Major issues:

Vaccine development

Analysis of tumor genes

Synthetic biology and

Genome evolution

Diagnostic tests

Gene therapy, and

ELSI-Social, ethical, legal

And Regulatory issues

First Biotech Patent
launched commercial biotechnology

1980 US Supreme Court decision in
Diamond v. Chakrabarty granted patent
protection of a genetically modified life-form
of an oil-digesting bacterium
Pseudomonas putida

Supreme Court Ruling, June 2013

The Supreme Court ruled that human genes cannot be patented, a decision with both immediate benefits for some breast and ovarian cancer patients and long-lasting repercussions for biotechnology research.

It is a victory for cancer patients, researchers and geneticists who claimed that Myriad's patent raised costs, restricted research and sometimes forced women to have breasts or ovaries removed without sufficient facts or second opinions.

FDA
July 31, 2014

The Food and Drug Administration announced recently that it would start regulating medical laboratory testing, saying that tests used to make important treatment decisions must be validated before they go into use. Some widely used commercial tests have never had to be reviewed by the agency. These include Myriad Genetics' breast cancer risk test, the subject of a Supreme Court patent decision last year.

FDA's view of Genetic Tests

FDA has observed the following problems with some LDTs in recent years:

Faulty data analysis
Exaggerated clinical claims
Fraudulent data
Lack of traceability/change control
Poor clinical study design
Unacceptable clinical performance

GENETIC TESTING

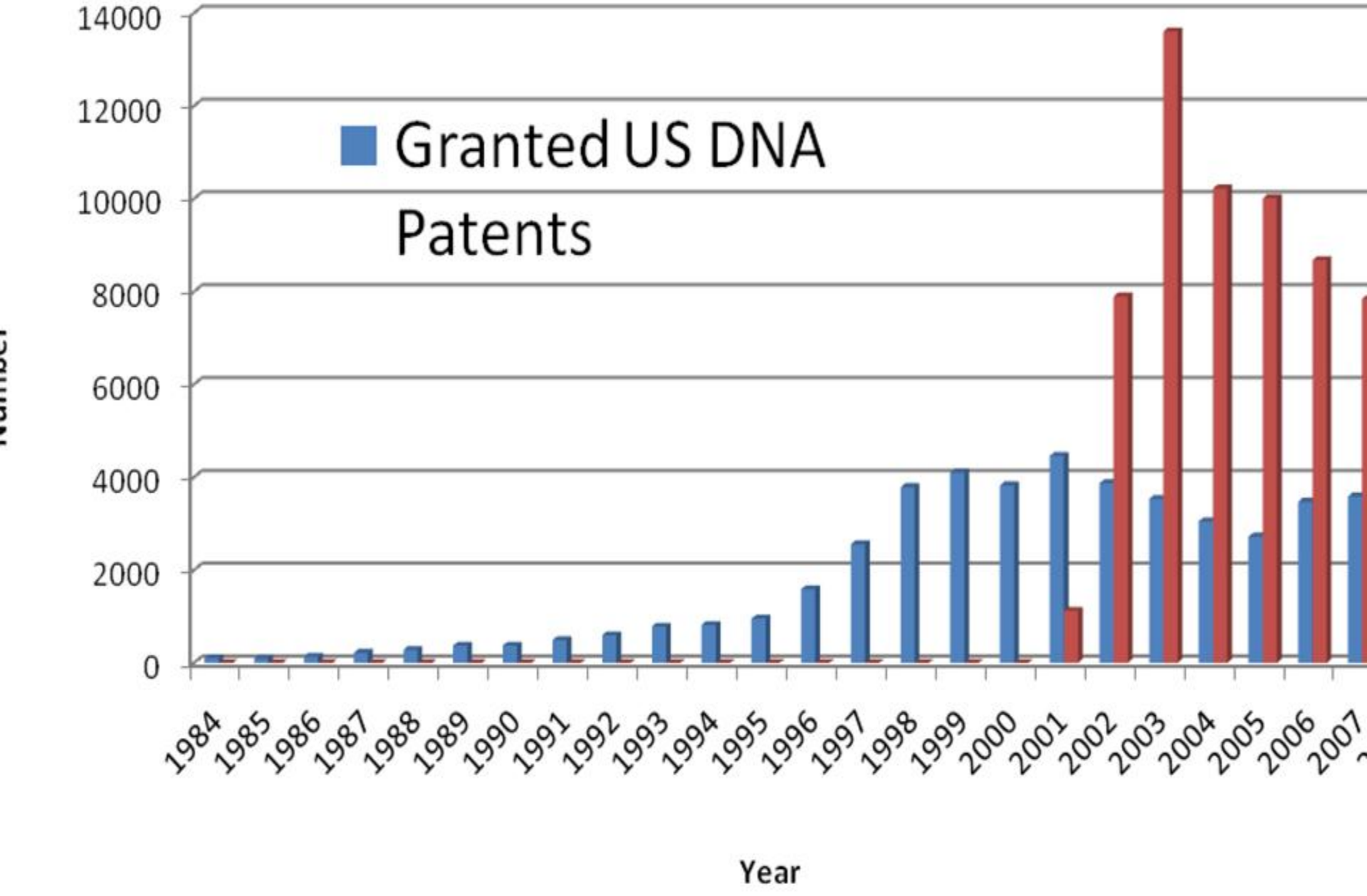
The term "genetic testing" covers an array of techniques including analysis of human DNA, RNA, or protein.

