolio for African Sleeping Sickness



Suppository for treatment of infectious pyrexia



Metastatic melanoma



*C.difficile* infection



Drug sensitive cancer genotype characterisation

## Vaccines



Oral formulation for pandemic flu

HumAbs Inc

Antibodies for avian flu



TB booster vaccine

## Diagnostics



PoC sexually transmitted disease tests



PoC TB diagnosis

## Devices



iSnake robotic endoscope



Bio-engineered teeth



New models:

## Major New Facilities: UKCMRI

A National centre for major collaborative, interdisciplinary and translational medical research between the UK's principal medical research organisations

Enabling the partners to:

- take a long-term approach to important research and innovation problems
- promote interdisciplinary and translational research
- provide cost-effective access to world-class infrastructure, resources, services and specialist facilities
- exploit intellectual property more effectively
- enable the UK to secure access to or retain world-class scientists
- provide a high-quality environment in which to recruit, train and develop future scientists
- develop close links between the Institute and the outstanding research intensive hospitals nearby



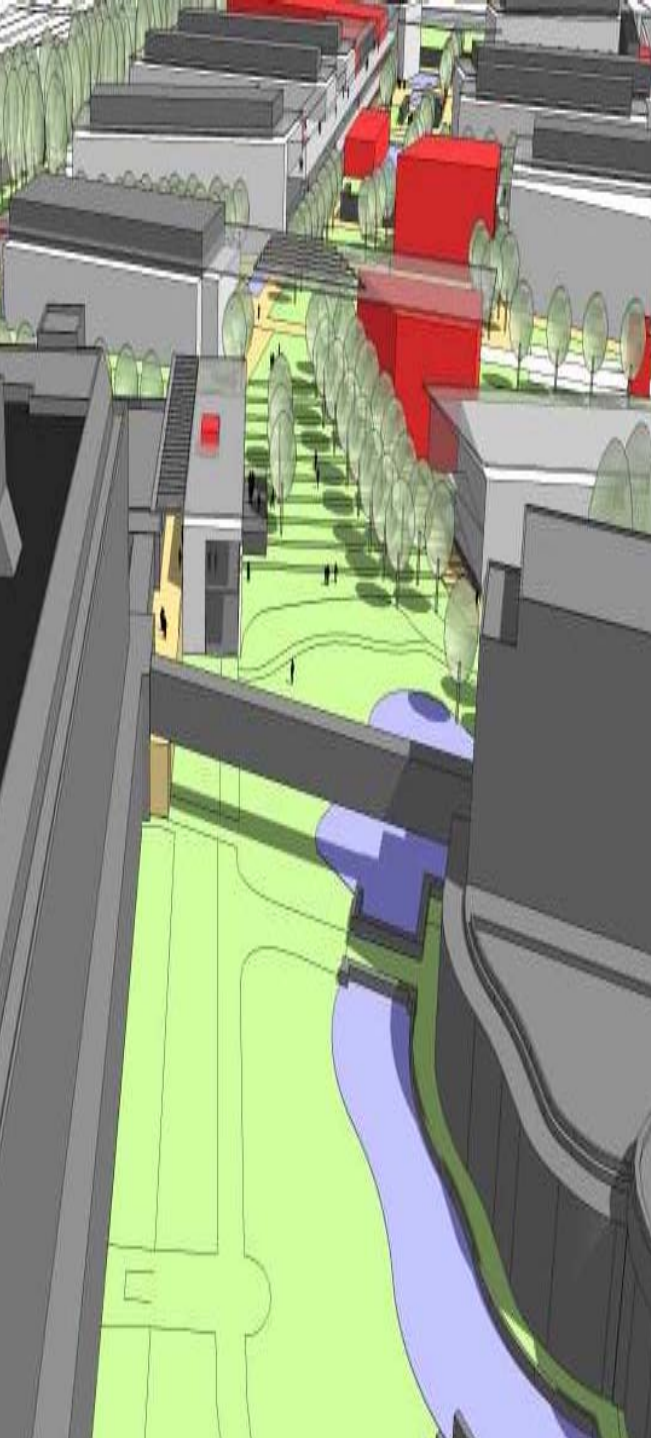
New models:

## MSD WT Hilleman Laboratories

- Vision: A sustainable, not-for-profit operating model to turn innovative science into practical solutions for those in greatest need
- £90 million joint venture between Merck & Co and WT
- turning innovative basic science into practical and affordable vaccines
- new vaccines in areas of unmet need and optimization of existing vaccines
- range of business models to ensure fair results for all



MSD • Wellcome Trust  
Hilleman Laboratories



New models:

# Stevenage Bio-Incubator

- Vision: Create a place that will enable, enhance and inspire improved performance in translational research, leading to a strong and relevant pipeline of healthcare products in areas of unmet need
- Partnership:
  - Business Innovation & Skill (£11.7m)
  - GlaxoSmithKline (£11m)
  - Regional Development Agency (£4m)
  - Wellcome Trust (£6m)
  - Technology Strategy Board (£5m)



# Improving innovation

Key to an innovative healthcare and biomedical industry we must:

- develop bench to bedside – bedside to bench approaches
- optimize academia & industry links
- develop innovative public private partnerships
- BRIC recognition of IP
- provide fiscal incentives:
  - encouraging exploitation
  - encouraging collaboration
- ensure regulation is facilitative and proportional:
  - EU regulations
  - streamline approval processes
  - animals in research
  - ‘consent for consent’



# Civil society and a sustainable globe

- Universities as knowledge hubs
- Partnership for a sustainable globe