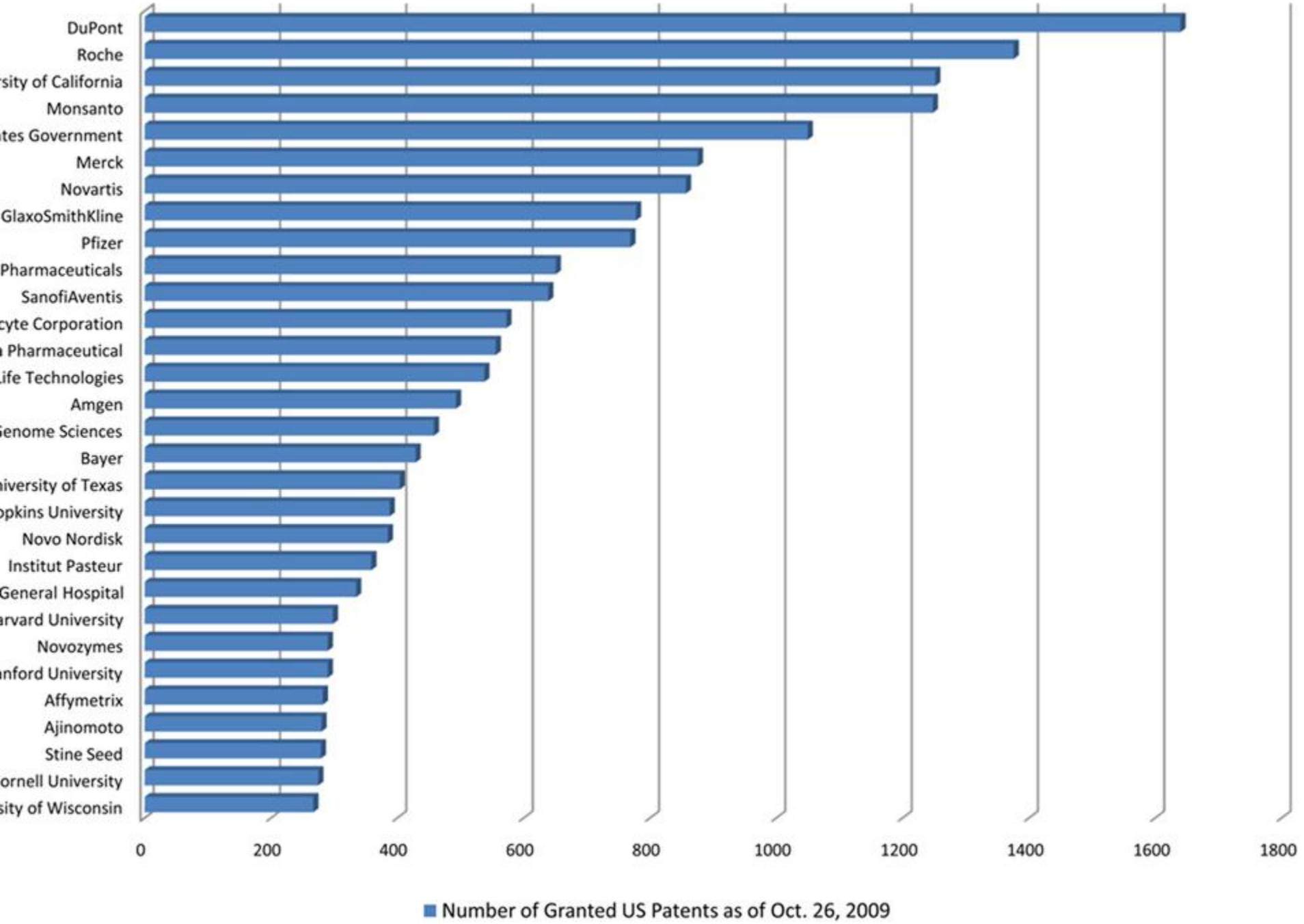
Genetic tests are used as a health care tool to detect gene variants associated with a specific disease or condition, as well as for non-clinical uses such as paternity testing and forensics.

In the clinical setting

Genetic tests are performed to determine the genetic cause of a disease, confirm a suspected diagnosis, predict future illness, detect when an individual might pass a genetic mutation to his or her children, and predict response to therapy.

Growth of DNA Patents




DuPont
Roche
sity of California
Monsanto
tes Government
Merck
Novartis
GlaxoSmithKline
Pfizer
Pharmaceuticals
SanofiAventis
cyte Corporation
Pharmaceutical
Life Technologies
Amgen
Genome Sciences
Bayer
niversity of Texas
opkins University
Novo Nordisk
Institut Pasteur
General Hospital
arvard University
Novozymes
nford University
Affymetrix
Ajinomoto
Stine Seed
ornell University
sity of Wisconsin
0
200
400
600
800
1000
1200
1400
1600
1800
Number of Granted US Patents as of Oct. 26, 2009

Biobanks/Repositories

(for conservation, research, data sharing)

Disease/Control Biobanks - Biological samples from patients with specific diseases and healthy controls

Population-based Biobanks – Samples from populations with or without a disease

Population isolate Biobanks – genetically homogeneous samples from a population isolate with or without a disease

Twin registries – Samples from mono- and di-zygotic twins

Infectious disease Biobanks – Samples from patients with specific infectious diseases

4 Potential Uses of DNA Patents (U.K.)

Diagnostic tests: Inventiveness criterion needs stringent application.
Research tools: Strict application of utility criterion.
Gene therapy: The identification of a disease-specific gene should not be granted a product patent, but rather encourage the invention of safe and effective methods of gene delivery, and
Therapeutic proteins: Not the DNA sequence as such, but the protein described.

Anne Wojcicki, Co-founder of 23andMe



Warning Letter

Food and Drug Administration
Document Number: GEN1300666
Re: Personal Genome Service (PGS)

WARNING LETTER
Dear Ms. Wojcicki,
The Food and Drug Administration (FDA) is sending you this letter because you are marketing the 23andMe Saliva Collection Kit and Personal Genome Service (PGS) without marketing clearance or approval in violation of the Federal Food, Drug and Cosmetic Act (the FD&C Act).

23andMe:
Ethical and Privacy Concerns

Users can still purchase the $99 at-home DNA kit, but they will not receive the health reports that formerly accompanied the purchase. Buyers will only get an ancestry report and uninterpreted raw genetic data.

Gene association with Parkinson's was determined for the first time - six loci, or locations of specific genes on chromosomes, to be associated with Parkinson's. The study was enormous. It looked at 7 million variants in about 13,000 patients with Parkinson's and, for a control group, more than 100,000 people who did not have the disease.

Charges of Discrimination

Burlington Northern began secret genetic testing of its workforce in 2000, hoping to prove a "pre-existing condition" and avoid paying workers' compensation costs. The EEOC sued for discrimination based upon predictive genetic testing.
Case settled in 2002 with Burlington Northern paying $2.2 million to the workers.

Ethical Issues

The shared nature and ownership of genetic information.
Confidentiality of the individual
Dissemination of genetic test results within the family
The discovery of variation in a particular gene and its implications
Inappropriate applications of genetic testing, e.g. sexing of a fetus
The potential for discrimination with the use of predictive/presymptomatic testing
Impact of results on life insurance applications and employment
Forensic DNA databanks. Ensuring that they are used for the purpose for which they were collected and protected from misuse.

Benefits of University - Industry Collaboration

Financial support.
Broaden the experience of students.
Expose students and faculty to significant, interesting, and relevant "real world" problems.
Enhance regional economic growth.
Increase employment opportunities for students.
Provide Sabbaticals

Access to expertise and knowledge not typically available in industrial laboratories.
Aid in the renewal and expansion of a company's science and technology base.
Gain access to students as potential employees.
Gain access to faculty consultants.
Leverage internal research Capabilities, opportunities for faculty.

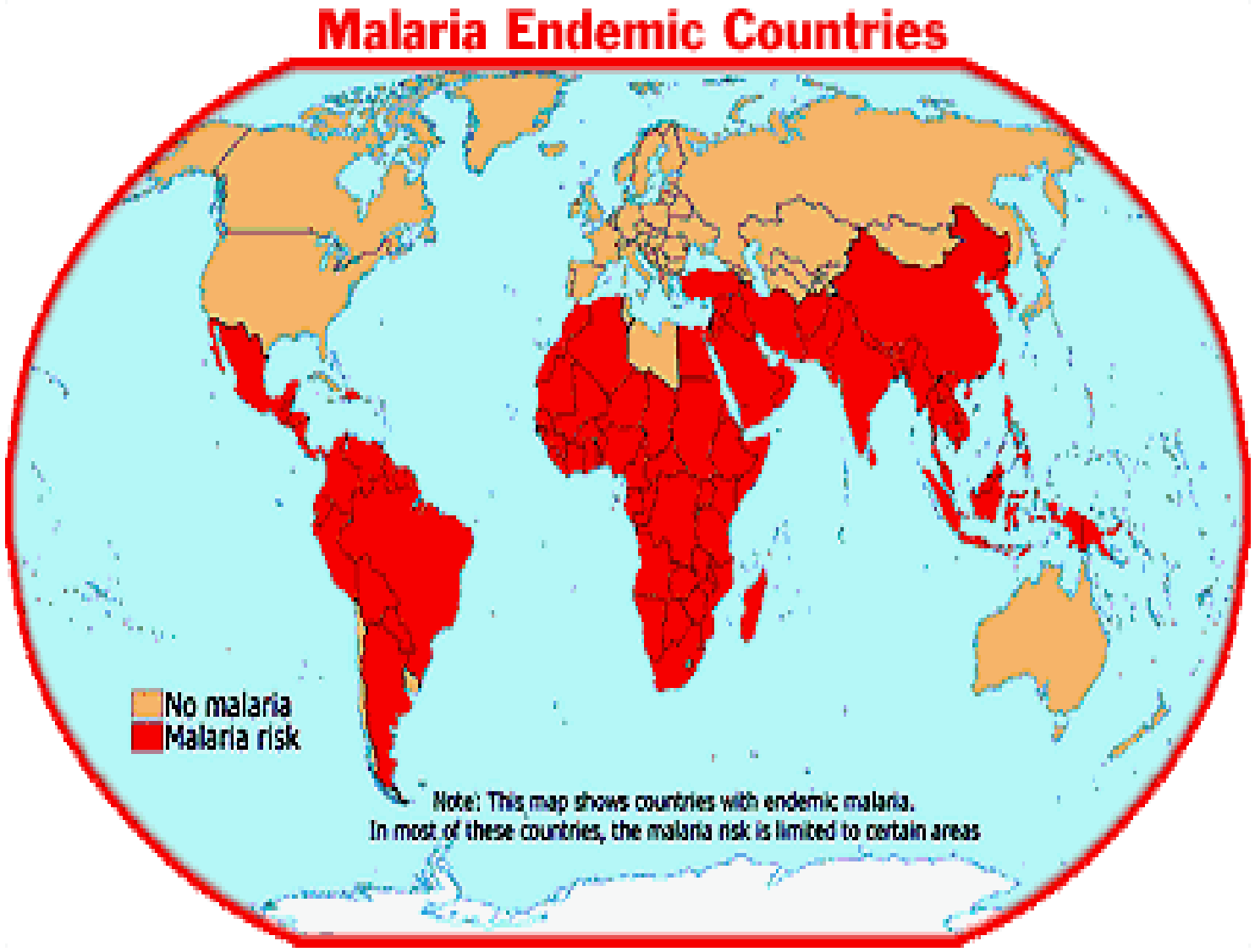
Vaccine Production

Malaria vaccine
Ebola Vaccine

Oxford and NIH trials for Ebola Vaccine



Areas with Endemic Malaria



Malaria Statistics

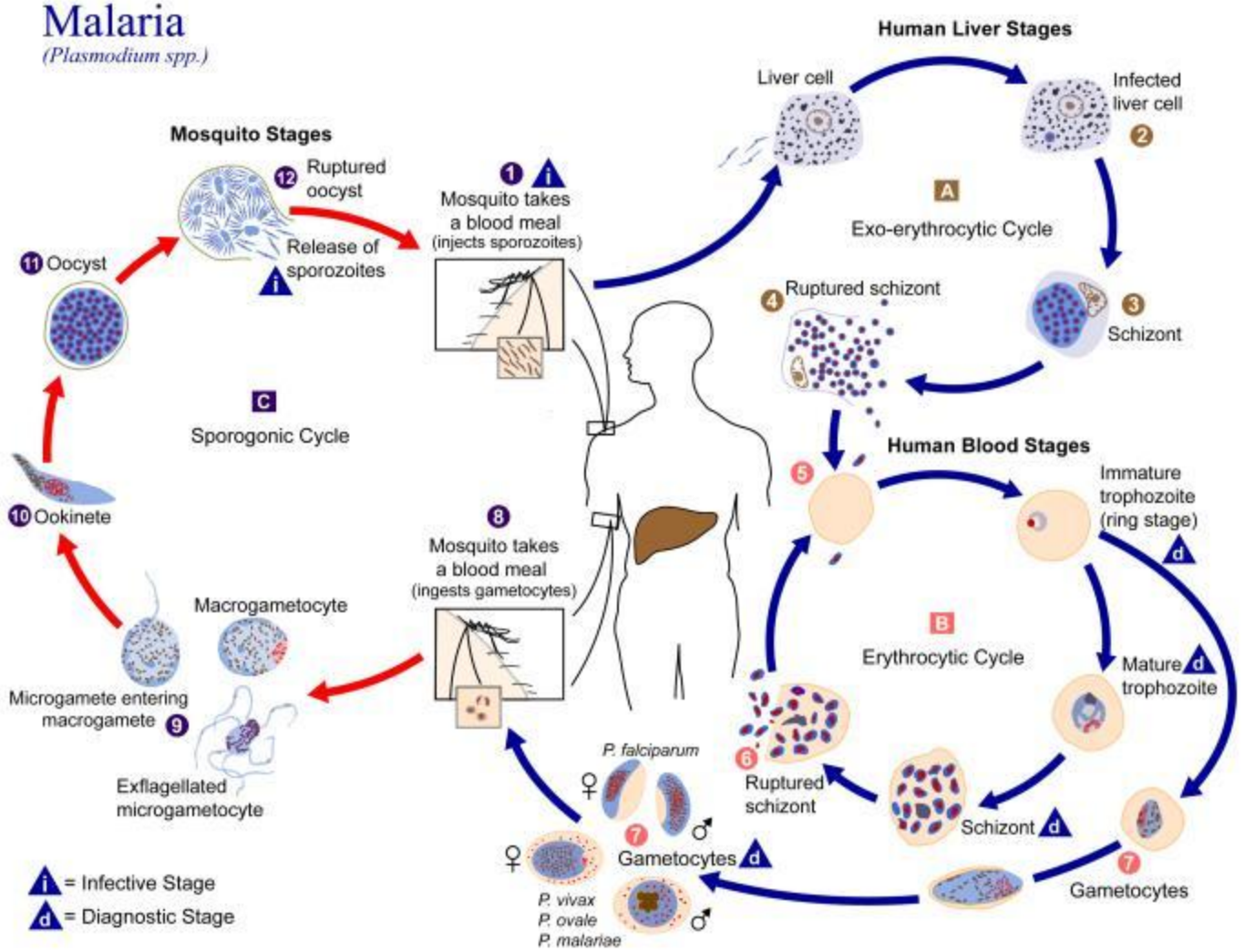
In 2012, 207 million people were infected and 627,000 died as a result of the disease. Of those deaths, 91% were in sub-Saharan Africa and 77% were children under the age of 5. Malaria is prevalent across 106 countries, impacting nearly 3.4 billion people, according to the Centers for Disease Control. The CDC estimates that direct costs from illness, treatments and premature death amounts to around $12 billion per year.

Ronald Ross (1857-1932) in Hyderabad, India
Nobel Prize 1902



Malaria

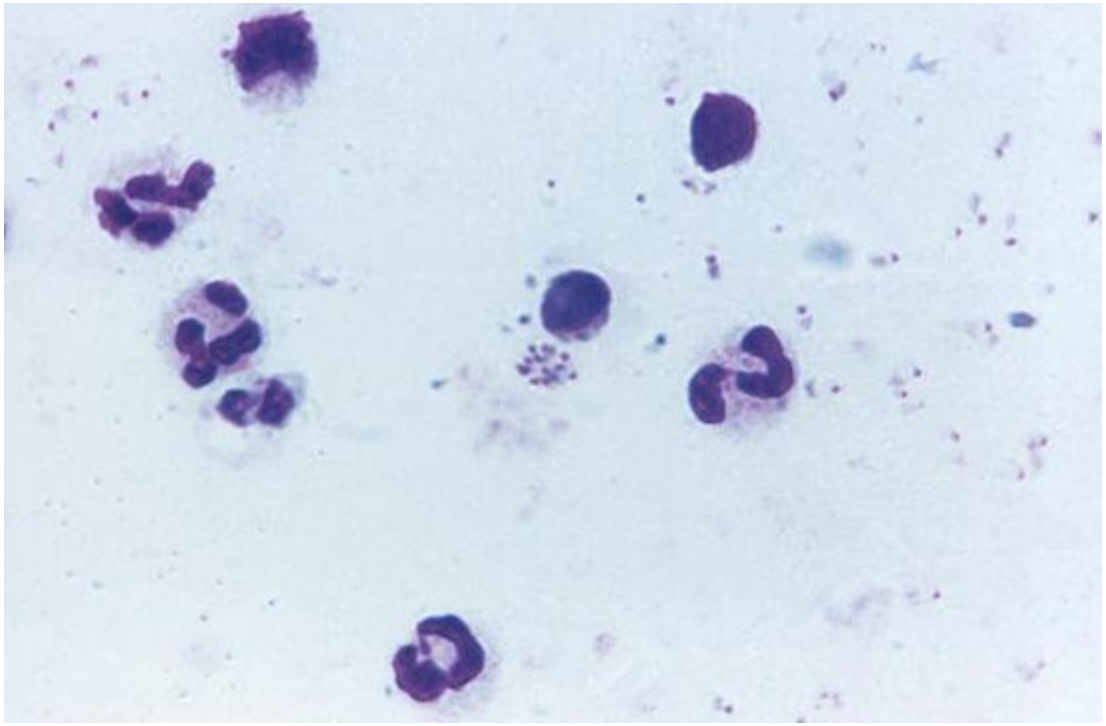
(Plasmodium spp.)



Malaria parasite: *Plasmodium falciparum*



Ring-form trophozoites of P. falciparum



Female Anopheles



Anopheles genome

The *Anopheles* genome, at 230–284 million base pairs (Mbp), is comparable in size to that of the fruitfly *Drosophila*.

The genome is diploid with six chromosomes

Ideal habitat for Anopheles breeding in Cambodia



Sequencing the Malaria parasite
Plasmodium falciparum

The parasites that causes the most common form of malaria share the same genetic variations — even when the organisms are separated across continents. This discovery raises concerns that mutations to resist existing medications could spread worldwide, making global eradication efforts even more difficult.

International collaboration: the Jenner Institute in Oxford, NIH, and the University of Maryland

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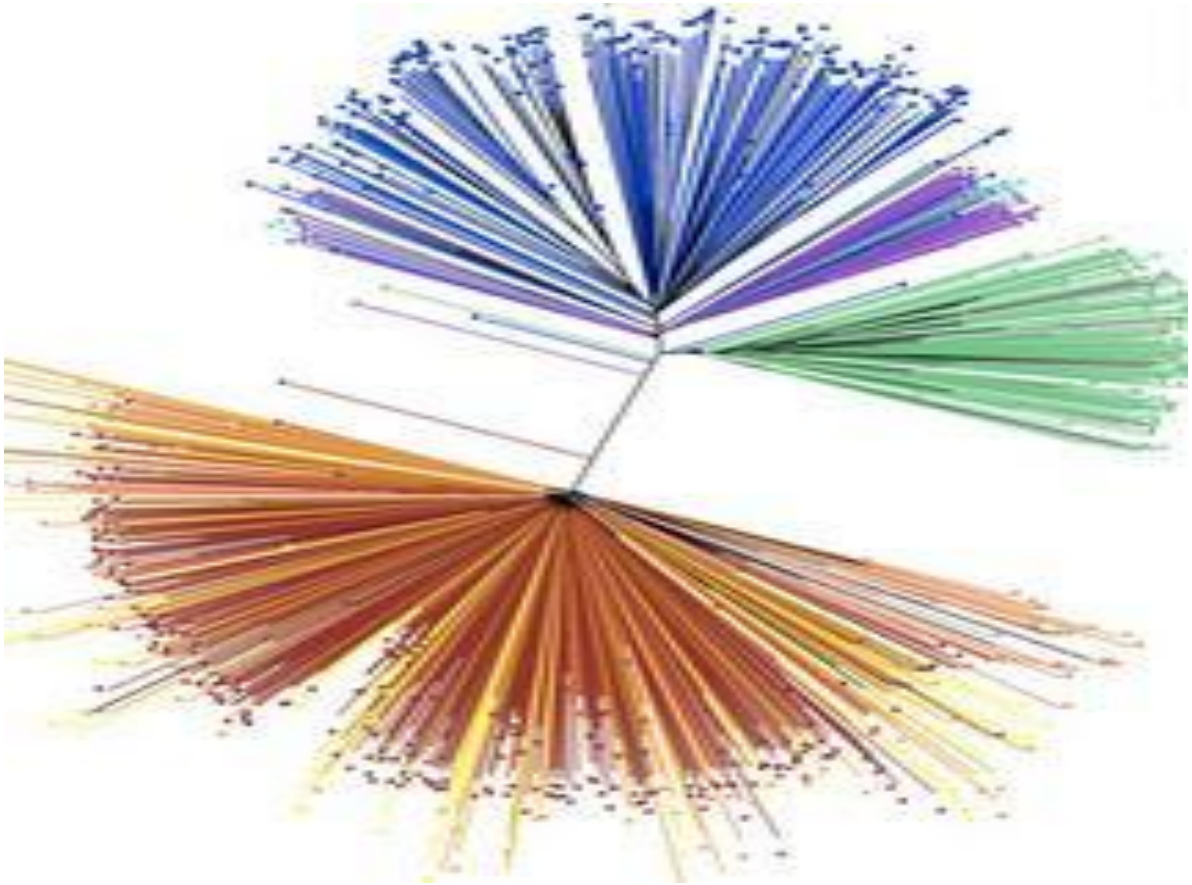
The phase 1 trials will begin as soon as they receive ethical and regulatory approvals, which will be considered on an expedited basis. If approvals are granted, the UK research teams could start vaccinating volunteers from mid-September.

June 2014

GlaxoSmithKline (GSK.L)

GSK is applying for regulatory approval for the world's first vaccine against malaria, designed for children in Africa. Regulatory submission is a first step toward launching the vaccine in malaria-infected regions which will change the lives of billions of people around the world. Glaxo has submitted a regulatory application to the European Medicines Agency (EMA) for its malaria vaccine RTS,S.

Genetic fingerprinting detected strains of drug-resistant malaria parasites



JBS Haldane (1892-1964)

- 1959



The Haldane hypothesis (Malaria hypothesis)

- Haldane's prediction (1948)
- that Sickle cell carriers (heterozygotes)
- are resistant to malarial infection

- Allison's confirmation (1954) in Africa, later re-confirmed by many other scientists

Malaria-Sickle Cell Dynamics

- Sickle cell disease (Si Si – homozygote)
- Die of sickle cell disease
- Sickle cell carriers (Si si – heterozygote)
- Survivors
- Sickle cell homozygote (si si) – Malaria
- Die of Malaria